Dossier zur Nutzenbewertung gemäß § 35a SGB V

Ganaxolon (Ztalmy®)

Immedica Pharma Germany GmbH

Modul 4A Anhang 4-G

Zusatzbehandlung von CDD-induzierten epileptischen Anfällen bei Patienten ab 2 Jahren

Ergänzende Informationen zur Studie Marigold

Stand: 26.06.2026

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4 Anhang 4-G – Ergänzende Informationen und Analysen aus der Studie Marigold

4.1 Charakterisierung der Studienpopulation aus der Studie Marigold

4.1.1 Vor- und Begleittherapien in der Studie Marigold

4.1.1.1 Medikamentöse Vortherapien mit Antikonvulsiva

Table 14.1.5
Summary of Prior AED Medications and Historical Seizure Information
(Safety Population)

Item	Placebo (N=51)	Ganaxolone (N=50)	Total (N=101)
Prior AEDs Used And Discontinued, n(%)			
Any	50 (98.0)	48 (96.0)	98 (97.0)
Levetiracetam (Keppra)	32 (62.7)	36 (72.0)	68 (67.3)
Topiramate (Topamax)	30 (58.8)	31 (62.0)	61 (60.4)
Vigabtrin (Sabril)	25 (49.0)	28 (56.0)	53 (52.5)
Phenobarbital	28 (54.9)	23 (46.0)	51 (50.5)
Valproate/ Valproic Acid (Depakote)	23 (45.1)	26 (52.0)	49 (48.5)
Clobazam	26 (51.0)	21 (42.0)	47 (46.5)
Lamotrigine (Lamictal)	15 (29.4)	19 (38.0)	34 (33.7)
Clonazepam	17 (33.3)	14 (28.0)	31 (30.7)
Rufinamide (Banzel)	17 (33.3)	12 (24.0)	29 (28.7)
Cannabidiol (Cbd)	9 (17.6)	17 (34.0)	26 (25.7)
Carbamazepine (Tegretal)	13 (25.5)	10 (20.0)	23 (22.8)
Oxcarbazepine (Trileptal)	10 (19.6)	13 (26.0)	23 (22.8)
Zonisamide (Zonegran)	12 (23.5)	11 (22.0)	23 (22.8)
Ethosuximide	4 (7.8)	9 (18.0)	13 (12.9)
Lacosamide (Vimpat)	7 (13.7)	6 (12.0)	13 (12.9)
Diazepam	7 (13.7)	5 (10.0)	12 (11.9)
Lorazepam	6 (11.8)	6 (12.0)	12 (11.9)
Phenytoin (Dilantin)	3 (5.9)	9 (18.0)	12 (11.9)
Felbamate (Febatol)	2 (3.9)	9 (18.0)	11 (10.9)
Perampanel (Fycompa)	4 (7.8)	6 (12.0)	10 (9.9)
Nitrazepam	4 (7.8)	3 (6.0)	7 (6.9)
Gabapentin (Neurontin)	2 (3.9)	1 (2.0)	3 (3.0)
Pregabalin (Lyrica)	0	1 (2.0)	1 (1.0)
Primidone	0	1 (2.0)	1 (1.0)
Other	29 (56.9)	37 (74.0)	66 (65.3)

Source Data: Listing 16.2.3.4

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4.1.1.2 Medikamentöse Vortherapien (nicht antikonvulsiv)

Table 14.1.7.1
Summary of Prior Non-AED Medications
(Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
Any Prior Non-AED Medications	14 (27.5)	7 (14.0)	21 (20.8)
ANILIDES	2 (3.9)	3 (6.0)	5 (5.0)
PARACETAMOL	2 (3.9)	3 (6.0)	5 (5.0)
H2-RECEPTOR ANTAGONISTS	1 (2.0)	2 (4.0)	3 (3.0)
RANITIDINE HYDROCHLORIDE	1 (2.0)	1 (2.0)	2 (2.0)
RANITIDINE	0	1 (2.0)	1 (1.0)
INFLUENZA VACCINES	2 (3.9)	0	2 (2.0)
INFLUENZA VACCINE	2 (3.9)	0	2 (2.0)
OSMOTICALLY ACTING LAXATIVES	1 (2.0)	1 (2.0)	2 (2.0)
MACROGOL 3350	1 (2.0)	1 (2.0)	2 (2.0)
PROTON PUMP INHIBITORS	1 (2.0)	1 (2.0)	2 (2.0)
ESOMEPRAZOLE SODIUM	1 (2.0)	0	1 (1.0)
OMEPRAZOLE	0	1 (2.0)	1 (1.0)
ANESTHETICS, LOCAL	1 (2.0)	0	1 (1.0)
AMYLOCAINE HYDROCHLORIDE	1 (2.0)	0	1 (1.0)
ANTIBACTERIALS FOR SYSTEMIC USE	1 (2.0)	0	1 (1.0)
ANTIBIOTICS	1 (2.0)	0	1 (1.0)
ANTIDOTES	1 (2.0)	0	1 (1.0)
SUGAMMADEX	1 (2.0)	0	1 (1.0)
ANTIINFECTIVES FOR SYSTEMIC USE	0	1 (2.0)	1 (1.0)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)

Note: Prior medications are defined as medications that started and stopped prior to the first dosing day of study drug.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1

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Table 14.1.7.1
Summary of Prior Non-AED Medications
(Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
ANTIINFECTIVES FOR SYSTEMIC USE	0	1 (2.0)	1 (1.0)
BELLADONNA ALKALOIDS, TERTIARY AMINES	1 (2.0)	0	1 (1.0)
ATROPINE	1 (2.0)	0	1 (1.0)
BETA-LACTAM ANTIBACTERIALS, PENICILLINS	1 (2.0)	0	1 (1.0)
PENICILLIN NOS	1 (2.0)	0	1 (1.0)
ESTERS OF BENZOIC ACID	1 (2.0)	0	1 (1.0)
AMYLOCAINE HYDROCHLORIDE;SODIUM BENZOATE	1 (2.0)	0	1 (1.0)
ESTREN DERIVATIVES	1 (2.0)	0	1 (1.0)
NORETHISTERONE	1 (2.0)	0	1 (1.0)
FIRST-GENERATION CEPHALOSPORINS	1 (2.0)	0	1 (1.0)
CEFALEXIN	1 (2.0)	0	1 (1.0)
GLUCOCORTICOIDS	1 (2.0)	0	1 (1.0)
PREDNISONE	1 (2.0)	0	1 (1.0)
IMIDAZOLE AND TRIAZOLE DERIVATIVES	1 (2.0)	0	1 (1.0)
CLOTRIMAZOLE	1 (2.0)	0	1 (1.0)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
IMIDAZOLE DERIVATIVES	0	1 (2.0)	1 (1.0)
METRONIDAZOLE	0	1 (2.0)	1 (1.0)
MACROLIDES	1 (2.0)	0	1 (1.0)
AZITHROMYCIN	1 (2.0)	0	1 (1.0)
MELATONIN RECEPTOR AGONISTS	1 (2.0)	0	1 (1.0)

Note: Prior medications are defined as medications that started and stopped prior to the first dosing day of study drug.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1

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Table 14.1.7.1
Summary of Prior Non-AED Medications
(Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
MELATONIN	1 (2.0)	0	1 (1.0)
OPIOID ANESTHETICS	1 (2.0)	0	1 (1.0)
FENTANYL	1 (2.0)	0	1 (1.0)
REMIFENTANIL	1 (2.0)	0	1 (1.0)
OTHER COLD PREPARATIONS	1 (2.0)	0	1 (1.0)
CYSTINE;RETINOL ACETATE;SACCHAROMYCES CEREVISIAE;SULFUR	1 (2.0)	0	1 (1.0)
OTHER GENERAL ANESTHETICS	1 (2.0)	0	1 (1.0)
PROPOFOL	1 (2.0)	0	1 (1.0)
OTHER OPIOIDS	1 (2.0)	0	1 (1.0)
TRAMADOL HYDROCHLORIDE	1 (2.0)	0	1 (1.0)

OTHER QUATERNARY AMMONIUM COMPOUNDS	1 (2.0)	0	1 (1.0)
ROCURONIUM	1 (2.0)	0	1 (1.0)
PENICILLINS WITH EXTENDED SPECTRUM	1 (2.0)	0	1 (1.0)
AMOXICILLIN	1 (2.0)	0	1 (1.0)
PROPIONIC ACID DERIVATIVES	0	1 (2.0)	1 (1.0)
IBUPROFEN	0	1 (2.0)	1 (1.0)
SEROTONIN (5HT3) ANTAGONISTS	1 (2.0)	0	1 (1.0)
ONDANSETRON	1 (2.0)	0	1 (1.0)
SOMATOSTATIN AND ANALOGUES	0	1 (2.0)	1 (1.0)
OCTREOTIDE	0	1 (2.0)	1 (1.0)

Note: Prior medications are defined as medications that started and stopped prior to the first dosing day of study drug.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1

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Table 14.1.7.1
Summary of Prior Non-AED Medications
(Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
UNSPECIFIED HERBAL AND TRADITIONAL MEDICINE	0	1 (2.0)	1 (1.0)
SAMBUCUS NIGRA	0	1 (2.0)	1 (1.0)
VARIOUS ALIMENTARY TRACT AND METABOLISM PRODUCTS	1 (2.0)	0	1 (1.0)
PHOSPHORUS	1 (2.0)	0	1 (1.0)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)

Note: Prior medications are defined as medications that started and stopped prior to the first dosing day of study drug.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1

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4.1.1.3 Medikamentöse Begleittherapien mit Antikonvulsiva

Table 14.1.7.3
Summary of Concomitant AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
Any Concomitant AED Medications	48 (94.1)	49 (98.0)	97 (96.0)
OTHER ANTIEPILEPTICS	33 (64.7)	28 (56.0)	61 (60.4)
LEVETIRACETAM	13 (25.5)	13 (26.0)	26 (25.7)
TOPIRAMATE	8 (15.7)	6 (12.0)	14 (13.9)
ZONISAMIDE	6 (11.8)	7 (14.0)	13 (12.9)
LAMOTRIGINE	6 (11.8)	4 (8.0)	10 (9.9)
PERAMPANEL	3 (5.9)	3 (6.0)	6 (5.9)
FELBAMATE	4 (7.8)	1 (2.0)	5 (5.0)
LACOSAMIDE	3 (5.9)	1 (2.0)	4 (4.0)
GABAPENTIN	1 (2.0)	2 (4.0)	3 (3.0)
BRIVARACETAM	0	2 (4.0)	2 (2.0)
SULTIAME	1 (2.0)	1 (2.0)	2 (2.0)
CANNABIDIOL	0	1 (2.0)	1 (1.0)
STIRIPENTOL	1 (2.0)	0	1 (1.0)
FATTY ACID DERIVATIVES	25 (49.0)	26 (52.0)	51 (50.5)
VALPROATE SEMISODIUM	16 (31.4)	18 (36.0)	34 (33.7)
VIGABATRIN	12 (23.5)	10 (20.0)	22 (21.8)
BENZODIAZEPINE DERIVATIVES	21 (41.2)	26 (52.0)	47 (46.5)
CLOBAZAM	13 (25.5)	12 (24.0)	25 (24.8)
CLONAZEPAM	4 (7.8)	6 (12.0)	10 (9.9)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
[1] WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.2

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Table 14.1.7.3
Summary of Concomitant AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
NITRAZEPAM	2 (3.9)	3 (6.0)	5 (5.0)
DIAZEPAM	1 (2.0)	3 (6.0)	4 (4.0)
MIDAZOLAM HYDROCHLORIDE	2 (3.9)	2 (4.0)	4 (4.0)
LORAZEPAM	0	1 (2.0)	1 (1.0)
CARBOXAMIDE DERIVATIVES	6 (11.8)	9 (18.0)	15 (14.9)
RUFINAMIDE	5 (9.8)	6 (12.0)	11 (10.9)
OXCARBAZEPINE	0	3 (6.0)	3 (3.0)
CARBAMAZEPINE	1 (2.0)	1 (2.0)	2 (2.0)
BARBITURATES AND DERIVATIVES	3 (5.9)	4 (8.0)	7 (6.9)
PHENOBARBITAL	3 (5.9)	4 (8.0)	7 (6.9)
SUCCINIMIDE DERIVATIVES	1 (2.0)	2 (4.0)	3 (3.0)
ETHOSUXIMIDE	1 (2.0)	2 (4.0)	3 (3.0)
HYDANTOIN DERIVATIVES	2 (3.9)	0	2 (2.0)
PHENYTOIN	2 (3.9)	0	2 (2.0)
ALDEHYDES AND DERIVATIVES	0	1 (2.0)	1 (1.0)
CHLORAL HYDRATE	0	1 (2.0)	1 (1.0)
IMMUNOGLOBULINS	0	1 (2.0)	1 (1.0)
IMMUNOGLOBULINS NOS	0	1 (2.0)	1 (1.0)
OTHER ANALGESICS AND ANTIPYRETICS	1 (2.0)	0	1 (1.0)
CANNABIDIOL	1 (2.0)	0	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
[1] WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.2

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-07-03-cm-aed.rtf

Table 14.1.7.3
Summary of Concomitant AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
OTHER PLAIN VITAMIN PREPARATIONS	0	1 (2.0)	1 (1.0)
PYRIDOXINE	0	1 (2.0)	1 (1.0)
PROGESTOGENS	1 (2.0)	0	1 (1.0)
MEDROXYPROGESTERONE ACETATE	1 (2.0)	0	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
[1] WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.2

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4.1.1.4 Medikamentöse Begleittherapien (nicht anti-konvulsiv)

Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
Any Concomitant Non-AED Medications	47 (92.2)	42 (84.0)	89 (88.1)
ANILIDES	16 (31.4)	15 (30.0)	31 (30.7)
PARACETAMOL	15 (29.4)	15 (30.0)	30 (29.7)
IBUPROFEN;PARACETAMOL	1 (2.0)	0	1 (1.0)
OSMOTICALLY ACTING LAXATIVES	16 (31.4)	13 (26.0)	29 (28.7)
MACROGOL 3350	11 (21.6)	5 (10.0)	16 (15.8)
MACROGOL	3 (5.9)	4 (8.0)	7 (6.9)
MACROGOL 3350;POTASSIUM CHLORIDE;SODIUM BICARBONATE;SODIUM CHLORIDE	1 (2.0)	2 (4.0)	3 (3.0)
MACROGOL 4000	0	2 (4.0)	2 (2.0)
LACTULOSE	0	1 (2.0)	1 (1.0)
LACTULOSE;LIGNIN	1 (2.0)	0	1 (1.0)
MAGNESIUM HYDROXIDE	1 (2.0)	0	1 (1.0)
PROTON PUMP INHIBITORS	14 (27.5)	13 (26.0)	27 (26.7)
LANSOPRAZOLE	6 (11.8)	7 (14.0)	13 (12.9)
OMEPRAZOLE	5 (9.8)	5 (10.0)	10 (9.9)
ESOMEPRAZOLE MAGNESIUM	3 (5.9)	1 (2.0)	4 (4.0)
ESOMEPRAZOLE	1 (2.0)	1 (2.0)	2 (2.0)
ESOMEPRAZOLE SODIUM	1 (2.0)	0	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
[1] WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1

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Table 14.1.7.2
Summary of Concomitant Non-AED Medications

(17-Week Double-Blind Phase, Safety Population) WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
VITAMIN D AND ANALOGUES	11 (21.6)	9 (18.0)	20 (19.8)
COLECALCIFEROL	6 (11.8)	5 (10.0)	11 (10.9)
VITAMIN D NOS	5 (9.8)	4 (8.0)	9 (8.9)
PROPIONIC ACID DERIVATIVES	10 (19.6)	8 (16.0)	18 (17.8)
IBUPROFEN	8 (15.7)	7 (14.0)	15 (14.9)
PROPIONIC ACID DERIVATIVES	1 (2.0)	1 (2.0)	2 (2.0)
KETOPROFEN	1 (2.0)	0	1 (1.0)
ANTIDIARRHEAL MICROORGANISMS	9 (17.6)	5 (10.0)	14 (13.9)
LACTOBACILLUS ACIDOPHILUS	3 (5.9)	1 (2.0)	4 (4.0)
PROBIOTICS NOS	3 (5.9)	1 (2.0)	4 (4.0)
LACTOBACILLUS NOS	0	3 (6.0)	3 (3.0)
ANTIDIARRHEAL MICROORGANISMS	2 (3.9)	0	2 (2.0)
BIFIDOBACTERIUM BIFIDUM;BIFIDOBACTERIUM BREVE;LACTOBACILLUS ACIDOPHILUS;LACTOBACILLUS CASEI;LACTOBACILLUS PLANTARUM;LACTOBACILLUS RHAMNOSUS;PEDIOCOCCUS ACIDILACTICI;STREPTOCOCCUS LACTIS	1 (2.0)	0	1 (1.0)
LACTOBACILLUS RHAMNOSUS	1 (2.0)	0	1 (1.0)
MULTIVITAMINS, PLAIN	10 (19.6)	4 (8.0)	14 (13.9)
MULTIVITAMINS, PLAIN	7 (13.7)	3 (6.0)	10 (9.9)
VITAMINS NOS	3 (5.9)	1 (2.0)	4 (4.0)
H2-RECEPTOR ANTAGONISTS	7 (13.7)	5 (10.0)	12 (11.9)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
RANITIDINE HYDROCHLORIDE	4 (7.8)	3 (6.0)	7 (6.9)
RANITIDINE	3 (5.9)	2 (4.0)	5 (5.0)
SELECTIVE BETA-2-ADRENORECEPTOR AGONISTS	6 (11.8)	6 (12.0)	12 (11.9)
SALBUTAMOL	5 (9.8)	6 (12.0)	11 (10.9)
SALBUTAMOL SULFATE	1 (2.0)	1 (2.0)	2 (2.0)
FORMOTEROL	1 (2.0)	0	1 (1.0)
LEVOSALBUTAMOL HYDROCHLORIDE	1 (2.0)	0	1 (1.0)
MELATONIN RECEPTOR AGONISTS	4 (7.8)	7 (14.0)	11 (10.9)
MELATONIN	4 (7.8)	7 (14.0)	11 (10.9)
AMINO ACIDS AND DERIVATIVES	6 (11.8)	4 (8.0)	10 (9.9)
LEVOCARNITINE	5 (9.8)	4 (8.0)	9 (8.9)
CARNITINE	1 (2.0)	0	1 (1.0)
GLUCOCORTICOIDS	6 (11.8)	3 (6.0)	9 (8.9)
BUDESONIDE	5 (9.8)	1 (2.0)	6 (5.9)
BECLOMETASONE DIPROPIONATE	1 (2.0)	1 (2.0)	2 (2.0)
FLUTICASONE	1 (2.0)	0	1 (1.0)
FLUTICASONE PROPIONATE	1 (2.0)	0	1 (1.0)
METHYLPREDNISOLONE	1 (2.0)	0	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications

(17-Week Double-Blind Phase, Safety Population) WHO Preferred Term [1]	Drug Class	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
METHYLPREDNISOLONE SODIUM SUCCINATE		1 (2.0)	0	1 (1.0)
MOMETASONE FUROATE		1 (2.0)	0	1 (1.0)
PREDNISOLONE		0	1 (2.0)	1 (1.0)
PREDNISONE		1 (2.0)	0	1 (1.0)
OTHER ANTIHISTAMINES FOR SYSTEMIC USE		5 (9.8)	3 (6.0)	8 (7.9)
LORATADINE		1 (2.0)	2 (4.0)	3 (3.0)
CYPROHEPTADINE HYDROCHLORIDE		2 (3.9)	0	2 (2.0)
DESLORATADINE		2 (3.9)	0	2 (2.0)
FEXOFENADINE HYDROCHLORIDE		0	1 (2.0)	1 (1.0)
SYNTHETIC ANTICHOLINERGICS, QUATERNARY AMMONIUM COMPOUNDS		4 (7.8)	4 (8.0)	8 (7.9)
GLYCOPYRRONIUM		3 (5.9)	4 (8.0)	7 (6.9)
GLYCOPYRRONIUM BROMIDE		1 (2.0)	0	1 (1.0)
THIRD-GENERATION CEPHALOSPORINS		8 (15.7)	0	8 (7.9)
CEFDINIR		3 (5.9)	0	3 (3.0)
CEFIXIME		2 (3.9)	0	2 (2.0)
CEFOTAXIME		1 (2.0)	0	1 (1.0)
CEFTAZIDIME		1 (2.0)	0	1 (1.0)
CEFTRIAXONE		1 (2.0)	0	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Preferred Term [1]	Drug	Class	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
CORTICOSTEROIDS			5 (9.8)	2 (4.0)	7 (6.9)
FLUTICASONE			4 (7.8)	2 (4.0)	6 (5.9)
TIXOCORTOL PIVALATE			1 (2.0)	0	1 (1.0)
MACROLIDES			5 (9.8)	2 (4.0)	7 (6.9)
AZITHROMYCIN			2 (3.9)	1 (2.0)	3 (3.0)
CLARITHROMYCIN			0	1 (2.0)	1 (1.0)
ERYTHROMYCIN			1 (2.0)	0	1 (1.0)
ERYTHROMYCIN ETHYLSUCCINATE			1 (2.0)	0	1 (1.0)
ROXITHROMYCIN			1 (2.0)	0	1 (1.0)
CALCIUM			4 (7.8)	2 (4.0)	6 (5.9)
CALCIUM			4 (7.8)	1 (2.0)	5 (5.0)
CALCIUM CARBONATE			1 (2.0)	1 (2.0)	2 (2.0)
CENTRALLY ACTING SYMPATHOMIMETICS			4 (7.8)	2 (4.0)	6 (5.9)
CLONIDINE			4 (7.8)	2 (4.0)	6 (5.9)
COMBINATIONS OF PENICILLINS, INCL. BETA-LACTAMASE INHIBITORS			2 (3.9)	4 (8.0)	6 (5.9)
AMOXICILLIN;CLAVULANIC ACID			2 (3.9)	2 (4.0)	4 (4.0)
AMOXICILLIN;CLAVULANATE POTASSIUM			0	2 (4.0)	2 (2.0)
AMPICILLIN;SULBACTAM			0	1 (2.0)	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
MULTIVITAMINS WITH MINERALS	5 (9.8)	1 (2.0)	6 (5.9)
MINERALS NOS;VITAMINS NOS	1 (2.0)	1 (2.0)	2 (2.0)
ASCORBIC ACID;BIOTIN;CALCIUM;CHROMIUM;COLECALCIFEROL;COPPER;CYANOCOBALAMIN;FOLATE SODIUM;IODINE;IRON;MAGNESIUM;MANGANESE;MENADIONE;MOLYBDENUM;NICOTINIC ACID;PANTOTHENIC ACID;PHOSPHORUS;POTASSIUM;PYR	1 (2.0)	0	1 (1.0)
ASCORBIC ACID;BIOTIN;CALCIUM;COLECALCIFEROL;COPPER;CYANOCOBALAMIN;FOLIC ACID;IODINE;IRON;NICOTINIC ACID;PANTOTHENIC ACID;PHYTOMENADIONE;PYRIDOXINE HYDROCHLORIDE;RETINOL;RIBOFLAVIN;SODIUM;THIAMINE;TO	1 (2.0)	0	1 (1.0)
FOLIC ACID;MINERALS NOS;VITAMINS NOS	1 (2.0)	0	1 (1.0)
MULTIVITAMINS WITH MINERALS [UMBRELLA TERM]	1 (2.0)	0	1 (1.0)
OTHER PLAIN VITAMIN PREPARATIONS	4 (7.8)	2 (4.0)	6 (5.9)
PYRIDOXINE HYDROCHLORIDE	2 (3.9)	1 (2.0)	3 (3.0)
PYRIDOXINE	1 (2.0)	1 (2.0)	2 (2.0)
BIOTIN	1 (2.0)	0	1 (1.0)
PENICILLINS WITH EXTENDED SPECTRUM	3 (5.9)	3 (6.0)	6 (5.9)
AMOXICILLIN	3 (5.9)	3 (6.0)	6 (5.9)
PIPERAZINE DERIVATIVES	3 (5.9)	3 (6.0)	6 (5.9)
CETIRIZINE HYDROCHLORIDE	2 (3.9)	3 (6.0)	5 (5.0)
CETIRIZINE	0	1 (2.0)	1 (1.0)
HYDROXYZINE HYDROCHLORIDE	1 (2.0)	0	1 (1.0)
UNSPECIFIED HERBAL AND TRADITIONAL MEDICINE	2 (3.9)	4 (8.0)	6 (5.9)
ANGELICA ARCHANGELICA ROOT;CARUM CARVI FRUIT;CHELIDONIUM MAJUS HERB;GLYCYRRHIZA GLABRA ROOT;IBERIS AMARA;MATRICARIA RECUTITA FLOWER;MELISSA OFFICINALIS LEAF;MENTHA X PIPERITA LEAF;SILYBUM MARIANUM F	0	1 (2.0)	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
CALENDULA OFFICINALIS	1 (2.0)	0	1 (1.0)
CETRARIA ISLANDICA	0	1 (2.0)	1 (1.0)
ENZYMES NOS;HONEY;PRUNUS DOMESTICA	1 (2.0)	0	1 (1.0)
SAMBUCUS NIGRA	0	1 (2.0)	1 (1.0)
SENNA ALEXANDRINA	0	1 (2.0)	1 (1.0)
TRITICUM AESTIVUM	1 (2.0)	0	1 (1.0)
CONTACT LAXATIVES	3 (5.9)	2 (4.0)	5 (5.0)
SENNA ALEXANDRINA	1 (2.0)	2 (4.0)	3 (3.0)
SENNA ALEXANDRINA GLYCOSIDE EXTRACT	1 (2.0)	0	1 (1.0)
SENNOSIDE A+B	1 (2.0)	0	1 (1.0)
CALCIUM, COMBINATIONS WITH VITAMIN D AND/OR OTHER DRUGS	3 (5.9)	1 (2.0)	4 (4.0)
CALCIUM;COLECALCIFEROL	1 (2.0)	0	1 (1.0)
CALCIUM;COLECALCIFEROL;MAGNESIUM	0	1 (2.0)	1 (1.0)
CALCIUM;MAGNESIUM	1 (2.0)	0	1 (1.0)
CALCIUM;VITAMIN D NOS	1 (2.0)	0	1 (1.0)
CORTICOSTEROIDS, WEAK (GROUP I)	1 (2.0)	3 (6.0)	4 (4.0)
HYDROCORTISONE	1 (2.0)	3 (6.0)	4 (4.0)
GENERAL NUTRIENTS	2 (3.9)	2 (4.0)	4 (4.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications

(17-Week Double-Blind Phase, Safety Population) WHO Preferred Term [1]	Drug Class	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
NUTRIENTS NOS		2 (3.9)	2 (4.0)	4 (4.0)
MAGNESIUM		3 (5.9)	1 (2.0)	4 (4.0)
MAGNESIUM CARBONATE		1 (2.0)	0	1 (1.0)
MAGNESIUM HYDROXIDE		0	1 (2.0)	1 (1.0)
MAGNESIUM LACTATE		1 (2.0)	0	1 (1.0)
MAGNESIUM OXIDE		1 (2.0)	0	1 (1.0)
MULTIVITAMINS, OTHER COMBINATIONS		3 (5.9)	1 (2.0)	4 (4.0)
MINERALS NOS;VITAMINS NOS		2 (3.9)	0	2 (2.0)
ASCORBIC ACID;BETACAROTENE;BIOTIN;CALCIUM CARBONATE;CALCIUM PANTOTHENATE;CHROMIC CHLORIDE;COLECALCIFEROL;COPPER SULFATE;CYANOCOBALAMIN;FERROUS FUMARATE;FOLIC ACID;LYCOPENE;MAGNESIUM OXIDE;MANGANESE		0	1 (2.0)	1 (1.0)
MULTIVITAMINS, OTHER COMBINATIONS		1 (2.0)	0	1 (1.0)
OTHER COMBINATIONS OF NUTRIENTS		2 (3.9)	2 (4.0)	4 (4.0)
FISH OIL		1 (2.0)	2 (4.0)	3 (3.0)
CALCIUM PHOSPHATE;FUMARIC ACID;POTASSIUM CHLORIDE		1 (2.0)	0	1 (1.0)
CREATINE		0	1 (2.0)	1 (1.0)
OTHER DRUGS FOR CONSTIPATION		4 (7.8)	0	4 (4.0)
GLYCEROL		4 (7.8)	0	4 (4.0)
POTASSIUM		1 (2.0)	3 (6.0)	4 (4.0)
POTASSIUM CITRATE		1 (2.0)	2 (4.0)	3 (3.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Preferred Term [1]	Drug	Class	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
POTASSIUM			0	1 (2.0)	1 (1.0)
AMINOALKYL ETHERS			1 (2.0)	2 (4.0)	3 (3.0)
DIPHENHYDRAMINE			1 (2.0)	1 (2.0)	2 (2.0)
DIPHENHYDRAMINE HYDROCHLORIDE			0	1 (2.0)	1 (1.0)
ASCORBIC ACID (VITAMIN C), PLAIN			2 (3.9)	1 (2.0)	3 (3.0)
ASCORBIC ACID			2 (3.9)	1 (2.0)	3 (3.0)
LEUKOTRIENE RECEPTOR ANTAGONISTS			2 (3.9)	1 (2.0)	3 (3.0)
MONTELUKAST SODIUM			2 (3.9)	0	2 (2.0)
MONTELUKAST			0	1 (2.0)	1 (1.0)
OTHER DRUGS FOR FUNCTIONAL GASTROINTESTINAL DISORDERS			3 (5.9)	0	3 (3.0)
SIMETICONE			3 (5.9)	0	3 (3.0)
OTHER NERVOUS SYSTEM DRUGS			1 (2.0)	2 (4.0)	3 (3.0)
GABAPENTIN			0	1 (2.0)	1 (1.0)
TETRABENAZINE			1 (2.0)	0	1 (1.0)
THIOCTIC ACID			0	1 (2.0)	1 (1.0)
SALT SOLUTIONS			2 (3.9)	1 (2.0)	3 (3.0)
SODIUM BICARBONATE			1 (2.0)	1 (2.0)	2 (2.0)
SODIUM CITRATE			1 (2.0)	0	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
SECOND-GENERATION CEPHALOSPORINS	1 (2.0)	2 (4.0)	3 (3.0)
CEFACLOR	0	1 (2.0)	1 (1.0)
CEFUROXIME	1 (2.0)	0	1 (1.0)
CEFUROXIME AXETIL	0	1 (2.0)	1 (1.0)
SELENIUM	1 (2.0)	2 (4.0)	3 (3.0)
SELENIUM	1 (2.0)	2 (4.0)	3 (3.0)
SOLUTIONS AFFECTING THE ELECTROLYTE BALANCE	3 (5.9)	0	3 (3.0)
SODIUM CHLORIDE	2 (3.9)	0	2 (2.0)
GLUCONATE SODIUM;MAGNESIUM CHLORIDE;POTASSIUM CHLORIDE;SODIUM ACETATE TRIHYDRATE;SODIUM CHLORIDE	1 (2.0)	0	1 (1.0)
SOLUTIONS AFFECTING THE ELECTROLYTE BALANCE	1 (2.0)	0	1 (1.0)
VARIOUS ALIMENTARY TRACT AND METABOLISM PRODUCTS	2 (3.9)	1 (2.0)	3 (3.0)
CITRIC ACID;SODIUM CITRATE	1 (2.0)	0	1 (1.0)
PHOSPHORUS	1 (2.0)	0	1 (1.0)
PROBIOTICS NOS	0	1 (2.0)	1 (1.0)
ANTACIDS	1 (2.0)	1 (2.0)	2 (2.0)
SODIUM CITRATE	1 (2.0)	1 (2.0)	2 (2.0)
ANTICHOLINERGICS	1 (2.0)	1 (2.0)	2 (2.0)
GLYCOPYRRONIUM	1 (2.0)	1 (2.0)	2 (2.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
[1] WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1

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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
BELLADONNA ALKALOIDS, TERTIARY AMINES	1 (2.0)	1 (2.0)	2 (2.0)
ATROPINE	1 (2.0)	1 (2.0)	2 (2.0)
CORTICOSTEROIDS, POTENT, COMBINATIONS WITH ANTIBIOTICS	1 (2.0)	1 (2.0)	2 (2.0)
BETAMETHASONE VALERATE;FUSIDIC ACID	0	1 (2.0)	1 (1.0)
BETAMETHASONE VALERATE;GENTAMICIN SULFATE	1 (2.0)	0	1 (1.0)
ENEMAS	1 (2.0)	1 (2.0)	2 (2.0)
GLYCEROL	1 (2.0)	0	1 (1.0)
SODIUM CITRATE;SODIUM LAURYL SULFOACETATE;SORBITOL	0	1 (2.0)	1 (1.0)
ESTREN DERIVATIVES	1 (2.0)	1 (2.0)	2 (2.0)
NORETHISTERONE	1 (2.0)	1 (2.0)	2 (2.0)
FIRST-GENERATION CEPHALOSPORINS	1 (2.0)	1 (2.0)	2 (2.0)
CEFALEXIN	1 (2.0)	1 (2.0)	2 (2.0)
MEDICAL GASES	1 (2.0)	1 (2.0)	2 (2.0)
OXYGEN	1 (2.0)	1 (2.0)	2 (2.0)
MUCOLYTICS	1 (2.0)	1 (2.0)	2 (2.0)
AMBROXOL ACEFYLLINATE	1 (2.0)	0	1 (1.0)
SODIUM CHLORIDE	0	1 (2.0)	1 (1.0)
NEURAMINIDASE INHIBITORS	1 (2.0)	1 (2.0)	2 (2.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Preferred Term [1]	Drug Class	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
OSELTAMIVIR PHOSPHATE		1 (2.0)	1 (2.0)	2 (2.0)
ORAL REHYDRATION SALT FORMULATIONS		2 (3.9)	0	2 (2.0)
GLUCOSE;POTASSIUM CHLORIDE;SODIUM CHLORIDE;SODIUM CITRATE ACID		1 (2.0)	0	1 (1.0)
GLUCOSE;POTASSIUM GLUCONATE;SODIUM CHLORIDE;SODIUM CITRATE;SUCROSE		1 (2.0)	0	1 (1.0)
ORAL REHYDRATION SALT FORMULATIONS		1 (2.0)	0	1 (1.0)
OTHER ANTIBIOTICS FOR TOPICAL USE		1 (2.0)	1 (2.0)	2 (2.0)
CHLORAMPHENICOL;METHYLURACIL		0	1 (2.0)	1 (1.0)
MUPIROCIN		1 (2.0)	0	1 (1.0)
OTHER ANTIDEPRESSANTS		1 (2.0)	1 (2.0)	2 (2.0)
TRAZODONE		1 (2.0)	1 (2.0)	2 (2.0)
OTHER ANTIEPILEPTICS		0	2 (4.0)	2 (2.0)
ACETAZOLAMIDE		0	1 (2.0)	1 (1.0)
GABAPENTIN		0	1 (2.0)	1 (1.0)
OTHER CICATRIZANTS		2 (3.9)	0	2 (2.0)
COPPER GLUCONATE;HYALURONATE SODIUM;MADECASSOSIDE;MANGANESE GLUCONATE;ZINC GLUCONATE		1 (2.0)	0	1 (1.0)
DEXPANTHENOL		1 (2.0)	0	1 (1.0)
OTHER LIPID MODIFYING AGENTS		1 (2.0)	1 (2.0)	2 (2.0)
OTHER LIPID MODIFYING AGENTS		0	1 (2.0)	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
TRIGLYCERIDES	1 (2.0)	0	1 (1.0)
OTHER MINERAL PRODUCTS	1 (2.0)	1 (2.0)	2 (2.0)
POTASSIUM PHOSPHATE DIBASIC;POTASSIUM PHOSPHATE MONOBASIC;SODIUM PHOSPHATE DIBASIC;SODIUM PHOSPHATE MONOBASIC	1 (2.0)	0	1 (1.0)
SODIUM PHOSPHATE DIBASIC	0	1 (2.0)	1 (1.0)
OTHER THERAPEUTIC PRODUCTS	2 (3.9)	0	2 (2.0)
HOMEOPATHIC PREPARATION	1 (2.0)	0	1 (1.0)
TAURINE	1 (2.0)	0	1 (1.0)
SEROTONIN (5HT3) ANTAGONISTS	0	2 (4.0)	2 (2.0)
ONDANSETRON	0	2 (4.0)	2 (2.0)
ZINC	1 (2.0)	1 (2.0)	2 (2.0)
ZINC	1 (2.0)	1 (2.0)	2 (2.0)
ACETIC ACID DERIVATIVES AND RELATED SUBSTANCES	1 (2.0)	0	1 (1.0)
DICLOFENAC	1 (2.0)	0	1 (1.0)
ACID PREPARATIONS	0	1 (2.0)	1 (1.0)
CITRIC ACID	0	1 (2.0)	1 (1.0)
ALDEHYDES AND DERIVATIVES	1 (2.0)	0	1 (1.0)
CHLORAL HYDRATE	1 (2.0)	0	1 (1.0)
ALL OTHER THERAPEUTIC PRODUCTS	1 (2.0)	0	1 (1.0)

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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Preferred Term [1]	Drug	Class	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
PROPOLIS			1 (2.0)	0	1 (1.0)
ALUMINIUM COMPOUNDS			1 (2.0)	0	1 (1.0)
ALUMINIUM PHOSPHATE			1 (2.0)	0	1 (1.0)
ANTIALLERGIC AGENTS, EXCL. CORTICOSTEROIDS			1 (2.0)	0	1 (1.0)
AZELASTINE			1 (2.0)	0	1 (1.0)
OLOPATADINE HYDROCHLORIDE			1 (2.0)	0	1 (1.0)
ANTIBIOTICS			0	1 (2.0)	1 (1.0)
POLYMYXIN B SULFATE;TRIMETHOPRIM			0	1 (2.0)	1 (1.0)
APPETITE STIMULANTS			0	1 (2.0)	1 (1.0)
CYPROHEPTADINE HYDROCHLORIDE			0	1 (2.0)	1 (1.0)
BELLADONNA ALKALOIDS, SEMISYNTHETIC, QUATERNARY AMMONIUM COMPOUNDS			1 (2.0)	0	1 (1.0)
HYOSCINE BUTYLBROMIDE			1 (2.0)	0	1 (1.0)
CALCIUM COMPOUNDS			1 (2.0)	0	1 (1.0)
CALCIUM CARBONATE			1 (2.0)	0	1 (1.0)
CARBOHYDRATES			0	1 (2.0)	1 (1.0)
DEXTRIN			0	1 (2.0)	1 (1.0)
CHARCOAL PREPARATIONS			1 (2.0)	0	1 (1.0)
CHARCOAL, ACTIVATED			1 (2.0)	0	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
CORTICOSTEROIDS, MODERATELY POTENT (GROUP II)	0	1 (2.0)	1 (1.0)
TRIAMCINOLONE	0	1 (2.0)	1 (1.0)
CORTICOSTEROIDS, PLAIN	0	1 (2.0)	1 (1.0)
DESONIDE	0	1 (2.0)	1 (1.0)
COUGH AND COLD PREPARATIONS	1 (2.0)	0	1 (1.0)
COUGH AND COLD PREPARATIONS	1 (2.0)	0	1 (1.0)
DIAZEPINES, OXAZEPINES, THIAZEPINES AND OXEPINES	0	1 (2.0)	1 (1.0)
OLANZAPINE	0	1 (2.0)	1 (1.0)
ENZYME PREPARATIONS	1 (2.0)	0	1 (1.0)
PANCREATIN	1 (2.0)	0	1 (1.0)
FAT/CARBOHYDRATES/PROTEINS/MINERALS/VITAMINS, COMBINATIONS	0	1 (2.0)	1 (1.0)
CARNITINE;CAROTENOIDS NOS;CHOLINE;FISH OIL;HERBAL OIL NOS;MALTODEXTRIN;MINERALS NOS;PROTEINS NOS;TAURINE;VITAMINS NOS	0	1 (2.0)	1 (1.0)
FATTY ACID DERIVATIVES	0	1 (2.0)	1 (1.0)
VIGABATRIN	0	1 (2.0)	1 (1.0)
HEPATITIS VACCINES	1 (2.0)	0	1 (1.0)
HEPATITIS A VACCINE	1 (2.0)	0	1 (1.0)
HERBAL ANTIEMETICS, OTHER	1 (2.0)	0	1 (1.0)
ZINGIBER OFFICINALE	1 (2.0)	0	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Preferred Term [1]	Drug	Class	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
HERBAL DIAPHORETICS AND OTHER HERBAL COUGH AND COLD REMEDIES			1 (2.0)	0	1 (1.0)
GRINDELIA SPP.;HELICHRYSUM SPP.;HONEY;PLANTAGO LANCEOLATA			1 (2.0)	0	1 (1.0)
HERBAL PREPARATIONS FOR TREATMENT OF WOUNDS AND ULCERS, OTHER			0	1 (2.0)	1 (1.0)
CALENDULA OFFICINALIS			0	1 (2.0)	1 (1.0)
IMIDAZOLE AND TRIAZOLE DERIVATIVES			1 (2.0)	0	1 (1.0)
KETOCONAZOLE			1 (2.0)	0	1 (1.0)
IMIDAZOLINE RECEPTOR AGONISTS			1 (2.0)	0	1 (1.0)
CLONIDINE			1 (2.0)	0	1 (1.0)
IMMUNOGLOBULINS			0	1 (2.0)	1 (1.0)
IMMUNOGLOBULINS NOS			0	1 (2.0)	1 (1.0)
INFLUENZA VACCINES			1 (2.0)	0	1 (1.0)
INFLUENZA VACCINE			1 (2.0)	0	1 (1.0)
INTERFERONS			0	1 (2.0)	1 (1.0)
IMMUNOGLOBULIN HUMAN NORMAL;INTERFERON ALFA-2B			0	1 (2.0)	1 (1.0)
IRON BIVALENT, ORAL PREPARATIONS			1 (2.0)	0	1 (1.0)
FERROUS SULFATE			1 (2.0)	0	1 (1.0)
LINCOSAMIDES			1 (2.0)	0	1 (1.0)
CLINDAMYCIN			1 (2.0)	0	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
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Table 14.1.7.2
Summary of Concomitant Non-AED Medications
(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
LIVER THERAPY	0	1 (2.0)	1 (1.0)
CHOLINE BITARTRATE;INOSITOL;METHIONINE	0	1 (2.0)	1 (1.0)
NITROFURAN DERIVATIVES	1 (2.0)	0	1 (1.0)
AKRITOIN	1 (2.0)	0	1 (1.0)
OPIUM ALKALOIDS AND DERIVATIVES	1 (2.0)	0	1 (1.0)
CODEINE;SISYMBRIUM OFFICINALE	1 (2.0)	0	1 (1.0)
OPIUM DERIVATIVES AND EXPECTORANTS	1 (2.0)	0	1 (1.0)
CHLORPHENAMINE MALEATE;DEXTROMETHORPHAN HYDROBROMIDE;GUAIFENESIN;PHENYLEPHRINE	1 (2.0)	0	1 (1.0)
OTHER AGENTS FOR LOCAL ORAL TREATMENT	1 (2.0)	0	1 (1.0)
OTHER AGENTS FOR LOCAL ORAL TREATMENT	1 (2.0)	0	1 (1.0)
OTHER ANTIEMETICS	0	1 (2.0)	1 (1.0)
HYOSCINE	0	1 (2.0)	1 (1.0)
OTHER ANTIFUNGALS FOR TOPICAL USE	0	1 (2.0)	1 (1.0)
CICLOPIROX	0	1 (2.0)	1 (1.0)
OTHER ANTIINFLAMMATORY AND ANTIRHEUMATIC AGENTS, NON-STEROIDS	1 (2.0)	0	1 (1.0)
BENZYDAMINE HYDROCHLORIDE	1 (2.0)	0	1 (1.0)
OTHER ANTISEPTICS AND DISINFECTANTS	0	1 (2.0)	1 (1.0)
BRILLIANT GREEN CI 42040	0	1 (2.0)	1 (1.0)

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(17-Week Double-Blind Phase, Safety Population)

WHO Preferred Term [1]	Drug	Class	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
OTHER ANTIVIRALS			1 (2.0)	0	1 (1.0)
UMIFENOVIR			1 (2.0)	0	1 (1.0)
OTHER CARDIAC PREPARATIONS			0	1 (2.0)	1 (1.0)
UBIDECARENONE			0	1 (2.0)	1 (1.0)
OTHER CARDIAC STIMULANTS			1 (2.0)	0	1 (1.0)
IPRATROPIUM BROMIDE			1 (2.0)	0	1 (1.0)
OTHER CENTRALLY ACTING AGENTS			1 (2.0)	0	1 (1.0)
BACLOFEN			1 (2.0)	0	1 (1.0)
OTHER COLD PREPARATIONS			0	1 (2.0)	1 (1.0)
ESSENTIAL OILS NOS			0	1 (2.0)	1 (1.0)
OTHER COUGH SUPPRESSANTS			1 (2.0)	0	1 (1.0)
BUTAMIRATE			1 (2.0)	0	1 (1.0)
OTHER DRUGS FOR PEPTIC ULCER AND GASTRO-OESOPHAGEAL REFLUX DISEASE (GORD)			1 (2.0)	0	1 (1.0)
SUCRALFATE			1 (2.0)	0	1 (1.0)
OTHER ECTOPARASITICIDES, INCL. SCABICIDES			0	1 (2.0)	1 (1.0)
DIMETICONE			0	1 (2.0)	1 (1.0)
OTHER INTESTINAL ADSORBENTS			0	1 (2.0)	1 (1.0)
GELATIN TANNATE			0	1 (2.0)	1 (1.0)

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(17-Week Double-Blind Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
OTHER INTESTINAL ANTIINFECTIVES	1 (2.0)	0	1 (1.0)
NIFUROXAZIDE	1 (2.0)	0	1 (1.0)
OTHER NASAL PREPARATIONS	0	1 (2.0)	1 (1.0)
SODIUM CHLORIDE	0	1 (2.0)	1 (1.0)
OTHER THROAT PREPARATIONS	1 (2.0)	0	1 (1.0)
PROPOLIS	1 (2.0)	0	1 (1.0)
OTHER UROLOGICALS	1 (2.0)	0	1 (1.0)
CITRIC ACID;POTASSIUM CITRATE	1 (2.0)	0	1 (1.0)
OXICAMS	1 (2.0)	0	1 (1.0)
PIROXICAM	1 (2.0)	0	1 (1.0)
PNEUMOCOCCAL VACCINES	1 (2.0)	0	1 (1.0)
PNEUMOCOCCAL VACCINE	1 (2.0)	0	1 (1.0)
PREGNEN (4) DERIVATIVES	0	1 (2.0)	1 (1.0)
MEDROXYPROGESTERONE ACETATE	0	1 (2.0)	1 (1.0)
PROGESTOGENS	0	1 (2.0)	1 (1.0)
DESOGESTREL	0	1 (2.0)	1 (1.0)
PROPULSIVES	1 (2.0)	0	1 (1.0)
DOMPERIDONE	1 (2.0)	0	1 (1.0)

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(17-Week Double-Blind Phase, Safety Population)

WHO Preferred Term [1]	Drug	Class	Placebo (N=51) n (%)	Ganaxol (N=50) n (%)	Total (N=101) n (%)
SILVER COMPOUNDS			1 (2.0)	0	1 (1.0)
SILVER NITRATE			1 (2.0)	0	1 (1.0)
SOFTENERS, EMOLLIENTS			0	1 (2.0)	1 (1.0)
PARAFFIN			0	1 (2.0)	1 (1.0)
SYNTHETIC ANTICHOLINERGICS, ESTERS WITH TERTIARY AMINO GROUP			1 (2.0)	0	1 (1.0)
TRIMEBUTINE			1 (2.0)	0	1 (1.0)
THYROID HORMONES			1 (2.0)	0	1 (1.0)
LEVOTHYROXINE SODIUM			1 (2.0)	0	1 (1.0)
URINARY CONCREMENT SOLVENTS			1 (2.0)	0	1 (1.0)
CITRIC ACID;POTASSIUM CITRATE;SODIUM CITRATE ACID			1 (2.0)	0	1 (1.0)
VITAMINS, OTHER COMBINATIONS			1 (2.0)	0	1 (1.0)
VITAMINS, OTHER COMBINATIONS			1 (2.0)	0	1 (1.0)
XANTHINE DERIVATIVES			0	1 (2.0)	1 (1.0)
CAFFEINE			0	1 (2.0)	1 (1.0)

Note: Concomitant medications are defined as medications (other than the study drug) taken on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were taken prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
[1] WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-cm-nonaed.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-07-02-cm-nonaed.rtf

4.1.1.5 Nicht medikamentöse Vor- und Begleittherapien

Table 14.1.7.4
Summary of Prior and Concomitant Therapies
(17-Week Double-Blind Phase, Safety Population)

Therapy [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
Prior Therapies	8 (15.7)	9 (18.0)	17 (16.8)
Ketogenic Diet	3 (5.9)	3 (6.0)	6 (5.9)
Gastrostomy	2 (3.9)	2 (4.0)	4 (4.0)
Speech Rehabilitation	1 (2.0)	2 (4.0)	3 (3.0)
Corpus Callosotomy	1 (2.0)	1 (2.0)	2 (2.0)
Brain Operation	1 (2.0)	0	1 (1.0)
Compression Garment Application	1 (2.0)	0	1 (1.0)
Hip Arthroplasty	0	1 (2.0)	1 (1.0)
Hydrotherapy	0	1 (2.0)	1 (1.0)
Laparoscopic Surgery	0	1 (2.0)	1 (1.0)
Occupational Therapy	1 (2.0)	0	1 (1.0)
Oesophagogastric Fundoplasty	0	1 (2.0)	1 (1.0)
Psychotherapy	0	1 (2.0)	1 (1.0)
Spinal Fusion Surgery	1 (2.0)	0	1 (1.0)
Strabismus Correction	1 (2.0)	0	1 (1.0)
Tooth Extraction	1 (2.0)	0	1 (1.0)
Tooth Repair	1 (2.0)	0	1 (1.0)

Note: Prior therapies are defined as therapies that started and stopped prior to the first dosing day of study drug.
Concomitant therapies are defined as therapies administered on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were started prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
[1] Therapies are coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.7.3

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-prct.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-07-04-prct.rtf

Table 14.1.7.4
Summary of Prior and Concomitant Therapies
(17-Week Double-Blind Phase, Safety Population)

Therapy [1]	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
Concomitant Therapies	26 (51.0)	29 (58.0)	55 (54.5)
Physiotherapy	21 (41.2)	20 (40.0)	41 (40.6)
Speech Rehabilitation	18 (35.3)	15 (30.0)	33 (32.7)
Occupational Therapy	15 (29.4)	12 (24.0)	27 (26.7)
Therapeutic Procedure	7 (13.7)	7 (14.0)	14 (13.9)
Vagal Nerve Stimulator Implantation	6 (11.8)	7 (14.0)	13 (12.9)
Ketogenic Diet	7 (13.7)	4 (8.0)	11 (10.9)
Gastrostomy	4 (7.8)	1 (2.0)	5 (5.0)
Hydrotherapy	4 (7.8)	1 (2.0)	5 (5.0)
Oxygen Therapy	2 (3.9)	2 (4.0)	4 (4.0)
Psychotherapy	1 (2.0)	3 (6.0)	4 (4.0)
Airway Secretion Clearance Therapy	2 (3.9)	0	2 (2.0)
Orthosis User	1 (2.0)	1 (2.0)	2 (2.0)
Acupuncture	0	1 (2.0)	1 (1.0)
Adenotonsillectomy	0	1 (2.0)	1 (1.0)
Eye Exercises	0	1 (2.0)	1 (1.0)
Eyeglasses Therapy	1 (2.0)	0	1 (1.0)
Jejunostomy	1 (2.0)	0	1 (1.0)
Joint Dislocation	1 (2.0)	0	1 (1.0)
Physiotherapy Chest	1 (2.0)	0	1 (1.0)
Therapeutic Aspiration	0	1 (2.0)	1 (1.0)

Note: Prior therapies are defined as therapies that started and stopped prior to the first dosing day of study drug.
Concomitant therapies are defined as therapies administered on or after the first dosing day of the study drug during the 17-week double blind phase of the study, regardless of whether they were started prior to the first dosing day and, for subjects entering the 52-week open-label extension phase, before the first dosing day of that phase.
[1] Therapies are coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.7.3

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-prct.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-07-04-prct.rtf

4.1.2 Medizinische Vorgeschichte

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
Any Medical History Terms	51 (100)	50 (100)	101 (100)
CONGENITAL, FAMILIAL AND GENETIC DISORDERS	51 (100)	48 (96.0)	99 (98.0)
X-LINKED CHROMOSOMAL DISORDER	34 (66.7)	31 (62.0)	65 (64.4)
CDKL5 DEFICIENCY DISORDER	16 (31.4)	18 (36.0)	34 (33.7)
DEVELOPMENTAL HIP DYSPLASIA	4 (7.8)	2 (4.0)	6 (5.9)
RETT SYNDROME	2 (3.9)	1 (2.0)	3 (3.0)
ATRIAL SEPTAL DEFECT	1 (2.0)	1 (2.0)	2 (2.0)
CEREBRAL PALSY	0	2 (4.0)	2 (2.0)
CONGENITAL NYSTAGMUS	1 (2.0)	1 (2.0)	2 (2.0)
BRAIN MALFORMATION	1 (2.0)	0	1 (1.0)
CLEFT LIP	1 (2.0)	0	1 (1.0)
CONGENITAL CEREBROVASCULAR ANOMALY	1 (2.0)	0	1 (1.0)
CRYPTORCHISM	1 (2.0)	0	1 (1.0)
DUCTUS ARTERIOSUS PREMATURE CLOSURE	0	1 (2.0)	1 (1.0)
INTESTINAL MALROTATION	0	1 (2.0)	1 (1.0)
MICROCEPHALY	0	1 (2.0)	1 (1.0)
VESTIBULOCEREBELLAR SYNDROME	1 (2.0)	0	1 (1.0)
NERVOUS SYSTEM DISORDERS	31 (60.8)	30 (60.0)	61 (60.4)
EPILEPSY	20 (39.2)	18 (36.0)	38 (37.6)
HYPOTONIA	9 (17.6)	9 (18.0)	18 (17.8)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
SEIZURE	3 (5.9)	5 (10.0)	8 (7.9)
LENNOX-GASTAUT SYNDROME	5 (9.8)	2 (4.0)	7 (6.9)
INFANTILE SPASMS	3 (5.9)	2 (4.0)	5 (5.0)
INTELLECTUAL DISABILITY	2 (3.9)	3 (6.0)	5 (5.0)
SPEECH DISORDER DEVELOPMENTAL	2 (3.9)	2 (4.0)	4 (4.0)
DYSKINESIA	2 (3.9)	1 (2.0)	3 (3.0)
DYSTONIA	2 (3.9)	1 (2.0)	3 (3.0)
ENCEPHALOPATHY	2 (3.9)	1 (2.0)	3 (3.0)
DROOLING	1 (2.0)	1 (2.0)	2 (2.0)
QUADRIPARESIS	0	2 (4.0)	2 (2.0)
ARACHNOID CYST	0	1 (2.0)	1 (1.0)
CHOREA	0	1 (2.0)	1 (1.0)
COGNITIVE DISORDER	0	1 (2.0)	1 (1.0)
COORDINATION ABNORMAL	0	1 (2.0)	1 (1.0)
DELAYED VISUAL MATURATION	0	1 (2.0)	1 (1.0)
EPILEPTIC ENCEPHALOPATHY	0	1 (2.0)	1 (1.0)
EXAGGERATED STARTLE RESPONSE	1 (2.0)	0	1 (1.0)
GENERALISED TONIC-CLONIC SEIZURE	1 (2.0)	0	1 (1.0)
HYPERKINESIA	0	1 (2.0)	1 (1.0)
LANGUAGE DISORDER	0	1 (2.0)	1 (1.0)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
MOTOR DEVELOPMENTAL DELAY	1 (2.0)	0	1 (1.0)
MUSCLE SPASTICITY	1 (2.0)	0	1 (1.0)
NYSTAGMUS	0	1 (2.0)	1 (1.0)
QUADRIPLEGIA	1 (2.0)	0	1 (1.0)
SEIZURE CLUSTER	1 (2.0)	0	1 (1.0)
SENSORY PROCESSING DISORDER	0	1 (2.0)	1 (1.0)
TONIC CONVULSION	1 (2.0)	0	1 (1.0)
UNRESPONSIVE TO STIMULI	0	1 (2.0)	1 (1.0)
GASTROINTESTINAL DISORDERS	26 (51.0)	27 (54.0)	53 (52.5)
CONSTIPATION	16 (31.4)	17 (34.0)	33 (32.7)
GASTROOESOPHAGEAL REFLUX DISEASE	14 (27.5)	14 (28.0)	28 (27.7)
DYSPHAGIA	3 (5.9)	1 (2.0)	4 (4.0)
PANCREATITIS	0	3 (6.0)	3 (3.0)
SALIVARY HYPERSECRETION	0	3 (6.0)	3 (3.0)
VOMITING	1 (2.0)	2 (4.0)	3 (3.0)
DIARRHOEA	0	2 (4.0)	2 (2.0)
ABDOMINAL DISTENSION	0	1 (2.0)	1 (1.0)
EOSINOPHILIC OESOPHAGITIS	1 (2.0)	0	1 (1.0)
FUNCTIONAL GASTROINTESTINAL DISORDER	0	1 (2.0)	1 (1.0)
GASTRIC ULCER	0	1 (2.0)	1 (1.0)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
IMPAIRED GASTRIC EMPTYING	0	1 (2.0)	1 (1.0)
INTUSSUSCEPTION	0	1 (2.0)	1 (1.0)
IRRITABLE BOWEL SYNDROME	0	1 (2.0)	1 (1.0)
MALOCCLUSION	1 (2.0)	0	1 (1.0)
PANCREATIC STEATOSIS	1 (2.0)	0	1 (1.0)
REGURGITATION	1 (2.0)	0	1 (1.0)
TEETHING	0	1 (2.0)	1 (1.0)
TOOTHACHE	1 (2.0)	0	1 (1.0)
VOLVULUS	0	1 (2.0)	1 (1.0)
EYE DISORDERS	18 (35.3)	20 (40.0)	38 (37.6)
CORTICAL VISUAL IMPAIRMENT	9 (17.6)	12 (24.0)	21 (20.8)
STRABISMUS	4 (7.8)	2 (4.0)	6 (5.9)
VISUAL IMPAIRMENT	3 (5.9)	3 (6.0)	6 (5.9)
BLINDNESS CORTICAL	2 (3.9)	1 (2.0)	3 (3.0)
ASTIGMATISM	1 (2.0)	1 (2.0)	2 (2.0)
HYPERMETROPIA	1 (2.0)	1 (2.0)	2 (2.0)
BLEPHARITIS	0	1 (2.0)	1 (1.0)
GAZE PALSY	0	1 (2.0)	1 (1.0)
GENERAL DISORDERS AND ADMINISTRATION SITE CONDITIONS	14 (27.5)	13 (26.0)	27 (26.7)
DEVELOPMENTAL DELAY	14 (27.5)	12 (24.0)	26 (25.7)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
GAIT INABILITY	1 (2.0)	0	1 (1.0)
INFUSION SITE EXTRAVASATION	0	1 (2.0)	1 (1.0)
PAIN	0	1 (2.0)	1 (1.0)
PYREXIA	1 (2.0)	0	1 (1.0)
INFECTIONS AND INFESTATIONS	12 (23.5)	11 (22.0)	23 (22.8)
PNEUMONIA	3 (5.9)	2 (4.0)	5 (5.0)
URINARY TRACT INFECTION	1 (2.0)	2 (4.0)	3 (3.0)
BRONCHITIS	1 (2.0)	1 (2.0)	2 (2.0)
VARICELLA	1 (2.0)	1 (2.0)	2 (2.0)
BODY TINEA	1 (2.0)	0	1 (1.0)
CHRONIC TONSILLITIS	1 (2.0)	0	1 (1.0)
LARYNGITIS	0	1 (2.0)	1 (1.0)
LOWER RESPIRATORY TRACT INFECTION	0	1 (2.0)	1 (1.0)
MEASLES	1 (2.0)	0	1 (1.0)
NASOPHARYNGITIS	1 (2.0)	0	1 (1.0)
ORAL CANDIDIASIS	0	1 (2.0)	1 (1.0)
OTITIS MEDIA	0	1 (2.0)	1 (1.0)
OTITIS MEDIA CHRONIC	1 (2.0)	0	1 (1.0)
OTOSALPINGITIS	0	1 (2.0)	1 (1.0)
PHARYNGITIS	0	1 (2.0)	1 (1.0)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
RESPIRATORY TRACT INFECTION	0	1 (2.0)	1 (1.0)
RESPIRATORY TRACT INFECTION VIRAL	1 (2.0)	0	1 (1.0)
ROSEOLA	1 (2.0)	0	1 (1.0)
ROTAVIRUS INFECTION	0	1 (2.0)	1 (1.0)
TINEA CAPITIS	1 (2.0)	0	1 (1.0)
UPPER RESPIRATORY TRACT INFECTION	1 (2.0)	0	1 (1.0)
VIRAL INFECTION	0	1 (2.0)	1 (1.0)
RESPIRATORY, THORACIC AND MEDIASTINAL DISORDERS	8 (15.7)	14 (28.0)	22 (21.8)
ASTHMA	4 (7.8)	1 (2.0)	5 (5.0)
CHRONIC RESPIRATORY DISEASE	3 (5.9)	2 (4.0)	5 (5.0)
SLEEP APNOEA SYNDROME	0	5 (10.0)	5 (5.0)
PNEUMONIA ASPIRATION	0	3 (6.0)	3 (3.0)
RHINITIS ALLERGIC	2 (3.9)	0	2 (2.0)
BRONCHIAL HYPERREACTIVITY	0	1 (2.0)	1 (1.0)
CHILDHOOD ASTHMA	0	1 (2.0)	1 (1.0)
CHRONIC OBSTRUCTIVE PULMONARY DISEASE	0	1 (2.0)	1 (1.0)
DYSPNOEA	0	1 (2.0)	1 (1.0)
EPISTAXIS	0	1 (2.0)	1 (1.0)
HYPOXIA	0	1 (2.0)	1 (1.0)
LOWER RESPIRATORY TRACT CONGESTION	0	1 (2.0)	1 (1.0)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
PULMONARY HYPERTENSION	0	1 (2.0)	1 (1.0)
REFLUX LARYNGITIS	1 (2.0)	0	1 (1.0)
RESPIRATORY DISORDER	1 (2.0)	0	1 (1.0)
RESPIRATORY FAILURE	0	1 (2.0)	1 (1.0)
RESTRICTIVE PULMONARY DISEASE	1 (2.0)	0	1 (1.0)
TONSILLAR HYPERTROPHY	0	1 (2.0)	1 (1.0)
WHEEZING	0	1 (2.0)	1 (1.0)
SURGICAL AND MEDICAL PROCEDURES	9 (17.6)	13 (26.0)	22 (21.8)
GASTROSTOMY	7 (13.7)	7 (14.0)	14 (13.9)
VAGAL NERVE STIMULATOR IMPLANTATION	2 (3.9)	4 (8.0)	6 (5.9)
GASTROINTESTINAL TUBE INSERTION	0	3 (6.0)	3 (3.0)
EAR TUBE INSERTION	1 (2.0)	1 (2.0)	2 (2.0)
KETOGENIC DIET	1 (2.0)	1 (2.0)	2 (2.0)
TONSILLECTOMY	0	2 (4.0)	2 (2.0)
ADENOIDECTOMY	0	1 (2.0)	1 (1.0)
ENTERAL NUTRITION	0	1 (2.0)	1 (1.0)
EXTERNAL VAGAL NERVE STIMULATION	0	1 (2.0)	1 (1.0)
OXYGEN THERAPY	1 (2.0)	0	1 (1.0)
PROPHYLAXIS	1 (2.0)	0	1 (1.0)
SPINAL FUSION SURGERY	0	1 (2.0)	1 (1.0)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
STRABISMUS CORRECTION	0	1 (2.0)	1 (1.0)
TOOTH EXTRACTION	0	1 (2.0)	1 (1.0)
MUSCULOSKELETAL AND CONNECTIVE TISSUE DISORDERS	12 (23.5)	6 (12.0)	18 (17.8)
SCOLIOSIS	9 (17.6)	5 (10.0)	14 (13.9)
OSTEOPENIA	2 (3.9)	0	2 (2.0)
ARTHRALGIA	1 (2.0)	0	1 (1.0)
ARTHROPATHY	0	1 (2.0)	1 (1.0)
OSTEOPOROSIS	1 (2.0)	0	1 (1.0)
PATHOLOGICAL FRACTURE	1 (2.0)	0	1 (1.0)
PSYCHIATRIC DISORDERS	10 (19.6)	8 (16.0)	18 (17.8)
INSOMNIA	7 (13.7)	2 (4.0)	9 (8.9)
AUTISM SPECTRUM DISORDER	1 (2.0)	2 (4.0)	3 (3.0)
AGGRESSION	0	1 (2.0)	1 (1.0)
ANXIETY	0	1 (2.0)	1 (1.0)
BRUXISM	0	1 (2.0)	1 (1.0)
EATING DISORDER	0	1 (2.0)	1 (1.0)
INTENTIONAL SELF-INJURY	0	1 (2.0)	1 (1.0)
IRRITABILITY	1 (2.0)	0	1 (1.0)
LEARNING DISABILITY	0	1 (2.0)	1 (1.0)
ORGANIC BRAIN SYNDROME	1 (2.0)	0	1 (1.0)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
POVERTY OF SPEECH	1 (2.0)	0	1 (1.0)
SLEEP DISORDER	0	1 (2.0)	1 (1.0)
METABOLISM AND NUTRITION DISORDERS	10 (19.6)	6 (12.0)	16 (15.8)
POOR FEEDING INFANT	1 (2.0)	2 (4.0)	3 (3.0)
FEEDING DISORDER	2 (3.9)	0	2 (2.0)
FEEDING INTOLERANCE	2 (3.9)	0	2 (2.0)
VITAMIN D DEFICIENCY	1 (2.0)	1 (2.0)	2 (2.0)
WEIGHT GAIN POOR	0	2 (4.0)	2 (2.0)
CARNITINE DEFICIENCY	1 (2.0)	0	1 (1.0)
DECREASED APPETITE	1 (2.0)	0	1 (1.0)
DEHYDRATION	1 (2.0)	0	1 (1.0)
FAILURE TO THRIVE	0	1 (2.0)	1 (1.0)
HYPOVITAMINOSIS	0	1 (2.0)	1 (1.0)
MALNUTRITION	1 (2.0)	0	1 (1.0)
MITOCHONDRIAL CYTOPATHY	0	1 (2.0)	1 (1.0)
INJURY, POISONING AND PROCEDURAL COMPLICATIONS	6 (11.8)	3 (6.0)	9 (8.9)
FEMUR FRACTURE	2 (3.9)	1 (2.0)	3 (3.0)
JOINT DISLOCATION	3 (5.9)	0	3 (3.0)
FACIAL BONES FRACTURE	0	1 (2.0)	1 (1.0)
SCAR	0	1 (2.0)	1 (1.0)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
STOMA SITE HYPERGRANULATION	1 (2.0)	0	1 (1.0)
STOMA SITE REACTION	0	1 (2.0)	1 (1.0)
IMMUNE SYSTEM DISORDERS	2 (3.9)	6 (12.0)	8 (7.9)
SEASONAL ALLERGY	2 (3.9)	3 (6.0)	5 (5.0)
DRUG HYPERSENSITIVITY	0	2 (4.0)	2 (2.0)
HYPOGAMMAGLOBULINAEMIA	0	1 (2.0)	1 (1.0)
SKIN AND SUBCUTANEOUS TISSUE DISORDERS	4 (7.8)	4 (8.0)	8 (7.9)
ECZEMA	3 (5.9)	2 (4.0)	5 (5.0)
ACNE	1 (2.0)	1 (2.0)	2 (2.0)
DERMATITIS CONTACT	0	2 (4.0)	2 (2.0)
DERMATITIS ACNEIFORM	1 (2.0)	0	1 (1.0)
INVESTIGATIONS	3 (5.9)	4 (8.0)	7 (6.9)
BODY MASS INDEX DECREASED	1 (2.0)	0	1 (1.0)
ENDOSCOPY	0	1 (2.0)	1 (1.0)
GAMMA-GLUTAMYLTRANSFERASE INCREASED	1 (2.0)	0	1 (1.0)
LIPASE INCREASED	1 (2.0)	0	1 (1.0)
ULTRASOUND KIDNEY ABNORMAL	0	1 (2.0)	1 (1.0)
URINARY SEDIMENT	1 (2.0)	0	1 (1.0)
VITAMIN D DECREASED	0	1 (2.0)	1 (1.0)
WEIGHT DECREASED	0	1 (2.0)	1 (1.0)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
CARDIAC DISORDERS	3 (5.9)	3 (6.0)	6 (5.9)
LEFT VENTRICULAR DYSFUNCTION	1 (2.0)	1 (2.0)	2 (2.0)
BUNDLE BRANCH BLOCK RIGHT	0	1 (2.0)	1 (1.0)
CARDIAC VENTRICULAR DISORDER	0	1 (2.0)	1 (1.0)
MITRAL VALVE INCOMPETENCE	1 (2.0)	0	1 (1.0)
MITRAL VALVE PROLAPSE	1 (2.0)	0	1 (1.0)
RIGHT VENTRICULAR DILATATION	1 (2.0)	0	1 (1.0)
SINUS ARRHYTHMIA	1 (2.0)	0	1 (1.0)
TACHYARRHYTHMIA	0	1 (2.0)	1 (1.0)
RENAL AND URINARY DISORDERS	2 (3.9)	4 (8.0)	6 (5.9)
URINARY RETENTION	0	2 (4.0)	2 (2.0)
METABOLIC NEPHROPATHY	0	1 (2.0)	1 (1.0)
MIXED INCONTINENCE	0	1 (2.0)	1 (1.0)
NEPHROLITHIASIS	1 (2.0)	0	1 (1.0)
RENAL CYST	1 (2.0)	0	1 (1.0)
PREGNANCY, PUERPERIUM AND PERINATAL CONDITIONS	1 (2.0)	2 (4.0)	3 (3.0)
PERINATAL BRAIN DAMAGE	1 (2.0)	2 (4.0)	3 (3.0)
PREMATURE BABY	0	1 (2.0)	1 (1.0)
VASCULAR DISORDERS	2 (3.9)	1 (2.0)	3 (3.0)
CIRCULATORY COLLAPSE	0	1 (2.0)	1 (1.0)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=51) n (%)	Ganaxolone (N=50) n (%)	Total (N=101) n (%)
HYPERTENSION	1 (2.0)	0	1 (1.0)
VASOCONSTRICTION	1 (2.0)	0	1 (1.0)
EAR AND LABYRINTH DISORDERS	1 (2.0)	1 (2.0)	2 (2.0)
DEAFNESS NEUROSENSORY	1 (2.0)	0	1 (1.0)
LABYRINTHINE FISTULA	0	1 (2.0)	1 (1.0)
ENDOCRINE DISORDERS	1 (2.0)	1 (2.0)	2 (2.0)
HYPOTHYROIDISM	1 (2.0)	0	1 (1.0)
PRECOCIOUS PUBERTY	0	1 (2.0)	1 (1.0)
HEPATOBILIARY DISORDERS	0	2 (4.0)	2 (2.0)
BILIARY TRACT DISORDER	0	1 (2.0)	1 (1.0)
GALLBLADDER DISORDER	0	1 (2.0)	1 (1.0)
BLOOD AND LYMPHATIC SYSTEM DISORDERS	0	1 (2.0)	1 (1.0)
HYPOCHROMIC ANAEMIA	0	1 (2.0)	1 (1.0)
SOCIAL CIRCUMSTANCES	1 (2.0)	0	1 (1.0)
WALKING DISABILITY	1 (2.0)	0	1 (1.0)
NOT CODED	0	1 (2.0)	1 (1.0)
NOT CODED	0	1 (2.0)	1 (1.0)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-03-mh.rtf

4.1.3 Ergebnisse der genetischen Testung

Table 14.1.4
Summary of Genetic Testing
(Safety Population)

Variable	Placebo (N=51)	Ganaxolone (N=50)	Total (N=101)
Mutations in other genes, n(%)			
None	39 (76.5)	36 (72.0)	75 (74.3)
POLG	7 (13.7)	8 (16.0)	15 (14.9)
CDKL5	2 (3.9)	1 (2.0)	3 (3.0)
MECP2	1 (2.0)	1 (2.0)	2 (2.0)
SCN2A	1 (2.0)	1 (2.0)	2 (2.0)
ARX	1 (2.0)	0	1 (1.0)
FOLR1	1 (2.0)	0	1 (1.0)
FOXG1	0	1 (2.0)	1 (1.0)
PCDH19	1 (2.0)	0	1 (1.0)
RS1 GENE DELETED	1 (2.0)	0	1 (1.0)
SCN1A	0	1 (2.0)	1 (1.0)
SLC2A1	0	1 (2.0)	1 (1.0)
SPTAN1	1 (2.0)	0	1 (1.0)
TSC1	0	1 (2.0)	1 (1.0)
THE PATIENT IS HETEROZYGOUS FOR A LIKELY PATHOGENIC VARIANT IN THE CDLK5 GENE WHICH IS LIKELY CONSISTENT WITH THE DIAGNOSIS OF A CDLK5-RELATED DISORDER.	0	1 (2.0)	1 (1.0)

Source Data: Listing 16.2.3.3

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-gt.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-04-gt.rtf

Table 14.1.4
Summary of Genetic Testing
(Safety Population)

Variable	Placebo (N=51)	Ganaxolone (N=50)	Total (N=101)
Genetic test classification, n(%)			
n	51	50	101
Pathogenic	26 (51.0)	33 (66.0)	59 (58.4)
Likely Pathogenic	20 (39.2)	11 (22.0)	31 (30.7)
Variant of Uncertain Significance (VUS)	5 (9.8)	6 (12.0)	11 (10.9)
Mutation Of a de novo variant of unknown significance (Vus) in the kinase domain, n(%)			
Yes	5 (9.8)	4 (8.0)	9 (8.9)
No	0	2 (4.0)	2 (2.0)
Seizure onset by 1 year of age, n(%)			
n	51	50	101
Yes	51 (100)	49 (98.0)	100 (99.0)
No	0	1 (2.0)	1 (1.0)
Lack of independent ambulation by 2 years of age			
n	51	50	101
Yes	51 (100)	50 (100)	101 (100)

Source Data: Listing 16.2.3.3

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\t-gt.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\final_unblinded\prog\tlfs\output\t-14-01-04-gt.rtf

4.2 Ergänzende Analysen zum Endpunkt Morbidität – Veränderung der Anfallshäufigkeit

4.2.1 Prozentuale Veränderung der Anfallshäufigkeit sekundärer und tertiärer Anfälle pro 28 Tage während der 17-wöchigen doppelblinden Behandlungsphase

Study No. 1042-CDD-3001

Table 14.2.5.6.1 Summary of 28-Day Seizure Frequency for Secondary Seizure Types

28-day seizure frequency (secondary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	4.16 (12.637)	0.00 (0.00, 0.00)	0.00, 0.00	0.0, 64.0			
	17-Week Post-Baseline Phase	50	0 (0.0)	4.40 (11.729)	0.00 (0.00, 0.00)	0.00, 0.23	0.0, 51.7			
	Arithmetic Change from Baseline	49	1 (2.0)	0.33 (5.570)	0.00 (0.00, 0.00)	0.00, 0.00	-18.8, 16.7			
	Percent Change from Baseline	12	38 (76.0)	267.00 (775.935)	-23.33 (-95.73, 84.31)	-95.13, 73.09	-100.0, 2498.3			
Placebo, N=51	Baseline	51	0 (0.0)	2.87 (8.837)	0.00 (0.00, 0.00)	0.00, 0.00	0.0, 46.7	0.00 (0.00, 0.00)	0.4272	0.7941
	17-Week Post-Baseline Phase	51	0 (0.0)	1.37 (5.360)	0.00 (0.00, 0.00)	0.00, 0.00	0.0, 36.1	0.00 (0.00, 0.00)	0.1769	1.3503
	Arithmetic Change from Baseline	51	0 (0.0)	-1.51 (4.632)	0.00 (0.00, 0.00)	0.00, 0.00	-25.5, 0.0	0.00 (0.00, 0.00)	0.1242	1.5373
	Percent Change from Baseline	9	42 (82.4)	-57.80 (29.389)	-58.76 (-89.18, -22.71)	-71.72, -47.06	-100.0, -8.6	28.92 (-29.80, 136.56)	0.4550	0.7472

Study No. 1042-CDD-3001

Table 14.2.5.6.2 Summary of 28-Day Seizure Frequency for Tertiary Seizure Types

28-day seizure frequency (tertiary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	59.27 (103.606)	9.33 (0.00, 29.33)	0.00, 63.33	0.0, 441.9			
	17-Week Post-Baseline Phase	50	0 (0.0)	50.74 (109.814)	4.63 (0.00, 18.19)	0.00, 32.31	0.0, 558.9			
	Arithmetic Change from Baseline	49	1 (2.0)	-7.67 (69.143)	0.00 (-3.70, 0.00)	-14.87, 0.00	-299.7, 222.4			
	Percent Change from Baseline	27	23 (46.0)	61.61 (354.348)	-25.90 (-64.41, 1.03)	-76.77, 21.44	-100.0, 1733.3			
Placebo, N=51	Baseline	51	0 (0.0)	34.25 (103.552)	0.00 (0.00, 2.73)	0.00, 12.21	0.0, 669.1	0.00 (0.00, 12.98)	0.0886	1.7028
	17-Week Post-Baseline Phase	51	0 (0.0)	32.83 (87.380)	0.00 (0.00, 2.35)	0.00, 25.69	0.0, 554.6	0.00 (0.00, 5.69)	0.1148	1.5769
	Arithmetic Change from Baseline	51	0 (0.0)	-1.43 (33.186)	0.00 (0.00, 0.00)	0.00, 0.00	-114.5, 109.7	0.00 (-6.07, 0.00)	0.1854	-1.3245
	Percent Change from Baseline	22	29 (56.9)	227.47 (1034.580)	-18.83 (-46.03, 39.38)	-59.05, 39.38	-100.0, 4833.3	-15.49 (-56.44, 25.74)	0.4880	-0.6935

4.2.1 Sensitivitätsanalysen

4.2.1.1 Sensitivitätsanalysen: Prozentuale Veränderung der Gesamtanfallshäufigkeit pro 28 Tage gegenüber Baseline

Study No. 1042-CDD-3001
Table 14.2.5.1.s1 Summary of 28-Day Seizure Frequency for All Seizure Types
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

28-day seizure frequency (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	178.80 (174.002)	116.00 (75.33, 173.33)	45.33, 223.33	18.4, 651.3			
	17-Week Post-Baseline Phase	50	0 (0.0)	144.10 (183.701)	69.06 (65.59, 108.38)	32.59, 146.83	0.5, 817.7			
	Arithmetic Change from Baseline	49	1 (2.0)	-32.92 (148.029)	-17.95 (-30.59, -0.86)	-76.97, 3.62	-501.8, 373.7			
	Percent Change from Baseline	49	1 (2.0)	-10.77 (65.959)	-21.26 (-39.67, -3.28)	-49.93, 8.63	-97.3, 274.8			
Placebo, N=51	Baseline	51	0 (0.0)	141.07 (200.875)	60.00 (46.00, 120.00)	26.67, 162.00	7.0, 1021.3	26.33 (-1.33, 76.00)	0.0636	1.8548
	17-Week Post-Baseline Phase	51	0 (0.0)	161.31 (489.989)	67.50 (55.50, 82.55)	39.55, 111.49	3.1, 3542.1	3.96 (-16.27, 31.75)	0.5450	0.6053
	Arithmetic Change from Baseline	51	0 (0.0)	20.24 (429.112)	-5.00 (-10.56, 13.92)	-25.83, 21.50	-953.8, 2772.1	-19.17 (-43.86, 0.32)	0.0527	-1.9376
	Percent Change from Baseline	51	0 (0.0)	41.66 (143.033)	-6.90 (-24.13, 12.32)	-31.18, 37.29	-93.4, 540.4	-18.86 (-42.10, 1.35)	0.0607	-1.8755

Study No. 1042-CDD-3001
Table 14.2.5.1.s2 Summary of 28-Day Seizure Frequency for All Seizure Types
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

28-day seizure frequency (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	178.80 (174.002)	116.00 (75.33, 173.33)	45.33, 223.33	18.4, 651.3			
	17-Week Post-Baseline Phase	50	0 (0.0)	169.71 (189.120)	93.30 (65.59, 146.83)	32.59, 285.03	0.5, 817.7			
	Arithmetic Change from Baseline	49	1 (2.0)	-6.78 (135.889)	-12.78 (-25.35, 2.08)	-51.67, 7.58	-501.8, 373.7			
	Percent Change from Baseline	49	1 (2.0)	14.08 (158.672)	-12.45 (-32.86, 1.82)	-40.61, 12.64	-97.3, 1020.7			
Placebo, N=51	Baseline	51	0 (0.0)	141.07 (200.875)	60.00 (46.00, 120.00)	26.67, 162.00	7.0, 1021.3	26.33 (-1.33, 76.00)	0.0636	1.8548
	17-Week Post-Baseline Phase	51	0 (0.0)	201.49 (491.171)	82.55 (55.50, 157.44)	39.55, 277.86	3.1, 3542.1	1.34 (-28.04, 38.47)	0.8277	0.2176
	Arithmetic Change from Baseline	51	0 (0.0)	60.41 (417.237)	-0.39 (-7.77, 15.80)	-19.76, 46.84	-697.7, 2772.1	-19.49 (-47.28, 0.69)	0.0570	-1.9031
	Percent Change from Baseline	51	0 (0.0)	135.57 (389.217)	-1.51 (-20.52, 17.52)	-29.31, 57.05	-83.2, 1778.1	-17.70 (-41.57, 1.56)	0.0687	-1.8203

4.2.1.2 Sensitivitätsanalysen Anteil der Patienten mit einer Reduktion der Gesamtanfallshäufigkeit pro 28 Tage um ≥ 25 %, ≥ 50 % und ≥ 75 %

Study No. 1042-CDD-3001
Table 14.2.3.2.1.s1 Summary of Notable Reduction Rates in 28-Day Seizure Frequency
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Seizure type	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Primary Seizures	GNX, N=50	49	25% Responder	1 (2.0)	28 (57.1)				
			50% Responder	1 (2.0)	11 (22.4)				
			75% Responder	1 (2.0)	4 (8.2)				
	Placebo, N=51	51	25% Responder	0 (0.0)	13 (25.5)	3.90 (1.67, 9.09)	2.24 (1.32, 3.80)	31.7 (11.7, 49.3)	0.0021
			50% Responder	0 (0.0)	5 (9.8)	2.66 (0.85, 8.34)	2.29 (0.86, 6.11)	12.6 (-6.7, 31.9)	0.1055
			75% Responder	0 (0.0)	2 (3.9)	2.18 (0.38, 12.47)	2.08 (0.40, 10.86)	4.2 (-15.3, 24.0)	0.4316
All Seizures	GNX, N=50	49	25% Responder	1 (2.0)	23 (46.9)				
			50% Responder	1 (2.0)	12 (24.5)				
			75% Responder	1 (2.0)	5 (10.2)				
	Placebo, N=51	51	25% Responder	0 (0.0)	18 (35.3)	1.62 (0.73, 3.62)	1.33 (0.83, 2.14)	11.6 (-8.5, 30.6)	0.3096
			50% Responder	0 (0.0)	8 (15.7)	1.74 (0.64, 4.72)	1.56 (0.70, 3.49)	8.8 (-10.6, 28.1)	0.3226
			75% Responder	0 (0.0)	3 (5.9)	1.82 (0.41, 8.06)	1.73 (0.44, 6.87)	4.3 (-15.1, 24.1)	0.4829

Study No. 1042-CDD-3001
Table 14.2.3.2.1.s1 Summary of Notable Reduction Rates in 28-Day Seizure Frequency
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Seizure type	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Primary Seizures	GNX, N=50	49	25% Responder	1 (2.0)	26 (53.1)				
			50% Responder	1 (2.0)	11 (22.4)				
			75% Responder	1 (2.0)	4 (8.2)				
	Placebo, N=51	51	25% Responder	0 (0.0)	12 (23.5)	3.67 (1.56, 8.65)	2.26 (1.29, 3.95)	29.5 (9.6, 47.1)	0.0037
			50% Responder	0 (0.0)	5 (9.8)	2.66 (0.85, 8.34)	2.29 (0.86, 6.11)	12.6 (-6.7, 31.9)	0.1055
			75% Responder	0 (0.0)	2 (3.9)	2.18 (0.38, 12.47)	2.08 (0.40, 10.86)	4.2 (-15.3, 24.0)	0.4316
All Seizures	GNX, N=50	49	25% Responder	1 (2.0)	20 (40.8)				
			50% Responder	1 (2.0)	8 (16.3)				
			75% Responder	1 (2.0)	3 (6.1)				
	Placebo, N=51	51	25% Responder	0 (0.0)	16 (31.4)	1.51 (0.66, 3.43)	1.30 (0.77, 2.21)	9.4 (-10.6, 28.1)	0.4055
			50% Responder	0 (0.0)	6 (11.8)	1.46 (0.47, 4.58)	1.39 (0.52, 3.71)	4.6 (-14.6, 24.3)	0.5734
			75% Responder	0 (0.0)	1 (2.0)	3.26 (0.33, 32.47)	3.12 (0.34, 29.01)	4.2 (-15.5, 23.9)	0.3574

4.2.1.3 Sensitivitätsanalysen: Prozentzahl der Tage ohne Anfälle; Veränderung gegenüber Baseline

Study No. 1042-CDD-3001
Table 14.2.3.1.1.s1 Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Percentage of seizure free days (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	22.57 (25.761)	11.90 (0.00, 30.95)	0.00, 40.48	0.0, 82.9			
	17-Week Post-Baseline Phase	50	0 (0.0)	33.12 (29.984)	32.35 (15.25, 42.34)	1.72, 55.37	0.0, 98.2			
	Arithmetic Change from Baseline	49	1 (2.0)	10.46 (20.459)	4.91 (0.00, 11.11)	0.00, 15.60	-29.4, 91.4			
Placebo, N=51	Baseline	51	0 (0.0)	30.32 (27.070)	23.81 (10.53, 35.71)	2.63, 54.76	0.0, 97.5	-6.67 (-17.07, 0.00)	0.0909	-1.6909
	17-Week Post-Baseline Phase	51	0 (0.0)	36.76 (28.279)	33.33 (25.64, 39.50)	13.51, 64.23	0.0, 95.4	-2.96 (-16.75, 6.36)	0.4120	-0.8204
	Arithmetic Change from Baseline	51	0 (0.0)	6.44 (17.864)	2.32 (-0.98, 9.47)	-5.75, 18.97	-29.8, 53.9	2.38 (-2.71, 9.22)	0.2956	1.0458

Study No. 1042-CDD-3001
Table 14.2.3.1.1.s2 Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Percentage of seizure free days (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	22.57 (25.761)	11.90 (0.00, 30.95)	0.00, 40.48	0.0, 82.9			
	17-Week Post-Baseline Phase	50	0 (0.0)	29.79 (31.617)	16.02 (3.36, 42.34)	0.00, 55.37	0.0, 98.2			
	Arithmetic Change from Baseline	49	1 (2.0)	7.06 (22.182)	1.71 (0.00, 10.00)	0.00, 15.25	-35.7, 91.4			
Placebo, N=51	Baseline	51	0 (0.0)	30.32 (27.070)	23.81 (10.53, 35.71)	2.63, 54.76	0.0, 97.5	-6.67 (-17.07, 0.00)	0.0909	-1.6909
	17-Week Post-Baseline Phase	51	0 (0.0)	31.53 (31.403)	21.37 (7.14, 39.50)	0.86, 64.23	0.0, 95.4	0.00 (-10.49, 5.73)	0.7114	-0.3700
	Arithmetic Change from Baseline	51	0 (0.0)	1.21 (21.331)	0.00 (-1.10, 7.48)	-6.17, 11.81	-63.2, 53.9	2.42 (-1.84, 9.56)	0.2398	1.1756

Study No. 1042-CDD-3001
Table 14.2.5.2.s1 Summary of the Percentage of Seizure-Free Days for All Seizure Types
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Percentage of seizure free days (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	11.99 (19.044)	0.00 (0.00, 9.52)	0.00, 17.14	0.0, 75.6			
	17-Week Post-Baseline Phase	50	0 (0.0)	19.74 (24.965)	9.62 (0.98, 20.51)	0.00, 24.51	0.0, 98.2			
	Arithmetic Change from Baseline	49	1 (2.0)	7.70 (16.397)	0.87 (0.00, 3.75)	0.00, 18.78	-29.4, 56.2			
Placebo, N=51	Baseline	51	0 (0.0)	21.62 (22.853)	16.22 (9.52, 23.81)	0.00, 40.48	0.0, 81.0	-7.14 (-14.69, 0.00)	0.0143	-2.4485
	17-Week Post-Baseline Phase	51	0 (0.0)	27.63 (25.803)	20.51 (17.24, 26.50)	4.31, 41.88	0.0, 89.7	-5.82 (-17.42, 0.00)	0.0637	-1.8542
	Arithmetic Change from Baseline	51	0 (0.0)	6.02 (18.172)	0.00 (-0.64, 11.34)	-4.80, 20.51	-42.6, 53.9	0.87 (-3.97, 5.78)	0.4843	0.6993

Study No. 1042-CDD-3001
Table 14.2.5.2.s2 Summary of the Percentage of Seizure-Free Days for All Seizure Types
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Percentage of seizure free days (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	11.99 (19.044)	0.00 (0.00, 9.52)	0.00, 17.14	0.0, 75.6			
	17-Week Post-Baseline Phase	50	0 (0.0)	17.69 (25.665)	2.54 (0.00, 14.69)	0.00, 24.51	0.0, 98.2			
	Arithmetic Change from Baseline	49	1 (2.0)	5.61 (16.703)	0.00 (0.00, 0.98)	0.00, 6.78	-29.4, 56.2			
Placebo, N=51	Baseline	51	0 (0.0)	21.62 (22.853)	16.22 (9.52, 23.81)	0.00, 40.48	0.0, 81.0	-7.14 (-14.69, 0.00)	0.0143	-2.4485
	17-Week Post-Baseline Phase	51	0 (0.0)	24.41 (27.736)	12.61 (1.00, 26.50)	0.00, 41.88	0.0, 89.7	-0.32 (-11.75, 0.00)	0.2318	-1.1957
	Arithmetic Change from Baseline	51	0 (0.0)	2.80 (21.164)	0.00 (-0.64, 4.95)	-4.80, 16.12	-63.2, 53.9	0.26 (-1.99, 3.88)	0.4668	0.7277

4.2.1.4 Sensitivitätsanalysen: Längstes anfallsfreies Intervall; Änderung gegenüber Baseline

Study No. 1042-CDD-3001
Table 14.2.5.3.1.s1 Summary of the Longest Seizure-Free Interval (Percentage of Days) for Primary Seizure Types
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Longest Period of Time Seizure Free (%) (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	7.86 (10.907)	2.56 (0.00, 7.14)	0.00, 11.90	0.0, 51.2			
	17-Week Post-Baseline Phase	50	0 (0.0)	6.82 (9.208)	5.08 (1.75, 6.25)	0.87, 7.63	0.0, 50.0			
	Arithmetic Change from Baseline	49	1 (2.0)	-1.20 (6.710)	0.00 (-1.17, 0.38)	-2.06, 1.72	-20.5, 20.7			
Placebo, N=51	Baseline	51	0 (0.0)	12.35 (17.707)	7.14 (4.76, 10.26)	2.38, 14.29	0.0, 95.0	-2.38 (-4.84, 0.00)	0.0799	-1.7510
	17-Week Post-Baseline Phase	51	0 (0.0)	7.58 (8.973)	5.08 (2.94, 5.60)	1.79, 7.83	0.0, 42.2	-0.20 (-2.52, 0.88)	0.4747	-0.7149
	Arithmetic Change from Baseline	51	0 (0.0)	-4.77 (14.838)	-1.70 (-4.39, 0.00)	-9.39, 1.63	-67.2, 35.1	1.72 (-0.18, 3.99)	0.1026	1.6326

Study No. 1042-CDD-3001
Table 14.2.5.3.1.s2 Summary of the Longest Seizure-Free Interval (Percentage of Days) for Primary Seizure Types
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Longest Period of Time Seizure Free (%) (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	7.86 (10.907)	2.56 (0.00, 7.14)	0.00, 11.90	0.0, 51.2			
	17-Week Post-Baseline Phase	50	0 (0.0)	6.31 (9.432)	2.60 (1.67, 6.25)	0.00, 7.63	0.0, 50.0			
	Arithmetic Change from Baseline	49	1 (2.0)	-1.72 (6.682)	0.00 (-1.52, 0.12)	-3.42, 1.69	-20.5, 20.7			
Placebo, N=51	Baseline	51	0 (0.0)	12.35 (17.707)	7.14 (4.76, 10.26)	2.38, 14.29	0.0, 95.0	-2.38 (-4.84, 0.00)	0.0799	-1.7510
	17-Week Post-Baseline Phase	51	0 (0.0)	6.78 (9.384)	2.80 (1.71, 5.60)	0.85, 7.83	0.0, 42.2	0.00 (-1.72, 1.12)	0.7089	-0.3734
	Arithmetic Change from Baseline	51	0 (0.0)	-5.56 (14.716)	-1.87 (-7.14, 0.00)	-9.94, 0.00	-67.2, 35.1	2.01 (0.00, 4.35)	0.0563	1.9086

Study No. 1042-CDD-3001
Table 14.2.5.3.2.s1 Summary of the Longest Seizure-Free Interval (Percentage of Days) for All Seizure Types
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Longest Period of Time Seizure Free (%) (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	4.61 (9.130)	0.00 (0.00, 4.76)	0.00, 6.25	0.0, 51.2			
	17-Week Post-Baseline Phase	50	0 (0.0)	3.84 (7.474)	1.74 (0.87, 2.56)	0.00, 4.90	0.0, 50.0			
	Arithmetic Change from Baseline	49	1 (2.0)	-0.84 (4.469)	0.00 (0.00, 0.31)	-2.44, 1.42	-20.5, 7.8			
Placebo, N=51	Baseline	51	0 (0.0)	8.23 (12.720)	4.76 (2.38, 7.14)	0.00, 10.53	0.0, 59.5	-2.38 (-3.38, 0.00)	0.0251	-2.2398
	17-Week Post-Baseline Phase	51	0 (0.0)	5.37 (7.423)	2.56 (2.19, 3.51)	1.00, 7.65	0.0, 42.2	-0.86 (-1.87, 0.00)	0.0652	-1.8440
	Arithmetic Change from Baseline	51	0 (0.0)	-2.86 (11.112)	-1.50 (-2.24, 0.00)	-4.96, 1.01	-41.2, 35.1	1.18 (-0.14, 2.56)	0.1403	1.4748

Study No. 1042-CDD-3001
Table 14.2.5.3.2.s2 Summary of the Longest Seizure-Free Interval (Percentage of Days) for All Seizure Types
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Longest Period of Time Seizure Free (%) (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=50	Baseline	49	1 (2.0)	4.61 (9.130)	0.00 (0.00, 4.76)	0.00, 6.25	0.0, 51.2			
	17-Week Post-Baseline Phase	50	0 (0.0)	3.58 (7.558)	1.32 (0.00, 2.26)	0.00, 4.90	0.0, 50.0			
	Arithmetic Change from Baseline	49	1 (2.0)	-1.10 (4.439)	0.00 (-0.70, 0.00)	-2.44, 0.87	-20.5, 7.8			
Placebo, N=51	Baseline	51	0 (0.0)	8.23 (12.720)	4.76 (2.38, 7.14)	0.00, 10.53	0.0, 59.5	-2.38 (-3.38, 0.00)	0.0251	-2.2398
	17-Week Post-Baseline Phase	51	0 (0.0)	4.97 (7.635)	1.87 (0.87, 3.51)	0.00, 7.65	0.0, 42.2	-0.03 (-1.72, 0.00)	0.2147	-1.2409
	Arithmetic Change from Baseline	51	0 (0.0)	-3.26 (11.130)	-1.50 (-2.24, 0.00)	-7.14, 0.00	-41.2, 35.1	1.00 (0.00, 2.38)	0.0913	1.6888

4.3 Ergänzende Analysen zum Endpunkt Morbidität – Globaler Betreuereindruck (CGI)

4.3.1 Sensitivitätsanalysen

4.3.1.1 Sensitivitätsanalysen: Mittlere Änderung der Anfallsintensität und -dauer (CGI-CSID), der Aufmerksamkeit (CGICA), des Verhaltens (CGI-C – Zielverhalten) und des globalen klinischen Eindrucks der Verbesserung (CGI-I – Eltern/Betreuer) im Studienverlauf zu den Erhebungszeitpunkten in Woche 1–17 (MMRM)

Study No. 1042-CDD-3001

Table 14.2.3.3.1 Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID) Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

					Differeces of LSM		
Visit		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 3 (End of Week 5)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGI-CSID, n (%)	Very Much Improved	4 (8.0)	2 (3.9)		.	
		Much Improved	12 (24.0)	6 (11.8)		.	
		Minimally Improved	24 (48.0)	15 (29.4)		.	
		No Change	8 (16.0)	25 (49.0)		.	
		Minimally Worse	2 (4.0)	3 (5.9)		.	
	Mean (SD)	.	2.8 (0.93)	3.4 (0.92)		.	-0.62 (-1.016, -0.218)
	LS Mean (SE)	.	2.8 (0.16)	3.4 (0.16)	-0.57 (0.230) [-1.025, -0.118]	0.0137	
Visit 4 (End of Week 9)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGI-CSID, n (%)	Very Much Improved	5 (10.0)	3 (5.9)		.	
		Much Improved	11 (22.0)	6 (11.8)		.	
		Minimally Improved	13 (26.0)	10 (19.6)		.	
		No Change	13 (26.0)	26 (51.0)		.	
		Minimally Worse	4 (8.0)	5 (9.8)		.	
		Much Worse	3 (6.0)	1 (2.0)		.	
		Very Much Worse	1 (2.0)	0		.	

					Differeces of LSM		
Visit		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Mean (SD)	.	3.3 (1.43)	3.5 (1.08)		.	-0.21 (-0.604, 0.178)
	LS Mean (SE)	.	3.3 (0.16)	3.5 (0.16)	-0.27 (0.230) [-0.723, 0.184]	0.2429	
Visit 5 (End of Week 17)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGI-CSID, n (%)	Very Much Improved	2 (4.0)	1 (2.0)		.	
		Much Improved	15 (30.0)	5 (9.8)		.	
		Minimally Improved	11 (22.0)	11 (21.6)		.	
		No Change	15 (30.0)	25 (49.0)		.	
		Minimally Worse	3 (6.0)	5 (9.8)		.	
		Much Worse	2 (4.0)	4 (7.8)		.	
		Very Much Worse	2 (4.0)	0		.	
	Mean (SD)	.	3.3 (1.39)	3.8 (1.08)		.	-0.37 (-0.766, 0.021)
	LS Mean (SE)	.	3.3 (0.16)	3.8 (0.16)	-0.46 (0.230) [-0.918, -0.011]	0.0448	
Post-baseline	Mean (SD)	.	3.1 (1.28)	3.6 (1.04)		.	
Overall MMRM treatment effect	LS Mean (SE)	.	3.1 (0.13)	3.6 (0.13)	-0.44 (0.182) [-0.796, -0.074]	0.0186	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001

Table 14.2.3.3.1 Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID)
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

					Differeces of LSM		
Visit		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 3 (End of Week 5)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGI-CSID, n (%)	Very Much Improved	4 (8.0)	2 (3.9)		.	
		Much Improved	12 (24.0)	6 (11.8)		.	
		Minimally Improved	24 (48.0)	15 (29.4)		.	
		No Change	6 (12.0)	22 (43.1)		.	
		Minimally Worse	4 (8.0)	6 (11.8)		.	
	Mean (SD)	.	2.9 (1.00)	3.5 (0.99)		.	-0.59 (-0.992, -0.195)
	LS Mean (SE)	.	2.9 (0.18)	3.5 (0.18)	-0.59 (0.252) [-1.087, -0.094]	0.0199	
Visit 4 (End of Week 9)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGI-CSID, n (%)	Very Much Improved	5 (10.0)	3 (5.9)		.	
		Much Improved	11 (22.0)	6 (11.8)		.	
		Minimally Improved	13 (26.0)	10 (19.6)		.	
		No Change	9 (18.0)	23 (45.1)		.	
		Minimally Worse	8 (16.0)	8 (15.7)		.	
		Much Worse	3 (6.0)	1 (2.0)		.	
		Very Much Worse	1 (2.0)	0		.	
	Mean (SD)	.	3.3 (1.49)	3.6 (1.13)		.	-0.19 (-0.578, 0.203)
	LS Mean (SE)	.	3.3 (0.18)	3.6 (0.18)	-0.25 (0.252) [-0.744, 0.248]	0.3251	
Visit 5 (End of Week 17)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGI-CSID, n (%)	Very Much Improved	2 (4.0)	1 (2.0)		.	

					Differeces of LSM		
Visit		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
		Much Improved	15 (30.0)	5 (9.8)		.	
		Minimally Improved	11 (22.0)	11 (21.6)		.	
		No Change	10 (20.0)	21 (41.2)		.	
		Minimally Worse	3 (6.0)	5 (9.8)		.	
		Much Worse	7 (14.0)	8 (15.7)		.	
		Very Much Worse	2 (4.0)	0		.	
	Mean (SD)	.	3.5 (1.61)	3.9 (1.24)		.	-0.29 (-0.686, 0.098)
	LS Mean (SE)	.	3.5 (0.18)	3.9 (0.18)	-0.42 (0.252) [-0.917, 0.075]	0.0957	
Post-baseline	Mean (SD)	.	3.2 (1.41)	3.7 (1.14)		.	
Overall MMRM treatment effect	LS Mean (SE)	.	3.2 (0.14)	3.7 (0.14)	-0.42 (0.203) [-0.822, -0.018]	0.0408	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001

Table 14.2.4.1.1.s1 Summary of Caregiver Global Impression of Change in Attention (CGICA)

Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

					Differeces of LSM		
Visit		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 3 (End of Week 5)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGICA, n (%)	Very Much Improved	0	1 (2.0)		.	
		Much Improved	5 (10.0)	7 (13.7)		.	
		Minimally Improved	15 (30.0)	6 (11.8)		.	
		No Change	27 (54.0)	37 (72.5)		.	
		Minimally Worse	1 (2.0)	0		.	
		Much Worse	2 (4.0)	0		.	
	Mean (SD)	.	3.6 (0.86)	3.5 (0.81)		.	0.06 (-0.329, 0.451)
	LS Mean (SE)	.	3.6 (0.13)	3.5 (0.13)	0.05 (0.178) [-0.300, 0.402]	0.7752	
Visit 4 (End of Week 9)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGICA, n (%)	Very Much Improved	1 (2.0)	2 (3.9)		.	
		Much Improved	8 (16.0)	4 (7.8)		.	
		Minimally Improved	16 (32.0)	11 (21.6)		.	
		No Change	21 (42.0)	32 (62.7)		.	
		Minimally Worse	2 (4.0)	2 (3.9)		.	
		Much Worse	2 (4.0)	0		.	
	Mean (SD)	.	3.4 (1.01)	3.5 (0.86)		.	-0.14 (-0.528, 0.253)
	LS Mean (SE)	.	3.4 (0.13)	3.5 (0.13)	-0.13 (0.178) [-0.480, 0.222]	0.4701	
Visit 5 (End of Week 17)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGICA, n (%)	Very Much Improved	1 (2.0)	1 (2.0)		.	

					Differeces of LSM		
Visit		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
		Much Improved	2 (4.0)	7 (13.7)		.	
		Minimally Improved	21 (42.0)	14 (27.5)		.	
		No Change	23 (46.0)	27 (52.9)		.	
		Minimally Worse	1 (2.0)	1 (2.0)		.	
		Much Worse	1 (2.0)	1 (2.0)		.	
		Very Much Worse	1 (2.0)	0		.	
	Mean (SD)	.	3.6 (0.93)	3.5 (0.90)		.	0.12 (-0.271, 0.510)
	LS Mean (SE)	.	3.6 (0.13)	3.5 (0.13)	0.11 (0.178) [-0.242, 0.460]	0.5415	
Post-baseline	Mean (SD)	.	3.5 (0.93)	3.5 (0.85)		.	
Overall MMRM treatment effect	LS Mean (SE)	.	3.5 (0.10)	3.5 (0.10)	0.01 (0.138) [-0.264, 0.285]	0.9407	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001

Table 14.2.4.1.1.s2 Summary of Caregiver Global Impression of Change in Attention (CGICA)

Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

					Differeces of LSM		
Visit		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 3 (End of Week 5)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGICA, n (%)	Very Much Improved	0	1 (2.0)		.	
		Much Improved	5 (10.0)	7 (13.7)		.	
		Minimally Improved	15 (30.0)	6 (11.8)		.	
		No Change	27 (54.0)	37 (72.5)		.	
		Minimally Worse	1 (2.0)	0		.	
		Much Worse	2 (4.0)	0		.	
	Mean (SD)	.	3.6 (0.86)	3.5 (0.81)		.	0.06 (-0.329, 0.451)
	LS Mean (SE)	.	3.6 (0.13)	3.5 (0.13)	0.05 (0.178) [-0.300, 0.402]	0.7752	
Visit 4 (End of Week 9)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGICA, n (%)	Very Much Improved	1 (2.0)	2 (3.9)		.	
		Much Improved	8 (16.0)	4 (7.8)		.	
		Minimally Improved	16 (32.0)	11 (21.6)		.	
		No Change	21 (42.0)	32 (62.7)		.	
		Minimally Worse	2 (4.0)	2 (3.9)		.	
		Much Worse	2 (4.0)	0		.	
	Mean (SD)	.	3.4 (1.01)	3.5 (0.86)		.	-0.14 (-0.528, 0.253)
	LS Mean (SE)	.	3.4 (0.13)	3.5 (0.13)	-0.13 (0.178) [-0.480, 0.222]	0.4701	
Visit 5 (End of Week 17)	Total observed, n	.	50	51		.	
	Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
	n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
	CGICA, n (%)	Very Much Improved	1 (2.0)	1 (2.0)		.	

					Differeces of LSM		
Visit		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
		Much Improved	2 (4.0)	7 (13.7)		.	
		Minimally Improved	21 (42.0)	14 (27.5)		.	
		No Change	23 (46.0)	27 (52.9)		.	
		Minimally Worse	1 (2.0)	1 (2.0)		.	
		Much Worse	1 (2.0)	1 (2.0)		.	
		Very Much Worse	1 (2.0)	0		.	
	Mean (SD)	.	3.6 (0.93)	3.5 (0.90)		.	0.12 (-0.271, 0.510)
	LS Mean (SE)	.	3.6 (0.13)	3.5 (0.13)	0.11 (0.178) [-0.242, 0.460]	0.5415	
Post-baseline	Mean (SD)	.	3.5 (0.93)	3.5 (0.85)		.	
Overall MMRM treatment effect	LS Mean (SE)	.	3.5 (0.10)	3.5 (0.10)	0.01 (0.138) [-0.264, 0.285]	0.9407	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001

Table 14.2.4.2.1.s1 Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

						Differeces of LSM		
Visit	**Variable**		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 1 (Screening)	Chosen Target Behavior:	Total observed, n	.	44	47		.	
		Missing, n (%)	.	6 (12.0)	4 (7.8)		.	
		n/M*, (%)	.	44/ 50 (88.0)	47/ 51 (92.2)		.	
		CGI-C, n (%)	Sociabilty	13 (29.5)	12 (25.5)		.	
			Communication	14 (31.8)	17 (36.2)		.	
			Irritability	13 (29.5)	8 (17.0)		.	
			Hyperactivity	4 (9.1)	10 (21.3)		.	
Visit 3 (End of Week 5)	Chosen Target Behavior:	Total observed, n	.	46	47		.	
		Missing, n (%)	.	4 (8.0)	4 (7.8)		.	
		n/M*, (%)	.	46/ 50 (92.0)	47/ 49 (95.9)		.	
		CGI-C, n (%)	Sociabilty	13 (28.3)	14 (29.8)		.	
			Communication	15 (32.6)	16 (34.0)		.	
			Irritability	13 (28.3)	8 (17.0)		.	
			Hyperactivity	5 (10.9)	9 (19.1)		.	
	Score:	Total observed, n	.	50	51		.	
		Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
		n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
		CGI-C, n (%)	Very Much Improved	1 (2.0)	1 (2.0)		.	
			Much Improved	7 (14.0)	3 (5.9)		.	
			Minimally Improved	15 (30.0)	11 (21.6)		.	
			No Change	23 (46.0)	31 (60.8)		.	
			Minimally Worse	4 (8.0)	2 (3.9)		.	
			Much Worse	0	3 (5.9)		.	
		Mean (SD)	.	3.4 (0.91)	3.8 (0.93)		.	-0.35 (-0.747, 0.040)
		LS Mean (SE)	.	3.4 (0.12)	3.8 (0.12)	-0.32 (0.174) [-0.668, 0.018]	0.0633	

						Differeces of LSM		
Visit	**Variable**		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 4 (End of Week 9)	Chosen Target Behavior:	Total observed, n	.	41	45		.	
		Missing, n (%)	.	9 (18.0)	6 (11.8)		.	
		n/M*, (%)	.	41/ 48 (85.4)	45/ 49 (91.8)		.	
		CGI-C, n (%)	Sociabilty	12 (29.3)	13 (28.9)		.	
			Communication	12 (29.3)	16 (35.6)		.	
			Irritability	13 (31.7)	7 (15.6)		.	
			Hyperactivity	4 (9.8)	9 (20.0)		.	
	Score:	Total observed, n	.	50	51		.	
		Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
		n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
		CGI-C, n (%)	Very Much Improved	1 (2.0)	0		.	
			Much Improved	8 (16.0)	3 (5.9)		.	
			Minimally Improved	17 (34.0)	14 (27.5)		.	
			No Change	21 (42.0)	32 (62.7)		.	
			Minimally Worse	1 (2.0)	2 (3.9)		.	
			Much Worse	2 (4.0)	0		.	
		Mean (SD)	.	3.4 (0.99)	3.6 (0.66)		.	-0.32 (-0.711, 0.074)
		LS Mean (SE)	.	3.4 (0.12)	3.6 (0.12)	-0.27 (0.174) [-0.610, 0.076]	0.1262	

						Differeces of LSM		
Visit	**Variable**		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 5 (End of Week 17)	Chosen Target Behavior:	Total observed, n	.	42	43		.	
		Missing, n (%)	.	8 (16.0)	8 (15.7)		.	
		n/M*, (%)	.	42/ 42 (100.0)	43/ 43 (100.0)		.	
		CGI-C, n (%)	Sociabilty	12 (28.6)	14 (32.6)		.	
			Communication	13 (31.0)	14 (32.6)		.	
			Irritability	12 (28.6)	6 (14.0)		.	

						Differeces of LSM		
Visit	**Variable**		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
			Hyperactivity	5 (11.9)	9 (20.9)		.	
	Score:	Total observed, n	.	50	51		.	
		Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
		n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
		CGI-C, n (%)	Much Improved	4 (8.0)	6 (11.8)		.	
			Minimally Improved	20 (40.0)	14 (27.5)		.	
			No Change	24 (48.0)	27 (52.9)		.	
			Minimally Worse	2 (4.0)	1 (2.0)		.	
			Much Worse	0	2 (3.9)		.	
			Very Much Worse	0	1 (2.0)		.	
		Mean (SD)	.	3.5 (0.71)	3.6 (1.00)		.	-0.19 (-0.584, 0.198)
		LS Mean (SE)	.	3.5 (0.12)	3.6 (0.12)	-0.17 (0.174) [-0.510, 0.176]	0.3380	
Post-baseline	Score:	Mean (SD)	.	3.4 (0.87)	3.7 (0.87)		.	
Overall MMRM treatment effect	Score:	LS Mean (SE)	.	3.4 (0.09)	3.7 (0.09)	-0.25 (0.132) [-0.515, 0.009]	0.0580	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001

Table 14.2.4.2.1.s2 Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)

Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

						Differeces of LSM		
Visit	**Variable**		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 1 (Screening)	Chosen Target Behavior:	Total observed, n	.	44	47		.	
		Missing, n (%)	.	6 (12.0)	4 (7.8)		.	
		n/M*, (%)	.	44/ 50 (88.0)	47/ 51 (92.2)		.	
		CGI-C, n (%)	Sociabilty	13 (29.5)	12 (25.5)		.	
			Communication	14 (31.8)	17 (36.2)		.	
			Irritability	13 (29.5)	8 (17.0)		.	
			Hyperactivity	4 (9.1)	10 (21.3)		.	
Visit 3 (End of Week 5)	Chosen Target Behavior:	Total observed, n	.	46	47		.	
		Missing, n (%)	.	4 (8.0)	4 (7.8)		.	
		n/M*, (%)	.	46/ 50 (92.0)	47/ 49 (95.9)		.	
		CGI-C, n (%)	Sociabilty	13 (28.3)	14 (29.8)		.	
			Communication	15 (32.6)	16 (34.0)		.	
			Irritability	13 (28.3)	8 (17.0)		.	
			Hyperactivity	5 (10.9)	9 (19.1)		.	
	Score:	Total observed, n	.	50	51		.	
		Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
		n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
		CGI-C, n (%)	Very Much Improved	1 (2.0)	1 (2.0)		.	
			Much Improved	7 (14.0)	3 (5.9)		.	
			Minimally Improved	15 (30.0)	11 (21.6)		.	
			No Change	20 (40.0)	28 (54.9)		.	
			Minimally Worse	4 (8.0)	2 (3.9)		.	
			Much Worse	3 (6.0)	6 (11.8)		.	
		Mean (SD)	.	3.6 (1.09)	3.9 (1.07)		.	-0.30 (-0.691, 0.094)

						Differeces of LSM		
Visit	**Variable**		**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
		LS Mean (SE)	.	3.6 (0.15)	3.9 (0.14)	-0.32 (0.204) [-0.725, 0.080]	0.1159	
Visit 4 (End of Week 9)	Chosen Target Behavior:	Total observed, n	.	41	45		.	
		Missing, n (%)	.	9 (18.0)	6 (11.8)		.	
		n/M*, (%)	.	41/ 48 (85.4)	45/ 49 (91.8)		.	
		CGI-C, n (%)	Sociabilty	12 (29.3)	13 (28.9)		.	
			Communication	12 (29.3)	16 (35.6)		.	
			Irritability	13 (31.7)	7 (15.6)		.	
			Hyperactivity	4 (9.8)	9 (20.0)		.	
	Score:	Total observed, n	.	50	51		.	
		Missing, n (%)	.	0 (0.0)	0 (0.0)		.	
		n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.	
		CGI-C, n (%)	Very Much Improved	1 (2.0)	0		.	
			Much Improved	8 (16.0)	3 (5.9)		.	
			Minimally Improved	17 (34.0)	14 (27.5)		.	
			No Change	21 (42.0)	32 (62.7)		.	
			Minimally Worse	1 (2.0)	2 (3.9)		.	
			Much Worse	2 (4.0)	0		.	
		Mean (SD)	.	3.4 (0.99)	3.6 (0.66)		.	-0.32 (-0.711, 0.074)
		LS Mean (SE)	.	3.4 (0.15)	3.6 (0.14)	-0.27 (0.204) [-0.669, 0.135]	0.1923	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001

Table 14.2.4.3.1.s1 Summary of Clinical Global Impression of Improvement (CGI-I)

Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Visit 3 (End of Week 5)

		Parent/Caregiver					Clinician				
	Category	**Ganaxolone N=50**	**Placebo N=51**	**Differeces of LSM (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**	**Ganaxolone N=50**	**Placebo N=51**	**Differeces of LSM (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Total observed, n	.	50	51		.		50	51		.	
Missing, n (%)	.	0 (0.0)	0 (0.0)		.		0 (0.0)	0 (0.0)		.	
n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.		50/ 50 (100.0)	51/ 51 (100.0)		.	
CGI-CSID, n (%)	Very Much Improved	2 (4.0)	3 (5.9)		.		1 (2.0)	2 (3.9)		.	
	Much Improved	5 (10.0)	6 (11.8)		.		10 (20.0)	4 (7.8)		.	
	Minimally Improved	24 (48.0)	15 (29.4)		.		20 (40.0)	16 (31.4)		.	
	No Change	16 (32.0)	27 (52.9)		.		15 (30.0)	26 (51.0)		.	
	Minimally Worse	2 (4.0)	0		.		2 (4.0)	3 (5.9)		.	
	Much Worse	1 (2.0)	0		.		2 (4.0)	0		.	
Mean (SD)	.	3.3 (0.93)	3.3 (0.90)		.	-0.02 (-0.406, 0.375)	3.3 (1.03)	3.5 (0.88)		.	-0.22 (-0.612, 0.171)
LS Mean (SE)	.	3.3 (0.14)	3.3 (0.14)	-0.01 (0.193) [-0.394, 0.366]	0.9416		3.3 (0.13)	3.5 (0.13)	-0.21 (0.189) [-0.583, 0.162]	0.2664	

Visit 4 (End of Week 9)

		Parent/Caregiver					Clinician				
	Category	**Ganaxolone N=50**	**Placebo N=51**	**Differeces of LSM (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**	**Ganaxolone N=50**	**Placebo N=51**	**Differeces of LSM (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Total observed, n	.	50	51		.		50	51		.	

		Parent/Caregiver					Clinician				
	Category	Ganaxolone N=50	Placebo N=51	Differeces of LSM (SE) [95% CI]	p-value	Hedges' g (95% CI)	Ganaxolone N=50	Placebo N=51	Differeces of LSM (SE) [95% CI]	p-value	Hedges' g (95% CI)
Missing, n (%)	.	0 (0.0)	0 (0.0)		.		0 (0.0)	0 (0.0)		.	
n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.		50/ 50 (100.0)	51/ 51 (100.0)		.	
CGI-CSID, n (%)	Very Much Improved	2 (4.0)	2 (3.9)		.		1 (2.0)	0		.	
	Much Improved	9 (18.0)	4 (7.8)		.		12 (24.0)	6 (11.8)		.	
	Minimally Improved	17 (34.0)	12 (23.5)		.		16 (32.0)	13 (25.5)		.	
	No Change	17 (34.0)	31 (60.8)		.		19 (38.0)	26 (51.0)		.	
	Minimally Worse	3 (6.0)	1 (2.0)		.		2 (4.0)	6 (11.8)		.	
	Much Worse	1 (2.0)	1 (2.0)		.					.	
	Very Much Worse	1 (2.0)	0		.					.	
Mean (SD)	.	3.3 (1.15)	3.5 (0.90)		.	-0.20 (-0.593, 0.189)	3.2 (0.92)	3.6 (0.85)		.	-0.51 (-0.903, -0.110)
LS Mean (SE)	.	3.3 (0.14)	3.5 (0.14)	-0.21 (0.193) [-0.589, 0.171]	0.2792		3.2 (0.13)	3.6 (0.13)	-0.45 (0.189) [-0.820, -0.075]	0.0188	

Visit 5 (End of Week 17)

		Parent/Caregiver					Clinician				
	Category	Ganaxolone N=50	Placebo N=51	Differeces of LSM (SE) [95% CI]	p-value	Hedges' g (95% CI)	Ganaxolone N=50	Placebo N=51	Differeces of LSM (SE) [95% CI]	p-value	Hedges' g (95% CI)
Total observed, n	.	50	51		.		50	51		.	
Missing, n (%)	.	0 (0.0)	0 (0.0)		.		0 (0.0)	0 (0.0)		.	
n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.		50/ 50 (100.0)	51/ 51 (100.0)		.	
CGI-CSID, n (%)	Very Much Improved	0	1 (2.0)		.					.	
	Much Improved	12 (24.0)	7 (13.7)		.		7 (14.0)	7 (13.7)		.	
	Minimally Improved	15 (30.0)	13 (25.5)		.		18 (36.0)	13 (25.5)		.	
	No Change	20 (40.0)	26 (51.0)		.		20 (40.0)	23 (45.1)		.	

		Parent/Caregiver					Clinician				
	Category	**Ganaxolone N=50**	**Placebo N=51**	**Differeces of LSM (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**	**Ganaxolone N=50**	**Placebo N=51**	**Differeces of LSM (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Minimally Worse	1 (2.0)	4 (7.8)		.		1 (2.0)	8 (15.7)		.	
	Much Worse	2 (4.0)	0		.		3 (6.0)	0		.	
	Very Much Worse				.		1 (2.0)	0		.	
Proportional Odds Ratio (95% CI)	.		1.59 (0.77, 3.30)		.			1.40 (0.68, 2.88)		.	
P-value	.		0.2083		.			0.3585		.	
Mean (SD)	.	3.3 (1.00)	3.5 (0.90)		.	-0.18 (-0.570, 0.212)	3.6 (1.09)	3.6 (0.92)		.	-0.07 (-0.457, 0.323)
LS Mean (SE)	.	3.3 (0.14)	3.5 (0.14)	-0.17 (0.193) [-0.550, 0.210]	0.3780		3.6 (0.13)	3.6 (0.13)	-0.07 (0.189) [-0.440, 0.305]	0.7215	
Post-baseline	Mean (SD)	3.3 (1.02)	3.4 (0.90)		.		3.3 (1.02)	3.6 (0.88)		.	
Overall MMRM treatment effect	LS Mean (SE)	3.3 (0.11)	3.4 (0.11)	-0.13 (0.159) [-0.447, 0.185]	0.4122		3.3 (0.11)	3.6 (0.10)	-0.24 (0.148) [-0.536, 0.052]	0.1059	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001

Table 14.2.4.3.1.s2 Summary of Clinical Global Impression of Improvement (CGI-I)
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Visit 3 (End of Week 5)

		Parent/Caregiver					Clinician				
	Category	**Ganaxolone N=50**	**Placebo N=51**	**Differeces of LSM (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**	**Ganaxolone N=50**	**Placebo N=51**	**Differeces of LSM (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Total observed, n	.	50	51		.		50	51		.	
Missing, n (%)	.	0 (0.0)	0 (0.0)		.		0 (0.0)	0 (0.0)		.	
n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.		50/ 50 (100.0)	51/ 51 (100.0)		.	
CGI-CSID, n (%)	Very Much Improved	2 (4.0)	3 (5.9)		.		1 (2.0)	2 (3.9)		.	
	Much Improved	5 (10.0)	6 (11.8)		.		10 (20.0)	4 (7.8)		.	
	Minimally Improved	24 (48.0)	15 (29.4)		.		20 (40.0)	16 (31.4)		.	
	No Change	16 (32.0)	27 (52.9)		.		10 (20.0)	21 (41.2)		.	
	Minimally Worse	2 (4.0)	0		.		7 (14.0)	8 (15.7)		.	
	Much Worse	1 (2.0)	0		.		2 (4.0)	0		.	
Mean (SD)	.	3.3 (0.93)	3.3 (0.90)		.	-0.02 (-0.406, 0.375)	3.4 (1.14)	3.6 (0.98)		.	-0.20 (-0.587, 0.195)
LS Mean (SE)	.	3.3 (0.14)	3.3 (0.14)	-0.01 (0.201) [-0.410, 0.381]	0.9439		3.4 (0.15)	3.6 (0.15)	-0.21 (0.209) [-0.620, 0.203]	0.3183	

Visit 4 (End of Week 9)

		Parent/Caregiver					Clinician				
	Category	Ganaxolone N=50	Placebo N=51	Differeces of LSM (SE) [95% CI]	p-value	Hedges' g (95% CI)	Ganaxolone N=50	Placebo N=51	Differeces of LSM (SE) [95% CI]	p-value	Hedges' g (95% CI)
Total observed, n	.	50	51		.		50	51		.	
Missing, n (%)	.	0 (0.0)	0 (0.0)		.		0 (0.0)	0 (0.0)		.	
n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.		50/ 50 (100.0)	51/ 51 (100.0)		.	
CGI-CSID, n (%)	Very Much Improved	2 (4.0)	2 (3.9)		.		1 (2.0)	0		.	
	Much Improved	9 (18.0)	4 (7.8)		.		12 (24.0)	6 (11.8)		.	
	Minimally Improved	17 (34.0)	12 (23.5)		.		16 (32.0)	13 (25.5)		.	
	No Change	17 (34.0)	31 (60.8)		.		14 (28.0)	23 (45.1)		.	
	Minimally Worse	3 (6.0)	1 (2.0)		.		7 (14.0)	9 (17.6)		.	
	Much Worse	1 (2.0)	1 (2.0)		.					.	
	Very Much Worse	1 (2.0)	0		.					.	
Mean (SD)	.	3.3 (1.15)	3.5 (0.90)		.	-0.20 (-0.593, 0.189)	3.3 (1.05)	3.7 (0.91)		.	-0.41 (-0.809, -0.020)
LS Mean (SE)	.	3.3 (0.14)	3.5 (0.14)	-0.21 (0.201) [-0.604, 0.186]	0.2985		3.3 (0.15)	3.7 (0.15)	-0.41 (0.209) [-0.817, 0.005]	0.0528	

Visit 5 (End of Week 17)

		Parent/Caregiver					Clinician				
	Category	Ganaxolone N=50	Placebo N=51	Differeces of LSM (SE) [95% CI]	p-value	Hedges' g (95% CI)	Ganaxolone N=50	Placebo N=51	Differeces of LSM (SE) [95% CI]	p-value	Hedges' g (95% CI)
Total observed, n	.	50	51		.		50	51		.	
Missing, n (%)	.	0 (0.0)	0 (0.0)		.		0 (0.0)	0 (0.0)		.	
n/M*, (%)	.	50/ 50 (100.0)	51/ 51 (100.0)		.		50/ 50 (100.0)	51/ 51 (100.0)		.	
CGI-CSID, n (%)	Very Much Improved	0	1 (2.0)		.					.	
	Much Improved	12 (24.0)	7 (13.7)		.		7 (14.0)	7 (13.7)		.	

		Parent/Caregiver					Clinician				
	Category	**Ganaxolone N=50**	**Placebo N=51**	**Differeces of LSM (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**	**Ganaxolone N=50**	**Placebo N=51**	**Differeces of LSM (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Minimally Improved	15 (30.0)	13 (25.5)		.		18 (36.0)	13 (25.5)		.	
	No Change	13 (26.0)	22 (43.1)		.		14 (28.0)	18 (35.3)		.	
	Minimally Worse	8 (16.0)	8 (15.7)		.		7 (14.0)	13 (25.5)		.	
	Much Worse	2 (4.0)	0		.		3 (6.0)	0		.	
	Very Much Worse				.		1 (2.0)	0		.	
Proportional Odds Ratio (95% CI)	.		1.32 (0.66, 2.68)		.			1.25 (0.62, 2.51)		.	
P-value	.		0.4340		.			0.5404		.	
Mean (SD)	.	3.5 (1.15)	3.6 (0.98)		.	-0.10 (-0.492, 0.289)	3.7 (1.19)	3.7 (1.00)		.	-0.04 (-0.432, 0.349)
LS Mean (SE)	.	3.5 (0.14)	3.6 (0.14)	-0.11 (0.201) [-0.504, 0.287]	0.5886		3.7 (0.15)	3.7 (0.15)	-0.05 (0.209) [-0.457, 0.366]	0.8275	
Post-baseline	Mean (SD)	3.4 (1.08)	3.5 (0.93)		.		3.4 (1.13)	3.7 (0.96)		.	
Overall MMRM treatment effect	LS Mean (SE)	3.4 (0.12)	3.5 (0.12)	-0.11 (0.166) [-0.439, 0.218]	0.5062		3.4 (0.12)	3.7 (0.12)	-0.22 (0.167) [-0.552, 0.112]	0.1914	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

4.4 Ergänzende Analysen zum Endpunkt Morbidität – Veränderung des CSHQ gegenüber Baseline

4.4.1 Sensitivitätsanalysen

4.4.1.1 Sensitivitätsanalysen: Mittlere Änderung in den Subskalen und dem Gesamtscore des CSHQ gegenüber Baseline im Studienverlauf zu den Erhebungszeitpunkten in Woche 1–17 (MMRM)

Study No. 1042-CDD-3001

Table 14.2.6.1.s1 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Bedtime Resistance Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	8.2 (2.40)	8.5 (2.63)			
	95% Confidence Interval of Mean	7.6 , 8.9	7.7 , 9.2			
	Median	8.0	8.0			
	Q1, Q3	6.0 , 9.0	6.0 ,10.0			
	Min, Max	6.0 ,15.0	6.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.2,0.8)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	7.8 (2.25)	8.0 (2.63)			
	95% Confidence Interval of Mean	7.2 , 8.5	7.3 , 8.7			
	Median	7.0	6.5			
	Q1, Q3	6.0 , 9.0	6.0 ,10.0			
	Min, Max	6.0 ,14.0	6.0 ,14.0			

	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.1,0.8)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.4 (1.45)	-0.4 (1.67)			0.03 (-0.364, 0.416)
	95% Confidence Interval of Mean	-0.8 , 0.0	-0.9 , 0.0			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 0.0	-1.0 , 0.0			
	Min, Max	-5.0 , 3.0	-5.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.6,0.7)				
	LS Mean (SE)	-0.4 (0.22)	-0.4 (0.22)	-0.02 (0.310) [-0.629, 0.595]	0.9572	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	8.0 (2.31)	8.2 (2.39)			
	95% Confidence Interval of Mean	7.4 , 8.7	7.5 , 8.9			
	Median	7.5	8.0			
	Q1, Q3	6.0 ,10.0	6.0 , 9.0			
	Min, Max	6.0 ,14.0	6.0 ,16.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.1,0.7)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.2 (1.61)	-0.2 (1.56)			0.01 (-0.380, 0.400)
	95% Confidence Interval of Mean	-0.7 , 0.2	-0.7 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 0.0	-1.0 , 1.0			
	Min, Max	-3.0 , 6.0	-4.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.6,0.6)				
	LS Mean (SE)	-0.2 (0.22)	-0.2 (0.22)	-0.04 (0.310) [-0.655, 0.569]	0.8910	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			

	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	7.9 (2.42)	8.0 (2.39)			
	95% Confidence Interval of Mean	7.2 , 8.6	7.3 , 8.7			
	Median	7.0	7.0			
	Q1, Q3	6.0 , 9.0	6.0 , 9.0			
	Min, Max	6.0 ,14.0	6.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-1.0,0.9)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.3 (1.93)	-0.5 (1.92)			0.07 (-0.322, 0.458)
	95% Confidence Interval of Mean	-0.9 , 0.2	-1.0 , 0.1			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 0.0	-2.0 , 1.0			
	Min, Max	-4.0 , 6.0	-8.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.6,0.9)				
	LS Mean (SE)	-0.4 (0.22)	-0.4 (0.22)	0.07 (0.310) [-0.539, 0.685]	0.8149	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s2 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Bedtime Resistance Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	8.6 (2.77)	8.6 (2.74)			
	95% Confidence Interval of Mean	7.8 , 9.4	7.8 , 9.3			
	Median	8.0	8.0			
	Q1, Q3	6.0 ,11.0	6.0 ,10.0			
	Min, Max	6.0 ,15.0	6.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-1.1,1.1)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	8.7 (2.94)	8.7 (3.12)			
	95% Confidence Interval of Mean	7.9 , 9.6	7.9 , 9.6			
	Median	8.0	8.0			
	Q1, Q3	6.0 ,11.0	6.0 ,12.0			
	Min, Max	6.0 ,14.0	6.0 ,14.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.0 (-1.2,1.2)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.1 (2.73)	0.2 (2.82)			-0.01 (-0.403, 0.377)
	95% Confidence Interval of Mean	-0.6 , 0.9	-0.6 , 1.0			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-6.0 , 8.0	-6.0 , 8.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.0 (-1.1,1.1)				
	LS Mean (SE)	0.1 (0.35)	0.2 (0.35)	-0.02 (0.491) [-0.991, 0.946]	0.9631	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	8.6 (2.83)	8.7 (2.79)			
	95% Confidence Interval of Mean	7.8 , 9.4	7.9 , 9.5			
	Median	7.5	8.0			
	Q1, Q3	6.0 ,11.0	6.0 ,11.0			
	Min, Max	6.0 ,14.0	6.0 ,16.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-1.2,1.0)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.0 (2.48)	0.1 (2.43)			-0.05 (-0.438, 0.342)
	95% Confidence Interval of Mean	-0.7 , 0.7	-0.5 , 0.8			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 1.0			
	Min, Max	-8.0 , 7.0	-7.0 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-1.1,0.9)				
	LS Mean (SE)	0.0 (0.35)	0.1 (0.35)	-0.10 (0.491) [-1.072, 0.865]	0.8331	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	8.7 (2.95)	8.6 (2.79)			
	95% Confidence Interval of Mean	7.9 , 9.6	7.8 , 9.4			
	Median	7.0	7.0			
	Q1, Q3	6.0 ,12.0	6.0 ,11.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	6.0 ,14.0	6.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-1.0,1.3)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.1 (3.08)	0.0 (2.83)			0.05 (-0.343, 0.438)
	95% Confidence Interval of Mean	-0.7 , 1.0	-0.8 , 0.8			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-2.0 , 1.0			
	Min, Max	-8.0 , 7.0	-8.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-1.0,1.3)				
	LS Mean (SE)	0.1 (0.35)	-0.0 (0.35)	0.15 (0.491) [-0.815, 1.122]	0.7544	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s1 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Sleep Onset Delay Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.8 (0.80)	1.9 (0.73)			
	95% Confidence Interval of Mean	1.6 , 2.0	1.7 , 2.1			
	Median	2.0	2.0			
	Q1, Q3	1.0 , 2.0	1.0 , 2.0			
	Min, Max	1.0 , 3.0	1.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.4,0.2)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.8 (0.74)	1.9 (0.72)			
	95% Confidence Interval of Mean	1.6 , 2.0	1.7 , 2.1			
	Median	2.0	2.0			
	Q1, Q3	1.0 , 2.0	1.0 , 2.0			
	Min, Max	1.0 , 3.0	1.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.4,0.2)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.0 (0.86)	-0.0 (0.66)			-0.00 (-0.391, 0.389)
	95% Confidence Interval of Mean	-0.3 , 0.2	-0.2 , 0.1			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 0.0	0.0 , 0.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-2.0 , 2.0	-1.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.0 (-0.3,0.3)				
	LS Mean (SE)	-0.1 (0.09)	-0.0 (0.09)	-0.04 (0.125) [-0.290, 0.201]	0.7192	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.7 (0.65)	1.8 (0.76)			
	95% Confidence Interval of Mean	1.5 , 1.9	1.6 , 2.1			
	Median	2.0	2.0			
	Q1, Q3	1.0 , 2.0	1.0 , 2.0			
	Min, Max	1.0 , 3.0	1.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.4,0.1)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.1 (0.87)	-0.1 (0.61)			-0.08 (-0.472, 0.309)
	95% Confidence Interval of Mean	-0.4 , 0.1	-0.2 , 0.1			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 0.0	0.0 , 0.0			
	Min, Max	-2.0 , 2.0	-1.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.4,0.2)				
	LS Mean (SE)	-0.1 (0.09)	-0.0 (0.09)	-0.11 (0.125) [-0.351, 0.140]	0.3993	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.7 (0.65)	1.9 (0.78)			
	95% Confidence Interval of Mean	1.5 , 1.9	1.6 , 2.1			
	Median	2.0	2.0			
	Q1, Q3	1.0 , 2.0	1.0 , 2.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	1.0 , 3.0	1.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-0.5,0.1)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.1 (0.86)	-0.0 (0.56)			-0.14 (-0.530, 0.251)
	95% Confidence Interval of Mean	-0.4 , 0.1	-0.2 , 0.1			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 0.0	0.0 , 0.0			
	Min, Max	-2.0 , 2.0	-1.0 , 1.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.4,0.2)				
	LS Mean (SE)	-0.2 (0.09)	-0.0 (0.09)	-0.14 (0.125) [-0.390, 0.101]	0.2463	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s2 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Sleep Onset Delay Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.8 (0.80)	1.9 (0.74)			
	95% Confidence Interval of Mean	1.6 , 2.0	1.7 , 2.1			
	Median	2.0	2.0			
	Q1, Q3	1.0 , 2.0	1.0 , 2.0			
	Min, Max	1.0 , 3.0	1.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.4,0.2)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.8 (0.79)	1.9 (0.77)			
	95% Confidence Interval of Mean	1.6 , 2.1	1.7 , 2.1			
	Median	2.0	2.0			
	Q1, Q3	1.0 , 2.0	1.0 , 3.0			
	Min, Max	1.0 , 3.0	1.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.4,0.2)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.0 (0.89)	0.0 (0.69)			0.03 (-0.365, 0.415)
	95% Confidence Interval of Mean	-0.2 , 0.3	-0.2 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	0.0 , 0.0	0.0 , 0.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-2.0 , 2.0	-1.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.3,0.3)				
	LS Mean (SE)	-0.0 (0.10)	0.0 (0.10)	-0.03 (0.140) [-0.310, 0.243]	0.8118	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.8 (0.76)	1.9 (0.81)			
	95% Confidence Interval of Mean	1.6 , 2.0	1.7 , 2.1			
	Median	2.0	2.0			
	Q1, Q3	1.0 , 2.0	1.0 , 3.0			
	Min, Max	1.0 , 3.0	1.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.4,0.2)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.0 (0.94)	-0.0 (0.68)			-0.00 (-0.391, 0.390)
	95% Confidence Interval of Mean	-0.3 , 0.2	-0.2 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 0.0	0.0 , 0.0			
	Min, Max	-2.0 , 2.0	-1.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.0 (-0.3,0.3)				
	LS Mean (SE)	-0.0 (0.10)	0.0 (0.10)	-0.05 (0.140) [-0.330, 0.222]	0.7014	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.8 (0.78)	1.9 (0.83)			
	95% Confidence Interval of Mean	1.6 , 2.0	1.7 , 2.2			
	Median	2.0	2.0			

	Q1, Q3	1.0 , 2.0	1.0 , 3.0			
	Min, Max	1.0 , 3.0	1.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.5,0.2)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.0 (0.96)	0.0 (0.65)			-0.05 (-0.439, 0.342)
	95% Confidence Interval of Mean	-0.3 , 0.3	-0.2 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	0.0 , 0.0			
	Min, Max	-2.0 , 2.0	-1.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.0 (-0.4,0.3)				
	LS Mean (SE)	-0.0 (0.10)	0.0 (0.10)	-0.09 (0.140) [-0.369, 0.183]	0.5075	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s1 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Sleep Duration Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.5 (1.69)	4.6 (1.76)			
	95% Confidence Interval of Mean	4.0 , 5.0	4.1 , 5.1			
	Median	4.0	4.0			
	Q1, Q3	3.0 , 6.0	3.0 , 6.0			
	Min, Max	3.0 , 8.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.8,0.6)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.7 (1.52)	4.8 (1.59)			
	95% Confidence Interval of Mean	4.3 , 5.1	4.4 , 5.3			
	Median	5.0	5.0			
	Q1, Q3	3.0 , 6.0	3.0 , 6.0			
	Min, Max	3.0 , 9.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.7,0.5)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.2 (1.50)	0.2 (1.51)			0.02 (-0.374, 0.406)
	95% Confidence Interval of Mean	-0.2 , 0.6	-0.2 , 0.6			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	0.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-3.0 , 5.0	-5.0 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.6,0.6)				
	LS Mean (SE)	0.2 (0.21)	0.2 (0.21)	-0.04 (0.293) [-0.620, 0.536]	0.8857	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.4 (1.49)	4.8 (1.95)			
	95% Confidence Interval of Mean	4.0 , 4.8	4.3 , 5.4			
	Median	4.0	4.0			
	Q1, Q3	3.0 , 5.0	3.0 , 6.0			
	Min, Max	3.0 , 8.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.4 (-1.1,0.3)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.1 (1.91)	0.2 (2.06)			-0.14 (-0.529, 0.252)
	95% Confidence Interval of Mean	-0.6 , 0.5	-0.4 , 0.8			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 1.0			
	Min, Max	-5.0 , 5.0	-5.0 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.3 (-1.1,0.5)				
	LS Mean (SE)	-0.1 (0.21)	0.2 (0.21)	-0.34 (0.293) [-0.920, 0.236]	0.2451	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.5 (1.54)	4.7 (1.97)			
	95% Confidence Interval of Mean	4.1 , 4.9	4.2 , 5.3			
	Median	4.0	4.0			

	Q1, Q3	3.0 , 5.0	3.0 , 6.0			
	Min, Max	3.0 , 8.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-0.9,0.5)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.0 (1.42)	0.1 (1.80)			-0.07 (-0.462, 0.318)
	95% Confidence Interval of Mean	-0.4 , 0.4	-0.4 , 0.6			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 1.0			
	Min, Max	-5.0 , 3.0	-5.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.8,0.5)				
	LS Mean (SE)	-0.0 (0.21)	0.2 (0.21)	-0.18 (0.293) [-0.761, 0.395]	0.5324	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s2 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Sleep Duration Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.6 (1.76)	4.8 (1.87)			
	95% Confidence Interval of Mean	4.1 , 5.1	4.2 , 5.3			
	Median	4.0	4.0			
	Q1, Q3	3.0 , 6.0	3.0 , 6.0			
	Min, Max	3.0 , 8.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-0.9,0.5)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.9 (1.71)	5.0 (1.81)			
	95% Confidence Interval of Mean	4.4 , 5.4	4.5 , 5.5			
	Median	5.0	5.0			
	Q1, Q3	3.0 , 6.0	3.0 , 7.0			
	Min, Max	3.0 , 9.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-0.9,0.5)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.3 (1.50)	0.3 (1.55)			0.03 (-0.360, 0.420)
	95% Confidence Interval of Mean	-0.1 , 0.7	-0.2 , 0.7			
	Median	0.0	0.0			

	Q1, Q3	-1.0 , 1.0	0.0 , 1.0			
	Min, Max	-3.0 , 4.0	-5.0 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.6,0.6)				
	LS Mean (SE)	0.3 (0.25)	0.3 (0.25)	-0.05 (0.359) [-0.753, 0.662]	0.8992	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.0 (2.09)	5.2 (2.23)			
	95% Confidence Interval of Mean	4.4 , 5.6	4.6 , 5.8			
	Median	4.5	5.0			
	Q1, Q3	3.0 , 6.0	3.0 , 7.0			
	Min, Max	3.0 , 9.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.1,0.7)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.5 (2.28)	0.5 (2.21)			0.00 (-0.386, 0.394)
	95% Confidence Interval of Mean	-0.2 , 1.1	-0.2 , 1.1			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 2.0	0.0 , 1.0			
	Min, Max	-5.0 , 5.0	-5.0 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.9,0.9)				
	LS Mean (SE)	0.4 (0.25)	0.5 (0.25)	-0.08 (0.359) [-0.789, 0.625]	0.8195	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.1 (2.11)	5.2 (2.32)			
	95% Confidence Interval of Mean	4.5 , 5.7	4.6 , 5.9			
	Median	5.0	5.0			
	Q1, Q3	3.0 , 7.0	3.0 , 8.0			
	Min, Max	3.0 , 9.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-1.0,0.7)				

Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.5 (2.09)	0.5 (2.02)			0.03 (-0.356, 0.424)
	95% Confidence Interval of Mean	-0.1 , 1.1	-0.1 , 1.0			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	0.0 , 1.0			
	Min, Max	-5.0 , 6.0	-5.0 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.7,0.9)				
	LS Mean (SE)	0.5 (0.25)	0.5 (0.25)	-0.02 (0.359) [-0.729, 0.686]	0.9521	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s1 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Sleep Anxiety Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.9 (1.24)	5.3 (1.59)			
	95% Confidence Interval of Mean	4.6 , 5.3	4.8 , 5.7			
	Median	4.0	5.0			
	Q1, Q3	4.0 , 6.0	4.0 , 6.0			
	Min, Max	4.0 , 8.0	4.0 ,10.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.3 (-0.9,0.2)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.7 (1.08)	5.2 (1.63)			
	95% Confidence Interval of Mean	4.4 , 5.0	4.7 , 5.6			
	Median	4.0	4.0			
	Q1, Q3	4.0 , 5.0	4.0 , 6.0			
	Min, Max	4.0 , 8.0	4.0 ,10.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.4 (-1.0,0.1)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.2 (1.21)	-0.1 (1.24)			-0.07 (-0.457, 0.323)
	95% Confidence Interval of Mean	-0.5 , 0.1	-0.5 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 0.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-4.0 , 2.0	-2.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.6,0.4)				
	LS Mean (SE)	-0.3 (0.15)	-0.0 (0.15)	-0.25 (0.210) [-0.660, 0.167]	0.2420	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.9 (1.14)	5.0 (1.09)			
	95% Confidence Interval of Mean	4.6 , 5.2	4.7 , 5.3			
	Median	5.0	5.0			
	Q1, Q3	4.0 , 5.0	4.0 , 6.0			
	Min, Max	4.0 , 9.0	4.0 , 8.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.5,0.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.1 (1.10)	-0.3 (1.14)			0.23 (-0.164, 0.618)
	95% Confidence Interval of Mean	-0.4 , 0.3	-0.6 , 0.0			
	Median	0.0	0.0			
	Q1, Q3	0.0 , 1.0	-1.0 , 0.0			
	Min, Max	-3.0 , 2.0	-4.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.3 (-0.2,0.7)				
	LS Mean (SE)	-0.1 (0.15)	-0.2 (0.15)	0.09 (0.210) [-0.324, 0.503]	0.6687	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.7 (1.16)	4.9 (1.43)			
	95% Confidence Interval of Mean	4.4 , 5.0	4.5 , 5.3			
	Median	4.0	4.0			
	Q1, Q3	4.0 , 5.0	4.0 , 6.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	4.0 , 9.0	4.0 ,10.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-0.7,0.3)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.2 (1.19)	-0.4 (1.60)			0.09 (-0.296, 0.484)
	95% Confidence Interval of Mean	-0.6 , 0.1	-0.8 , 0.1			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 0.0	-1.0 , 0.0			
	Min, Max	-3.0 , 3.0	-6.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.4,0.7)				
	LS Mean (SE)	-0.3 (0.15)	-0.3 (0.15)	-0.03 (0.210) [-0.445, 0.382]	0.8817	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s2 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Sleep Anxiety Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.5 (1.89)	5.6 (1.88)			
	95% Confidence Interval of Mean	5.0 , 6.0	5.1 , 6.1			
	Median	4.0	5.0			
	Q1, Q3	4.0 , 7.0	4.0 , 6.0			
	Min, Max	4.0 , 9.0	4.0 ,10.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.8,0.7)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.4 (1.79)	5.7 (1.98)			
	95% Confidence Interval of Mean	4.9 , 5.9	5.2 , 6.3			
	Median	5.0	5.0			
	Q1, Q3	4.0 , 6.0	4.0 , 7.0			
	Min, Max	4.0 , 9.0	4.0 ,10.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.3 (-1.0,0.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.1 (2.09)	0.2 (1.91)			-0.11 (-0.499, 0.282)
	95% Confidence Interval of Mean	-0.7 , 0.5	-0.4 , 0.7			
	Median	0.0	0.0			
	Q1, Q3	0.0 , 1.0	-1.0 , 0.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-5.0 , 5.0	-5.0 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.0,0.6)				
	LS Mean (SE)	-0.1 (0.22)	0.2 (0.21)	-0.28 (0.305) [-0.878, 0.324]	0.3643	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.2 (1.41)	5.2 (1.28)			
	95% Confidence Interval of Mean	4.8 , 5.6	4.8 , 5.6			
	Median	5.0	5.0			
	Q1, Q3	4.0 , 7.0	4.0 , 6.0			
	Min, Max	4.0 , 9.0	4.0 , 8.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.5,0.6)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.3 (1.87)	-0.4 (1.64)			0.08 (-0.315, 0.465)
	95% Confidence Interval of Mean	-0.8 , 0.3	-0.9 , 0.1			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 0.0			
	Min, Max	-5.0 , 3.0	-5.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.6,0.8)				
	LS Mean (SE)	-0.3 (0.22)	-0.4 (0.21)	0.07 (0.305) [-0.529, 0.673]	0.8140	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.2 (1.55)	5.5 (1.72)			
	95% Confidence Interval of Mean	4.7 , 5.6	5.0 , 5.9			
	Median	4.0	5.0			
	Q1, Q3	4.0 , 6.0	4.0 , 7.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	4.0 , 9.0	4.0 ,10.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.3 (-0.9,0.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.3 (2.15)	-0.1 (2.25)			-0.08 (-0.473, 0.307)
	95% Confidence Interval of Mean	-0.9 , 0.3	-0.8 , 0.5			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 0.0	-1.0 , 1.0			
	Min, Max	-5.0 , 4.0	-6.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.1,0.7)				
	LS Mean (SE)	-0.4 (0.22)	-0.1 (0.21)	-0.24 (0.305) [-0.844, 0.358]	0.4261	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s1 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Night Wakings Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.0 (1.29)	5.1 (1.21)			
	95% Confidence Interval of Mean	4.6 , 5.3	4.7 , 5.4			
	Median	5.0	5.0			
	Q1, Q3	4.0 , 6.0	5.0 , 6.0			
	Min, Max	3.0 , 7.0	3.0 , 8.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.6,0.4)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.1 (1.34)	5.0 (1.17)			
	95% Confidence Interval of Mean	4.8 , 5.5	4.7 , 5.3			
	Median	5.0	5.0			
	Q1, Q3	4.0 , 6.0	4.0 , 6.0			
	Min, Max	3.0 , 8.0	3.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.4,0.6)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.2 (1.32)	-0.1 (1.14)			0.19 (-0.197, 0.585)
	95% Confidence Interval of Mean	-0.2 , 0.6	-0.4 , 0.3			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	0.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-2.0 , 3.0	-3.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-0.2,0.7)				
	LS Mean (SE)	0.2 (0.16)	-0.0 (0.16)	0.18 (0.227) [-0.265, 0.630]	0.4217	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.0 (1.24)	4.9 (1.19)			
	95% Confidence Interval of Mean	4.7 , 5.4	4.5 , 5.2			
	Median	5.0	5.0			
	Q1, Q3	5.0 , 6.0	4.0 , 5.0			
	Min, Max	3.0 , 8.0	3.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.3,0.6)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.1 (1.25)	-0.2 (1.21)			0.19 (-0.199, 0.583)
	95% Confidence Interval of Mean	-0.3 , 0.4	-0.5 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 0.0			
	Min, Max	-3.0 , 3.0	-4.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-0.2,0.7)				
	LS Mean (SE)	0.0 (0.16)	-0.1 (0.16)	0.18 (0.227) [-0.267, 0.628]	0.4277	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.9 (1.29)	4.7 (1.32)			
	95% Confidence Interval of Mean	4.6 , 5.3	4.4 , 5.1			
	Median	5.0	5.0			
	Q1, Q3	4.0 , 5.0	3.0 , 5.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	3.0 , 8.0	3.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-0.3,0.7)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.0 (1.62)	-0.3 (1.45)			0.18 (-0.212, 0.569)
	95% Confidence Interval of Mean	-0.5 , 0.4	-0.7 , 0.1			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 0.0			
	Min, Max	-4.0 , 4.0	-4.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.3 (-0.3,0.9)				
	LS Mean (SE)	-0.1 (0.16)	-0.3 (0.16)	0.22 (0.227) [-0.230, 0.665]	0.3388	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s2 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Night Wakings Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.2 (1.46)	5.4 (1.40)			
	95% Confidence Interval of Mean	4.8 , 5.6	5.0 , 5.8			
	Median	5.0	5.0			
	Q1, Q3	4.0 , 7.0	5.0 , 7.0			
	Min, Max	3.0 , 7.0	3.0 , 8.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-0.7,0.4)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.4 (1.49)	5.3 (1.36)			
	95% Confidence Interval of Mean	5.0 , 5.8	4.9 , 5.7			
	Median	5.0	5.0			
	Q1, Q3	4.0 , 7.0	4.0 , 7.0			
	Min, Max	3.0 , 8.0	3.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.4,0.7)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.2 (1.62)	-0.1 (1.42)			0.21 (-0.182, 0.600)
	95% Confidence Interval of Mean	-0.2 , 0.7	-0.5 , 0.3			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-3.0 , 4.0	-4.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.3 (-0.3,0.9)				
	LS Mean (SE)	0.2 (0.19)	-0.0 (0.19)	0.21 (0.270) [-0.319, 0.745]	0.4303	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.4 (1.47)	5.2 (1.41)			
	95% Confidence Interval of Mean	5.0 , 5.8	4.8 , 5.6			
	Median	5.0	5.0			
	Q1, Q3	5.0 , 7.0	4.0 , 7.0			
	Min, Max	3.0 , 8.0	3.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.3 (-0.3,0.8)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.2 (1.39)	-0.2 (1.53)			0.30 (-0.094, 0.691)
	95% Confidence Interval of Mean	-0.2 , 0.6	-0.6 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	0.0 , 1.0	-1.0 , 1.0			
	Min, Max	-3.0 , 4.0	-4.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.4 (-0.1,1.0)				
	LS Mean (SE)	0.2 (0.19)	-0.2 (0.19)	0.33 (0.270) [-0.201, 0.863]	0.2216	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.2 (1.48)	5.1 (1.57)			
	95% Confidence Interval of Mean	4.8 , 5.6	4.6 , 5.5			
	Median	5.0	5.0			
	Q1, Q3	4.0 , 7.0	3.0 , 7.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	3.0 , 8.0	3.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.5,0.7)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.0 (1.81)	-0.3 (1.82)			0.17 (-0.218, 0.564)
	95% Confidence Interval of Mean	-0.5 , 0.5	-0.8 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 1.0			
	Min, Max	-4.0 , 4.0	-4.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.3 (-0.4,1.0)				
	LS Mean (SE)	-0.1 (0.19)	-0.3 (0.19)	0.21 (0.270) [-0.323, 0.741]	0.4397	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s1 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Parasomnias Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	10.2 (1.92)	10.3 (2.12)			
	95% Confidence Interval of Mean	9.6 ,10.7	9.7 ,10.9			
	Median	10.0	10.0			
	Q1, Q3	9.0 ,11.0	9.0 ,11.0			
	Min, Max	7.0 ,15.0	7.0 ,19.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.0,0.6)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	10.1 (1.69)	10.3 (2.20)			
	95% Confidence Interval of Mean	9.7 ,10.6	9.7 ,10.9			
	Median	10.0	10.0			
	Q1, Q3	9.0 ,11.0	9.0 ,11.0			
	Min, Max	7.0 ,13.0	7.0 ,18.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.9,0.6)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.0 (1.96)	-0.1 (1.27)			0.01 (-0.379, 0.402)
	95% Confidence Interval of Mean	-0.6 , 0.5	-0.4 , 0.3			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 1.0	-1.0 , 0.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-5.0 , 4.0	-3.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.6,0.7)				
	LS Mean (SE)	-0.1 (0.22)	-0.0 (0.22)	-0.06 (0.314) [-0.682, 0.554]	0.8390	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	9.9 (1.72)	9.8 (1.71)			
	95% Confidence Interval of Mean	9.4 ,10.4	9.4 ,10.3			
	Median	10.0	10.0			
	Q1, Q3	9.0 ,11.0	9.0 ,10.0			
	Min, Max	7.0 ,13.0	7.0 ,16.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.6,0.7)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.3 (2.06)	-0.5 (1.94)			0.10 (-0.285, 0.495)
	95% Confidence Interval of Mean	-0.9 , 0.3	-1.0 , 0.1			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 1.0	-1.0 , 0.0			
	Min, Max	-6.0 , 4.0	-9.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-0.6,1.0)				
	LS Mean (SE)	-0.3 (0.22)	-0.4 (0.22)	0.13 (0.314) [-0.490, 0.746]	0.6848	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	9.4 (1.65)	9.6 (1.93)			
	95% Confidence Interval of Mean	8.9 , 9.9	9.1 ,10.2			
	Median	9.0	9.0			
	Q1, Q3	9.0 ,10.0	9.0 ,10.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	7.0 ,16.0	7.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-0.9,0.5)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.8 (1.96)	-0.7 (2.16)			-0.02 (-0.407, 0.373)
	95% Confidence Interval of Mean	-1.3 ,-0.2	-1.3 ,-0.1			
	Median	-1.0	-1.0			
	Q1, Q3	-2.0 , 0.0	-1.0 , 0.0			
	Min, Max	-4.0 , 5.0	-10 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.0 (-0.9,0.8)				
	LS Mean (SE)	-0.8 (0.22)	-0.7 (0.22)	-0.12 (0.314) [-0.735, 0.501]	0.7093	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s2 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Parasomnias Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	10.7 (2.34)	11.0 (2.53)			
	95% Confidence Interval of Mean	10.1 ,11.4	10.3 ,11.8			
	Median	11.0	10.0			
	Q1, Q3	9.0 ,13.0	9.0 ,13.0			
	Min, Max	7.0 ,15.0	7.0 ,19.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.3 (-1.3,0.7)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	11.2 (2.63)	11.1 (2.79)			
	95% Confidence Interval of Mean	10.5 ,12.0	10.3 ,11.8			
	Median	11.0	10.0			
	Q1, Q3	9.0 ,13.0	9.0 ,14.0			
	Min, Max	7.0 ,15.0	7.0 ,18.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-0.9,1.3)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.5 (3.20)	0.0 (2.30)			0.17 (-0.218, 0.564)
	95% Confidence Interval of Mean	-0.4 , 1.4	-0.6 , 0.7			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-6.0 , 8.0	-6.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.5 (-0.6,1.6)				
	LS Mean (SE)	0.4 (0.31)	0.1 (0.31)	0.29 (0.443) [-0.582, 1.165]	0.5110	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	10.7 (2.21)	10.5 (2.19)			
	95% Confidence Interval of Mean	10.1 ,11.3	9.9 ,11.2			
	Median	11.0	10.0			
	Q1, Q3	9.0 ,13.0	9.0 ,13.0			
	Min, Max	7.0 ,13.0	7.0 ,16.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.7,1.0)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.1 (2.74)	-0.5 (2.46)			0.17 (-0.226, 0.556)
	95% Confidence Interval of Mean	-0.8 , 0.7	-1.2 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 1.0	-1.0 , 1.0			
	Min, Max	-7.0 , 6.0	-9.0 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.4 (-0.6,1.5)				
	LS Mean (SE)	-0.2 (0.31)	-0.4 (0.31)	0.24 (0.443) [-0.632, 1.115]	0.5862	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	10.2 (2.16)	10.4 (2.31)			
	95% Confidence Interval of Mean	9.6 ,10.8	9.7 ,11.0			
	Median	10.0	10.0			
	Q1, Q3	9.0 ,13.0	9.0 ,13.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	7.0 ,16.0	7.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.1,0.7)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.5 (2.84)	-0.6 (2.56)			0.05 (-0.343, 0.437)
	95% Confidence Interval of Mean	-1.3 , 0.3	-1.4 , 0.1			
	Median	-1.0	0.0			
	Q1, Q3	-2.0 , 1.0	-1.0 , 1.0			
	Min, Max	-6.0 , 6.0	-10 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.9,1.2)				
	LS Mean (SE)	-0.6 (0.31)	-0.6 (0.31)	-0.06 (0.443) [-0.935, 0.812]	0.8898	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s1 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Sleep Disordered Breathing Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	3.7 (1.12)	3.6 (1.22)			
	95% Confidence Interval of Mean	3.4 , 4.1	3.3 , 4.0			
	Median	3.0	3.0			
	Q1, Q3	3.0 , 4.0	3.0 , 4.0			
	Min, Max	3.0 , 7.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.3,0.6)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	3.8 (1.08)	3.9 (1.29)			
	95% Confidence Interval of Mean	3.5 , 4.1	3.5 , 4.3			
	Median	3.5	3.5			
	Q1, Q3	3.0 , 4.0	3.0 , 4.0			
	Min, Max	3.0 , 8.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.6,0.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.1 (1.38)	0.3 (0.86)			-0.20 (-0.587, 0.195)
	95% Confidence Interval of Mean	-0.3 , 0.5	0.0 , 0.5			
	Median	0.0	0.0			
	Q1, Q3	-0.5 , 0.0	0.0 , 0.5			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-3.0 , 5.0	-2.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-0.7,0.2)				
	LS Mean (SE)	0.1 (0.12)	0.2 (0.12)	-0.14 (0.175) [-0.481, 0.209]	0.4373	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	3.6 (0.93)	3.5 (0.83)			
	95% Confidence Interval of Mean	3.3 , 3.8	3.3 , 3.8			
	Median	3.0	3.0			
	Q1, Q3	3.0 , 4.0	3.0 , 4.0			
	Min, Max	3.0 , 7.0	3.0 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.3,0.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.2 (1.23)	-0.1 (1.22)			-0.08 (-0.473, 0.308)
	95% Confidence Interval of Mean	-0.5 , 0.2	-0.4 , 0.3			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 0.0	0.0 , 0.0			
	Min, Max	-4.0 , 2.0	-6.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.6,0.4)				
	LS Mean (SE)	-0.1 (0.12)	-0.1 (0.12)	-0.01 (0.175) [-0.358, 0.332]	0.9407	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	3.5 (0.84)	3.3 (0.65)			
	95% Confidence Interval of Mean	3.2 , 3.7	3.1 , 3.5			
	Median	3.0	3.0			
	Q1, Q3	3.0 , 4.0	3.0 , 4.0			
	Min, Max	3.0 , 6.0	3.0 , 6.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.1,0.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.3 (1.16)	-0.3 (1.13)			0.01 (-0.377, 0.403)
	95% Confidence Interval of Mean	-0.6 , 0.1	-0.6 , 0.0			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 0.0	0.0 , 0.0			
	Min, Max	-4.0 , 2.0	-6.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.4,0.5)				
	LS Mean (SE)	-0.2 (0.12)	-0.3 (0.12)	0.10 (0.175) [-0.242, 0.447]	0.5581	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s2 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Sleep Disordered Breathing Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.1 (1.41)	3.9 (1.51)			
	95% Confidence Interval of Mean	3.7 , 4.5	3.5 , 4.3			
	Median	3.0	3.0			
	Q1, Q3	3.0 , 5.0	3.0 , 4.0			
	Min, Max	3.0 , 7.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.4,0.7)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.1 (1.38)	4.2 (1.58)			
	95% Confidence Interval of Mean	3.7 , 4.5	3.8 , 4.7			
	Median	4.0	4.0			
	Q1, Q3	3.0 , 5.0	3.0 , 5.0			
	Min, Max	3.0 , 8.0	3.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-0.7,0.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.0 (1.46)	0.3 (1.46)			-0.20 (-0.592, 0.190)
	95% Confidence Interval of Mean	-0.4 , 0.4	-0.1 , 0.7			
	Median	0.0	0.0			
	Q1, Q3	0.0 , 0.0	0.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-4.0 , 5.0	-4.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.3 (-0.9,0.3)				
	LS Mean (SE)	0.1 (0.15)	0.3 (0.15)	-0.20 (0.213) [-0.618, 0.223]	0.3557	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.0 (1.02)	3.9 (0.94)			
	95% Confidence Interval of Mean	3.7 , 4.3	3.6 , 4.1			
	Median	4.0	4.0			
	Q1, Q3	3.0 , 5.0	3.0 , 5.0			
	Min, Max	3.0 , 7.0	3.0 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-0.2,0.5)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.0 (1.48)	-0.1 (1.49)			0.01 (-0.377, 0.403)
	95% Confidence Interval of Mean	-0.5 , 0.4	-0.5 , 0.4			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	0.0 , 1.0			
	Min, Max	-4.0 , 2.0	-6.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-0.6,0.6)				
	LS Mean (SE)	0.0 (0.15)	-0.1 (0.15)	0.12 (0.213) [-0.305, 0.535]	0.5904	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	3.8 (0.96)	3.7 (0.88)			
	95% Confidence Interval of Mean	3.5 , 4.0	3.4 , 3.9			
	Median	3.0	3.0			
	Q1, Q3	3.0 , 5.0	3.0 , 4.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	3.0 , 6.0	3.0 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.3,0.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.3 (1.40)	-0.2 (1.53)			-0.04 (-0.434, 0.346)
	95% Confidence Interval of Mean	-0.7 , 0.1	-0.7 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 0.0	0.0 , 0.0			
	Min, Max	-4.0 , 2.0	-6.0 , 2.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-0.6,0.5)				
	LS Mean (SE)	-0.3 (0.15)	-0.3 (0.15)	0.03 (0.213) [-0.389, 0.452]	0.8827	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s1 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Daytime Sleepiness Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	12.1 (2.34)	12.2 (2.61)			
	95% Confidence Interval of Mean	11.4 ,12.7	11.5 ,13.0			
	Median	12.0	12.0			
	Q1, Q3	11.0 ,13.0	11.0 ,13.0			
	Min, Max	8.0 ,18.0	8.0 ,19.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.2,0.8)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	12.8 (2.81)	12.5 (2.74)			
	95% Confidence Interval of Mean	12.0 ,13.6	11.7 ,13.2			
	Median	12.0	12.0			
	Q1, Q3	11.0 ,15.0	10.0 ,14.0			
	Min, Max	8.0 ,20.0	8.0 ,19.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.3 (-0.8,1.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.7 (2.27)	0.2 (2.31)			0.20 (-0.188, 0.594)
	95% Confidence Interval of Mean	0.1 , 1.3	-0.4 , 0.9			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 1.0	-1.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-3.0 , 8.0	-5.0 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.5 (-0.4,1.4)				
	LS Mean (SE)	0.7 (0.31)	0.3 (0.31)	0.40 (0.438) [-0.463, 1.263]	0.3616	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	12.6 (2.46)	12.5 (2.52)			
	95% Confidence Interval of Mean	11.9 ,13.3	11.8 ,13.2			
	Median	12.5	12.5			
	Q1, Q3	12.0 ,13.0	11.0 ,14.0			
	Min, Max	8.0 ,20.0	8.0 ,20.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.9,1.1)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.5 (2.16)	0.2 (2.21)			0.12 (-0.269, 0.512)
	95% Confidence Interval of Mean	-0.1 , 1.1	-0.4 , 0.9			
	Median	0.5	0.0			
	Q1, Q3	-1.0 , 2.0	-1.0 , 1.0			
	Min, Max	-4.0 , 6.0	-5.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.3 (-0.6,1.1)				
	LS Mean (SE)	0.5 (0.31)	0.3 (0.31)	0.20 (0.438) [-0.662, 1.064]	0.6475	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	12.3 (2.44)	11.9 (3.13)			
	95% Confidence Interval of Mean	11.6 ,13.0	11.0 ,12.8			
	Median	11.0	11.0			
	Q1, Q3	11.0 ,14.0	10.0 ,13.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	8.0 ,20.0	8.0 ,22.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.4 (-0.7,1.5)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.2 (2.23)	-0.4 (2.96)			0.22 (-0.173, 0.609)
	95% Confidence Interval of Mean	-0.4 , 0.9	-1.2 , 0.5			
	Median	0.0	0.0			
	Q1, Q3	-1.0 , 2.0	-2.0 , 1.0			
	Min, Max	-6.0 , 6.0	-8.0 ,11.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.6 (-0.5,1.6)				
	LS Mean (SE)	0.2 (0.31)	-0.3 (0.31)	0.51 (0.438) [-0.354, 1.372]	0.2468	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s2 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Daytime Sleepiness Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	13.4 (3.41)	13.6 (3.57)			
	95% Confidence Interval of Mean	12.4 ,14.3	12.6 ,14.7			
	Median	12.5	13.0			
	Q1, Q3	11.0 ,17.0	11.0 ,18.0			
	Min, Max	8.0 ,18.0	8.0 ,19.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.3 (-1.6,1.1)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	14.3 (3.54)	13.6 (3.49)			
	95% Confidence Interval of Mean	13.3 ,15.3	12.7 ,14.6			
	Median	15.0	13.0			
	Q1, Q3	11.0 ,18.0	10.0 ,18.0			
	Min, Max	8.0 ,20.0	8.0 ,19.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.7 (-0.7,2.1)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.9 (3.85)	0.0 (3.45)			0.26 (-0.134, 0.649)
	95% Confidence Interval of Mean	-0.2 , 2.0	-1.0 , 1.0			
	Median	1.0	0.0			
	Q1, Q3	-1.0 , 3.0	-1.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-9.0 , 8.0	-9.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.9 (-0.5,2.4)				
	LS Mean (SE)	0.9 (0.47)	0.1 (0.47)	0.78 (0.664) [-0.531, 2.088]	0.2423	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	13.5 (2.89)	13.4 (2.96)			
	95% Confidence Interval of Mean	12.7 ,14.3	12.5 ,14.2			
	Median	13.0	14.0			
	Q1, Q3	12.0 ,16.0	11.0 ,16.0			
	Min, Max	8.0 ,20.0	8.0 ,20.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-1.0,1.3)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.1 (3.39)	-0.3 (3.34)			0.11 (-0.279, 0.502)
	95% Confidence Interval of Mean	-0.9 , 1.1	-1.2 , 0.7			
	Median	0.0	-1.0			
	Q1, Q3	-2.0 , 2.0	-2.0 , 2.0			
	Min, Max	-8.0 , 6.0	-9.0 , 8.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.4 (-1.0,1.7)				
	LS Mean (SE)	0.0 (0.47)	-0.2 (0.47)	0.21 (0.664) [-1.096, 1.522]	0.7486	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	14.3 (3.87)	14.0 (4.57)			
	95% Confidence Interval of Mean	13.2 ,15.4	12.7 ,15.3			
	Median	13.5	13.0			
	Q1, Q3	11.0 ,18.0	10.0 ,20.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	8.0 ,20.0	8.0 ,22.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.3 (-1.4,1.9)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.9 (4.46)	0.4 (4.89)			0.11 (-0.278, 0.503)
	95% Confidence Interval of Mean	-0.4 , 2.1	-1.0 , 1.7			
	Median	1.0	0.0			
	Q1, Q3	-1.0 , 3.0	-3.0 , 2.0			
	Min, Max	-8.0 ,10.0	-9.0 ,12.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.5 (-1.3,2.4)				
	LS Mean (SE)	0.8 (0.47)	0.4 (0.47)	0.37 (0.664) [-0.944, 1.675]	0.5825	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s1 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Total of All CSHQ Items

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	48.1 (5.12)	49.4 (7.89)			
	95% Confidence Interval of Mean	46.6 ,49.6	47.2 ,51.7			
	Median	49.0	49.0			
	Q1, Q3	46.0 ,50.0	46.0 ,51.0			
	Min, Max	37.0 ,60.0	35.0 ,76.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-1.3 (-4.0,1.3)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	49.2 (4.70)	49.4 (7.81)			
	95% Confidence Interval of Mean	47.9 ,50.6	47.2 ,51.6			
	Median	49.0	49.0			
	Q1, Q3	48.0 ,50.0	44.0 ,54.0			
	Min, Max	40.0 ,64.0	35.0 ,74.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-2.7,2.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	1.1 (5.12)	-0.1 (4.94)			0.24 (-0.153, 0.630)
	95% Confidence Interval of Mean	-0.3 , 2.6	-1.4 , 1.3			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 4.0	-2.0 , 2.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-9.0 ,14.0	-11 ,11.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.2 (-0.8,3.2)				
	LS Mean (SE)	0.8 (0.71)	0.3 (0.70)	0.46 (1.001) [-1.514, 2.430]	0.6478	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	47.7 (3.62)	47.8 (6.24)			
	95% Confidence Interval of Mean	46.7 ,48.7	46.0 ,49.5			
	Median	47.0	47.0			
	Q1, Q3	47.0 ,49.0	45.0 ,49.0			
	Min, Max	39.0 ,60.0	37.0 ,67.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-2.1,1.9)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.4 (5.23)	-1.6 (7.71)			0.19 (-0.202, 0.580)
	95% Confidence Interval of Mean	-1.9 , 1.1	-3.8 , 0.5			
	Median	-1.0	-2.0			
	Q1, Q3	-2.0 , 3.0	-4.0 , 2.0			
	Min, Max	-12 ,13.0	-29 ,17.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.2 (-1.4,3.9)				
	LS Mean (SE)	-0.8 (0.71)	-1.3 (0.70)	0.51 (1.001) [-1.466, 2.479]	0.6136	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	46.8 (5.42)	46.9 (6.13)			
	95% Confidence Interval of Mean	45.3 ,48.4	45.2 ,48.6			
	Median	46.0	46.0			
	Q1, Q3	43.0 ,50.0	45.0 ,49.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	37.0 ,60.0	35.0 ,70.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-2.4,2.2)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.3 (6.02)	-2.5 (7.66)			0.18 (-0.210, 0.572)
	95% Confidence Interval of Mean	-3.0 , 0.4	-4.7 ,-0.4			
	Median	-2.5	-3.0			
	Q1, Q3	-6.0 , 3.0	-4.0 , 1.0			
	Min, Max	-13 ,14.0	-33 ,12.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.2 (-1.5,4.0)				
	LS Mean (SE)	-1.7 (0.71)	-2.2 (0.70)	0.51 (1.001) [-1.464, 2.481]	0.6120	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.1.s2 Summary of Childrens Sleep Habit Questionnaire
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Total of All CSHQ Items

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	53.7 (10.90)	55.7 (12.03)			
	95% Confidence Interval of Mean	50.6 ,56.8	52.3 ,59.1			
	Median	51.0	54.0			
	Q1, Q3	46.0 ,69.0	46.0 ,69.0			
	Min, Max	37.0 ,69.0	35.0 ,76.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-2.0 (-6.5,2.5)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	55.4 (9.66)	53.3 (10.67)			
	95% Confidence Interval of Mean	52.6 ,58.1	50.3 ,56.3			
	Median	53.0	53.0			
	Q1, Q3	48.0 ,67.0	44.0 ,67.0			
	Min, Max	40.0 ,67.0	35.0 ,74.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.1 (-1.9,6.1)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	1.7 (12.42)	-2.5 (9.91)			0.37 (-0.027, 0.760)
	95% Confidence Interval of Mean	-1.9 , 5.2	-5.2 , 0.3			
	Median	0.5	-2.0			
	Q1, Q3	-3.0 , 6.0	-4.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-26 ,29.0	-30 ,28.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	4.1 (-0.3,8.5)				
	LS Mean (SE)	0.9 (1.19)	-1.7 (1.18)	2.67 (1.681) [-0.643, 5.984]	0.1136	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	53.8 (8.12)	52.8 (9.34)			
	95% Confidence Interval of Mean	51.5 ,56.1	50.2 ,55.4			
	Median	51.5	50.0			
	Q1, Q3	48.0 ,63.0	45.0 ,63.0			
	Min, Max	39.0 ,63.0	37.0 ,67.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.0 (-2.5,4.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.1 (11.78)	-2.9 (11.63)			0.25 (-0.137, 0.646)
	95% Confidence Interval of Mean	-3.3 , 3.4	-6.2 , 0.4			
	Median	-1.0	-3.0			
	Q1, Q3	-6.0 , 6.0	-6.0 , 2.0			
	Min, Max	-27 ,25.0	-29 ,25.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	3.0 (-1.6,7.6)				
	LS Mean (SE)	-0.6 (1.19)	-2.2 (1.18)	1.54 (1.681) [-1.772, 4.855]	0.3602	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	50.7 (7.68)	50.7 (8.14)			
	95% Confidence Interval of Mean	48.5 ,52.9	48.4 ,53.0			
	Median	52.0	50.0			
	Q1, Q3	43.0 ,59.0	45.0 ,59.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	37.0 ,60.0	35.0 ,70.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.0 (-3.1,3.1)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-3.0 (12.19)	-5.0 (12.51)			0.16 (-0.229, 0.553)
	95% Confidence Interval of Mean	-6.4 , 0.5	-8.5 ,-1.5			
	Median	-2.5	-3.0			
	Q1, Q3	-10 , 6.0	-10 , 2.0			
	Min, Max	-31 ,21.0	-34 ,21.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.0 (-2.9,6.9)				
	LS Mean (SE)	-3.7 (1.19)	-4.3 (1.18)	0.56 (1.681) [-2.754, 3.873]	0.7395	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

4.5 Ergänzende Analysen zum Endpunkt Morbidität – Veränderung der ADAMS gegenüber Baseline

4.5.1 Sensitivitätsanalysen

4.5.1.1 Sensitivitätsanalysen: Mittlere Änderung in den Subskalen des ADAMS gegenüber Baseline im Studienverlauf zu den Erhebungszeitpunkten in Woche 1–17 (MMRM)

Study No. 1042-CDD-3001

Table 14.2.6.2.s1 Summary of Anxiety Depression and Mood Scale
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Manic Hyperactive Behavior Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	6.9 (4.25)	6.3 (3.93)			
	95% Confidence Interval of Mean	5.7 , 8.1	5.1 , 7.4			
	Median	7.0	6.0			
	Q1, Q3	3.0 ,11.0	2.0 ,10.0			
	Min, Max	0.0 ,15.0	0.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.7 (-1.0,2.3)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.7 (3.67)	5.5 (3.62)			
	95% Confidence Interval of Mean	4.7 , 6.7	4.5 , 6.5			
	Median	6.0	6.0			
	Q1, Q3	3.0 , 8.0	2.0 , 8.0			
	Min, Max	0.0 ,15.0	0.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-1.3,1.6)				

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.2 (3.59)	-0.7 (3.74)			-0.13 (-0.525, 0.256)
	95% Confidence Interval of Mean	-2.2 ,-0.2	-1.8 , 0.3			
	Median	-1.0	0.0			
	Q1, Q3	-3.0 , 2.0	-3.0 , 0.0			
	Min, Max	-11 , 5.0	-10 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.5 (-1.9,1.0)				
	LS Mean (SE)	-1.1 (0.41)	-0.9 (0.41)	-0.18 (0.581) [-1.330, 0.961]	0.7506	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.4 (3.64)	4.7 (3.50)			
	95% Confidence Interval of Mean	4.4 , 6.4	3.7 , 5.7			
	Median	5.0	5.0			
	Q1, Q3	2.0 , 8.0	1.0 , 7.0			
	Min, Max	0.0 ,12.0	0.0 ,13.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.7 (-0.7,2.1)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.5 (3.48)	-1.6 (3.40)			0.01 (-0.376, 0.404)
	95% Confidence Interval of Mean	-2.5 ,-0.5	-2.5 ,-0.6			
	Median	-1.0	-2.0			
	Q1, Q3	-4.0 , 1.0	-4.0 , 0.0			
	Min, Max	-11 , 5.0	-8.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-1.3,1.4)				
	LS Mean (SE)	-1.4 (0.41)	-1.7 (0.41)	0.36 (0.581) [-0.787, 1.504]	0.5380	

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.7 (3.45)	5.2 (3.91)			
	95% Confidence Interval of Mean	3.7 , 5.7	4.1 , 6.3			
	Median	4.5	5.0			
	Q1, Q3	2.0 , 7.0	2.0 , 8.0			
	Min, Max	0.0 ,13.0	0.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.5 (-1.9,1.0)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-2.2 (3.79)	-1.1 (2.72)			-0.34 (-0.733, 0.052)
	95% Confidence Interval of Mean	-3.3 ,-1.1	-1.8 ,-0.3			
	Median	-1.5	-1.0			
	Q1, Q3	-5.0 , 0.0	-2.0 , 0.0			
	Min, Max	-12 , 4.0	-8.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-1.1 (-2.4,0.2)				
	LS Mean (SE)	-2.0 (0.41)	-1.2 (0.41)	-0.81 (0.581) [-1.957, 0.333]	0.1637	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.2.s2 Summary of Anxiety Depression and Mood Scale
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Manic Hyperactive Behavior Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	7.2 (4.36)	6.5 (4.09)			
	95% Confidence Interval of Mean	5.9 , 8.4	5.3 , 7.6			
	Median	7.5	7.0			
	Q1, Q3	3.0 ,11.0	2.0 ,10.0			
	Min, Max	0.0 ,15.0	0.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.7 (-1.0,2.3)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	6.3 (4.14)	6.0 (4.02)			
	95% Confidence Interval of Mean	5.1 , 7.5	4.9 , 7.1			
	Median	6.0	6.0			
	Q1, Q3	3.0 , 9.0	2.0 , 9.0			
	Min, Max	0.0 ,15.0	0.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.3 (-1.3,1.9)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.9 (4.33)	-0.5 (4.00)			-0.09 (-0.479, 0.302)
	95% Confidence Interval of Mean	-2.1 , 0.4	-1.6 , 0.6			
	Median	-1.0	0.0			
	Q1, Q3	-3.0 , 2.0	-3.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-11 ,11.0	-10 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.4 (-2.0,1.3)				
	LS Mean (SE)	-0.7 (0.51)	-0.7 (0.50)	-0.03 (0.716) [-1.447, 1.379]	0.9619	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	6.2 (4.12)	5.0 (3.81)			
	95% Confidence Interval of Mean	5.1 , 7.4	4.0 , 6.1			
	Median	6.0	5.0			
	Q1, Q3	2.0 ,11.0	1.0 , 8.0			
	Min, Max	0.0 ,12.0	0.0 ,13.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.2 (-0.4,2.8)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.9 (4.19)	-1.5 (3.62)			0.14 (-0.255, 0.526)
	95% Confidence Interval of Mean	-2.1 , 0.3	-2.5 ,-0.4			
	Median	-1.0	-1.0			
	Q1, Q3	-3.0 , 1.0	-4.0 , 0.0			
	Min, Max	-11 ,10.0	-12 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.5 (-1.0,2.1)				
	LS Mean (SE)	-0.8 (0.51)	-1.6 (0.50)	0.87 (0.716) [-0.547, 2.280]	0.2279	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.8 (4.39)	5.9 (4.41)			
	95% Confidence Interval of Mean	4.6 , 7.1	4.6 , 7.1			
	Median	5.0	6.0			
	Q1, Q3	2.0 ,10.0	2.0 ,10.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,13.0	0.0 ,15.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.0 (-1.8,1.7)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.3 (5.28)	-0.6 (3.26)			-0.16 (-0.549, 0.232)
	95% Confidence Interval of Mean	-2.8 , 0.2	-1.5 , 0.3			
	Median	-1.0	0.0			
	Q1, Q3	-5.0 , 1.0	-2.0 , 1.0			
	Min, Max	-12 ,11.0	-12 ,10.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.7 (-2.4,1.0)				
	LS Mean (SE)	-1.2 (0.51)	-0.8 (0.50)	-0.36 (0.716) [-1.770, 1.056]	0.6187	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.2.s1 Summary of Anxiety Depression and Mood Scale
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Depressed Mood Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.7 (3.75)	4.8 (3.58)			
	95% Confidence Interval of Mean	4.6 , 6.7	3.8 , 5.8			
	Median	5.0	5.0			
	Q1, Q3	3.0 , 7.0	2.0 , 8.0			
	Min, Max	0.0 ,16.0	0.0 ,13.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.9 (-0.6,2.3)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.6 (3.59)	4.3 (3.96)			
	95% Confidence Interval of Mean	4.6 , 6.6	3.2 , 5.5			
	Median	5.0	3.5			
	Q1, Q3	3.5 , 7.0	1.0 , 6.0			
	Min, Max	0.0 ,15.0	0.0 ,18.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.2 (-0.2,2.7)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.1 (4.22)	-0.5 (3.56)			0.09 (-0.295, 0.485)
	95% Confidence Interval of Mean	-1.3 , 1.1	-1.5 , 0.5			
	Median	0.0	-1.0			
	Q1, Q3	-2.0 , 3.0	-3.0 , 2.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-10 ,11.0	-9.0 ,10.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.4 (-1.2,1.9)				
	LS Mean (SE)	0.1 (0.43)	-0.7 (0.42)	0.79 (0.603) [-0.397, 1.979]	0.1909	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.1 (3.67)	3.3 (3.42)			
	95% Confidence Interval of Mean	4.0 , 6.1	2.4 , 4.3			
	Median	4.0	2.5			
	Q1, Q3	2.5 , 7.0	0.0 , 5.0			
	Min, Max	0.0 ,16.0	0.0 ,12.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.8 (0.4,3.2)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.6 (3.64)	-1.5 (3.02)			0.27 (-0.126, 0.658)
	95% Confidence Interval of Mean	-1.6 , 0.4	-2.3 ,-0.6			
	Median	-0.8	-2.0			
	Q1, Q3	-3.0 , 1.0	-3.0 , 0.0			
	Min, Max	-10 , 8.0	-10 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.9 (-0.4,2.2)				
	LS Mean (SE)	-0.4 (0.43)	-1.7 (0.42)	1.31 (0.603) [0.122, 2.499]	0.0308	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.2 (3.13)	3.9 (3.53)			
	95% Confidence Interval of Mean	4.3 , 6.1	2.9 , 4.9			
	Median	4.0	3.0			
	Q1, Q3	3.0 , 7.0	1.0 , 6.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,13.0	0.0 ,12.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.3 (-0.0,2.6)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.5 (3.59)	-0.9 (2.64)			0.13 (-0.257, 0.524)
	95% Confidence Interval of Mean	-1.5 , 0.5	-1.7 ,-0.2			
	Median	0.0	-1.0			
	Q1, Q3	-3.0 , 2.0	-3.0 , 1.0			
	Min, Max	-10 , 6.0	-7.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.4 (-0.8,1.7)				
	LS Mean (SE)	-0.3 (0.43)	-1.1 (0.42)	0.84 (0.603) [-0.347, 2.030]	0.1641	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.2.s2 Summary of Anxiety Depression and Mood Scale
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Depressed Mood Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.9 (3.90)	5.0 (3.78)			
	95% Confidence Interval of Mean	4.8 , 7.0	4.0 , 6.1			
	Median	5.0	5.0			
	Q1, Q3	3.0 , 8.0	2.0 , 8.0			
	Min, Max	0.0 ,16.0	0.0 ,13.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.9 (-0.6,2.4)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	6.3 (3.85)	4.8 (4.25)			
	95% Confidence Interval of Mean	5.2 , 7.4	3.6 , 6.0			
	Median	6.0	4.0			
	Q1, Q3	4.0 , 9.0	1.0 , 8.0			
	Min, Max	0.0 ,15.0	0.0 ,18.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.6 (-0.0,3.2)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.4 (4.50)	-0.3 (3.71)			0.16 (-0.227, 0.554)
	95% Confidence Interval of Mean	-0.9 , 1.7	-1.3 , 0.8			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 3.0	-2.0 , 2.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-10 ,11.0	-9.0 ,10.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.7 (-1.0,2.3)				
	LS Mean (SE)	0.6 (0.48)	-0.5 (0.48)	1.12 (0.682) [-0.225, 2.467]	0.1020	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	6.1 (3.85)	3.8 (3.76)			
	95% Confidence Interval of Mean	5.0 , 7.2	2.7 , 4.8			
	Median	5.5	3.0			
	Q1, Q3	3.0 ,10.0	0.0 , 6.0			
	Min, Max	0.0 ,16.0	0.0 ,12.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.4 (0.9,3.9)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.2 (4.14)	-1.3 (3.24)			0.40 (0.009, 0.797)
	95% Confidence Interval of Mean	-1.0 , 1.4	-2.2 ,-0.4			
	Median	0.0	-1.0			
	Q1, Q3	-2.0 , 2.0	-3.0 , 1.0			
	Min, Max	-10 , 9.0	-11 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.5 (0.0,3.0)				
	LS Mean (SE)	0.4 (0.48)	-1.5 (0.48)	1.94 (0.682) [0.595, 3.286]	0.0049	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	6.5 (3.59)	4.5 (4.01)			
	95% Confidence Interval of Mean	5.4 , 7.5	3.4 , 5.6			
	Median	6.5	3.0			
	Q1, Q3	3.0 ,10.0	1.0 , 8.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,13.0	0.0 ,12.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.0 (0.4,3.5)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.5 (4.60)	-0.5 (3.07)			0.27 (-0.118, 0.666)
	95% Confidence Interval of Mean	-0.8 , 1.8	-1.4 , 0.3			
	Median	0.5	0.0			
	Q1, Q3	-3.0 , 4.0	-2.0 , 1.0			
	Min, Max	-10 ,11.0	-11 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.1 (-0.5,2.6)				
	LS Mean (SE)	0.8 (0.48)	-0.8 (0.48)	1.52 (0.682) [0.170, 2.861]	0.0275	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.2.s1 Summary of Anxiety Depression and Mood Scale
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Social Avoidance Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	7.6 (4.52)	6.2 (4.30)			
	95% Confidence Interval of Mean	6.3 , 8.9	5.0 , 7.4			
	Median	6.0	5.0			
	Q1, Q3	4.0 ,10.0	3.0 , 9.0			
	Min, Max	0.0 ,18.0	0.0 ,18.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.4 (-0.4,3.1)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	6.3 (4.35)	6.1 (4.81)			
	95% Confidence Interval of Mean	5.1 , 7.6	4.8 , 7.5			
	Median	6.0	5.0			
	Q1, Q3	4.0 , 9.0	3.0 , 9.0			
	Min, Max	0.0 ,18.0	0.0 ,17.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-1.6,2.0)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.3 (3.11)	-0.1 (3.84)			-0.34 (-0.731, 0.055)
	95% Confidence Interval of Mean	-2.1 ,-0.4	-1.2 , 1.0			
	Median	-0.5	0.0			
	Q1, Q3	-3.0 , 1.0	-2.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-9.0 , 4.0	-10 ,14.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-1.2 (-2.6,0.2)				
	LS Mean (SE)	-1.0 (0.47)	-0.3 (0.46)	-0.67 (0.661) [-1.978, 0.630]	0.3094	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	5.8 (3.92)	5.3 (4.05)			
	95% Confidence Interval of Mean	4.7 , 6.9	4.2 , 6.5			
	Median	5.0	4.5			
	Q1, Q3	3.0 , 8.0	2.0 , 8.0			
	Min, Max	0.0 ,17.0	0.0 ,17.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.4 (-1.1,2.0)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.8 (3.83)	-0.9 (3.79)			-0.24 (-0.632, 0.151)
	95% Confidence Interval of Mean	-2.9 ,-0.7	-2.0 , 0.2			
	Median	-1.0	-1.0			
	Q1, Q3	-3.0 , 0.0	-2.0 , 2.0			
	Min, Max	-12 , 5.0	-10 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.9 (-2.4,0.6)				
	LS Mean (SE)	-1.6 (0.47)	-1.1 (0.46)	-0.41 (0.661) [-1.715, 0.894]	0.5358	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	6.5 (4.21)	5.7 (4.39)			
	95% Confidence Interval of Mean	5.3 , 7.7	4.5 , 6.9			
	Median	5.0	5.0			
	Q1, Q3	4.0 , 9.0	3.0 , 9.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,18.0	0.0 ,17.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.8 (-0.9,2.5)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.1 (4.11)	-0.5 (3.26)			-0.15 (-0.544, 0.237)
	95% Confidence Interval of Mean	-2.2 , 0.1	-1.4 , 0.4			
	Median	-1.0	0.0			
	Q1, Q3	-3.0 , 2.0	-2.0 , 1.0			
	Min, Max	-13 , 6.0	-9.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.6 (-2.0,0.9)				
	LS Mean (SE)	-0.8 (0.47)	-0.8 (0.46)	-0.06 (0.661) [-1.367, 1.242]	0.9246	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.2.s2 Summary of Anxiety Depression and Mood Scale
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Social Avoidance Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	8.2 (4.80)	6.6 (4.63)			
	95% Confidence Interval of Mean	6.8 , 9.5	5.3 , 7.9			
	Median	6.5	5.0			
	Q1, Q3	4.0 ,11.0	3.0 ,10.0			
	Min, Max	0.0 ,18.0	0.0 ,18.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.6 (-0.3,3.4)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	7.4 (5.21)	7.0 (5.48)			
	95% Confidence Interval of Mean	5.9 , 8.9	5.5 , 8.5			
	Median	6.5	5.0			
	Q1, Q3	4.0 ,11.0	3.0 ,10.0			
	Min, Max	0.0 ,18.0	0.0 ,17.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.4 (-1.7,2.5)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.8 (4.40)	0.4 (5.22)			-0.24 (-0.630, 0.153)
	95% Confidence Interval of Mean	-2.0 , 0.5	-1.1 , 1.9			
	Median	0.0	0.0			
	Q1, Q3	-4.0 , 1.0	-2.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-9.0 ,12.0	-15 ,14.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-1.2 (-3.1,0.8)				
	LS Mean (SE)	-0.4 (0.64)	0.0 (0.63)	-0.41 (0.906) [-2.196, 1.379]	0.6527	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	7.0 (4.60)	5.8 (4.43)			
	95% Confidence Interval of Mean	5.7 , 8.3	4.6 , 7.1			
	Median	6.0	5.0			
	Q1, Q3	3.0 ,11.0	2.0 , 9.0			
	Min, Max	0.0 ,17.0	0.0 ,17.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.1 (-0.6,2.9)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.2 (4.46)	-0.8 (4.58)			-0.10 (-0.487, 0.294)
	95% Confidence Interval of Mean	-2.5 , 0.0	-2.1 , 0.5			
	Median	-1.0	-1.0			
	Q1, Q3	-3.0 , 2.0	-3.0 , 2.0			
	Min, Max	-12 , 9.0	-12 ,11.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.4 (-2.2,1.3)				
	LS Mean (SE)	-0.8 (0.64)	-1.2 (0.63)	0.31 (0.906) [-1.479, 2.095]	0.7342	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	8.5 (5.46)	7.0 (5.50)			
	95% Confidence Interval of Mean	6.9 ,10.0	5.5 , 8.5			
	Median	8.0	6.0			
	Q1, Q3	4.0 ,14.0	3.0 , 9.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,18.0	0.0 ,17.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.5 (-0.7,3.6)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.3 (5.97)	0.4 (5.28)			-0.02 (-0.406, 0.374)
	95% Confidence Interval of Mean	-1.4 , 2.0	-1.1 , 1.9			
	Median	0.0	0.0			
	Q1, Q3	-3.0 , 4.0	-3.0 , 3.0			
	Min, Max	-13 ,13.0	-12 ,14.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-2.3,2.1)				
	LS Mean (SE)	0.7 (0.64)	0.0 (0.63)	0.65 (0.906) [-1.136, 2.439]	0.4729	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.2.s1 Summary of Anxiety Depression and Mood Scale
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

General Anxiety Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.0 (3.49)	4.5 (3.99)			
	95% Confidence Interval of Mean	3.0 , 5.0	3.4 , 5.6			
	Median	3.0	4.0			
	Q1, Q3	1.0 , 6.0	2.0 , 6.0			
	Min, Max	0.0 ,14.0	0.0 ,18.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.5 (-2.0,0.9)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	2.8 (2.84)	3.6 (4.08)			
	95% Confidence Interval of Mean	2.0 , 3.6	2.4 , 4.7			
	Median	2.0	2.0			
	Q1, Q3	1.0 , 3.0	1.0 , 5.0			
	Min, Max	0.0 ,12.0	0.0 ,18.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.7 (-2.1,0.6)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.1 (2.54)	-0.9 (2.55)			-0.08 (-0.468, 0.312)
	95% Confidence Interval of Mean	-1.9 ,-0.4	-1.7 ,-0.2			
	Median	-1.0	-1.0			
	Q1, Q3	-2.0 , 0.0	-3.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-8.0 , 4.0	-8.0 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.2,0.8)				
	LS Mean (SE)	-1.3 (0.34)	-0.8 (0.34)	-0.42 (0.485) [-1.380, 0.531]	0.3821	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	2.6 (2.74)	3.3 (3.58)			
	95% Confidence Interval of Mean	1.8 , 3.4	2.2 , 4.3			
	Median	2.0	2.0			
	Q1, Q3	1.0 , 4.0	1.0 , 4.0			
	Min, Max	0.0 ,14.0	0.0 ,13.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.7 (-1.9,0.6)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.4 (3.21)	-1.3 (2.91)			-0.04 (-0.431, 0.349)
	95% Confidence Interval of Mean	-2.3 ,-0.5	-2.1 ,-0.4			
	Median	-1.0	-1.0			
	Q1, Q3	-2.0 , 0.0	-3.0 , 0.0			
	Min, Max	-14 , 5.0	-13 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.1 (-1.3,1.1)				
	LS Mean (SE)	-1.5 (0.34)	-1.1 (0.34)	-0.35 (0.485) [-1.306, 0.604]	0.4700	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	2.6 (2.75)	2.8 (3.44)			
	95% Confidence Interval of Mean	1.9 , 3.4	1.9 , 3.8			
	Median	2.0	2.0			
	Q1, Q3	0.0 , 3.0	1.0 , 3.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,11.0	0.0 ,14.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.2 (-1.4,1.0)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.3 (3.13)	-1.7 (2.81)			0.12 (-0.274, 0.507)
	95% Confidence Interval of Mean	-2.2 ,-0.4	-2.5 ,-0.9			
	Median	-1.0	-2.0			
	Q1, Q3	-2.0 , 0.0	-2.0 , 0.0			
	Min, Max	-14 , 4.0	-14 , 5.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.3 (-0.8,1.5)				
	LS Mean (SE)	-1.4 (0.34)	-1.6 (0.34)	0.12 (0.485) [-0.834, 1.076]	0.8029	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.2.s2 Summary of Anxiety Depression and Mood Scale
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

General Anxiety Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.4 (4.01)	4.9 (4.40)			
	95% Confidence Interval of Mean	3.2 , 5.5	3.7 , 6.1			
	Median	3.0	4.0			
	Q1, Q3	1.0 , 7.0	2.0 , 7.0			
	Min, Max	0.0 ,14.0	0.0 ,18.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.5 (-2.2,1.1)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	3.9 (4.16)	4.2 (4.62)			
	95% Confidence Interval of Mean	2.7 , 5.1	2.9 , 5.5			
	Median	2.0	3.0			
	Q1, Q3	1.0 , 6.0	1.0 , 5.0			
	Min, Max	0.0 ,13.0	0.0 ,18.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.3 (-2.0,1.4)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.4 (3.86)	-0.7 (3.10)			0.07 (-0.320, 0.461)
	95% Confidence Interval of Mean	-1.5 , 0.7	-1.6 , 0.2			
	Median	-1.0	-1.0			
	Q1, Q3	-2.0 , 1.0	-2.0 , 1.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-8.0 ,12.0	-14 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-1.1,1.6)				
	LS Mean (SE)	-0.6 (0.53)	-0.6 (0.53)	0.01 (0.748) [-1.463, 1.488]	0.9863	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.1 (4.53)	3.9 (4.24)			
	95% Confidence Interval of Mean	2.8 , 5.4	2.7 , 5.1			
	Median	2.5	2.0			
	Q1, Q3	1.0 , 6.0	1.0 , 6.0			
	Min, Max	0.0 ,14.0	0.0 ,13.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.2 (-1.5,2.0)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.2 (4.80)	-1.0 (3.42)			0.18 (-0.208, 0.573)
	95% Confidence Interval of Mean	-1.6 , 1.1	-2.0 ,-0.0			
	Median	-1.0	-1.0			
	Q1, Q3	-2.0 , 2.0	-2.0 , 0.0			
	Min, Max	-14 ,12.0	-14 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.8 (-0.9,2.4)				
	LS Mean (SE)	-0.4 (0.53)	-0.9 (0.53)	0.53 (0.748) [-0.949, 2.002]	0.4822	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	4.4 (4.68)	3.7 (4.39)			
	95% Confidence Interval of Mean	3.1 , 5.7	2.5 , 4.9			
	Median	3.0	2.0			
	Q1, Q3	0.0 , 8.0	1.0 , 6.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,13.0	0.0 ,14.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.7 (-1.1,2.5)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.0 (5.51)	-1.2 (3.79)			0.26 (-0.130, 0.654)
	95% Confidence Interval of Mean	-1.5 , 1.6	-2.3 ,-0.1			
	Median	-1.0	-1.0			
	Q1, Q3	-2.0 , 2.0	-2.0 , 0.0			
	Min, Max	-14 ,12.0	-14 ,11.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.2 (-0.6,3.1)				
	LS Mean (SE)	-0.1 (0.53)	-1.1 (0.53)	1.00 (0.748) [-0.473, 2.478]	0.1816	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.2.s1 Summary of Anxiety Depression and Mood Scale
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Obsessive Compulsive Behavior Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	2.2 (2.28)	2.2 (2.54)			
	95% Confidence Interval of Mean	1.6 , 2.9	1.5 , 3.0			
	Median	2.0	2.0			
	Q1, Q3	0.0 , 4.0	0.0 , 4.0			
	Min, Max	0.0 , 8.0	0.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.0 (-1.0,0.9)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.3 (1.59)	1.8 (2.33)			
	95% Confidence Interval of Mean	0.8 , 1.7	1.2 , 2.5			
	Median	1.0	1.0			
	Q1, Q3	0.0 , 2.0	0.0 , 3.0			
	Min, Max	0.0 , 8.0	0.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.6 (-1.4,0.2)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.9 (2.01)	-0.4 (1.65)			-0.30 (-0.690, 0.094)
	95% Confidence Interval of Mean	-1.5 ,-0.4	-0.9 , 0.1			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 0.0	-1.0 , 0.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-8.0 , 2.0	-5.0 , 3.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.5 (-1.3,0.2)				
	LS Mean (SE)	-0.9 (0.21)	-0.4 (0.21)	-0.55 (0.295) [-1.137, 0.027]	0.0615	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.2 (1.69)	1.6 (2.36)			
	95% Confidence Interval of Mean	0.7 , 1.6	1.0 , 2.3			
	Median	0.0	0.0			
	Q1, Q3	0.0 , 2.0	0.0 , 3.0			
	Min, Max	0.0 , 7.0	0.0 , 8.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.5 (-1.3,0.3)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.1 (2.07)	-0.6 (1.83)			-0.23 (-0.622, 0.160)
	95% Confidence Interval of Mean	-1.6 ,-0.5	-1.1 ,-0.1			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 0.0	-2.0 , 0.0			
	Min, Max	-7.0 , 3.0	-6.0 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.5 (-1.2,0.3)				
	LS Mean (SE)	-1.1 (0.21)	-0.6 (0.21)	-0.46 (0.295) [-1.041, 0.123]	0.1212	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.1 (1.31)	1.6 (2.26)			
	95% Confidence Interval of Mean	0.7 , 1.5	1.0 , 2.3			
	Median	0.5	0.5			
	Q1, Q3	0.0 , 2.0	0.0 , 3.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 , 5.0	0.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.5 (-1.3,0.2)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.1 (1.79)	-0.6 (1.69)			-0.29 (-0.686, 0.098)
	95% Confidence Interval of Mean	-1.6 ,-0.6	-1.1 ,-0.1			
	Median	-1.0	0.0			
	Q1, Q3	-2.0 , 0.0	-2.0 , 0.0			
	Min, Max	-6.0 , 2.0	-5.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.5 (-1.2,0.2)				
	LS Mean (SE)	-1.1 (0.21)	-0.6 (0.21)	-0.52 (0.295) [-1.101, 0.063]	0.0801	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.2.s2 Summary of Anxiety Depression and Mood Scale
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Obsessive Compulsive Behavior Total

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	2.4 (2.47)	2.4 (2.71)			
	95% Confidence Interval of Mean	1.7 , 3.1	1.7 , 3.2			
	Median	2.0	2.0			
	Q1, Q3	0.0 , 4.0	0.0 , 4.0			
	Min, Max	0.0 , 8.0	0.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.0 (-1.0,1.0)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	1.9 (2.34)	2.3 (2.69)			
	95% Confidence Interval of Mean	1.2 , 2.5	1.6 , 3.1			
	Median	1.0	1.0			
	Q1, Q3	0.0 , 3.0	0.0 , 4.0			
	Min, Max	0.0 , 8.0	0.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.4 (-1.4,0.6)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.5 (2.75)	-0.1 (1.87)			-0.18 (-0.571, 0.211)
	95% Confidence Interval of Mean	-1.3 , 0.2	-0.6 , 0.4			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 0.0	-1.0 , 0.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-8.0 , 7.0	-7.0 , 4.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.4 (-1.3,0.5)				
	LS Mean (SE)	-0.5 (0.32)	-0.1 (0.31)	-0.43 (0.447) [-1.310, 0.454]	0.3394	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	2.1 (2.56)	2.0 (2.64)			
	95% Confidence Interval of Mean	1.4 , 2.9	1.3 , 2.8			
	Median	1.0	0.0			
	Q1, Q3	0.0 , 3.0	0.0 , 4.0			
	Min, Max	0.0 , 7.0	0.0 , 8.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.9,1.1)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.3 (2.86)	-0.4 (2.10)			0.04 (-0.345, 0.435)
	95% Confidence Interval of Mean	-1.1 , 0.5	-1.0 , 0.2			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 0.0	-2.0 , 0.0			
	Min, Max	-7.0 , 7.0	-7.0 , 6.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.1 (-0.9,1.1)				
	LS Mean (SE)	-0.3 (0.32)	-0.4 (0.31)	0.11 (0.447) [-0.775, 0.988]	0.8118	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	2.3 (2.57)	2.3 (2.73)			
	95% Confidence Interval of Mean	1.5 , 3.0	1.5 , 3.0			
	Median	1.5	1.0			
	Q1, Q3	0.0 , 3.0	0.0 , 4.0			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 , 7.0	0.0 , 9.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-1.0,1.1)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.1 (3.26)	-0.2 (2.37)			0.01 (-0.384, 0.396)
	95% Confidence Interval of Mean	-1.1 , 0.8	-0.8 , 0.5			
	Median	0.0	0.0			
	Q1, Q3	-2.0 , 0.0	-1.0 , 0.0			
	Min, Max	-7.0 , 7.0	-7.0 , 7.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.0 (-1.1,1.1)				
	LS Mean (SE)	-0.1 (0.32)	-0.2 (0.31)	0.01 (0.447) [-0.871, 0.893]	0.9799	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

4.6 Ergänzende Analysen zum Endpunkt Lebensqualität – gemäß dem Quality of Life Inventory-Disability (QI-Disability)-Fragebogen

4.6.1 Sensitivitätsanalysen

4.6.1.1 Sensitivitätsanalysen: Mittlere Änderung in den Domänen und dem Gesamtscore des QI-Disability gegenüber Baseline im Studienverlauf zu den Erhebungszeitpunkten in Woche 1–17 (MMRM)

Study No. 1042-CDD-3001
Table 14.2.6.5.s1 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Physical Health Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	62.63 (16.535)	63.85 (20.475)			
	95% Confidence Interval of Mean	57.93 ,67.32	58.09 ,69.61			
	Median	68.75	68.75			
	Q1, Q3	56.25 ,68.75	56.25 ,75.00			
	Min, Max	25.0 , 93.8	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-1.22 (-8.58,6.13)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	59.75 (17.135)	64.22 (22.607)			
	95% Confidence Interval of Mean	54.88 ,64.62	57.86 ,70.57			
	Median	62.50	68.75			
	Q1, Q3	50.00 ,68.75	50.00 ,81.25			
	Min, Max	0.0 , 93.8	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-4.47 (-12.40,3.47)				

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-2.88 (19.933)	0.37 (15.279)			-0.18 (-0.574, 0.208)
	95% Confidence Interval of Mean	-8.54 , 2.79	-3.93 , 4.67			
	Median	-6.25	0.00			
	Q1, Q3	-12.5 , 6.25	-12.5 ,12.50			
	Min, Max	-56.3 , 50.0	-31.3 , 31.3			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-3.24 (-10.25,3.76)				
	LS Mean (SE)	-3.2 (2.13)	0.7 (2.10)	-3.83 (2.992) [-9.736, 2.067]	0.2016	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	61.75 (17.018)	65.81 (18.889)			
	95% Confidence Interval of Mean	56.91 ,66.59	60.50 ,71.12			
	Median	62.50	68.75			
	Q1, Q3	50.00 ,68.75	56.25 ,75.00			
	Min, Max	6.3 ,100.0	12.5 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-4.06 (-11.16,3.04)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.88 (20.203)	1.96 (15.983)			-0.16 (-0.547, 0.235)
	95% Confidence Interval of Mean	-6.62 , 4.87	-2.53 , 6.46			
	Median	0.00	0.00			
	Q1, Q3	-12.5 ,12.50	-6.25 ,12.50			
	Min, Max	-56.3 , 50.0	-25.0 , 43.8			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-2.84 (-10.02,4.35)				
	LS Mean (SE)	-1.2 (2.13)	2.3 (2.10)	-3.43 (2.992) [-9.329, 2.474]	0.2534	
Visit 5 (End of Week 17)	n	50	51			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	64.88 (14.391)	65.32 (15.477)			
	95% Confidence Interval of Mean	60.79 ,68.96	60.97 ,69.67			
	Median	68.75	68.75			
	Q1, Q3	56.25 ,75.00	56.25 ,75.00			
	Min, Max	25.0 ,100.0	18.8 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.44 (-6.35,5.46)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	2.25 (16.597)	1.47 (16.326)			0.05 (-0.343, 0.437)
	95% Confidence Interval of Mean	-2.47 , 6.97	-3.12 , 6.06			
	Median	0.00	0.00			
	Q1, Q3	-6.25 ,12.50	-6.25 ,12.50			
	Min, Max	-25.0 , 56.3	-43.8 , 43.8			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.78 (-5.72,7.28)				
	LS Mean (SE)	2.0 (2.13)	1.8 (2.10)	0.19 (2.992) [-5.714, 6.089]	0.9500	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s2 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Physical Health Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	62.63 (16.535)	62.87 (21.412)			
	95% Confidence Interval of Mean	57.93 ,67.32	56.85 ,68.89			
	Median	68.75	68.75			
	Q1, Q3	56.25 ,68.75	56.25 ,75.00			
	Min, Max	25.0 , 93.8	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.24 (-7.81,7.32)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	56.00 (21.203)	60.54 (26.413)			
	95% Confidence Interval of Mean	49.97 ,62.03	53.11 ,67.97			
	Median	59.38	68.75			
	Q1, Q3	43.75 ,68.75	50.00 ,81.25			
	Min, Max	0.0 , 93.8	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-4.54 (-14.01,4.93)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-6.63 (21.369)	-2.33 (21.649)			-0.20 (-0.591, 0.191)
	95% Confidence Interval of Mean	-12.7 ,-0.55	-8.42 , 3.76			
	Median	-6.25	0.00			
	Q1, Q3	-18.8 , 0.00	-12.5 ,12.50			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-56.3 , 50.0	-68.8 , 43.8			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-4.30 (-12.79,4.20)				
	LS Mean (SE)	-6.7 (2.61)	-2.3 (2.58)	-4.41 (3.669) [-11.650, 2.828]	0.2308	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	56.75 (21.161)	61.89 (22.751)			
	95% Confidence Interval of Mean	50.74 ,62.76	55.49 ,68.29			
	Median	62.50	62.50			
	Q1, Q3	43.75 ,68.75	50.00 ,75.00			
	Min, Max	6.3 ,100.0	12.5 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-5.14 (-13.82,3.54)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-5.88 (22.952)	-0.98 (21.116)			-0.22 (-0.613, 0.169)
	95% Confidence Interval of Mean	-12.4 , 0.65	-6.92 , 4.96			
	Median	-3.13	0.00			
	Q1, Q3	-18.8 , 6.25	-12.5 ,12.50			
	Min, Max	-56.3 , 50.0	-56.3 , 43.8			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-4.89 (-13.60,3.81)				
	LS Mean (SE)	-5.9 (2.61)	-0.9 (2.58)	-5.01 (3.669) [-12.248, 2.230]	0.1739	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	61.88 (15.834)	63.36 (16.490)			
	95% Confidence Interval of Mean	57.38 ,66.37	58.72 ,68.00			
	Median	62.50	68.75			
	Q1, Q3	50.00 ,75.00	50.00 ,75.00			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	25.0 ,100.0	18.8 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-1.48 (-7.87,4.90)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.75 (17.841)	0.49 (17.313)			-0.07 (-0.461, 0.320)
	95% Confidence Interval of Mean	-5.82 , 4.32	-4.38 , 5.36			
	Median	0.00	0.00			
	Q1, Q3	-12.5 ,12.50	-12.5 ,12.50			
	Min, Max	-43.8 , 56.3	-43.8 , 43.8			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-1.24 (-8.18,5.70)				
	LS Mean (SE)	-0.8 (2.61)	0.5 (2.58)	-1.35 (3.669) [-8.594, 5.884]	0.7124	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s1 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Positive Emotions Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	55.81 (25.348)	59.62 (24.406)			
	95% Confidence Interval of Mean	48.61 ,63.02	52.76 ,66.48			
	Median	50.00	59.38			
	Q1, Q3	37.50 ,75.00	50.00 ,75.00			
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-3.81 (-13.63,6.02)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	53.88 (21.350)	56.74 (25.643)			
	95% Confidence Interval of Mean	47.81 ,59.94	49.53 ,63.95			
	Median	50.00	50.00			
	Q1, Q3	37.50 ,62.50	43.75 ,75.00			
	Min, Max	6.3 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-2.87 (-12.19,6.46)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.94 (18.632)	-2.88 (14.589)			0.06 (-0.334, 0.447)
	95% Confidence Interval of Mean	-7.23 , 3.36	-6.98 , 1.22			
	Median	0.00	0.00			
	Q1, Q3	-12.5 ,12.50	-12.5 , 6.25			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-50.0 , 37.5	-43.8 , 25.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.94 (-5.66,7.54)				
	LS Mean (SE)	-2.8 (2.26)	-2.1 (2.24)	-0.71 (3.188) [-7.002, 5.579]	0.8237	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	58.25 (15.944)	59.19 (23.794)			
	95% Confidence Interval of Mean	53.72 ,62.78	52.50 ,65.88			
	Median	56.25	56.25			
	Q1, Q3	50.00 ,68.75	43.75 ,75.00			
	Min, Max	31.3 ,100.0	6.3 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.94 (-8.95,7.07)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	2.44 (23.935)	-0.43 (18.729)			0.13 (-0.257, 0.524)
	95% Confidence Interval of Mean	-4.36 , 9.24	-5.70 , 4.84			
	Median	0.00	0.00			
	Q1, Q3	-12.5 ,18.75	-12.5 , 6.25			
	Min, Max	-56.3 , 43.8	-37.5 , 56.3			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.87 (-5.61,11.34)				
	LS Mean (SE)	1.6 (2.26)	0.4 (2.24)	1.21 (3.188) [-5.078, 7.503]	0.7041	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	55.75 (17.716)	57.48 (21.361)			
	95% Confidence Interval of Mean	50.72 ,60.78	51.47 ,63.48			
	Median	56.25	56.25			
	Q1, Q3	43.75 ,62.50	43.75 ,68.75			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	25.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-1.73 (-9.48,6.03)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.06 (20.830)	-2.14 (17.617)			0.11 (-0.282, 0.498)
	95% Confidence Interval of Mean	-5.98 , 5.86	-7.10 , 2.81			
	Median	0.00	0.00			
	Q1, Q3	-12.5 ,18.75	-12.5 , 6.25			
	Min, Max	-43.8 , 50.0	-43.8 , 56.3			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.08 (-5.53,9.69)				
	LS Mean (SE)	-0.9 (2.26)	-1.3 (2.24)	0.43 (3.188) [-5.862, 6.718]	0.8933	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s2 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Positive Emotions Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	54.75 (26.292)	58.58 (25.525)			
	95% Confidence Interval of Mean	47.28 ,62.22	51.40 ,65.76			
	Median	50.00	56.25			
	Q1, Q3	37.50 ,75.00	43.75 ,75.00			
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-3.83 (-14.06,6.40)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	51.38 (23.429)	54.90 (27.166)			
	95% Confidence Interval of Mean	44.72 ,58.03	47.26 ,62.54			
	Median	50.00	50.00			
	Q1, Q3	37.50 ,62.50	37.50 ,75.00			
	Min, Max	6.3 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-3.53 (-13.55,6.50)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-3.38 (20.013)	-3.68 (19.169)			0.02 (-0.375, 0.405)
	95% Confidence Interval of Mean	-9.06 , 2.31	-9.07 , 1.71			
	Median	0.00	0.00			
	Q1, Q3	-12.5 ,12.50	-12.5 , 6.25			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-68.8 , 37.5	-75.0 , 37.5			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.30 (-7.44,8.04)				
	LS Mean (SE)	-4.3 (2.78)	-2.8 (2.75)	-1.49 (3.920) [-9.228, 6.253]	0.7048	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	53.75 (20.594)	56.99 (25.668)			
	95% Confidence Interval of Mean	47.90 ,59.60	49.77 ,64.20			
	Median	50.00	56.25			
	Q1, Q3	37.50 ,68.75	37.50 ,75.00			
	Min, Max	18.8 ,100.0	6.3 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-3.24 (-12.43,5.96)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.00 (26.557)	-1.59 (22.839)			0.02 (-0.366, 0.414)
	95% Confidence Interval of Mean	-8.55 , 6.55	-8.02 , 4.83			
	Median	0.00	0.00			
	Q1, Q3	-18.8 ,12.50	-12.5 , 6.25			
	Min, Max	-68.8 , 43.8	-75.0 , 56.3			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.59 (-9.18,10.37)				
	LS Mean (SE)	-1.9 (2.78)	-0.7 (2.75)	-1.20 (3.920) [-8.936, 6.545]	0.7607	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	49.75 (22.374)	54.53 (23.818)			
	95% Confidence Interval of Mean	43.39 ,56.11	47.84 ,61.23			
	Median	50.00	56.25			
	Q1, Q3	31.25 ,62.50	37.50 ,68.75			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	18.8 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-4.78 (-13.91,4.34)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-5.00 (26.426)	-4.04 (22.144)			-0.04 (-0.429, 0.351)
	95% Confidence Interval of Mean	-12.5 , 2.51	-10.3 , 2.18			
	Median	0.00	0.00			
	Q1, Q3	-18.8 ,12.50	-18.8 ,12.50			
	Min, Max	-81.3 , 50.0	-75.0 , 56.3			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.96 (-10.57,8.66)				
	LS Mean (SE)	-5.9 (2.78)	-3.2 (2.75)	-2.74 (3.920) [-10.485, 4.996]	0.4848	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s1 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Negative Emotions Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	79.57 (14.575)	77.94 (15.903)			
	95% Confidence Interval of Mean	75.43 ,83.71	73.47 ,82.41			
	Median	78.57	82.14			
	Q1, Q3	75.00 ,89.29	67.86 ,89.29			
	Min, Max	17.9 ,100.0	28.6 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.63 (-4.40,7.66)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	81.57 (13.075)	82.56 (14.475)			
	95% Confidence Interval of Mean	77.86 ,85.29	78.49 ,86.63			
	Median	83.93	85.71			
	Q1, Q3	75.00 ,89.29	75.00 ,92.86			
	Min, Max	32.1 ,100.0	32.1 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.99 (-6.44,4.46)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	2.00 (11.389)	4.62 (13.483)			-0.21 (-0.601, 0.181)
	95% Confidence Interval of Mean	-1.24 , 5.24	0.83 , 8.41			
	Median	3.57	3.57			
	Q1, Q3	-3.57 ,10.71	-3.57 ,10.71			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-28.6 , 25.0	-25.0 , 35.7			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-2.62 (-7.55,2.31)				
	LS Mean (SE)	2.4 (1.44)	4.2 (1.42)	-1.87 (2.025) [-5.861, 2.120]	0.3567	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	85.29 (9.989)	83.19 (13.176)			
	95% Confidence Interval of Mean	82.45 ,88.12	79.49 ,86.90			
	Median	85.71	85.71			
	Q1, Q3	78.57 ,92.86	78.57 ,92.86			
	Min, Max	60.7 ,100.0	39.3 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.09 (-2.53,6.72)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	5.71 (10.653)	5.25 (12.522)			0.04 (-0.350, 0.430)
	95% Confidence Interval of Mean	2.69 , 8.74	1.73 , 8.77			
	Median	3.57	3.57			
	Q1, Q3	0.00 ,10.71	-3.57 ,10.71			
	Min, Max	-17.9 , 42.9	-17.9 , 42.9			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.46 (-4.13,5.06)				
	LS Mean (SE)	6.1 (1.44)	4.9 (1.42)	1.21 (2.025) [-2.776, 5.204]	0.5496	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	84.21 (11.296)	83.12 (15.454)			
	95% Confidence Interval of Mean	81.00 ,87.42	78.78 ,87.47			
	Median	85.71	85.71			
	Q1, Q3	78.57 ,92.86	78.57 ,92.86			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	50.0 ,100.0	32.1 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.09 (-4.26,6.44)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	4.64 (11.949)	5.18 (13.690)			-0.04 (-0.432, 0.348)
	95% Confidence Interval of Mean	1.25 , 8.04	1.33 , 9.03			
	Median	3.57	3.57			
	Q1, Q3	0.00 ,10.71	-3.57 ,14.29			
	Min, Max	-28.6 , 32.1	-28.6 , 35.7			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.54 (-5.62,4.54)				
	LS Mean (SE)	5.0 (1.44)	4.8 (1.42)	0.21 (2.025) [-3.778, 4.202]	0.9166	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s2 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Negative Emotions Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	78.93 (15.157)	77.31 (16.364)			
	95% Confidence Interval of Mean	74.62 ,83.24	72.71 ,81.91			
	Median	78.57	82.14			
	Q1, Q3	75.00 ,89.29	67.86 ,89.29			
	Min, Max	17.9 ,100.0	28.6 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.62 (-4.61,7.85)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	78.71 (15.575)	79.76 (16.898)			
	95% Confidence Interval of Mean	74.29 ,83.14	75.01 ,84.51			
	Median	82.14	85.71			
	Q1, Q3	71.43 ,89.29	71.43 ,92.86			
	Min, Max	32.1 ,100.0	32.1 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-1.05 (-7.47,5.37)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.21 (13.999)	2.45 (16.296)			-0.18 (-0.566, 0.216)
	95% Confidence Interval of Mean	-4.19 , 3.76	-2.13 , 7.03			
	Median	1.79	3.57			
	Q1, Q3	-7.14 ,10.71	-3.57 ,10.71			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-39.3 , 25.0	-50.0 , 35.7			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-2.67 (-8.67,3.34)				
	LS Mean (SE)	0.2 (1.98)	2.1 (1.96)	-1.94 (2.781) [-7.426, 3.544]	0.4861	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	81.43 (14.413)	81.30 (14.908)			
	95% Confidence Interval of Mean	77.33 ,85.52	77.11 ,85.50			
	Median	82.14	85.71			
	Q1, Q3	75.00 ,92.86	75.00 ,92.86			
	Min, Max	53.6 ,100.0	39.3 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.13 (-5.66,5.92)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	2.50 (14.435)	3.99 (13.844)			-0.11 (-0.496, 0.285)
	95% Confidence Interval of Mean	-1.60 , 6.60	0.10 , 7.89			
	Median	3.57	3.57			
	Q1, Q3	0.00 , 7.14	-3.57 , 7.14			
	Min, Max	-35.7 , 42.9	-35.7 , 42.9			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-1.49 (-7.08,4.09)				
	LS Mean (SE)	2.9 (1.98)	3.6 (1.96)	-0.77 (2.781) [-6.252, 4.717]	0.7829	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	78.71 (17.331)	79.27 (18.912)			
	95% Confidence Interval of Mean	73.79 ,83.64	73.95 ,84.59			
	Median	83.93	85.71			
	Q1, Q3	71.43 ,92.86	71.43 ,92.86			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	46.4 ,100.0	32.1 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.56 (-7.72,6.61)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.21 (17.050)	1.96 (17.559)			-0.13 (-0.516, 0.265)
	95% Confidence Interval of Mean	-5.06 , 4.63	-2.98 , 6.90			
	Median	0.00	3.57			
	Q1, Q3	-7.14 ,10.71	-7.14 ,10.71			
	Min, Max	-42.9 , 32.1	-50.0 , 35.7			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-2.18 (-9.01,4.66)				
	LS Mean (SE)	0.2 (1.98)	1.6 (1.96)	-1.45 (2.781) [-6.935, 4.034]	0.6025	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s1 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Social Interaction Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	43.00 (26.599)	42.16 (26.419)			
	95% Confidence Interval of Mean	35.44 ,50.56	34.73 ,49.59			
	Median	42.86	42.86			
	Q1, Q3	17.86 ,64.29	25.00 ,60.71			
	Min, Max	0.0 ,100.0	0.0 , 96.4			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.84 (-9.62,11.31)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	46.29 (20.686)	43.14 (27.216)			
	95% Confidence Interval of Mean	40.41 ,52.16	35.48 ,50.79			
	Median	46.43	46.43			
	Q1, Q3	32.14 ,60.71	17.86 ,64.29			
	Min, Max	0.0 ,100.0	0.0 , 96.4			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	3.15 (-6.41,12.71)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	3.29 (19.102)	0.98 (13.999)			0.14 (-0.253, 0.528)
	95% Confidence Interval of Mean	-2.14 , 8.71	-2.96 , 4.92			
	Median	0.00	0.00			
	Q1, Q3	-10.7 ,14.29	-7.14 ,10.71			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-28.6 , 67.9	-25.0 , 35.7			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.31 (-4.30,8.91)				
	LS Mean (SE)	3.4 (2.42)	0.8 (2.40)	2.62 (3.406) [-4.099, 9.343]	0.4425	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	49.29 (19.455)	44.26 (26.284)			
	95% Confidence Interval of Mean	43.76 ,54.81	36.87 ,51.65			
	Median	46.43	42.86			
	Q1, Q3	42.86 ,57.14	25.00 ,60.71			
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	5.03 (-4.12,14.17)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	6.29 (24.538)	2.10 (18.422)			0.19 (-0.198, 0.584)
	95% Confidence Interval of Mean	-0.69 ,13.26	-3.08 , 7.28			
	Median	3.57	0.00			
	Q1, Q3	-10.7 ,21.43	-10.7 ,10.71			
	Min, Max	-42.9 , 78.6	-35.7 , 71.4			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	4.18 (-4.37,12.74)				
	LS Mean (SE)	6.4 (2.42)	1.9 (2.40)	4.50 (3.406) [-2.220, 11.223]	0.1880	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	46.79 (22.463)	42.51 (25.277)			
	95% Confidence Interval of Mean	40.40 ,53.17	35.40 ,49.62			
	Median	42.86	42.86			
	Q1, Q3	35.71 ,64.29	25.00 ,53.57			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	4.28 (-5.17,13.73)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	3.79 (22.512)	0.35 (18.340)			0.17 (-0.223, 0.558)
	95% Confidence Interval of Mean	-2.61 ,10.18	-4.81 , 5.51			
	Median	0.00	0.00			
	Q1, Q3	-10.7 ,14.29	-14.3 ,10.71			
	Min, Max	-57.1 , 78.6	-28.6 , 67.9			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	3.44 (-4.66,11.54)				
	LS Mean (SE)	3.9 (2.42)	0.2 (2.40)	3.75 (3.406) [-2.969, 10.473]	0.2721	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s2 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Social Interaction Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	42.14 (27.285)	40.48 (27.680)			
	95% Confidence Interval of Mean	34.39 ,49.90	32.69 ,48.26			
	Median	42.86	42.86			
	Q1, Q3	17.86 ,64.29	14.29 ,60.71			
	Min, Max	0.0 ,100.0	0.0 , 96.4			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.67 (-9.19,12.52)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	42.57 (24.264)	40.41 (29.054)			
	95% Confidence Interval of Mean	35.68 ,49.47	32.23 ,48.58			
	Median	42.86	46.43			
	Q1, Q3	32.14 ,60.71	17.86 ,64.29			
	Min, Max	0.0 ,100.0	0.0 , 96.4			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.17 (-8.41,12.74)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.43 (20.147)	-0.07 (19.891)			0.02 (-0.365, 0.415)
	95% Confidence Interval of Mean	-5.30 , 6.15	-5.66 , 5.52			
	Median	0.00	0.00			
	Q1, Q3	-14.3 ,14.29	-7.14 ,10.71			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-60.7 , 67.9	-60.7 , 50.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.50 (-7.41,8.40)				
	LS Mean (SE)	0.8 (3.12)	-0.4 (3.09)	1.18 (4.391) [-7.493, 9.843]	0.7893	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	44.14 (25.375)	40.90 (28.911)			
	95% Confidence Interval of Mean	36.93 ,51.35	32.76 ,49.03			
	Median	46.43	39.29			
	Q1, Q3	28.57 ,57.14	17.86 ,60.71			
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	3.25 (-7.50,13.99)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	2.00 (27.853)	0.42 (24.884)			0.06 (-0.330, 0.450)
	95% Confidence Interval of Mean	-5.92 , 9.92	-6.58 , 7.42			
	Median	0.00	0.00			
	Q1, Q3	-14.3 ,17.86	-10.7 ,14.29			
	Min, Max	-71.4 , 78.6	-60.7 , 71.4			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.58 (-8.84,12.00)				
	LS Mean (SE)	2.3 (3.12)	0.1 (3.09)	2.26 (4.391) [-6.411, 10.924]	0.6080	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	40.79 (27.899)	38.31 (28.312)			
	95% Confidence Interval of Mean	32.86 ,48.71	30.34 ,46.27			
	Median	42.86	39.29			
	Q1, Q3	14.29 ,64.29	17.86 ,53.57			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.48 (-8.62,13.58)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.36 (27.851)	-2.17 (25.972)			0.03 (-0.360, 0.420)
	95% Confidence Interval of Mean	-9.27 , 6.56	-9.48 , 5.13			
	Median	0.00	0.00			
	Q1, Q3	-10.7 ,10.71	-17.9 ,10.71			
	Min, Max	-100 , 78.6	-64.3 , 67.9			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.81 (-9.82,11.44)				
	LS Mean (SE)	-1.0 (3.12)	-2.5 (3.09)	1.49 (4.391) [-7.178, 10.158]	0.7348	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s1 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Leisure and the Outdoors Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	49.60 (26.989)	48.24 (30.196)			
	95% Confidence Interval of Mean	41.93 ,57.27	39.74 ,56.73			
	Median	50.00	50.00			
	Q1, Q3	30.00 ,70.00	25.00 ,70.00			
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.36 (-9.95,12.68)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	50.00 (21.237)	46.67 (32.104)			
	95% Confidence Interval of Mean	43.96 ,56.04	37.64 ,55.70			
	Median	50.00	45.00			
	Q1, Q3	35.00 ,65.00	10.00 ,70.00			
	Min, Max	0.0 , 90.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	3.33 (-7.44,14.10)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.40 (19.082)	-1.57 (19.659)			0.10 (-0.289, 0.492)
	95% Confidence Interval of Mean	-5.02 , 5.82	-7.10 , 3.96			
	Median	0.00	0.00			
	Q1, Q3	-15.0 ,15.00	-15.0 ,10.00			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-40.0 , 45.0	-50.0 , 65.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.97 (-5.68,9.62)				
	LS Mean (SE)	0.6 (2.78)	-1.8 (2.75)	2.46 (3.910) [-5.254, 10.181]	0.5295	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	54.30 (24.703)	52.84 (30.761)			
	95% Confidence Interval of Mean	47.28 ,61.32	44.19 ,61.49			
	Median	55.00	55.00			
	Q1, Q3	35.00 ,70.00	20.00 ,75.00			
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.46 (-9.57,12.49)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	4.70 (25.222)	4.61 (23.724)			0.00 (-0.386, 0.394)
	95% Confidence Interval of Mean	-2.47 ,11.87	-2.06 ,11.28			
	Median	5.00	5.00			
	Q1, Q3	-10.0 ,25.00	-10.0 ,15.00			
	Min, Max	-65.0 , 50.0	-40.0 , 75.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.09 (-9.57,9.76)				
	LS Mean (SE)	4.9 (2.78)	4.4 (2.75)	0.59 (3.910) [-7.130, 8.304]	0.8809	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	52.30 (21.529)	47.45 (28.042)			
	95% Confidence Interval of Mean	46.18 ,58.42	39.56 ,55.34			
	Median	50.00	50.00			
	Q1, Q3	40.00 ,60.00	30.00 ,70.00			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	4.85 (-5.04,14.73)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	2.70 (22.728)	-0.78 (21.917)			0.16 (-0.235, 0.547)
	95% Confidence Interval of Mean	-3.76 , 9.16	-6.95 , 5.38			
	Median	0.00	-5.00			
	Q1, Q3	-15.0 ,15.00	-15.0 , 5.00			
	Min, Max	-50.0 , 55.0	-40.0 , 75.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	3.48 (-5.33,12.30)				
	LS Mean (SE)	2.9 (2.78)	-1.0 (2.75)	3.98 (3.910) [-3.738, 11.696]	0.3103	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s2 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Leisure and the Outdoors Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	48.60 (27.885)	46.27 (31.636)			
	95% Confidence Interval of Mean	40.68 ,56.52	37.38 ,55.17			
	Median	50.00	50.00			
	Q1, Q3	25.00 ,70.00	20.00 ,70.00			
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.33 (-9.46,14.11)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	46.40 (25.295)	44.02 (33.971)			
	95% Confidence Interval of Mean	39.21 ,53.59	34.47 ,53.57			
	Median	50.00	45.00			
	Q1, Q3	35.00 ,65.00	10.00 ,70.00			
	Min, Max	0.0 , 90.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.38 (-9.46,14.22)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-2.20 (20.902)	-2.25 (24.785)			0.00 (-0.388, 0.392)
	95% Confidence Interval of Mean	-8.14 , 3.74	-9.23 , 4.72			
	Median	0.00	0.00			
	Q1, Q3	-15.0 ,10.00	-15.0 ,10.00			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-65.0 , 45.0	-80.0 , 65.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.05 (-9.01,9.12)				
	LS Mean (SE)	-1.7 (3.46)	-2.7 (3.42)	0.95 (4.866) [-8.657, 10.557]	0.8454	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	48.30 (29.513)	48.92 (33.366)			
	95% Confidence Interval of Mean	39.91 ,56.69	39.54 ,58.31			
	Median	50.00	50.00			
	Q1, Q3	25.00 ,70.00	10.00 ,75.00			
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.62 (-13.07,11.82)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.30 (28.310)	2.65 (29.109)			-0.10 (-0.493, 0.288)
	95% Confidence Interval of Mean	-8.35 , 7.75	-5.54 ,10.83			
	Median	5.00	5.00			
	Q1, Q3	-15.0 ,20.00	-15.0 ,20.00			
	Min, Max	-70.0 , 50.0	-75.0 , 75.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-2.95 (-14.29,8.39)				
	LS Mean (SE)	0.2 (3.46)	2.2 (3.42)	-2.05 (4.866) [-11.659, 7.555]	0.6738	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	45.30 (28.346)	42.55 (31.407)			
	95% Confidence Interval of Mean	37.24 ,53.36	33.72 ,51.38			
	Median	50.00	45.00			
	Q1, Q3	25.00 ,60.00	10.00 ,70.00			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 ,100.0	0.0 ,100.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.75 (-9.07,14.57)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-3.30 (30.010)	-3.73 (27.328)			0.01 (-0.375, 0.405)
	95% Confidence Interval of Mean	-11.8 , 5.23	-11.4 , 3.96			
	Median	0.00	-5.00			
	Q1, Q3	-20.0 ,10.00	-15.0 , 5.00			
	Min, Max	-100 , 55.0	-80.0 , 75.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.43 (-10.90,11.75)				
	LS Mean (SE)	-2.8 (3.46)	-4.2 (3.42)	1.32 (4.866) [-8.286, 10.928]	0.7864	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s1 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Independence Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	23.10 (20.724)	26.96 (21.191)			
	95% Confidence Interval of Mean	17.21 ,28.99	21.00 ,32.92			
	Median	17.50	25.00			
	Q1, Q3	5.00 ,30.00	10.00 ,40.00			
	Min, Max	0.0 , 90.0	0.0 , 75.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-3.86 (-12.14,4.42)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	23.55 (15.576)	26.81 (21.448)			
	95% Confidence Interval of Mean	19.12 ,27.98	20.78 ,32.85			
	Median	21.25	22.50			
	Q1, Q3	15.00 ,30.00	10.00 ,45.00			
	Min, Max	0.0 , 60.0	0.0 , 85.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-3.26 (-10.68,4.15)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.45 (16.184)	-0.15 (12.424)			0.04 (-0.349, 0.432)
	95% Confidence Interval of Mean	-4.15 , 5.05	-3.64 , 3.35			
	Median	0.00	0.00			
	Q1, Q3	-10.0 ,10.00	-5.00 , 5.00			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-40.0 , 40.0	-40.0 , 25.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.60 (-5.09,6.29)				
	LS Mean (SE)	-0.3 (2.27)	0.6 (2.25)	-0.88 (3.205) [-7.203, 5.444]	0.7840	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	31.40 (22.791)	29.02 (20.929)			
	95% Confidence Interval of Mean	24.92 ,37.88	23.13 ,34.91			
	Median	25.00	25.00			
	Q1, Q3	20.00 ,45.00	15.00 ,45.00			
	Min, Max	0.0 ,100.0	0.0 , 85.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.38 (-6.26,11.02)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	8.30 (21.395)	2.06 (15.139)			0.34 (-0.056, 0.730)
	95% Confidence Interval of Mean	2.22 ,14.38	-2.20 , 6.32			
	Median	10.00	5.00			
	Q1, Q3	-5.00 ,25.00	-10.0 ,10.00			
	Min, Max	-35.0 , 85.0	-50.0 , 30.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	6.24 (-1.07,13.55)				
	LS Mean (SE)	7.6 (2.27)	2.8 (2.25)	4.76 (3.205) [-1.559, 11.088]	0.1388	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	27.50 (20.806)	26.57 (21.130)			
	95% Confidence Interval of Mean	21.59 ,33.41	20.63 ,32.51			
	Median	20.00	20.00			
	Q1, Q3	10.00 ,35.00	10.00 ,45.00			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 , 85.0	0.0 , 85.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.93 (-7.35,9.21)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	4.40 (23.747)	-0.39 (16.151)			0.24 (-0.155, 0.628)
	95% Confidence Interval of Mean	-2.35 ,11.15	-4.93 , 4.15			
	Median	5.00	0.00			
	Q1, Q3	-10.0 ,20.00	-10.0 , 5.00			
	Min, Max	-55.0 , 65.0	-45.0 , 45.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	4.79 (-3.21,12.80)				
	LS Mean (SE)	3.7 (2.27)	0.3 (2.25)	3.32 (3.205) [-3.008, 9.639]	0.3023	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s2 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Independence Average

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	23.10 (20.724)	26.47 (21.524)			
	95% Confidence Interval of Mean	17.21 ,28.99	20.42 ,32.52			
	Median	17.50	25.00			
	Q1, Q3	5.00 ,30.00	10.00 ,40.00			
	Min, Max	0.0 , 90.0	0.0 , 75.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-3.37 (-11.72,4.97)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	22.20 (16.573)	25.49 (22.366)			
	95% Confidence Interval of Mean	17.49 ,26.91	19.20 ,31.78			
	Median	20.00	20.00			
	Q1, Q3	10.00 ,30.00	5.00 ,45.00			
	Min, Max	0.0 , 60.0	0.0 , 85.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-3.29 (-11.07,4.49)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-0.90 (16.058)	-0.98 (14.107)			0.01 (-0.385, 0.395)
	95% Confidence Interval of Mean	-5.46 , 3.66	-4.95 , 2.99			
	Median	0.00	0.00			
	Q1, Q3	-10.0 ,10.00	-5.00 , 5.00			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-40.0 , 40.0	-40.0 , 30.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.08 (-5.88,6.04)				
	LS Mean (SE)	-1.6 (2.57)	-0.3 (2.54)	-1.23 (3.617) [-8.364, 5.913]	0.7352	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	28.40 (25.019)	26.08 (22.985)			
	95% Confidence Interval of Mean	21.29 ,35.51	19.61 ,32.54			
	Median	25.00	20.00			
	Q1, Q3	10.00 ,45.00	5.00 ,45.00			
	Min, Max	0.0 ,100.0	0.0 , 85.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.32 (-7.16,11.80)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	5.30 (22.911)	-0.39 (20.146)			0.26 (-0.128, 0.656)
	95% Confidence Interval of Mean	-1.21 ,11.81	-6.06 , 5.27			
	Median	5.00	0.00			
	Q1, Q3	-10.0 ,20.00	-10.0 ,10.00			
	Min, Max	-60.0 , 85.0	-75.0 , 30.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	5.69 (-2.82,14.21)				
	LS Mean (SE)	4.6 (2.57)	0.3 (2.54)	4.39 (3.617) [-2.752, 11.525]	0.2269	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	24.70 (22.911)	24.61 (22.558)			
	95% Confidence Interval of Mean	18.19 ,31.21	18.26 ,30.95			
	Median	20.00	15.00			
	Q1, Q3	5.00 ,35.00	5.00 ,45.00			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	0.0 , 85.0	0.0 , 85.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.09 (-8.88,9.07)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	1.60 (25.663)	-1.86 (17.887)			0.16 (-0.234, 0.547)
	95% Confidence Interval of Mean	-5.69 , 8.89	-6.89 , 3.17			
	Median	0.00	0.00			
	Q1, Q3	-15.0 ,15.00	-10.0 , 5.00			
	Min, Max	-75.0 , 65.0	-50.0 , 45.0			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	3.46 (-5.26,12.18)				
	LS Mean (SE)	0.9 (2.57)	-1.2 (2.54)	2.16 (3.617) [-4.982, 9.295]	0.5518	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s1 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 1: Imputation of Median Placebo Group Counts

Average of All QI-Disability Items

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	53.05 (15.221)	53.35 (15.407)			
	95% Confidence Interval of Mean	48.72 ,57.37	49.02 ,57.69			
	Median	51.56	53.91			
	Q1, Q3	40.63 ,60.94	43.75 ,63.28			
	Min, Max	29.7 , 91.4	15.6 , 89.1			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.31 (-6.36,5.74)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	53.92 (11.906)	54.61 (16.685)			
	95% Confidence Interval of Mean	50.54 ,57.31	49.92 ,59.30			
	Median	54.69	56.25			
	Q1, Q3	46.88 ,60.16	39.06 ,66.41			
	Min, Max	26.6 , 82.8	21.1 , 89.1			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.69 (-6.42,5.04)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	0.88 (10.095)	1.26 (8.687)			-0.04 (-0.431, 0.350)
	95% Confidence Interval of Mean	-1.99 , 3.74	-1.19 , 3.70			
	Median	1.17	2.34			
	Q1, Q3	-5.47 , 5.47	-4.69 , 7.81			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-21.9 , 22.7	-16.4 , 24.2			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.38 (-4.10,3.33)				
	LS Mean (SE)	0.8 (1.42)	1.3 (1.41)	-0.49 (2.000) [-4.433, 3.459]	0.8081	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	57.64 (12.803)	55.64 (15.169)			
	95% Confidence Interval of Mean	54.00 ,61.28	51.37 ,59.90			
	Median	55.47	54.69			
	Q1, Q3	51.56 ,61.72	46.88 ,66.41			
	Min, Max	32.8 , 97.7	25.0 , 90.6			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.00 (-3.54,7.55)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	4.59 (13.174)	2.28 (11.107)			0.19 (-0.201, 0.581)
	95% Confidence Interval of Mean	0.85 , 8.34	-0.84 , 5.41			
	Median	3.52	2.34			
	Q1, Q3	-3.13 ,13.28	-3.91 , 6.25			
	Min, Max	-27.3 , 42.2	-21.9 , 38.3			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.31 (-2.50,7.12)				
	LS Mean (SE)	4.5 (1.42)	2.3 (1.41)	2.21 (2.000) [-1.740, 6.152]	0.2715	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	55.92 (12.596)	54.27 (15.314)			
	95% Confidence Interval of Mean	52.34 ,59.50	49.97 ,58.58			
	Median	53.91	53.91			
	Q1, Q3	48.44 ,60.94	43.75 ,62.50			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	32.0 , 94.5	21.9 , 90.6			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.65 (-3.89,7.19)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	2.88 (12.982)	0.92 (11.187)			0.16 (-0.229, 0.552)
	95% Confidence Interval of Mean	-0.81 , 6.56	-2.23 , 4.07			
	Median	3.13	-0.78			
	Q1, Q3	-2.34 , 8.59	-5.47 , 7.03			
	Min, Max	-34.4 , 39.1	-25.8 , 43.8			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	1.96 (-2.83,6.74)				
	LS Mean (SE)	2.8 (1.42)	1.0 (1.41)	1.85 (2.000) [-2.096, 5.796]	0.3561	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

Study No. 1042-CDD-3001
Table 14.2.6.5.s2 Summary of Quality of Life Inventory – Disability
Sensitivity Analysis 2: Imputation of Median of 5 Highest Placebo Group Counts

Average of All QI-Disability Items

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
Visit 2/Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	52.50 (15.674)	52.28 (16.275)			
	95% Confidence Interval of Mean	48.05 ,56.95	47.70 ,56.86			
	Median	51.17	53.91			
	Q1, Q3	39.84 ,60.94	39.84 ,63.28			
	Min, Max	26.6 , 91.4	15.6 , 89.1			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.22 (-6.09,6.53)				
Visit 3 (End of Week 5)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	51.48 (14.139)	52.22 (18.407)			
	95% Confidence Interval of Mean	47.47 ,55.50	47.04 ,57.40			
	Median	53.13	53.91			
	Q1, Q3	42.97 ,60.16	35.94 ,66.41			
	Min, Max	25.8 , 82.8	21.1 , 89.1			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.74 (-7.23,5.75)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.02 (10.616)	-0.06 (12.125)			-0.08 (-0.474, 0.307)
	95% Confidence Interval of Mean	-4.03 , 2.00	-3.47 , 3.35			
	Median	-0.78	1.56			
	Q1, Q3	-7.81 , 5.47	-5.47 , 7.81			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	-37.5 , 21.1	-39.8 , 26.6			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	-0.95 (-5.46,3.55)				
	LS Mean (SE)	-1.0 (1.96)	-0.1 (1.94)	-0.87 (2.757) [-6.309, 4.568]	0.7527	
Visit 4 (End of Week 9)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	54.55 (15.943)	52.10 (17.811)			
	95% Confidence Interval of Mean	50.02 ,59.08	47.09 ,57.11			
	Median	55.47	52.34			
	Q1, Q3	44.53 ,61.72	37.50 ,66.41			
	Min, Max	28.9 , 97.7	25.0 , 90.6			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.45 (-4.23,9.13)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	2.05 (15.246)	-0.18 (16.979)			0.14 (-0.252, 0.529)
	95% Confidence Interval of Mean	-2.29 , 6.38	-4.96 , 4.59			
	Median	2.34	2.34			
	Q1, Q3	-4.69 ,10.94	-6.25 , 8.59			
	Min, Max	-44.5 , 42.2	-47.7 , 38.3			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	2.23 (-4.14,8.61)				
	LS Mean (SE)	2.1 (1.96)	-0.2 (1.94)	2.31 (2.757) [-3.124, 7.753]	0.4022	
Visit 5 (End of Week 17)	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	n/M*, (%)	50 / 50 (100.0)	51 / 51 (100.0)			
	Mean (SD)	51.42 (16.902)	50.97 (17.910)			
	95% Confidence Interval of Mean	46.62 ,56.23	45.93 ,56.00			
	Median	53.91	53.13			
	Q1, Q3	38.28 ,60.94	38.28 ,62.50			

				Differeces of LSM		
Visit	**Category**	**Ganaxolone N=50**	**Placebo N=51**	**Mean (SE) [95% CI]**	**p-value**	**Hedges' g (95% CI)**
	Min, Max	25.8 , 94.5	21.9 , 90.6			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.46 (-6.42,7.34)				
Change from Baseline	n	50	51			
	Missing (%)	0 (0.0)	0 (0.0)			
	Mean (SD)	-1.08 (18.168)	-1.32 (16.204)			0.01 (-0.376, 0.404)
	95% Confidence Interval of Mean	-6.24 , 4.09	-5.87 , 3.24			
	Median	0.39	-0.78			
	Q1, Q3	-5.47 , 7.03	-8.59 , 7.81			
	Min, Max	-62.5 , 39.1	-39.8 , 43.8			
	Ganaxolone Minus Placebo, Mean (95% Confidence Interval)	0.24 (-6.55,7.03)				
	LS Mean (SE)	-1.0 (1.96)	-1.4 (1.94)	0.32 (2.757) [-5.115, 5.762]	0.9067	

**** Number of completed questionnaires (n) / Number of patients still remaining in the study (M)***

4.7 Subgruppenanalysen

4.7.1 Ergebnisse durchgeführter Subgruppenanalysen mit einem Interaktions-p-Wert ≥ 0,05

Study No. 1042-CDD-3001

Table 14.2.2.1 Summary of 28-Day Seizure Frequency for Primary Seizure Types - Subjects who are 2-6 years old

28-day seizure frequency (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=30	Baseline	29	1 (3.3)	141.84 (165.122)	95.61 (37.74, 173.33)	31.33, 212.67	5.5, 651.3			
	17-Week Post-Baseline Phase	30	0 (0.0)	118.00 (161.217)	52.28 (37.52, 106.26)	31.83, 132.75	0.5, 648.1			
	Arithmetic Change from Baseline	29	1 (3.3)	-21.45 (147.789)	-10.56 (-47.44, 0.97)	-75.34, 7.58	-501.8, 428.1			
	Percent Change from Baseline	29	1 (3.3)	-3.31 (78.635)	-30.66 (-35.94, 2.56)	-60.91, 16.66	-91.0, 215.4			
Placebo, N=22	Baseline	22	0 (0.0)	87.90 (147.747)	39.62 (18.67, 77.33)	16.67, 77.33	8.7, 681.3	22.46 (-4.56, 98.00)	0.0852	-1.7212
	17-Week Post-Baseline Phase	22	0 (0.0)	207.88 (706.172)	46.50 (29.73, 82.55)	24.43, 82.55	1.3, 3362.1	10.37 (-17.43, 42.03)	0.4758	-0.7131
	Arithmetic Change from Baseline	22	0 (0.0)	119.98 (573.795)	-3.41 (-7.77, 22.06)	-11.23, 22.06	-115.9, 2680.7	-20.89 (-62.08, 3.83)	0.1166	1.5690
	Percent Change from Baseline	22	0 (0.0)	57.05 (156.431)	-8.97 (-28.73, 59.29)	-33.68, 59.29	-89.7, 540.4	-26.99 (-63.15, 4.35)	0.0961	1.6641

Study No. 1042-CDD-3001

Table 14.2.2.1 Summary of 28-Day Seizure Frequency for Primary Seizure Types - Subjects who are >6 years old

28-day seizure frequency (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=20	Baseline	20	0 (0.0)	76.96 (74.628)	52.28 (32.67, 93.33)	29.00, 96.33	11.3, 302.7			
	17-Week Post-Baseline Phase	20	0 (0.0)	57.23 (64.972)	29.75 (16.43, 76.03)	15.91, 82.02	2.4, 258.5			
	Arithmetic Change from Baseline	20	0 (0.0)	-19.74 (33.546)	-9.08 (-28.61, -3.89)	-29.69, -3.06	-123.8, 32.5			
	Percent Change from Baseline	20	0 (0.0)	-29.47 (31.311)	-30.09 (-43.46, -9.90)	-46.48, -7.80	-96.2, 27.7			
Placebo, N=29	Baseline	29	0 (0.0)	116.11 (191.591)	50.00 (43.45, 120.00)	25.33, 142.67	0.7, 1021.3	-1.58 (-35.10, 25.33)	0.9190	-0.1017
	17-Week Post-Baseline Phase	29	0 (0.0)	107.84 (123.081)	65.17 (25.61, 141.27)	19.60, 154.00	2.5, 536.0	-21.81 (-68.55, 7.90)	0.1403	-1.4748
	Arithmetic Change from Baseline	29	0 (0.0)	-8.27 (102.267)	-0.39 (-7.34, 12.87)	-10.56, 13.69	-485.3, 124.7	-17.82 (-39.57, -1.84)	0.0288	-2.1868
	Percent Change from Baseline	29	0 (0.0)	70.37 (337.782)	-1.51 (-16.47, 24.19)	-20.32, 29.14	-83.2, 1796.9	-31.11 (-56.22, -9.36)	0.0075	-2.6750

Study No. 1042-CDD-3001

Table 14.2.2.1 Summary of 28-Day Seizure Frequency for Primary Seizure Types - Subjects not in US

28-day seizure frequency (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=32	Baseline	32	0 (0.0)	104.22 (125.508)	46.99 (37.74, 141.33)	31.70, 151.67	5.5, 651.3			
	17-Week Post-Baseline Phase	32	0 (0.0)	103.91 (162.516)	36.22 (23.46, 76.03)	18.30, 102.61	0.5, 648.1			
	Arithmetic Change from Baseline	32	0 (0.0)	-0.31 (104.798)	-8.07 (-25.03, -1.79)	-45.86, 0.18	-191.1, 428.1			
	Percent Change from Baseline	32	0 (0.0)	-13.04 (69.752)	-27.49 (-35.86, -7.28)	-56.17, 0.36	-96.2, 215.4			
Placebo, N=27	Baseline	27	0 (0.0)	54.48 (70.150)	25.33 (16.15, 46.67)	14.67, 58.80	0.7, 276.7	22.26 (2.67, 52.67)	0.0207	-2.3128
	17-Week Post-Baseline Phase	27	0 (0.0)	54.07 (61.293)	29.73 (14.97, 65.17)	13.69, 68.29	1.3, 241.9	7.92 (-10.91, 29.59)	0.3734	-0.8900
	Arithmetic Change from Baseline	27	0 (0.0)	-0.41 (34.315)	-4.47 (-7.34, 11.07)	-11.23, 12.58	-119.2, 56.4	-13.13 (-34.52, 1.35)	0.0988	1.6508
	Percent Change from Baseline	27	0 (0.0)	88.14 (362.759)	-11.64 (-24.13, 34.06)	-34.55, 39.66	-89.7, 1796.9	-19.74 (-53.13, 4.46)	0.1052	1.6203

Study No. 1042-CDD-3001

Table 14.2.2.1 Summary of 28-Day Seizure Frequency for Primary Seizure Types - Subjects in US

28-day seizure frequency (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=18	Baseline	17	1 (5.6)	136.33 (161.884)	84.67 (31.33, 146.67)	31.33, 146.67	15.3, 648.7			
	17-Week Post-Baseline Phase	18	0 (0.0)	75.51 (54.345)	63.42 (31.27, 106.26)	31.27, 106.26	10.4, 189.9			
	Arithmetic Change from Baseline	17	1 (5.6)	-59.23 (125.990)	-24.95 (-50.41, -0.86)	-50.41, -0.86	-501.8, 46.7			
	Percent Change from Baseline	17	1 (5.6)	-15.77 (55.316)	-31.99 (-43.46, -3.28)	-43.46, -3.28	-77.4, 149.2			
Placebo, N=24	Baseline	24	0 (0.0)	159.59 (231.049)	68.65 (53.73, 142.67)	49.59, 166.10	11.3, 1021.3	-6.00 (-45.33, 41.88)	0.8220	-0.2249
	17-Week Post-Baseline Phase	24	0 (0.0)	260.04 (671.950)	81.34 (63.18, 156.36)	44.82, 164.75	21.6, 3362.1	-25.26 (-71.11, 10.58)	0.1821	-1.3344
	Arithmetic Change from Baseline	24	0 (0.0)	100.45 (561.551)	0.81 (-9.97, 22.06)	-11.20, 35.04	-485.3, 2680.7	-34.85 (-93.17, -1.27)	0.0378	-2.0773
	Percent Change from Baseline	24	0 (0.0)	38.17 (105.838)	2.16 (-11.86, 36.36)	-18.79, 49.51	-59.1, 393.5	-34.21 (-65.08, -7.21)	0.0206	-2.3155

Study No. 1042-CDD-3001

Table 14.2.2.1 Summary of 28-Day Seizure Frequency for Primary Seizure Types - Females

28-day seizure frequency (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=39	Baseline	38	1 (2.6)	94.63 (121.657)	44.67 (31.33, 93.33)	28.88, 116.00	5.5, 648.7			
	17-Week Post-Baseline Phase	39	0 (0.0)	76.46 (114.101)	43.69 (26.56, 76.03)	16.43, 97.05	0.5, 648.1			
	Arithmetic Change from Baseline	38	1 (2.6)	-17.43 (123.144)	-6.39 (-24.95, -1.79)	-44.28, 0.97	-501.8, 428.1			
	Percent Change from Baseline	38	1 (2.6)	-11.07 (69.162)	-27.49 (-38.54, -5.71)	-53.68, 2.56	-96.2, 215.4			
Placebo, N=41	Baseline	41	0 (0.0)	109.16 (190.992)	44.10 (26.00, 60.67)	18.67, 84.00	0.7, 1021.3	4.90 (-14.67, 22.87)	0.5760	0.5593
	17-Week Post-Baseline Phase	41	0 (0.0)	164.46 (523.379)	44.17 (25.61, 74.41)	19.60, 87.86	1.3, 3362.1	-4.36 (-25.15, 14.55)	0.6579	-0.4428
	Arithmetic Change from Baseline	41	0 (0.0)	55.30 (429.624)	-1.79 (-7.34, 9.08)	-11.23, 18.51	-485.3, 2680.7	-12.31 (-33.44, 1.15)	0.0887	-1.7024
	Percent Change from Baseline	41	0 (0.0)	72.27 (301.743)	-10.17 (-21.11, 24.19)	-28.73, 36.36	-89.7, 1796.9	-22.15 (-48.35, -1.36)	0.0362	-2.0949

Study No. 1042-CDD-3001

Table 14.2.2.1 Summary of 28-Day Seizure Frequency for Primary Seizure Types - Males

28-day seizure frequency (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=11	Baseline	11	0 (0.0)	186.98 (172.831)	156.00 (54.00, 279.10)	54.00, 226.00	38.2, 651.3			
	17-Week Post-Baseline Phase	11	0 (0.0)	154.77 (181.913)	61.25 (31.27, 326.02)	31.27, 258.52	21.5, 603.9			
	Arithmetic Change from Baseline	11	0 (0.0)	-32.21 (83.684)	-34.36 (-75.34, 32.52)	-75.34, -6.41	-191.1, 152.7			
	Percent Change from Baseline	11	0 (0.0)	-24.04 (46.308)	-31.97 (-49.50, 14.39)	-49.50, -7.28	-89.9, 88.1			
Placebo, N=10	Baseline	10	0 (0.0)	82.54 (59.022)	63.94 (18.67, 142.67)	46.00, 120.00	16.7, 206.1	71.52 (-7.76, 158.12)	0.0845	-1.7252
	17-Week Post-Baseline Phase	10	0 (0.0)	95.80 (54.452)	91.40 (29.73, 156.36)	63.18, 154.00	11.1, 168.3	1.81 (-68.41, 158.28)	0.9719	-0.0352
	Arithmetic Change from Baseline	10	0 (0.0)	13.26 (41.344)	6.34 (-19.76, 56.42)	-6.11, 13.69	-37.8, 108.0	-41.77 (-90.78, -5.27)	0.0265	2.2182
	Percent Change from Baseline	10	0 (0.0)	33.29 (79.329)	7.45 (-18.32, 83.12)	-16.47, 59.29	-33.7, 234.8	-42.09 (-95.17, -8.41)	0.0221	2.2886

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Table 14.2.5.1 Summary of 28-Day Seizure Frequency for All Seizure Types - Patients who are 2-6 years old

28-day seizure frequency (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=30	Baseline	29	1 (3.3)	219.10 (189.956)	168.72 (77.85, 279.10)	45.33, 362.00	18.4, 651.3			
	17-Week Post-Baseline Phase	30	0 (0.0)	186.42 (205.418)	119.14 (68.83, 167.00)	53.08, 189.86	0.5, 817.7			
	Arithmetic Change from Baseline	29	1 (3.3)	-28.21 (184.158)	-11.67 (-59.92, 7.58)	-83.25, 8.65	-501.8, 373.7			
	Percent Change from Baseline	29	1 (3.3)	-0.74 (74.912)	-6.92 (-34.37, 16.66)	-38.54, 24.59	-97.3, 274.8			
Placebo, N=22	Baseline	22	0 (0.0)	102.12 (164.460)	41.77 (22.84, 119.00)	19.33, 119.00	8.7, 770.0	110.33 (14.07, 178.67)	0.0040	-2.8813
	17-Week Post-Baseline Phase	22	0 (0.0)	226.15 (743.421)	51.74 (32.75, 87.86)	29.73, 87.86	8.3, 3542.1	50.20 (4.51, 107.31)	0.0324	-2.1393
	Arithmetic Change from Baseline	22	0 (0.0)	124.03 (594.921)	-5.41 (-17.76, 13.92)	-23.62, 13.92	-119.3, 2772.1	-17.60 (-65.62, 13.69)	0.2272	1.2077
	Percent Change from Baseline	22	0 (0.0)	60.86 (167.193)	-18.23 (-28.73, 59.29)	-31.18, 59.29	-63.7, 540.4	-13.96 (-54.10, 19.99)	0.3465	0.9414

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Table 14.2.5.1 Summary of 28-Day Seizure Frequency for All Seizure Types - Patients who are >6 years old

28-day seizure frequency (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=20	Baseline	20	0 (0.0)	120.36 (131.301)	86.04 (45.33, 116.00)	43.33, 119.25	25.3, 589.3			
	17-Week Post-Baseline Phase	20	0 (0.0)	92.44 (128.310)	58.27 (29.10, 96.73)	28.67, 102.55	11.7, 600.1			
	Arithmetic Change from Baseline	20	0 (0.0)	-27.92 (38.247)	-21.97 (-30.59, -0.61)	-40.50, 0.99	-127.1, 23.7			
	Percent Change from Baseline	20	0 (0.0)	-25.38 (29.878)	-24.73 (-43.46, -1.85)	-45.01, -0.01	-81.1, 28.0			
Placebo, N=29	Baseline	29	0 (0.0)	170.62 (222.874)	96.60 (49.17, 187.33)	46.67, 203.90	7.0, 1021.3	-10.37 (-78.67, 31.89)	0.5898	-0.5391
	17-Week Post-Baseline Phase	29	0 (0.0)	154.11 (158.297)	100.24 (65.17, 168.47)	44.17, 171.93	3.1, 679.2	-37.62 (-101.20, 4.43)	0.0820	-1.7392
	Arithmetic Change from Baseline	29	0 (0.0)	-16.51 (103.606)	-0.39 (-10.56, 15.80)	-19.76, 18.47	-485.3, 119.7	-19.83 (-43.64, -0.88)	0.0430	-2.0240
	Percent Change from Baseline	29	0 (0.0)	17.69 (101.471)	-1.51 (-18.32, 12.32)	-21.11, 12.50	-83.2, 464.9	-22.46 (-45.04, -2.47)	0.0210	-2.3088

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Table 14.2.5.1 Summary of 28-Day Seizure Frequency for All Seizure Types - Patients not in US

28-day seizure frequency (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=32	Baseline	32	0 (0.0)	169.64 (178.363)	101.00 (45.50, 173.33)	43.49, 200.00	18.4, 651.3			
	17-Week Post-Baseline Phase	32	0 (0.0)	176.51 (220.980)	73.38 (46.91, 157.04)	31.12, 226.01	0.5, 817.7			
	Arithmetic Change from Baseline	32	0 (0.0)	6.88 (132.611)	-8.25 (-24.42, 5.26)	-32.52, 8.15	-376.5, 373.7			
	Percent Change from Baseline	32	0 (0.0)	0.58 (70.010)	-5.59 (-34.08, 11.47)	-37.69, 14.65	-97.3, 274.8			
Placebo, N=27	Baseline	27	0 (0.0)	72.54 (75.379)	40.21 (22.84, 96.60)	18.67, 119.00	7.0, 276.7	37.02 (9.95, 110.67)	0.0049	-2.8148
	17-Week Post-Baseline Phase	27	0 (0.0)	71.01 (68.021)	39.55 (24.43, 95.38)	18.83, 97.75	3.1, 241.9	27.11 (-2.68, 69.79)	0.0789	-1.7573
	Arithmetic Change from Baseline	27	0 (0.0)	-1.53 (33.732)	-5.04 (-7.81, 15.80)	-15.53, 18.51	-119.2, 54.6	-9.84 (-30.18, 9.24)	0.3264	0.9813
	Percent Change from Baseline	27	0 (0.0)	38.22 (146.322)	-16.61 (-24.13, 29.14)	-29.31, 34.06	-83.2, 540.4	-7.46 (-36.11, 20.90)	0.5580	0.5858

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Table 14.2.5.1 Summary of 28-Day Seizure Frequency for All Seizure Types - Patients in US

28-day seizure frequency (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=18	Baseline	17	1 (5.6)	196.04 (169.436)	154.67 (75.33, 279.10)	75.33, 279.10	26.3, 648.7			
	17-Week Post-Baseline Phase	18	0 (0.0)	99.61 (59.691)	99.13 (44.74, 146.83)	44.74, 146.83	10.4, 189.9			
	Arithmetic Change from Baseline	17	1 (5.6)	-93.91 (141.304)	-37.83 (-89.23, -12.78)	-89.23, -12.78	-501.8, 23.7			
	Percent Change from Baseline	17	1 (5.6)	-32.21 (32.971)	-39.67 (-49.93, -12.45)	-49.93, -12.45	-81.0, 29.9			
Placebo, N=24	Baseline	24	0 (0.0)	218.17 (263.779)	127.27 (60.00, 203.90)	53.00, 238.62	26.0, 1021.3	9.24 (-63.33, 105.67)	0.7913	0.2646
	17-Week Post-Baseline Phase	24	0 (0.0)	313.63 (707.245)	105.87 (67.50, 277.86)	59.26, 281.50	25.6, 3542.1	-30.25 (-123.73, 21.41)	0.1660	-1.3853
	Arithmetic Change from Baseline	24	0 (0.0)	95.46 (583.510)	-4.02 (-19.76, 13.92)	-54.81, 16.20	-485.3, 2772.1	-42.27 (-120.97, -5.05)	0.0221	-2.2890
	Percent Change from Baseline	24	0 (0.0)	34.17 (121.899)	-5.64 (-21.11, 12.50)	-27.05, 15.01	-59.1, 409.2	-32.16 (-55.96, -9.54)	0.0072	-2.6860

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Table 14.2.5.1 Summary of 28-Day Seizure Frequency for All Seizure Types - Females

28-day seizure frequency (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=39	Baseline	38	1 (2.6)	139.78 (145.869)	93.37 (45.33, 154.67)	36.11, 184.00	18.4, 648.7			
	17-Week Post-Baseline Phase	39	0 (0.0)	123.77 (171.543)	68.83 (44.74, 108.38)	30.06, 133.54	0.5, 817.7			
	Arithmetic Change from Baseline	38	1 (2.6)	-14.26 (138.811)	-14.74 (-29.95, 2.59)	-50.41, 5.26	-501.8, 373.7			
	Percent Change from Baseline	38	1 (2.6)	-8.54 (65.629)	-18.02 (-39.67, 7.65)	-43.46, 12.64	-97.3, 274.8			
Placebo, N=41	Baseline	41	0 (0.0)	151.50 (221.376)	56.00 (43.33, 150.00)	26.67, 187.33	7.0, 1021.3	9.86 (-16.39, 48.67)	0.4239	0.7997
	17-Week Post-Baseline Phase	41	0 (0.0)	205.09 (553.619)	65.17 (44.17, 111.49)	35.75, 168.47	3.1, 3542.1	-3.11 (-31.04, 24.82)	0.8398	-0.2021
	Arithmetic Change from Baseline	41	0 (0.0)	53.59 (445.731)	-5.00 (-10.56, 13.92)	-25.83, 18.51	-485.3, 2772.1	-13.84 (-35.20, 5.77)	0.1562	-1.4178
	Percent Change from Baseline	41	0 (0.0)	39.95 (144.865)	-10.17 (-24.13, 12.50)	-29.31, 29.14	-83.2, 540.4	-15.33 (-38.80, 5.86)	0.1346	-1.4963

Study No. 1042-CDD-3001
Table 14.2.5.1 Summary of 28-Day Seizure Frequency for All Seizure Types - Males

28-day seizure frequency (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=11	Baseline	11	0 (0.0)	313.57 (202.083)	279.10 (168.72, 589.33)	168.72, 482.00	54.4, 651.3			
	17-Week Post-Baseline Phase	11	0 (0.0)	237.68 (203.213)	157.04 (79.93, 600.07)	79.93, 326.26	29.1, 616.2			
	Arithmetic Change from Baseline	11	0 (0.0)	-75.89 (152.335)	-35.10 (-89.23, 10.74)	-89.23, 2.08	-376.5, 152.9			
	Percent Change from Baseline	11	0 (0.0)	-18.59 (45.879)	-21.26 (-46.55, 2.67)	-46.55, 1.82	-81.0, 88.2			
Placebo, N=10	Baseline	10	0 (0.0)	98.32 (61.909)	103.83 (18.67, 156.12)	46.00, 142.67	16.7, 206.1	181.00 (48.72, 383.28)	0.0067	-2.7111
	17-Week Post-Baseline Phase	10	0 (0.0)	103.58 (57.844)	97.81 (29.73, 168.30)	66.29, 156.36	11.1, 171.9	71.69 (-14.88, 230.88)	0.1300	-1.5140
	Arithmetic Change from Baseline	10	0 (0.0)	5.27 (39.997)	-4.33 (-23.62, 15.80)	-19.76, 13.69	-37.8, 108.0	-40.19 (-105.92, 2.66)	0.0528	1.9365
	Percent Change from Baseline	10	0 (0.0)	21.42 (79.196)	-5.64 (-19.85, 59.29)	-18.32, 10.12	-33.7, 234.8	-16.96 (-64.68, 9.55)	0.1300	1.5140

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Table 14.2.3.2.1 Summary of Notable Reduction Rates in 28-Day Seizure Frequency - Patients who are 2-6 years old

Seizure type	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Primary Seizures	GNX N=30	29	25% Responder	1 (3.3)	16 (55.2)				
			50% Responder	1 (3.3)	8 (27.6)				
			75% Responder	1 (3.3)	3 (10.3)				
	Placebo N=22	22	25% Responder	0 (0.0)	7 (31.8)	2.53 (0.82, 7.82)	1.69 (0.86, 3.30)	23.4 (-4.9, 48.5)	0.1552
			50% Responder	0 (0.0)	2 (9.1)	3.24 (0.70, 15.04)	2.61 (0.71, 9.58)	18.5 (-9.5, 44.0)	0.1565
			75% Responder	0 (0.0)	1 (4.5)	1.89 (0.26, 13.93)	1.79 (0.29, 11.20)	5.8 (-21.7, 32.6)	0.6247
All Seizures	GNX N=30	29	25% Responder	1 (3.3)	12 (41.4)				
			50% Responder	1 (3.3)	7 (24.1)				
			75% Responder	1 (3.3)	4 (13.8)				
	Placebo N=22	22	25% Responder	0 (0.0)	9 (40.9)	1.02 (0.34, 3.06)	1.01 (0.53, 1.92)	0.5 (-27.2, 27.6)	1.0000
			50% Responder	0 (0.0)	2 (9.1)	2.73 (0.58, 12.90)	2.30 (0.61, 8.65)	15.0 (-12.8, 40.9)	0.2684
			75% Responder	0 (0.0)	0 (0.0)	7.94 (0.40, 155.76)	6.90 (0.39, 121.79)	13.8 (-14.0, 39.9)	0.1243

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Table 14.2.3.2.1 Summary of Notable Reduction Rates in 28-Day Seizure Frequency - Patients who are >6 years old

Seizure type	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Primary Seizures	GNX N=20	20	25% Responder	0 (0.0)	12 (60.0)				
			50% Responder	0 (0.0)	4 (20.0)				
			75% Responder	0 (0.0)	2 (10.0)				
	Placebo N=29	29	25% Responder	0 (0.0)	5 (17.2)	7.20 (1.93, 26.81)	3.48 (1.45, 8.34)	42.8 (15.0, 66.2)	0.0051
			50% Responder	0 (0.0)	3 (10.3)	2.17 (0.43, 10.96)	1.93 (0.48, 7.72)	9.7 (-19.0, 36.8)	0.4221
			75% Responder	0 (0.0)	1 (3.4)	3.11 (0.26, 36.87)	2.90 (0.28, 29.86)	6.6 (-21.9, 33.7)	0.5593
All Seizures	GNX N=20	20	25% Responder	0 (0.0)	10 (50.0)				
			50% Responder	0 (0.0)	3 (15.0)				
			75% Responder	0 (0.0)	1 (5.0)				
	Placebo N=29	29	25% Responder	0 (0.0)	6 (20.7)	3.83 (1.09, 13.45)	2.42 (1.05, 5.58)	29.3 (1.1, 54.9)	0.0611
			50% Responder	0 (0.0)	2 (6.9)	2.38 (0.36, 15.76)	2.17 (0.40, 11.86)	8.1 (-20.5, 35.2)	0.3868
			75% Responder	0 (0.0)	1 (3.4)	1.47 (0.09, 25.03)	1.45 (0.10, 21.85)	1.6 (-26.6, 29.0)	1.0000

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Table 14.2.3.2.1 Summary of Notable Reduction Rates in 28-Day Seizure Frequency - Patients not in US

Seizure type	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Primary Seizures	GNX N=32	32	25% Responder	0 (0.0)	17 (53.1)				
			50% Responder	0 (0.0)	8 (25.0)				
			75% Responder	0 (0.0)	4 (12.5)				
	Placebo N=27	27	25% Responder	0 (0.0)	8 (29.6)	2.69 (0.91, 7.92)	1.79 (0.92, 3.49)	23.5 (-2.2, 47.0)	0.1120
			50% Responder	0 (0.0)	4 (14.8)	1.92 (0.51, 7.24)	1.69 (0.57, 5.00)	10.2 (-15.3, 34.7)	0.5177
			75% Responder	0 (0.0)	2 (7.4)	1.79 (0.30, 10.60)	1.69 (0.33, 8.51)	5.1 (-20.2, 30.2)	0.6780
All Seizures	GNX N=32	32	25% Responder	0 (0.0)	11 (34.4)				
			50% Responder	0 (0.0)	6 (18.8)				
			75% Responder	0 (0.0)	3 (9.4)				
	Placebo N=27	27	25% Responder	0 (0.0)	8 (29.6)	1.24 (0.41, 3.74)	1.16 (0.55, 2.46)	4.7 (-20.8, 29.7)	0.7836
			50% Responder	0 (0.0)	3 (11.1)	1.85 (0.41, 8.21)	1.69 (0.47, 6.12)	7.6 (-17.8, 32.5)	0.4877
			75% Responder	0 (0.0)	1 (3.7)	2.69 (0.26, 27.49)	2.53 (0.28, 22.95)	5.7 (-19.5, 30.8)	0.6175

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Table 14.2.3.2.1 Summary of Notable Reduction Rates in 28-Day Seizure Frequency - Patients in US

Seizure type	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Primary Seizures	GNX N=18	17	25% Responder	1 (5.6)	11 (64.7)				
			50% Responder	1 (5.6)	4 (23.5)				
			75% Responder	1 (5.6)	1 (5.9)				
	Placebo N=24	24	25% Responder	0 (0.0)	4 (16.7)	8.06 (1.98, 32.76)	3.55 (1.43, 8.78)	48.0 (17.6, 72.2)	0.0028
			50% Responder	0 (0.0)	1 (4.2)	5.22 (0.73, 37.27)	4.17 (0.73, 23.88)	19.4 (-11.8, 47.6)	0.1412
			75% Responder	0 (0.0)	0 (0.0)	4.45 (0.17, 116.14)	4.17 (0.18, 96.53)	5.9 (-24.8, 35.9)	0.4146
All Seizures	GNX N=18	17	25% Responder	1 (5.6)	11 (64.7)				
			50% Responder	1 (5.6)	4 (23.5)				
			75% Responder	1 (5.6)	2 (11.8)				
	Placebo N=24	24	25% Responder	0 (0.0)	7 (29.2)	4.13 (1.14, 14.95)	2.13 (1.07, 4.26)	35.5 (4.1, 62.0)	0.0308
			50% Responder	0 (0.0)	1 (4.2)	5.22 (0.73, 37.27)	4.17 (0.73, 23.88)	19.4 (-11.8, 47.6)	0.1412
			75% Responder	0 (0.0)	0 (0.0)	7.90 (0.36, 175.84)	6.94 (0.35, 136.08)	11.8 (-19.1, 41.1)	0.1659

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Table 14.2.3.2.1 Summary of Notable Reduction Rates in 28-Day Seizure Frequency - Females

Seizure type	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Primary Seizures	GNX N=39	38	25% Responder	1 (2.6)	21 (55.3)				
			50% Responder	1 (2.6)	10 (26.3)				
			75% Responder	1 (2.6)	4 (10.5)				
	Placebo N=41	41	25% Responder	0 (0.0)	11 (26.8)	3.26 (1.29, 8.23)	2.01 (1.14, 3.55)	28.4 (6.0, 48.4)	0.0125
			50% Responder	0 (0.0)	5 (12.2)	2.44 (0.78, 7.66)	2.06 (0.81, 5.24)	14.1 (-8.2, 35.5)	0.1527
			75% Responder	0 (0.0)	2 (4.9)	2.06 (0.41, 10.33)	1.94 (0.44, 8.54)	5.6 (-16.8, 27.0)	0.4204
All Seizures	GNX N=39	38	25% Responder	1 (2.6)	17 (44.7)				
			50% Responder	1 (2.6)	8 (21.1)				
			75% Responder	1 (2.6)	3 (7.9)				
	Placebo N=41	41	25% Responder	0 (0.0)	14 (34.1)	1.54 (0.63, 3.78)	1.30 (0.76, 2.24)	10.6 (-11.9, 31.8)	0.3649
			50% Responder	0 (0.0)	4 (9.8)	2.32 (0.67, 8.01)	2.03 (0.71, 5.85)	11.3 (-11.2, 32.8)	0.2148
			75% Responder	0 (0.0)	1 (2.4)	2.66 (0.37, 18.99)	2.51 (0.39, 16.18)	5.5 (-16.9, 26.9)	0.3467

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Table 14.2.3.2.1 Summary of Notable Reduction Rates in 28-Day Seizure Frequency - Males

Seizure type	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Primary Seizures	GNX N=11	11	25% Responder	0 (0.0)	7 (63.6)				
			50% Responder	0 (0.0)	2 (18.2)				
			75% Responder	0 (0.0)	1 (9.1)				
	Placebo N=10	10	25% Responder	0 (0.0)	1 (10.0)	15.75 (1.42, 174.25)	6.36 (0.94, 43.07)	53.6 (11.7, 83.5)	0.0237
			50% Responder	0 (0.0)	0 (0.0)	5.53 (0.23, 130.34)	4.58 (0.25, 85.33)	18.2 (-23.4, 56.6)	0.4762
			75% Responder	0 (0.0)	0 (0.0)	3.00 (0.11, 82.40)	2.75 (0.12, 60.70)	9.1 (-32.2, 48.8)	1.0000
All Seizures	GNX N=11	11	25% Responder	0 (0.0)	5 (45.5)				
			50% Responder	0 (0.0)	2 (18.2)				
			75% Responder	0 (0.0)	2 (18.2)				
	Placebo N=10	10	25% Responder	0 (0.0)	1 (10.0)	7.50 (0.69, 81.25)	4.55 (0.63, 32.56)	35.5 (-5.4, 70.9)	0.1486
			50% Responder	0 (0.0)	0 (0.0)	5.53 (0.23, 130.34)	4.58 (0.25, 85.33)	18.2 (-23.4, 56.6)	0.4762
			75% Responder	0 (0.0)	0 (0.0)	5.53 (0.23, 130.34)	4.58 (0.25, 85.33)	18.2 (-23.4, 56.6)	0.4762

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Table 14.2.5.3.1 Summary of the Longest Seizure-Free Interval (Percentage of Days) for Primary Seizure Types - Patients who are 2-6 years old

Longest Period of Time Seizure Free (%) (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=30	Baseline	29	1 (3.3)	8.07 (12.003)	2.56 (0.00, 9.52)	0.00, 11.90	0.0, 51.2			
	17-Week Post-Baseline Phase	30	0 (0.0)	9.25 (13.084)	5.29 (1.25, 7.81)	0.85, 9.40	0.0, 50.0			
	Arithmetic Change from Baseline	29	1 (3.3)	1.00 (9.986)	0.00 (0.00, 1.69)	-1.22, 1.71	-20.2, 35.8			
Placebo, N=22	Baseline	22	0 (0.0)	12.84 (18.040)	4.88 (2.63, 11.54)	2.44, 11.54	0.0, 59.5	-2.38 (-5.00, 1.09)	0.2889	1.0605
	17-Week Post-Baseline Phase	22	0 (0.0)	7.44 (7.067)	6.01 (2.52, 10.17)	1.80, 10.17	0.0, 25.0	-0.86 (-3.91, 2.75)	0.5586	0.5849
	Arithmetic Change from Baseline	22	0 (0.0)	-5.40 (13.823)	-1.35 (-4.49, 3.27)	-8.61, 3.27	-41.2, 9.0	2.06 (-1.63, 5.77)	0.2333	-1.1919

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Table 14.2.5.3.1 Summary of the Longest Seizure-Free Interval (Percentage of Days) for Primary Seizure Types - Patients who are >6 years old

Longest Period of Time Seizure Free (%) (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=20	Baseline	20	0 (0.0)	7.56 (9.384)	2.61 (2.38, 9.52)	1.19, 11.90	0.0, 33.3			
	17-Week Post-Baseline Phase	20	0 (0.0)	6.06 (5.901)	5.15 (1.72, 7.22)	1.70, 7.33	0.0, 20.7			
	Arithmetic Change from Baseline	20	0 (0.0)	-1.50 (8.439)	-0.35 (-3.01, 2.12)	-4.29, 2.29	-20.5, 20.7			
Placebo, N=29	Baseline	29	0 (0.0)	11.97 (17.762)	9.52 (2.44, 14.29)	2.38, 14.29	0.0, 95.0	-2.38 (-7.14, 2.12)	0.2347	-1.1882
	17-Week Post-Baseline Phase	29	0 (0.0)	7.84 (10.834)	3.23 (1.72, 7.65)	1.00, 7.83	0.0, 42.2	0.13 (-2.56, 3.02)	0.8227	0.2241
	Arithmetic Change from Baseline	29	0 (0.0)	-4.13 (15.773)	-1.53 (-3.77, 0.00)	-9.87, 0.00	-67.2, 35.1	1.72 (-1.67, 5.27)	0.3435	0.9473

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Table 14.2.5.3.1 Summary of the Longest Seizure-Free Interval (Percentage of Days) for Primary Seizure Types - Patients not in US

Longest Period of Time Seizure Free (%) (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=32	Baseline	32	0 (0.0)	8.96 (12.506)	2.53 (0.00, 11.90)	0.00, 13.55	0.0, 51.2			
	17-Week Post-Baseline Phase	32	0 (0.0)	8.79 (11.490)	5.29 (1.69, 9.40)	0.87, 12.55	0.0, 50.0			
	Arithmetic Change from Baseline	32	0 (0.0)	-0.17 (8.824)	0.00 (-1.22, 2.12)	-1.86, 2.57	-20.5, 26.9			
Placebo, N=27	Baseline	27	0 (0.0)	17.27 (22.102)	9.52 (6.06, 15.00)	4.76, 21.95	0.0, 95.0	-4.76 (-9.52, 0.00)	0.0372	2.0834
	17-Week Post-Baseline Phase	27	0 (0.0)	11.68 (11.040)	7.83 (3.64, 18.64)	2.80, 19.09	0.0, 42.2	-2.44 (-6.85, 0.80)	0.1150	1.5760
	Arithmetic Change from Baseline	27	0 (0.0)	-5.59 (19.490)	-1.87 (-4.49, 3.27)	-10.18, 5.60	-67.2, 35.1	2.06 (-2.41, 5.76)	0.2969	-1.0430

Study No. 1042-CDD-3001

Table 14.2.5.3.1 Summary of the Longest Seizure-Free Interval (Percentage of Days) for Primary Seizure Types - Patients in US

Longest Period of Time Seizure Free (%) (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=18	Baseline	17	1 (5.6)	5.78 (6.842)	4.76 (0.00, 7.14)	0.00, 7.14	0.0, 21.9			
	17-Week Post-Baseline Phase	18	0 (0.0)	6.53 (9.693)	4.20 (1.25, 7.63)	1.25, 7.63	0.0, 42.0			
	Arithmetic Change from Baseline	17	1 (5.6)	0.27 (10.617)	0.00 (-3.01, 1.48)	-3.01, 1.48	-14.5, 35.8			
Placebo, N=24	Baseline	24	0 (0.0)	6.81 (8.305)	2.54 (2.38, 10.53)	0.00, 10.67	0.0, 33.3	0.00 (-3.81, 2.44)	0.8195	-0.2282
	17-Week Post-Baseline Phase	24	0 (0.0)	3.15 (3.271)	2.16 (0.91, 5.08)	0.42, 5.11	0.0, 10.9	1.23 (-0.80, 4.54)	0.2300	1.2005
	Arithmetic Change from Baseline	24	0 (0.0)	-3.65 (6.834)	-0.65 (-4.39, 0.00)	-8.57, 0.00	-23.2, 7.8	1.48 (-0.70, 6.10)	0.1791	1.3434

Study No. 1042-CDD-3001

Table 14.2.5.3.1 Summary of the Longest Seizure-Free Interval (Percentage of Days) for Primary Seizure Types - Females

Longest Period of Time Seizure Free (%) (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=39	Baseline	38	1 (2.6)	8.97 (11.188)	5.98 (2.38, 9.52)	0.00, 12.82	0.0, 51.2			
	17-Week Post-Baseline Phase	39	0 (0.0)	8.28 (10.640)	5.88 (1.75, 7.63)	1.68, 9.40	0.0, 50.0			
	Arithmetic Change from Baseline	38	1 (2.6)	-0.85 (9.636)	0.00 (-1.63, 1.48)	-3.42, 1.72	-20.5, 35.8			
Placebo, N=41	Baseline	41	0 (0.0)	14.63 (19.020)	9.52 (5.00, 11.90)	2.63, 15.00	0.0, 95.0	-2.44 (-7.14, 0.00)	0.0816	-1.7416
	17-Week Post-Baseline Phase	41	0 (0.0)	8.85 (9.913)	5.13 (2.94, 8.04)	1.80, 10.92	0.0, 42.2	-0.17 (-2.90, 1.90)	0.7107	-0.3709
	Arithmetic Change from Baseline	41	0 (0.0)	-5.78 (16.238)	-1.87 (-4.39, 0.00)	-9.94, 0.15	-67.2, 35.1	2.06 (-0.39, 5.27)	0.1128	1.5858

Study No. 1042-CDD-3001

Table 14.2.5.3.1 Summary of the Longest Seizure-Free Interval (Percentage of Days) for Primary Seizure Types - Males

Longest Period of Time Seizure Free (%) (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=11	Baseline	11	0 (0.0)	4.03 (9.331)	0.00 (0.00, 4.88)	0.00, 2.78	0.0, 31.7			
	17-Week Post-Baseline Phase	11	0 (0.0)	6.89 (11.961)	1.25 (0.00, 26.92)	0.00, 5.22	0.0, 34.2			
	Arithmetic Change from Baseline	11	0 (0.0)	2.87 (8.162)	0.12 (0.00, 2.65)	0.00, 2.48	-3.6, 26.9			
Placebo, N=10	Baseline	10	0 (0.0)	2.99 (3.614)	1.19 (0.00, 7.14)	0.00, 6.06	0.0, 9.5	0.00 (-4.65, 2.38)	0.7036	0.3804
	17-Week Post-Baseline Phase	10	0 (0.0)	2.81 (3.553)	1.67 (0.00, 7.76)	0.00, 2.80	0.0, 10.5	0.00 (-1.95, 4.22)	1.0000	0.0000
	Arithmetic Change from Baseline	10	0 (0.0)	-0.18 (4.709)	0.00 (-4.49, 5.76)	-3.26, 1.63	-8.5, 7.8	0.85 (-1.63, 4.90)	0.2366	-1.1836

Study No. 1042-CDD-3001

Table 14.2.5.3.2 Summary of the Longest Seizure-Free Interval (Percentage of Days) for All Seizure Types - Patients who are 2-6 years old

Longest Period of Time Seizure Free (%) (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=30	Baseline	29	1 (3.3)	4.58 (10.311)	0.00 (0.00, 4.76)	0.00, 6.25	0.0, 51.2			
	17-Week Post-Baseline Phase	30	0 (0.0)	5.23 (11.534)	0.92 (0.00, 3.39)	0.00, 6.42	0.0, 50.0			
	Arithmetic Change from Baseline	29	1 (3.3)	0.57 (7.576)	0.00 (0.00, 0.87)	-0.72, 0.87	-14.2, 35.8			
Placebo, N=22	Baseline	22	0 (0.0)	9.38 (16.423)	4.76 (2.38, 9.52)	0.00, 9.52	0.0, 59.5	-2.44 (-4.76, 0.00)	0.0423	2.0309
	17-Week Post-Baseline Phase	22	0 (0.0)	6.36 (6.221)	5.36 (1.80, 9.32)	0.86, 9.32	0.0, 21.0	-1.91 (-5.92, 0.00)	0.0529	1.9361
	Arithmetic Change from Baseline	22	0 (0.0)	-3.03 (13.024)	-1.17 (-2.33, 3.27)	-2.38, 3.27	-41.2, 11.4	0.87 (-1.57, 2.38)	0.3523	-0.9301

Study No. 1042-CDD-3001

Table 14.2.5.3.2 Summary of the Longest Seizure-Free Interval (Percentage of Days) for All Seizure Types - Patients who are >6 years old

Longest Period of Time Seizure Free (%) (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=20	Baseline	20	0 (0.0)	4.64 (7.342)	2.58 (0.00, 5.71)	0.00, 6.43	0.0, 33.3			
	17-Week Post-Baseline Phase	20	0 (0.0)	3.55 (3.937)	2.03 (1.65, 4.90)	1.26, 5.15	0.0, 13.6			
	Arithmetic Change from Baseline	20	0 (0.0)	-1.08 (5.695)	0.00 (-2.96, 1.72)	-2.99, 1.92	-20.5, 7.8			
Placebo, N=29	Baseline	29	0 (0.0)	7.36 (9.209)	2.50 (2.38, 9.52)	0.00, 10.53	0.0, 37.5	-0.64 (-4.76, 0.40)	0.3244	-0.9854
	17-Week Post-Baseline Phase	29	0 (0.0)	5.36 (8.893)	1.87 (0.88, 4.35)	0.00, 5.13	0.0, 42.2	0.00 (-1.71, 1.70)	0.9347	0.0820
	Arithmetic Change from Baseline	29	0 (0.0)	-2.00 (9.617)	-0.60 (-2.22, 0.00)	-4.71, 0.00	-24.1, 35.1	1.42 (-1.00, 4.00)	0.3407	0.9529

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Table 14.2.5.3.2 Summary of the Longest Seizure-Free Interval (Percentage of Days) for All Seizure Types - Patients not in US

Longest Period of Time Seizure Free (%) (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=32	Baseline	32	0 (0.0)	5.03 (10.652)	0.00 (0.00, 4.76)	0.00, 7.14	0.0, 51.2			
	17-Week Post-Baseline Phase	32	0 (0.0)	4.36 (9.142)	1.66 (0.00, 3.85)	0.00, 5.15	0.0, 50.0			
	Arithmetic Change from Baseline	32	0 (0.0)	-0.66 (4.762)	0.00 (0.00, 0.87)	-1.83, 0.92	-20.5, 7.8			
Placebo, N=27	Baseline	27	0 (0.0)	11.77 (16.039)	5.41 (2.56, 11.54)	2.38, 11.90	0.0, 59.5	-4.40 (-7.14, -0.06)	0.0052	2.7940
	17-Week Post-Baseline Phase	27	0 (0.0)	8.68 (9.445)	5.60 (2.19, 12.07)	1.72, 13.40	0.0, 42.2	-2.88 (-7.65, -0.86)	0.0060	2.7495
	Arithmetic Change from Baseline	27	0 (0.0)	-3.09 (14.740)	-1.51 (-2.33, 1.01)	-4.49, 1.87	-41.2, 35.1	1.35 (-1.01, 3.02)	0.3709	-0.8948

Study No. 1042-CDD-3001

Table 14.2.5.3.2 Summary of the Longest Seizure-Free Interval (Percentage of Days) for All Seizure Types - Patients in US

Longest Period of Time Seizure Free (%) (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=18	Baseline	17	1 (5.6)	3.81 (5.406)	2.38 (0.00, 5.71)	0.00, 5.71	0.0, 21.9			
	17-Week Post-Baseline Phase	18	0 (0.0)	4.90 (9.676)	1.70 (0.81, 6.19)	0.81, 6.19	0.0, 42.0			
	Arithmetic Change from Baseline	17	1 (5.6)	0.95 (9.759)	0.00 (-1.52, 1.48)	-1.52, 1.48	-14.2, 35.8			
Placebo, N=24	Baseline	24	0 (0.0)	4.26 (5.459)	2.38 (0.00, 4.76)	0.00, 7.14	0.0, 15.0	0.00 (-2.38, 2.38)	0.9671	-0.0413
	17-Week Post-Baseline Phase	24	0 (0.0)	2.54 (3.238)	1.75 (0.00, 2.59)	0.00, 3.47	0.0, 10.9	0.48 (-0.88, 1.71)	0.5007	0.6734
	Arithmetic Change from Baseline	24	0 (0.0)	-1.72 (4.625)	-0.30 (-2.24, 0.00)	-2.54, 0.00	-11.7, 7.8	1.12 (-0.70, 2.50)	0.3414	0.9514

Study No. 1042-CDD-3001

Table 14.2.5.3.2 Summary of the Longest Seizure-Free Interval (Percentage of Days) for All Seizure Types - Females

Longest Period of Time Seizure Free (%) (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=39	Baseline	38	1 (2.6)	5.74 (10.094)	2.38 (0.00, 6.25)	0.00, 7.14	0.0, 51.2			
	17-Week Post-Baseline Phase	39	0 (0.0)	5.56 (10.229)	1.75 (1.56, 5.41)	0.85, 7.29	0.0, 50.0			
	Arithmetic Change from Baseline	38	1 (2.6)	-0.22 (7.758)	0.00 (-1.22, 0.87)	-2.96, 1.48	-20.5, 35.8			
Placebo, N=41	Baseline	41	0 (0.0)	9.66 (13.740)	4.76 (2.44, 9.52)	2.38, 11.90	0.0, 59.5	-2.38 (-4.76, 0.00)	0.0482	-1.9755
	17-Week Post-Baseline Phase	41	0 (0.0)	6.58 (8.350)	2.94 (1.80, 7.65)	0.87, 9.32	0.0, 42.2	-0.86 (-2.66, 0.80)	0.2342	-1.1897
	Arithmetic Change from Baseline	41	0 (0.0)	-3.08 (12.135)	-1.51 (-2.24, 0.00)	-3.22, 0.00	-41.2, 35.1	1.42 (-0.16, 2.89)	0.1332	1.5015

Study No. 1042-CDD-3001

Table 14.2.5.3.2 Summary of the Longest Seizure-Free Interval (Percentage of Days) for All Seizure Types - Males

Longest Period of Time Seizure Free (%) (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=11	Baseline	11	0 (0.0)	0.70 (1.195)	0.00 (0.00, 2.44)	0.00, 2.44	0.0, 2.8			
	17-Week Post-Baseline Phase	11	0 (0.0)	0.99 (1.744)	0.00 (0.00, 3.85)	0.00, 1.25	0.0, 4.9			
	Arithmetic Change from Baseline	11	0 (0.0)	0.29 (1.627)	0.00 (0.00, 2.12)	0.00, 0.87	-2.4, 3.8			
Placebo, N=10	Baseline	10	0 (0.0)	2.38 (3.549)	0.00 (0.00, 7.14)	0.00, 4.76	0.0, 9.5	0.00 (-2.38, 0.00)	0.4252	0.7974
	17-Week Post-Baseline Phase	10	0 (0.0)	2.55 (3.657)	1.35 (0.00, 7.76)	0.00, 2.65	0.0, 10.5	-0.54 (-2.65, 0.00)	0.2384	1.1789
	Arithmetic Change from Baseline	10	0 (0.0)	0.17 (4.593)	0.00 (-4.49, 5.76)	-0.67, 1.87	-8.5, 7.8	0.00 (-1.92, 2.12)	0.9110	-0.1118

Study No. 1042-CDD-3001

Table 14.2.3.1.1 Summary of the Percentage of Seizure-Free Days for Primary Seizure Types - Patients who are 2-6 years old

Percentage of seizure free days (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=30	Baseline	29	1 (3.3)	23.26 (27.153)	7.69 (0.00, 35.71)	0.00, 40.48	0.0, 82.9			
	17-Week Post-Baseline Phase	30	0 (0.0)	30.13 (30.042)	24.14 (3.75, 42.37)	0.87, 50.00	0.0, 98.2			
	Arithmetic Change from Baseline	29	1 (3.3)	6.62 (19.621)	1.71 (0.00, 15.25)	0.00, 15.32	-29.4, 46.2			
Placebo, N=22	Baseline	22	0 (0.0)	28.76 (27.267)	22.79 (2.63, 50.00)	2.44, 50.00	0.0, 81.0	-2.44 (-21.43, 4.76)	0.3504	0.9337
	17-Week Post-Baseline Phase	22	0 (0.0)	37.47 (28.608)	33.53 (18.97, 66.67)	13.51, 66.67	0.0, 95.4	-8.13 (-25.55, 7.89)	0.3296	0.9749
	Arithmetic Change from Baseline	22	0 (0.0)	8.71 (16.763)	10.27 (0.00, 18.97)	-2.97, 18.97	-24.9, 38.7	-3.55 (-13.75, 6.17)	0.5354	0.6198

Study No. 1042-CDD-3001

Table 14.2.3.1.1 Summary of the Percentage of Seizure-Free Days for Primary Seizure Types - Patients who are >6 years old

Percentage of seizure free days (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=20	Baseline	20	0 (0.0)	21.57 (24.255)	12.05 (2.38, 30.95)	1.19, 39.29	0.0, 66.7			
	17-Week Post-Baseline Phase	20	0 (0.0)	35.54 (31.955)	29.44 (8.77, 58.56)	6.07, 63.26	0.0, 91.5			
	Arithmetic Change from Baseline	20	0 (0.0)	13.96 (23.497)	8.14 (0.00, 15.60)	0.00, 19.32	-8.3, 91.4			
Placebo, N=29	Baseline	29	0 (0.0)	31.50 (27.342)	27.27 (9.52, 45.24)	9.52, 54.76	0.0, 97.5	-7.41 (-25.80, 3.51)	0.2202	-1.2259
	17-Week Post-Baseline Phase	29	0 (0.0)	35.19 (33.052)	26.17 (6.45, 63.56)	4.31, 64.23	0.0, 91.8	0.86 (-17.24, 16.68)	0.8306	0.2139
	Arithmetic Change from Baseline	29	0 (0.0)	3.69 (14.098)	0.00 (-1.10, 6.11)	-2.38, 7.48	-19.5, 53.9	6.28 (0.00, 13.67)	0.0427	2.0269

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Table 14.2.3.1.1 Summary of the Percentage of Seizure-Free Days for Primary Seizure Types - Patients not in US

Percentage of seizure free days (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=32	Baseline	32	0 (0.0)	24.07 (28.677)	8.01 (0.00, 45.24)	0.00, 48.81	0.0, 82.9			
	17-Week Post-Baseline Phase	32	0 (0.0)	36.08 (33.488)	35.77 (8.77, 58.56)	1.72, 63.42	0.0, 98.2			
	Arithmetic Change from Baseline	32	0 (0.0)	12.02 (22.978)	5.69 (0.00, 15.25)	0.00, 19.18	-29.4, 91.4			
Placebo, N=27	Baseline	27	0 (0.0)	39.30 (26.790)	35.71 (23.08, 57.14)	19.05, 57.14	0.0, 97.5	-18.20 (-33.21, 0.00)	0.0239	2.2593
	17-Week Post-Baseline Phase	27	0 (0.0)	49.11 (31.118)	60.87 (26.17, 67.80)	19.20, 71.68	0.0, 95.4	-12.99 (-31.87, 1.43)	0.1017	1.6369
	Arithmetic Change from Baseline	27	0 (0.0)	9.82 (17.044)	8.13 (0.00, 17.11)	-2.97, 19.20	-22.2, 53.9	-0.04 (-8.13, 8.31)	0.9696	0.0381

Study No. 1042-CDD-3001

Table 14.2.3.1.1 Summary of the Percentage of Seizure-Free Days for Primary Seizure Types - Patients in US

Percentage of seizure free days (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=18	Baseline	17	1 (5.6)	19.75 (19.613)	17.14 (0.00, 31.71)	0.00, 31.71	0.0, 61.9			
	17-Week Post-Baseline Phase	18	0 (0.0)	25.56 (24.112)	24.14 (3.36, 42.00)	3.36, 42.00	0.0, 75.0			
	Arithmetic Change from Baseline	17	1 (5.6)	5.11 (17.694)	0.00 (-6.28, 15.60)	-6.28, 15.60	-28.0, 44.0			
Placebo, N=24	Baseline	24	0 (0.0)	20.21 (24.095)	10.03 (2.38, 33.33)	0.00, 34.17	0.0, 76.2	0.00 (-9.93, 14.74)	0.9040	0.1206
	17-Week Post-Baseline Phase	24	0 (0.0)	21.62 (23.826)	15.38 (1.69, 37.39)	0.50, 38.44	0.0, 79.8	2.60 (-8.97, 20.63)	0.4824	0.7024
	Arithmetic Change from Baseline	24	0 (0.0)	1.40 (12.028)	0.00 (-1.67, 2.32)	-3.23, 4.24	-24.9, 38.7	0.98 (-5.96, 11.67)	0.4975	0.6784

Study No. 1042-CDD-3001

Table 14.2.3.1.1 Summary of the Percentage of Seizure-Free Days for Primary Seizure Types - Females

Percentage of seizure free days (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=39	Baseline	38	1 (2.6)	26.64 (27.220)	19.29 (2.38, 40.48)	0.00, 50.00	0.0, 82.9			
	17-Week Post-Baseline Phase	39	0 (0.0)	36.23 (31.938)	28.13 (10.17, 55.37)	5.36, 67.52	0.0, 98.2			
	Arithmetic Change from Baseline	38	1 (2.6)	9.56 (21.651)	5.14 (0.00, 11.39)	-0.93, 15.32	-29.4, 91.4			
Placebo, N=41	Baseline	41	0 (0.0)	34.49 (27.672)	33.33 (17.07, 50.00)	9.52, 57.14	0.0, 97.5	-7.14 (-19.05, 1.65)	0.1390	-1.4793
	17-Week Post-Baseline Phase	41	0 (0.0)	40.73 (31.418)	39.50 (19.20, 64.23)	7.14, 66.38	0.0, 95.4	-3.51 (-18.50, 8.11)	0.5601	-0.5828
	Arithmetic Change from Baseline	41	0 (0.0)	6.24 (16.382)	5.67 (-0.69, 11.07)	-4.07, 15.24	-24.9, 53.9	1.64 (-5.36, 8.63)	0.5657	0.5744

Study No. 1042-CDD-3001

Table 14.2.3.1.1 Summary of the Percentage of Seizure-Free Days for Primary Seizure Types - Males

Percentage of seizure free days (primary seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=11	Baseline	11	0 (0.0)	8.53 (12.814)	0.00 (0.00, 31.71)	0.00, 11.90	0.0, 34.1			
	17-Week Post-Baseline Phase	11	0 (0.0)	18.36 (21.037)	3.75 (0.00, 46.15)	0.00, 40.17	0.0, 51.3			
	Arithmetic Change from Baseline	11	0 (0.0)	9.83 (21.362)	1.71 (0.00, 43.61)	0.00, 23.04	-28.0, 46.2			
Placebo, N=10	Baseline	10	0 (0.0)	13.20 (16.220)	4.76 (0.00, 35.71)	0.00, 27.27	0.0, 40.5	0.00 (-19.05, 7.69)	0.5684	0.5705
	17-Week Post-Baseline Phase	10	0 (0.0)	17.50 (21.136)	13.55 (0.00, 32.74)	0.00, 26.17	0.0, 66.7	0.00 (-19.66, 25.14)	0.9143	-0.1076
	Arithmetic Change from Baseline	10	0 (0.0)	4.30 (10.619)	0.00 (-2.97, 18.97)	-1.10, 8.13	-8.5, 26.2	2.26 (-8.13, 19.96)	0.3699	-0.8967

Study No. 1042-CDD-3001

Table 14.2.5.2 Summary of the Percentage of Seizure-Free Days for All Seizure Types - Patients who are 2-6 years old

Percentage of seizure free days (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=30	Baseline	29	1 (3.3)	12.59 (21.286)	0.00 (0.00, 9.52)	0.00, 25.00	0.0, 75.6			
	17-Week Post-Baseline Phase	30	0 (0.0)	16.95 (26.907)	1.27 (0.00, 15.83)	0.00, 23.08	0.0, 98.2			
	Arithmetic Change from Baseline	29	1 (3.3)	4.20 (13.300)	0.00 (0.00, 3.85)	0.00, 6.78	-29.4, 44.0			
Placebo, N=22	Baseline	22	0 (0.0)	22.54 (24.254)	14.93 (2.38, 40.48)	0.00, 40.48	0.0, 81.0	-6.77 (-19.05, 0.00)	0.0366	2.0900
	17-Week Post-Baseline Phase	22	0 (0.0)	33.56 (27.487)	32.76 (12.61, 61.86)	0.86, 61.86	0.0, 72.2	-18.33 (-32.77, 0.00)	0.0506	1.9549
	Arithmetic Change from Baseline	22	0 (0.0)	11.02 (15.370)	11.58 (0.00, 20.95)	-2.38, 20.95	-10.8, 41.4	-8.03 (-17.55, 2.38)	0.2959	1.0452

Study No. 1042-CDD-3001

Table 14.2.5.2 Summary of the Percentage of Seizure-Free Days for All Seizure Types - Patients who are >6 years old

Percentage of seizure free days (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=20	Baseline	20	0 (0.0)	11.13 (15.723)	4.96 (0.00, 17.07)	0.00, 17.11	0.0, 66.7			
	17-Week Post-Baseline Phase	20	0 (0.0)	21.28 (24.091)	12.17 (1.72, 40.21)	1.29, 40.79	0.0, 69.2			
	Arithmetic Change from Baseline	20	0 (0.0)	10.16 (19.291)	1.35 (0.00, 18.78)	-0.46, 20.25	-9.1, 56.2			
Placebo, N=29	Baseline	29	0 (0.0)	20.92 (22.142)	16.22 (2.38, 34.48)	0.00, 35.71	0.0, 77.5	-6.70 (-17.34, 0.07)	0.2065	-1.2634
	17-Week Post-Baseline Phase	29	0 (0.0)	25.43 (29.282)	11.86 (1.74, 37.39)	0.00, 41.88	0.0, 89.7	0.00 (-13.49, 8.77)	0.8617	-0.1742
	Arithmetic Change from Baseline	29	0 (0.0)	4.51 (14.502)	0.00 (-0.64, 4.95)	-2.24, 5.67	-19.5, 53.9	1.72 (-1.99, 7.70)	0.3007	1.0348

Study No. 1042-CDD-3001

Table 14.2.5.2 Summary of the Percentage of Seizure-Free Days for All Seizure Types - Patients not in US

Percentage of seizure free days (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=32	Baseline	32	0 (0.0)	12.65 (21.343)	0.00 (0.00, 11.90)	0.00, 20.38	0.0, 75.6			
	17-Week Post-Baseline Phase	32	0 (0.0)	18.99 (27.382)	5.31 (0.00, 15.83)	0.00, 30.14	0.0, 98.2			
	Arithmetic Change from Baseline	32	0 (0.0)	6.34 (16.558)	0.47 (0.00, 3.85)	0.00, 8.05	-29.4, 56.2			
Placebo, N=27	Baseline	27	0 (0.0)	28.59 (24.628)	23.81 (10.00, 40.48)	9.52, 41.46	0.0, 81.0	-13.61 (-29.56, -2.38)	0.0032	2.9493
	17-Week Post-Baseline Phase	27	0 (0.0)	39.44 (29.835)	36.84 (19.20, 66.67)	4.39, 67.11	0.0, 89.7	-19.76 (-40.99, -2.06)	0.0056	2.7724
	Arithmetic Change from Baseline	27	0 (0.0)	10.85 (17.090)	7.88 (0.00, 20.95)	-2.24, 26.19	-19.5, 53.9	-4.06 (-13.61, 2.97)	0.4864	0.6960

Study No. 1042-CDD-3001

Table 14.2.5.2 Summary of the Percentage of Seizure-Free Days for All Seizure Types - Patients in US

Percentage of seizure free days (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=18	Baseline	17	1 (5.6)	10.75 (14.246)	4.88 (0.00, 17.14)	0.00, 17.14	0.0, 50.0			
	17-Week Post-Baseline Phase	18	0 (0.0)	18.15 (23.005)	3.56 (0.81, 40.21)	0.81, 40.21	0.0, 75.0			
	Arithmetic Change from Baseline	17	1 (5.6)	7.18 (15.688)	0.00 (-0.93, 17.00)	-0.93, 17.00	-8.7, 44.0			
Placebo, N=24	Baseline	24	0 (0.0)	13.78 (18.126)	5.98 (0.00, 19.05)	0.00, 20.24	0.0, 63.2	0.00 (-9.52, 4.88)	0.8154	-0.2334
	17-Week Post-Baseline Phase	24	0 (0.0)	17.12 (22.100)	9.50 (0.00, 26.50)	0.00, 29.63	0.0, 79.8	0.81 (-10.11, 9.23)	0.5257	0.6345
	Arithmetic Change from Baseline	24	0 (0.0)	3.35 (11.556)	0.00 (-0.98, 1.47)	-2.38, 5.82	-10.8, 41.4	0.98 (-2.38, 7.25)	0.4137	0.8174

Study No. 1042-CDD-3001

Table 14.2.5.2 Summary of the Percentage of Seizure-Free Days for All Seizure Types - Females

Percentage of seizure free days (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=39	Baseline	38	1 (2.6)	15.20 (20.560)	4.76 (0.00, 17.14)	0.00, 23.81	0.0, 75.6			
	17-Week Post-Baseline Phase	39	0 (0.0)	23.14 (27.239)	9.92 (1.72, 35.78)	0.86, 42.00	0.0, 98.2			
	Arithmetic Change from Baseline	38	1 (2.6)	7.98 (17.767)	0.98 (0.00, 6.78)	0.00, 17.00	-29.4, 56.2			
Placebo, N=41	Baseline	41	0 (0.0)	24.33 (23.611)	17.07 (9.52, 35.71)	2.38, 40.48	0.0, 81.0	-8.43 (-16.67, 0.00)	0.0420	-2.0337
	17-Week Post-Baseline Phase	41	0 (0.0)	32.35 (29.198)	26.50 (11.86, 50.91)	1.74, 61.86	0.0, 89.7	-4.62 (-21.17, 0.87)	0.2121	-1.2477
	Arithmetic Change from Baseline	41	0 (0.0)	8.02 (16.015)	0.00 (0.00, 11.81)	-2.38, 17.62	-19.5, 53.9	0.48 (-6.28, 4.80)	0.6720	0.4234

Study No. 1042-CDD-3001

Table 14.2.5.2 Summary of the Percentage of Seizure-Free Days for All Seizure Types - Males

Percentage of seizure free days (all seizure types)

									Wilcoxon Test	
Treatment	**Interval**	**n**	**Missing n (%)**	**Mean (SD)**	**Median (95% Distribution-Free CI)**	**Q1, Q3**	**Min, Max**	**Hodges-Lehmann Estimate of Location Shift (95% CI)**	**p-value**	**Zvalue**
GNX, N=11	Baseline	11	0 (0.0)	0.92 (1.679)	0.00 (0.00, 2.78)	0.00, 2.44	0.0, 4.9			
	17-Week Post-Baseline Phase	11	0 (0.0)	2.88 (7.287)	0.00 (0.00, 3.85)	0.00, 2.50	0.0, 24.5			
	Arithmetic Change from Baseline	11	0 (0.0)	1.97 (6.759)	0.00 (0.00, 3.85)	0.00, 0.87	-2.4, 21.7			
Placebo, N=10	Baseline	10	0 (0.0)	10.48 (15.881)	0.00 (0.00, 35.71)	0.00, 19.05	0.0, 40.5	0.00 (-16.61, 0.00)	0.2943	1.0487
	17-Week Post-Baseline Phase	10	0 (0.0)	14.92 (21.495)	5.17 (0.00, 32.74)	0.00, 20.51	0.0, 66.7	-1.00 (-19.64, 0.00)	0.1594	1.4071
	Arithmetic Change from Baseline	10	0 (0.0)	4.45 (10.640)	0.00 (-2.97, 18.97)	0.00, 9.35	-8.5, 26.2	0.00 (-9.35, 2.97)	0.7374	0.3354

Study No. 1042-CDD-3001

Table 14.2.3.3.1 Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID) - Patients who are 2-6 years old

Visit		Responder	Ganaxolone N=30	Placebo N=22	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	28	21				
	Missing, n (%)	.	2 (6.7)	1 (4.5)				
	CGI-CSID	No deterioration	26 (92.9)	20 (95.2)				
		Deterioration	2 (7.1)	1 (4.8)	1.54 (0.13, 18.19)	1.50 (0.15, 15.46)	2.4 (-26.0, 30.5)	1.0000
		No improvement	4 (14.3)	9 (42.9)				
		Improvement	24 (85.7)	12 (57.1)	4.50 (1.15, 17.65)	1.50 (1.01, 2.24)	28.6 (-0.3, 54.1)	0.0476
Visit 4 (End of Week 9)	Total observed, n	.	27	21				
	Missing, n (%)	.	3 (10.0)	1 (4.5)				
	CGI-CSID	No deterioration	22 (81.5)	20 (95.2)				
		Deterioration	5 (18.5)	1 (4.8)	4.55 (0.49, 42.31)	3.89 (0.49, 30.82)	13.8 (-15.1, 40.9)	0.2115
		No improvement	9 (33.3)	11 (52.4)				
		Improvement	18 (66.7)	10 (47.6)	2.20 (0.68, 7.10)	1.40 (0.83, 2.36)	19.0 (-9.8, 45.8)	0.2425
Visit 5 (End of Week 17)	Total observed, n	.	26	21				
	Missing, n (%)	.	4 (13.3)	1 (4.5)				
	CGI-CSID	No deterioration	21 (80.8)	16 (76.2)				
		Deterioration	5 (19.2)	5 (23.8)	0.76 (0.19, 3.09)	0.81 (0.27, 2.42)	-4.6 (-32.6, 23.6)	0.7339
		No improvement	10 (38.5)	13 (61.9)				
		Improvement	16 (61.5)	8 (38.1)	2.60 (0.80, 8.49)	1.62 (0.87, 3.02)	23.4 (-5.9, 49.9)	0.1468
Final OL/Taper Visit	Total observed, n	.	2					
	CGI-CSID	No deterioration	2 (100.0)	0				
		Deterioration			0.20 (0.00, 28.47)	0.33 (0.01, 8.18)		
		No improvement	1 (50.0)	0				
		Improvement	1 (50.0)	0	1.00 (0.01, 92.42)	1.00 (0.10, 9.61)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)

Study No. 1042-CDD-3001

Table 14.2.3.3.1 Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID) - Patients who are >6 years old

Visit		Responder	Ganaxolone N=20	Placebo N=29	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	20	27				
	Missing, n (%)	.	0 (0.0)	2 (6.9)				
	CGI-CSID	No deterioration	20 (100.0)	25 (92.6)				
		Deterioration	0	2 (7.4)	0.25 (0.01, 5.48)	0.27 (0.01, 5.27)	-7.4 (-35.2, 21.2)	0.5005
		No improvement	4 (20.0)	16 (59.3)				
		Improvement	16 (80.0)	11 (40.7)	5.82 (1.53, 22.17)	1.96 (1.19, 3.25)	39.3 (10.7, 63.4)	0.0089
Visit 4 (End of Week 9)	Total observed, n	.	19	27				
	Missing, n (%)	.	1 (5.0)	2 (6.9)				
	CGI-CSID	No deterioration	16 (84.2)	22 (81.5)				
		Deterioration	3 (15.8)	5 (18.5)	0.83 (0.17, 3.96)	0.85 (0.23, 3.15)	-2.7 (-31.0, 26.2)	1.0000
		No improvement	8 (42.1)	18 (66.7)				
		Improvement	11 (57.9)	9 (33.3)	2.75 (0.82, 9.24)	1.74 (0.90, 3.35)	24.6 (-5.2, 51.4)	0.1347
Visit 5 (End of Week 17)	Total observed, n	.	19	26				
	Missing, n (%)	.	1 (5.0)	3 (10.3)				
	CGI-CSID	No deterioration	17 (89.5)	22 (84.6)				
		Deterioration	2 (10.5)	4 (15.4)	0.65 (0.11, 3.96)	0.68 (0.14, 3.36)	-4.9 (-33.1, 24.5)	1.0000
		No improvement	7 (36.8)	17 (65.4)				
		Improvement	12 (63.2)	9 (34.6)	3.24 (0.94, 11.12)	1.82 (0.97, 3.43)	28.5 (-1.6, 55.0)	0.0752
Final OL/Taper Visit	Total observed, n	.	1					
	CGI-CSID	Deterioration	1 (100.0)	0	3.00 (0.02, 473.06)	1.50 (0.18, 12.46)		
		No improvement	1 (100.0)	0				
		Improvement			0.33 (0.00, 52.56)	0.50 (0.02, 11.09)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)

Study No. 1042-CDD-3001

Table 14.2.3.3.1 Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID) - Patients not in US

Visit		Responder	Ganaxolone N=32	Placebo N=27	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	31	26				
	Missing, n (%)	.	1 (3.1)	1 (3.7)				
	CGI-CSID	No deterioration	29 (93.5)	26 (100.0)				
		Deterioration	2 (6.5)	0	4.49 (0.21, 97.86)	4.22 (0.21, 84.13)	6.5 (-19.4, 31.7)	0.4950
		No improvement	7 (22.6)	12 (46.2)				
		Improvement	24 (77.4)	14 (53.8)	2.94 (0.94, 9.20)	1.44 (0.96, 2.15)	23.6 (-2.3, 47.6)	0.0907
Visit 4 (End of Week 9)	Total observed, n	.	30	26				
	Missing, n (%)	.	2 (6.3)	1 (3.7)				
	CGI-CSID	No deterioration	25 (83.3)	22 (84.6)				
		Deterioration	5 (16.7)	4 (15.4)	1.10 (0.26, 4.62)	1.08 (0.32, 3.62)	1.3 (-24.5, 27.2)	1.0000
		No improvement	14 (46.7)	15 (57.7)				
		Improvement	16 (53.3)	11 (42.3)	1.56 (0.54, 4.49)	1.26 (0.72, 2.21)	11.0 (-15.6, 36.6)	0.4357
Visit 5 (End of Week 17)	Total observed, n	.	30	26				
	Missing, n (%)	.	2 (6.3)	1 (3.7)				
	CGI-CSID	No deterioration	24 (80.0)	23 (88.5)				
		Deterioration	6 (20.0)	3 (11.5)	1.92 (0.43, 8.58)	1.73 (0.48, 6.25)	8.5 (-17.7, 33.9)	0.4808
		No improvement	15 (50.0)	16 (61.5)				
		Improvement	15 (50.0)	10 (38.5)	1.60 (0.55, 4.65)	1.30 (0.71, 2.38)	11.5 (-14.9, 37.0)	0.4296
Final OL/Taper Visit	Total observed, n	.	1					
	CGI-CSID	Deterioration	1 (100.0)	0	3.00 (0.02, 473.06)	1.50 (0.18, 12.46)		
		No improvement	1 (100.0)	0				
		Improvement			0.33 (0.00, 52.56)	0.50 (0.02, 11.09)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)

Study No. 1042-CDD-3001

Table 14.2.3.3.1 Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID) - Patients in US

Visit		Responder	Ganaxolone N=18	Placebo N=24	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	17	22				
	Missing, n (%)	.	1 (5.6)	2 (8.3)				
	CGI-CSID	No deterioration	17 (100.0)	19 (86.4)				
		Deterioration	0	3 (13.6)	0.16 (0.01, 3.30)	0.18 (0.01, 3.31)	-13.6 (-43.5, 17.5)	0.2429
		No improvement	1 (5.9)	13 (59.1)				
		Improvement	16 (94.1)	9 (40.9)	23.11 (2.58, 206.87)	2.30 (1.37, 3.85)	53.2 (22.7, 77.0)	0.0007
Visit 4 (End of Week 9)	Total observed, n	.	16	22				
	Missing, n (%)	.	2 (11.1)	2 (8.3)				
	CGI-CSID	No deterioration	13 (81.3)	20 (90.9)				
		Deterioration	3 (18.8)	2 (9.1)	2.31 (0.34, 15.75)	2.06 (0.39, 10.95)	9.7 (-22.6, 40.4)	0.6318
		No improvement	3 (18.8)	14 (63.6)				
		Improvement	13 (81.3)	8 (36.4)	7.58 (1.65, 34.90)	2.23 (1.23, 4.07)	44.9 (12.9, 70.1)	0.0088
Visit 5 (End of Week 17)	Total observed, n	.	15	21				
	Missing, n (%)	.	3 (16.7)	3 (12.5)				
	CGI-CSID	No deterioration	14 (93.3)	15 (71.4)				
		Deterioration	1 (6.7)	6 (28.6)	0.18 (0.02, 1.68)	0.23 (0.03, 1.74)	-21.9 (-52.2, 12.0)	0.2003
		No improvement	2 (13.3)	14 (66.7)				
		Improvement	13 (86.7)	7 (33.3)	13.00 (2.27, 74.32)	2.60 (1.38, 4.91)	53.3 (21.0, 78.7)	0.0022
Final OL/Taper Visit	Total observed, n	.	2					
	CGI-CSID	No deterioration	2 (100.0)	0				
		Deterioration			0.20 (0.00, 28.47)	0.33 (0.01, 8.18)		

Visit		Responder	Ganaxolone N=18	Placebo N=24	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
		No improvement	1 (50.0)	0				
		Improvement	1 (50.0)	0	1.00 (0.01, 92.42)	1.00 (0.10, 9.61)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)
Study No. 1042-CDD-3001

Table 14.2.3.3.1 Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID) - Females

Visit		Responder	Ganaxolone N=39	Placebo N=41	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	38	39				
	Missing, n (%)	.	1 (2.6)	2 (4.9)				
	CGI-CSID	No deterioration	37 (97.4)	36 (92.3)				
		Deterioration	1 (2.6)	3 (7.7)	0.32 (0.03, 3.26)	0.34 (0.04, 3.15)	-5.1 (-27.0, 17.0)	0.6151
		No improvement	7 (18.4)	22 (56.4)				
		Improvement	31 (81.6)	17 (43.6)	5.73 (2.03, 16.15)	1.87 (1.27, 2.76)	38.0 (16.7, 57.8)	0.0009
Visit 4 (End of Week 9)	Total observed, n	.	36	39				
	Missing, n (%)	.	3 (7.7)	2 (4.9)				
	CGI-CSID	No deterioration	29 (80.6)	35 (89.7)				
		Deterioration	7 (19.4)	4 (10.3)	2.11 (0.56, 7.93)	1.90 (0.61, 5.94)	9.2 (-13.9, 31.5)	0.3350
		No improvement	14 (38.9)	24 (61.5)				
		Improvement	22 (61.1)	15 (38.5)	2.51 (0.99, 6.37)	1.59 (0.99, 2.55)	22.6 (-0.5, 44.0)	0.0657
Visit 5 (End of Week 17)	Total observed, n	.	35	39				
	Missing, n (%)	.	4 (10.3)	2 (4.9)				
	CGI-CSID	No deterioration	29 (82.9)	33 (84.6)				
		Deterioration	6 (17.1)	6 (15.4)	1.14 (0.33, 3.92)	1.11 (0.40, 3.14)	1.8 (-21.1, 24.1)	1.0000
		No improvement	16 (45.7)	24 (61.5)				
		Improvement	19 (54.3)	15 (38.5)	1.90 (0.75, 4.80)	1.41 (0.86, 2.33)	15.8 (-7.4, 37.7)	0.2430

Visit		Responder	Ganaxolone N=39	Placebo N=41	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Final OL/Taper Visit	Total observed, n	.	3					
	CGI-CSID	No deterioration	2 (66.7)	0				
		Deterioration	1 (33.3)	0	0.60 (0.01, 49.45)	0.75 (0.07, 7.73)		
		No improvement	2 (66.7)	0				
		Improvement	1 (33.3)	0	0.60 (0.01, 49.45)	0.75 (0.07, 7.73)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)
Study No. 1042-CDD-3001
Table 14.2.3.3.1 Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID) - Males

Visit		Responder	Ganaxolone N=11	Placebo N=10	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	10	9				
	Missing, n (%)	.	1 (9.1)	1 (10.0)				
	CGI-CSID	No deterioration	9 (90.0)	9 (100.0)				
		Deterioration	1 (10.0)	0	3.00 (0.11, 83.36)	2.73 (0.12, 59.57)	10.0 (-33.3, 51.4)	1.0000
		No improvement	1 (10.0)	3 (33.3)				
		Improvement	9 (90.0)	6 (66.7)	4.50 (0.37, 54.16)	1.35 (0.81, 2.24)	23.3 (-23.6, 61.4)	0.3034
Visit 4 (End of Week 9)	Total observed, n	.	10	9				
	Missing, n (%)	.	1 (9.1)	1 (10.0)				
	CGI-CSID	No deterioration	9 (90.0)	7 (77.8)				
		Deterioration	1 (10.0)	2 (22.2)	0.39 (0.03, 5.21)	0.45 (0.05, 4.16)	-12.2 (-51.4, 33.3)	0.5820
		No improvement	3 (30.0)	5 (55.6)				
		Improvement	7 (70.0)	4 (44.4)	2.92 (0.44, 19.23)	1.58 (0.68, 3.63)	25.6 (-22.4, 65.3)	0.3698
Visit 5 (End of Week 17)	Total observed, n	.	10	8				
	Missing, n (%)	.	1 (9.1)	2 (20.0)				

Visit		Responder	Ganaxolone N=11	Placebo N=10	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
	CGI-CSID	No deterioration	9 (90.0)	5 (62.5)				
		Deterioration	1 (10.0)	3 (37.5)	0.19 (0.01, 2.29)	0.27 (0.03, 2.10)	-27.5 (-66.8, 19.3)	0.2745
		No improvement	1 (10.0)	6 (75.0)				
		Improvement	9 (90.0)	2 (25.0)	27.00 (1.98, 368.38)	3.60 (1.07, 12.17)	65.0 (17.4, 91.8)	0.0128

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)

Study No. 1042-CDD-3001

Table 14.2.4.1.1 Summary of Caregiver Global Impression of Change in Attention (CGICA) - Patients who are 2-6 years old

Visit		Responder	Ganaxolone N=30	Placebo N=22	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	28	21				
	Missing, n (%)	.	2 (6.7)	1 (4.5)				
	CGICA	No deterioration	28 (100.0)	21 (100.0)				
		Deterioration	0	0	0.75 (0.01, 39.56)	0.76 (0.02, 36.76)		
		No improvement	13 (46.4)	17 (81.0)				
		Improvement	15 (53.6)	4 (19.0)	4.90 (1.31, 18.33)	2.81 (1.09, 7.25)	34.5 (5.8, 59.1)	0.0190
Visit 4 (End of Week 9)	Total observed, n	.	27	21				
	Missing, n (%)	.	3 (10.0)	1 (4.5)				
	CGICA	No deterioration	26 (96.3)	19 (90.5)				
		Deterioration	1 (3.7)	2 (9.5)	0.37 (0.03, 4.33)	0.39 (0.04, 4.00)	-5.8 (-33.7, 22.5)	0.5738
		No improvement	10 (37.0)	13 (61.9)				
		Improvement	17 (63.0)	8 (38.1)	2.76 (0.85, 8.97)	1.65 (0.89, 3.06)	24.9 (-4.2, 50.9)	0.1449
Visit 5 (End of Week 17)	Total observed, n	.	26	21				
	Missing, n (%)	.	4 (13.3)	1 (4.5)				
	CGICA	No deterioration	23 (88.5)	21 (100.0)				
		Deterioration	3 (11.5)	0	6.40 (0.31, 131.28)	5.70 (0.31, 104.62)	11.5 (-17.2, 38.8)	0.2424
		No improvement	12 (46.2)	11 (52.4)				
		Improvement	14 (53.8)	10 (47.6)	1.28 (0.41, 4.06)	1.13 (0.64, 2.00)	6.2 (-22.7, 34.4)	0.7725
Final OL/Taper Visit	Total observed, n	.	2	0				
	CGICA	No deterioration	2 (100.0)	0				
		Deterioration	0	0	0.20 (0.00, 28.47)	0.33 (0.01, 8.18)		
		No improvement	1 (50.0)	0				
		Improvement	1 (50.0)	0	1.00 (0.01, 92.42)	1.00 (0.10, 9.61)		

Improvement defined as any improvement (minimally, much or very much improved)

Deterioration defined as any deterioration (minimally, much or very much worse)
Study No. 1042-CDD-3001
Table 14.2.4.1.1 Summary of Caregiver Global Impression of Change in Attention (CGICA) - Patients who are >6 years old

Visit		Responder	Ganaxolone N=20	Placebo N=29	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	19	27				
	Missing, n (%)	.	1 (5.0)	2 (6.9)				
	CGICA	No deterioration	16 (84.2)	27 (100.0)				
		Deterioration	3 (15.8)	0	11.67 (0.57, 240.36)	9.80 (0.54, 179.38)	15.8 (-13.4, 43.5)	0.0638
		No improvement	14 (73.7)	17 (63.0)				
		Improvement	5 (26.3)	10 (37.0)	0.61 (0.17, 2.20)	0.71 (0.29, 1.75)	-10.7 (-38.8, 18.7)	0.5329
Visit 4 (End of Week 9)	Total observed, n	.	19	27				
	Missing, n (%)	.	1 (5.0)	2 (6.9)				
	CGICA	No deterioration	16 (84.2)	27 (100.0)				
		Deterioration	3 (15.8)	0	11.67 (0.57, 240.36)	9.80 (0.54, 179.38)	15.8 (-13.4, 43.5)	0.0638
		No improvement	11 (57.9)	18 (66.7)				
		Improvement	8 (42.1)	9 (33.3)	1.45 (0.43, 4.89)	1.26 (0.60, 2.67)	8.8 (-20.4, 37.3)	0.7571
Visit 5 (End of Week 17)	Total observed, n	.	19	26				
	Missing, n (%)	.	1 (5.0)	3 (10.3)				
	CGICA	No deterioration	19 (100.0)	24 (92.3)				
		Deterioration	0	2 (7.7)	0.25 (0.01, 5.54)	0.27 (0.01, 5.32)	-7.7 (-36.2, 21.9)	0.5010
		No improvement	9 (47.4)	14 (53.8)				
		Improvement	10 (52.6)	12 (46.2)	1.30 (0.40, 4.24)	1.14 (0.63, 2.07)	6.5 (-23.3, 35.4)	0.7666
Final OL/Taper Visit	Total observed, n	.	1	0				
	CGICA	Deterioration	1 (100.0)	0	3.00 (0.02, 473.06)	1.50 (0.18, 12.46)		
		No improvement	1 (100.0)	0				
		Improvement	0	0	0.33 (0.00, 52.56)	0.50 (0.02, 11.09)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)
Study No. 1042-CDD-3001

Table 14.2.4.1.1 Summary of Caregiver Global Impression of Change in Attention (CGICA) - Patients not in US

Visit		Responder	Ganaxolone N=32	Placebo N=27	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	31	26				
	Missing, n (%)	.	1 (3.1)	1 (3.7)				
	CGICA	No deterioration	29 (93.5)	26 (100.0)				
		Deterioration	2 (6.5)	0	4.49 (0.21, 97.86)	4.22 (0.21, 84.13)	6.5 (-19.4, 31.7)	0.4950
		No improvement	21 (67.7)	18 (69.2)				
		Improvement	10 (32.3)	8 (30.8)	1.07 (0.35, 3.29)	1.05 (0.49, 2.26)	1.5 (-24.5, 26.9)	1.0000
Visit 4 (End of Week 9)	Total observed, n	.	30	26				
	Missing, n (%)	.	2 (6.3)	1 (3.7)				
	CGICA	No deterioration	27 (90.0)	25 (96.2)				
		Deterioration	3 (10.0)	1 (3.8)	2.78 (0.27, 28.48)	2.60 (0.29, 23.50)	6.2 (-20.3, 31.8)	0.6151
		No improvement	14 (46.7)	14 (53.8)				
		Improvement	16 (53.3)	12 (46.2)	1.33 (0.47, 3.82)	1.16 (0.68, 1.97)	7.2 (-19.4, 33.0)	0.7891
Visit 5 (End of Week 17)	Total observed, n	.	30	26				
	Missing, n (%)	.	2 (6.3)	1 (3.7)				
	CGICA	No deterioration	28 (93.3)	24 (92.3)				
		Deterioration	2 (6.7)	2 (7.7)	0.86 (0.11, 6.56)	0.87 (0.13, 5.73)	-1.0 (-27.2, 25.0)	1.0000
		No improvement	16 (53.3)	15 (57.7)				
		Improvement	14 (46.7)	11 (42.3)	1.19 (0.41, 3.44)	1.10 (0.61, 1.99)	4.4 (-21.9, 30.4)	0.7925
Final OL/Taper Visit	Total observed, n	.	1	0				
	CGICA	Deterioration	1 (100.0)	0	3.00 (0.02, 473.06)	1.50 (0.18, 12.46)		
		No improvement	1 (100.0)	0				
		Improvement	0	0	0.33 (0.00, 52.56)	0.50 (0.02, 11.09)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)

Study No. 1042-CDD-3001

Table 14.2.4.1.1 Summary of Caregiver Global Impression of Change in Attention (CGICA) - Patients in US

Visit		Responder	Ganaxolone N=18	Placebo N=24	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	16	22				
	Missing, n (%)	.	2 (11.1)	2 (8.3)				
	CGICA	No deterioration	15 (93.8)	22 (100.0)				
		Deterioration	1 (6.3)	0	4.35 (0.17, 114.05)	4.06 (0.18, 93.64)	6.3 (-26.1, 37.6)	0.4211
		No improvement	6 (37.5)	16 (72.7)				
		Improvement	10 (62.5)	6 (27.3)	4.44 (1.12, 17.67)	2.29 (1.05, 5.00)	35.2 (2.5, 62.7)	0.0471
Visit 4 (End of Week 9)	Total observed, n	.	16	22				
	Missing, n (%)	.	2 (11.1)	2 (8.3)				
	CGICA	No deterioration	15 (93.8)	21 (95.5)				
		Deterioration	1 (6.3)	1 (4.5)	1.40 (0.08, 24.20)	1.38 (0.09, 20.38)	1.7 (-30.4, 33.4)	1.0000
		No improvement	7 (43.8)	17 (77.3)				
		Improvement	9 (56.3)	5 (22.7)	4.37 (1.07, 17.79)	2.47 (1.02, 5.99)	33.5 (1.0, 61.3)	0.0468
Visit 5 (End of Week 17)	Total observed, n	.	15	21				
	Missing, n (%)	.	3 (16.7)	3 (12.5)				
	CGICA	No deterioration	14 (93.3)	21 (100.0)				
		Deterioration	1 (6.7)	0	4.45 (0.17, 116.94)	4.13 (0.18, 94.84)	6.7 (-26.2, 38.9)	0.4167
		No improvement	5 (33.3)	10 (47.6)				
		Improvement	10 (66.7)	11 (52.4)	1.82 (0.46, 7.18)	1.27 (0.74, 2.19)	14.3 (-19.4, 45.2)	0.5006
Final OL/Taper Visit	Total observed, n	.	2	0				
	CGICA	No deterioration	2 (100.0)	0				
		Deterioration	0	0	0.20 (0.00, 28.47)	0.33 (0.01, 8.18)		
		No improvement	1 (50.0)	0				

Visit		Responder	Ganaxolone N=18	Placebo N=24	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
		Improvement	1 (50.0)	0	1.00 (0.01, 92.42)	1.00 (0.10, 9.61)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)
Study No. 1042-CDD-3001
Table 14.2.4.1.1 Summary of Caregiver Global Impression of Change in Attention (CGICA) - Females

Visit		Responder	Ganaxolone N=39	Placebo N=41	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	37	39				
	Missing, n (%)	.	2 (5.1)	2 (4.9)				
	CGICA	No deterioration	34 (91.9)	39 (100.0)				
		Deterioration	3 (8.1)	0	8.01 (0.40, 160.68)	7.37 (0.39, 137.96)	8.1 (-14.5, 30.5)	0.1105
		No improvement	20 (54.1)	26 (66.7)				
		Improvement	17 (45.9)	13 (33.3)	1.70 (0.67, 4.30)	1.38 (0.78, 2.43)	12.6 (-10.6, 34.1)	0.3484
Visit 4 (End of Week 9)	Total observed, n	.	36	39				
	Missing, n (%)	.	3 (7.7)	2 (4.9)				
	CGICA	No deterioration	34 (94.4)	37 (94.9)				
		Deterioration	2 (5.6)	2 (5.1)	1.09 (0.15, 8.16)	1.08 (0.16, 7.29)	0.4 (-22.4, 22.6)	1.0000
		No improvement	14 (38.9)	24 (61.5)				
		Improvement	22 (61.1)	15 (38.5)	2.51 (0.99, 6.37)	1.59 (0.99, 2.55)	22.6 (-0.5, 44.0)	0.0657
Visit 5 (End of Week 17)	Total observed, n	.	35	39				
	Missing, n (%)	.	4 (10.3)	2 (4.9)				
	CGICA	No deterioration	34 (97.1)	37 (94.9)				
		Deterioration	1 (2.9)	2 (5.1)	0.54 (0.05, 6.28)	0.56 (0.05, 5.88)	-2.3 (-24.8, 20.6)	1.0000
		No improvement	15 (42.9)	18 (46.2)				

Visit		Responder	Ganaxolone N=39	Placebo N=41	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
		Improvement	20 (57.1)	21 (53.8)	1.14 (0.46, 2.86)	1.06 (0.71, 1.60)	3.3 (-19.5, 26.1)	0.8181
Final OL/Taper Visit	Total observed, n	.	3	0				
	CGICA	No deterioration	2 (66.7)	0				
		Deterioration	1 (33.3)	0	0.60 (0.01, 49.45)	0.75 (0.07, 7.73)		
		No improvement	2 (66.7)	0				
		Improvement	1 (33.3)	0	0.60 (0.01, 49.45)	0.75 (0.07, 7.73)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)
Study No. 1042-CDD-3001
Table 14.2.4.1.1 Summary of Caregiver Global Impression of Change in Attention (CGICA) - Males

Visit		Responder	Ganaxolone N=11	Placebo N=10	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	10	9				
	Missing, n (%)	.	1 (9.1)	1 (10.0)				
	CGICA	No deterioration	10 (100.0)	9 (100.0)				
		Deterioration	0	0	0.90 (0.02, 50.24)	0.91 (0.02, 41.68)		
		No improvement	7 (70.0)	8 (88.9)				
		Improvement	3 (30.0)	1 (11.1)	3.43 (0.29, 40.95)	2.70 (0.34, 21.53)	18.9 (-23.6, 59.7)	0.5820
Visit 4 (End of Week 9)	Total observed, n	.	10	9				
	Missing, n (%)	.	1 (9.1)	1 (10.0)				
	CGICA	No deterioration	8 (80.0)	9 (100.0)				
		Deterioration	2 (20.0)	0	5.59 (0.23, 133.61)	4.55 (0.25, 83.70)	20.0 (-23.6, 59.7)	0.4737
		No improvement	7 (70.0)	7 (77.8)				
		Improvement	3 (30.0)	2 (22.2)	1.50 (0.19, 11.93)	1.35 (0.29, 6.34)	7.8 (-34.9, 51.3)	1.0000

Visit		Responder	Ganaxolone N=11	Placebo N=10	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 5 (End of Week 17)	Total observed, n	.	10	8				
	Missing, n (%)	.	1 (9.1)	2 (20.0)				
	CGICA	No deterioration	8 (80.0)	8 (100.0)				
		Deterioration	2 (20.0)	0	5.00 (0.21, 120.44)	4.09 (0.22, 74.78)	20.0 (-28.1, 61.9)	0.4771
		No improvement	6 (60.0)	7 (87.5)				
		Improvement	4 (40.0)	1 (12.5)	4.67 (0.40, 53.95)	3.20 (0.44, 23.28)	27.5 (-19.3, 66.8)	0.3137

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)

Study No. 1042-CDD-3001

Table 14.2.4.2.1 Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C) - Patients who are 2-6 years old

Visit		Responder	Ganaxolone N=30	Placebo N=22	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	28	21				
	Missing, n (%)	.	2 (6.7)	1 (4.5)				
	CGI-C	No deterioration	27 (96.4)	18 (85.7)				
		Deterioration	1 (3.6)	3 (14.3)	0.22 (0.02, 2.31)	0.25 (0.03, 2.24)	-10.7 (-38.2, 18.0)	0.3006
		No improvement	11 (39.3)	16 (76.2)				
		Improvement	17 (60.7)	5 (23.8)	4.95 (1.41, 17.41)	2.55 (1.12, 5.80)	36.9 (8.3, 61.2)	0.0193
Visit 4 (End of Week 9)	Total observed, n	.	27	21				
	Missing, n (%)	.	3 (10.0)	1 (4.5)				
	CGI-C	No deterioration	27 (100.0)	19 (90.5)				
		Deterioration	0	2 (9.5)	0.14 (0.01, 3.12)	0.16 (0.01, 3.11)	-9.5 (-37.1, 18.9)	0.1862
		No improvement	8 (29.6)	14 (66.7)				
		Improvement	19 (70.4)	7 (33.3)	4.75 (1.39, 16.20)	2.11 (1.10, 4.05)	37.0 (8.3, 61.3)	0.0188
Visit 5 (End of Week 17)	Total observed, n	.	26	20				
	Missing, n (%)	.	4 (13.3)	2 (9.1)				
	CGI-C	No deterioration	24 (92.3)	20 (100.0)				
		Deterioration	2 (7.7)	0	4.18 (0.19, 92.18)	3.89 (0.20, 76.74)	7.7 (-21.6, 36.1)	0.4976
		No improvement	12 (46.2)	11 (55.0)				
		Improvement	14 (53.8)	9 (45.0)	1.43 (0.44, 4.60)	1.20 (0.66, 2.18)	8.8 (-20.6, 37.1)	0.7666
Final OL/Taper Visit	Total observed, n	.	2	0				
	CGI-C	No deterioration	2 (100.0)	0				
		Deterioration	0	0	0.20 (0.00, 28.47)	0.33 (0.01, 8.18)		
		No improvement	1 (50.0)	0				
		Improvement	1 (50.0)	0	1.00 (0.01, 92.42)	1.00 (0.10, 9.61)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)

Study No. 1042-CDD-3001

Table 14.2.4.2.1 Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C) - Patients who are >6 years old

Visit		Responder	Ganaxolone N=20	Placebo N=29	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	19	27				
	Missing, n (%)	.	1 (5.0)	2 (6.9)				
	CGI-C	No deterioration	16 (84.2)	25 (92.6)				
		Deterioration	3 (15.8)	2 (7.4)	2.34 (0.35, 15.61)	2.13 (0.39, 11.55)	8.4 (-20.4, 36.7)	0.6351
		No improvement	13 (68.4)	17 (63.0)				
		Improvement	6 (31.6)	10 (37.0)	0.78 (0.23, 2.72)	0.85 (0.37, 1.94)	-5.5 (-33.9, 23.8)	0.7618
Visit 4 (End of Week 9)	Total observed, n	.	18	26				
	Missing, n (%)	.	2 (10.0)	3 (10.3)				
	CGI-C	No deterioration	15 (83.3)	26 (100.0)				
		Deterioration	3 (16.7)	0	11.97 (0.58, 247.39)	9.95 (0.54, 181.60)	16.7 (-14.0, 44.5)	0.0616
		No improvement	11 (61.1)	16 (61.5)				
		Improvement	7 (38.9)	10 (38.5)	1.02 (0.30, 3.50)	1.01 (0.47, 2.15)	0.4 (-28.8, 30.3)	1.0000
Visit 5 (End of Week 17)	Total observed, n	.	19	26				
	Missing, n (%)	.	1 (5.0)	3 (10.3)				
	CGI-C	No deterioration	19 (100.0)	22 (84.6)				
		Deterioration	0	4 (15.4)	0.13 (0.01, 2.53)	0.15 (0.01, 2.63)	-15.4 (-43.0, 14.5)	0.1264
		No improvement	9 (47.4)	15 (57.7)				
		Improvement	10 (52.6)	11 (42.3)	1.52 (0.46, 4.98)	1.24 (0.67, 2.31)	10.3 (-19.6, 38.9)	0.5553
Final OL/Taper Visit	Total observed, n	.	1	0				
	CGI-C	Deterioration	1 (100.0)	0	3.00 (0.02, 473.06)	1.50 (0.18, 12.46)		
		No improvement	1 (100.0)	0				
		Improvement	0	0	0.33 (0.00, 52.56)	0.50 (0.02, 11.09)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)
Study No. 1042-CDD-3001

Table 14.2.4.2.1 Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C) - Patients not in US

Visit		Responder	Ganaxolone N=32	Placebo N=27	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	30	26				
	Missing, n (%)	.	2 (6.3)	1 (3.7)				
	CGI-C	No deterioration	27 (90.0)	23 (88.5)				
		Deterioration	3 (10.0)	3 (11.5)	0.85 (0.16, 4.64)	0.87 (0.19, 3.93)	-1.5 (-27.5, 24.3)	1.0000
		No improvement	16 (53.3)	18 (69.2)				
		Improvement	14 (46.7)	8 (30.8)	1.97 (0.66, 5.91)	1.52 (0.76, 3.03)	15.9 (-10.4, 40.8)	0.2785
Visit 4 (End of Week 9)	Total observed, n	.	30	25				
	Missing, n (%)	.	2 (6.3)	2 (7.4)				
	CGI-C	No deterioration	28 (93.3)	24 (96.0)				
		Deterioration	2 (6.7)	1 (4.0)	1.71 (0.15, 20.10)	1.67 (0.16, 17.32)	2.7 (-23.9, 29.0)	1.0000
		No improvement	13 (43.3)	15 (60.0)				
		Improvement	17 (56.7)	10 (40.0)	1.96 (0.67, 5.76)	1.42 (0.80, 2.51)	16.7 (-10.3, 41.9)	0.2823
Visit 5 (End of Week 17)	Total observed, n	.	30	26				
	Missing, n (%)	.	2 (6.3)	1 (3.7)				
	CGI-C	No deterioration	28 (93.3)	25 (96.2)				
		Deterioration	2 (6.7)	1 (3.8)	1.79 (0.15, 20.91)	1.73 (0.17, 18.04)	2.8 (-23.6, 28.8)	1.0000
		No improvement	18 (60.0)	13 (50.0)				
		Improvement	12 (40.0)	13 (50.0)	0.67 (0.23, 1.93)	0.80 (0.45, 1.43)	-10.0 (-35.5, 16.7)	0.5910
Final OL/Taper Visit	Total observed, n	.	1	0				
	CGI-C	Deterioration	1 (100.0)	0	3.00 (0.02, 473.06)	1.50 (0.18, 12.46)		
		No improvement	1 (100.0)	0				
		Improvement	0	0	0.33 (0.00, 52.56)	0.50 (0.02, 11.09)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)

Study No. 1042-CDD-3001

Table 14.2.4.2.1 Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C) - Patients in US

Visit		Responder	Ganaxolone N=18	Placebo N=24	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	17	22				
	Missing, n (%)	.	1 (5.6)	2 (8.3)				
	CGI-C	No deterioration	16 (94.1)	20 (90.9)				
		Deterioration	1 (5.9)	2 (9.1)	0.63 (0.05, 7.53)	0.65 (0.06, 6.55)	-3.2 (-34.1, 27.5)	1.0000
		No improvement	8 (47.1)	15 (68.2)				
		Improvement	9 (52.9)	7 (31.8)	2.41 (0.65, 8.92)	1.66 (0.78, 3.55)	21.1 (-11.0, 50.4)	0.2094
Visit 4 (End of Week 9)	Total observed, n	.	15	22				
	Missing, n (%)	.	3 (16.7)	2 (8.3)				
	CGI-C	No deterioration	14 (93.3)	21 (95.5)				
		Deterioration	1 (6.7)	1 (4.5)	1.50 (0.09, 26.01)	1.47 (0.10, 21.67)	2.1 (-29.9, 34.1)	1.0000
		No improvement	6 (40.0)	15 (68.2)				
		Improvement	9 (60.0)	7 (31.8)	3.21 (0.82, 12.63)	1.89 (0.90, 3.95)	28.2 (-5.2, 57.4)	0.1071
Visit 5 (End of Week 17)	Total observed, n	.	15	20				
	Missing, n (%)	.	3 (16.7)	4 (16.7)				
	CGI-C	No deterioration	15 (100.0)	17 (85.0)				
		Deterioration	0	3 (15.0)	0.16 (0.01, 3.38)	0.19 (0.01, 3.38)	-15.0 (-46.7, 19.2)	0.2437
		No improvement	3 (20.0)	13 (65.0)				
		Improvement	12 (80.0)	7 (35.0)	7.43 (1.56, 35.48)	2.29 (1.19, 4.37)	45.0 (11.4, 71.4)	0.0155
Final OL/Taper Visit	Total observed, n	.	2	0				
	CGI-C	No deterioration	2 (100.0)	0				
		Deterioration	0	0	0.20 (0.00, 28.47)	0.33 (0.01, 8.18)		
		No improvement	1 (50.0)	0				
		Improvement	1 (50.0)	0	1.00 (0.01, 92.42)	1.00 (0.10, 9.61)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)
Study No. 1042-CDD-3001
Table 14.2.4.2.1 Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C) - Females

Visit		Responder	Ganaxolone N=39	Placebo N=41	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	37	39				
	Missing, n (%)	.	2 (5.1)	2 (4.9)				
	CGI-C	No deterioration	35 (94.6)	37 (94.9)				
		Deterioration	2 (5.4)	2 (5.1)	1.06 (0.14, 7.92)	1.05 (0.16, 7.10)	0.3 (-22.1, 23.1)	1.0000
		No improvement	16 (43.2)	26 (66.7)				
		Improvement	21 (56.8)	13 (33.3)	2.63 (1.03, 6.66)	1.70 (1.01, 2.88)	23.4 (0.2, 44.5)	0.0642
Visit 4 (End of Week 9)	Total observed, n	.	35	38				
	Missing, n (%)	.	4 (10.3)	3 (7.3)				
	CGI-C	No deterioration	33 (94.3)	37 (97.4)				
		Deterioration	2 (5.7)	1 (2.6)	2.24 (0.19, 25.88)	2.17 (0.21, 22.91)	3.1 (-20.0, 25.5)	0.6044
		No improvement	12 (34.3)	24 (63.2)				
		Improvement	23 (65.7)	14 (36.8)	3.29 (1.26, 8.58)	1.78 (1.10, 2.88)	28.9 (5.6, 50.0)	0.0193
Visit 5 (End of Week 17)	Total observed, n	.	35	38				
	Missing, n (%)	.	4 (10.3)	3 (7.3)				
	CGI-C	No deterioration	33 (94.3)	35 (92.1)				
		Deterioration	2 (5.7)	3 (7.9)	0.71 (0.11, 4.50)	0.72 (0.13, 4.08)	-2.2 (-25.2, 20.3)	1.0000
		No improvement	17 (48.6)	20 (52.6)				
		Improvement	18 (51.4)	18 (47.4)	1.18 (0.47, 2.95)	1.09 (0.68, 1.73)	4.1 (-19.2, 27.0)	0.8163
Final OL/Taper Visit	Total observed, n	.	3	0				
	CGI-C	No deterioration	2 (66.7)	0				
		Deterioration	1 (33.3)	0	0.60 (0.01, 49.45)	0.75 (0.07, 7.73)		
		No improvement	2 (66.7)	0				
		Improvement	1 (33.3)	0	0.60 (0.01, 49.45)	0.75 (0.07, 7.73)		

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)
Study No. 1042-CDD-3001
Table 14.2.4.2.1 Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C) - Males

Visit		Responder	Ganaxolone N=11	Placebo N=10	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	10	9				
	Missing, n (%)	.	1 (9.1)	1 (10.0)				
	CGI-C	No deterioration	8 (80.0)	6 (66.7)				
		Deterioration	2 (20.0)	3 (33.3)	0.50 (0.06, 4.00)	0.60 (0.13, 2.82)	-13.3 (-53.7, 33.2)	0.6285
		No improvement	8 (80.0)	7 (77.8)				
		Improvement	2 (20.0)	2 (22.2)	0.88 (0.10, 7.95)	0.90 (0.16, 5.13)	-2.2 (-42.8, 42.5)	1.0000
Visit 4 (End of Week 9)	Total observed, n	.	10	9				
	Missing, n (%)	.	1 (9.1)	1 (10.0)				
	CGI-C	No deterioration	9 (90.0)	8 (88.9)				
		Deterioration	1 (10.0)	1 (11.1)	0.89 (0.05, 16.66)	0.90 (0.07, 12.38)	-1.1 (-42.5, 42.5)	1.0000
		No improvement	7 (70.0)	6 (66.7)				
		Improvement	3 (30.0)	3 (33.3)	0.86 (0.12, 5.94)	0.90 (0.24, 3.38)	-3.3 (-46.0, 42.3)	1.0000
Visit 5 (End of Week 17)	Total observed, n	.	10	8				
	Missing, n (%)	.	1 (9.1)	2 (20.0)				
	CGI-C	No deterioration	10 (100.0)	7 (87.5)				
		Deterioration	0	1 (12.5)	0.24 (0.01, 6.69)	0.27 (0.01, 5.92)	-12.5 (-56.0, 34.9)	0.4444
		No improvement	4 (40.0)	6 (75.0)				
		Improvement	6 (60.0)	2 (25.0)	4.50 (0.59, 34.61)	2.40 (0.65, 8.83)	35.0 (-13.5, 72.8)	0.1880

Improvement defined as any improvement (minimally, much or very much improved)
Deterioration defined as any deterioration (minimally, much or very much worse)

Study No. 1042-CDD-3001

Table 14.2.4.3.1 Summary of Clinical Global Impression of Improvement (CGI-I) - Patients who are 2-6 years old

Clinical Global Impression-Improvement - by Parent/Caregiver

Visit		Responder	Ganaxolone N=30	Placebo N=22	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	27	21				
	Missing, n (%)	.	3 (10.0)	1 (4.5)				
	CGI-I	No deterioration	26 (96.3)	21 (100.0)				
		Deterioration	1 (3.7)	0	2.43 (0.09, 62.82)	2.36 (0.10, 55.09)	3.7 (-24.6, 31.5)	1.0000
		No improvement	6 (22.2)	9 (42.9)				
		Improvement	21 (77.8)	12 (57.1)	2.63 (0.75, 9.19)	1.36 (0.89, 2.08)	20.6 (-8.1, 46.9)	0.2089
Visit 4 (End of Week 9)	Total observed, n	.	26	20				
	Missing, n (%)	.	4 (13.3)	2 (9.1)				
	CGI-I	No deterioration	23 (88.5)	19 (95.0)				
		Deterioration	3 (11.5)	1 (5.0)	2.48 (0.24, 25.81)	2.31 (0.26, 20.55)	6.5 (-22.5, 35.0)	0.6216
		No improvement	8 (30.8)	10 (50.0)				
		Improvement	18 (69.2)	10 (50.0)	2.25 (0.67, 7.54)	1.38 (0.83, 2.30)	19.2 (-10.1, 46.5)	0.2312
Visit 5 (End of Week 17)	Total observed, n	.	25	21				
	Missing, n (%)	.	5 (16.7)	1 (4.5)				
	CGI-I	No deterioration	23 (92.0)	18 (85.7)				
		Deterioration	2 (8.0)	3 (14.3)	0.52 (0.08, 3.46)	0.56 (0.10, 3.04)	-6.3 (-34.3, 22.3)	0.6476
		No improvement	9 (36.0)	11 (52.4)				
		Improvement	16 (64.0)	10 (47.6)	1.96 (0.60, 6.39)	1.34 (0.79, 2.30)	16.4 (-13.2, 43.8)	0.3721
Final OL/Taper Visit	Total observed, n	.	2	0				
	CGI-I	No deterioration	2 (100.0)	0				
		Deterioration	0	0	0.20 (0.00, 28.47)	0.33 (0.01, 8.18)		
		No improvement	1 (50.0)	0				
		Improvement	1 (50.0)	0	1.00 (0.01, 92.42)	1.00 (0.10, 9.61)		

Responder defined as any improvement (minimally, much or very much improved) for CGI-I - Parent/Caregiver
Responder defined as any deterioration (minimally, much or very much worse) for CGI-I – Clinician

Study No. 1042-CDD-3001

Table 14.2.4.3.1 Summary of Clinical Global Impression of Improvement (CGI-I) - Patients who are >6 years old

Clinical Global Impression-Improvement - by Parent/Caregiver

Visit		Responder	Ganaxolone N=20	Placebo N=29	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	20	27				
	Missing, n (%)	.	0 (0.0)	2 (6.9)				
	CGI-I	No deterioration	18 (90.0)	27 (100.0)				
		Deterioration	2 (10.0)	0	7.43 (0.34, 163.84)	6.67 (0.34, 131.67)	10.0 (-18.8, 37.5)	0.1758
		No improvement	10 (50.0)	15 (55.6)				
		Improvement	10 (50.0)	12 (44.4)	1.25 (0.39, 3.99)	1.13 (0.61, 2.07)	5.6 (-23.5, 34.0)	0.7729
Visit 4 (End of Week 9)	Total observed, n	.	19	26				
	Missing, n (%)	.	1 (5.0)	3 (10.3)				
	CGI-I	No deterioration	17 (89.5)	25 (96.2)				
		Deterioration	2 (10.5)	1 (3.8)	2.94 (0.25, 35.06)	2.74 (0.27, 28.03)	6.7 (-22.5, 35.4)	0.5648
		No improvement	9 (47.4)	18 (69.2)				
		Improvement	10 (52.6)	8 (30.8)	2.50 (0.73, 8.52)	1.71 (0.83, 3.50)	21.9 (-8.1, 49.2)	0.2182
Visit 5 (End of Week 17)	Total observed, n	.	18	26				
	Missing, n (%)	.	2 (10.0)	3 (10.3)				
	CGI-I	No deterioration	17 (94.4)	25 (96.2)				
		Deterioration	1 (5.6)	1 (3.8)	1.47 (0.09, 25.16)	1.44 (0.10, 21.62)	1.7 (-28.3, 31.1)	1.0000
		No improvement	7 (38.9)	15 (57.7)				
		Improvement	11 (61.1)	11 (42.3)	2.14 (0.63, 7.30)	1.44 (0.81, 2.58)	18.8 (-11.8, 46.7)	0.3580
Final OL/Taper Visit	Total observed, n	.	1	0				
	CGI-I	Deterioration	1 (100.0)	0	3.00 (0.02, 473.06)	1.50 (0.18, 12.46)		
		No improvement	1 (100.0)	0				
		Improvement	0	0	0.33 (0.00, 52.56)	0.50 (0.02, 11.09)		

Responder defined as any improvement (minimally, much or very much improved) for CGI-I - Parent/Caregiver

Responder defined as any deterioration (minimally, much or very much worse) for CGI-I – Clinician

Study No. 1042-CDD-3001
Table 14.2.4.3.1 Summary of Clinical Global Impression of Improvement (CGI-I) - Patients not in US

Clinical Global Impression-Improvement - by Parent/Caregiver

Visit		Responder	Ganaxolone N=32	Placebo N=27	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	30	26				
	Missing, n (%)	.	2 (6.3)	1 (3.7)				
	CGI-I	No deterioration	28 (93.3)	26 (100.0)				
		Deterioration	2 (6.7)	0	4.65 (0.21, 101.37)	4.35 (0.22, 86.79)	6.7 (-19.9, 32.5)	0.4935
		No improvement	13 (43.3)	12 (46.2)				
		Improvement	17 (56.7)	14 (53.8)	1.12 (0.39, 3.22)	1.05 (0.66, 1.69)	2.8 (-23.6, 28.8)	1.0000
Visit 4 (End of Week 9)	Total observed, n	.	30	25				
	Missing, n (%)	.	2 (6.3)	2 (7.4)				
	CGI-I	No deterioration	26 (86.7)	24 (96.0)				
		Deterioration	4 (13.3)	1 (4.0)	3.69 (0.39, 35.40)	3.33 (0.40, 27.94)	9.3 (-17.5, 35.2)	0.3623
		No improvement	13 (43.3)	15 (60.0)				
		Improvement	17 (56.7)	10 (40.0)	1.96 (0.67, 5.76)	1.42 (0.80, 2.51)	16.7 (-10.3, 41.9)	0.2823
Visit 5 (End of Week 17)	Total observed, n	.	29	26				
	Missing, n (%)	.	3 (9.4)	1 (3.7)				
	CGI-I	No deterioration	27 (93.1)	25 (96.2)				
		Deterioration	2 (6.9)	1 (3.8)	1.85 (0.16, 21.70)	1.79 (0.17, 18.64)	3.1 (-22.9, 29.3)	1.0000
		No improvement	14 (48.3)	14 (53.8)				
		Improvement	15 (51.7)	12 (46.2)	1.25 (0.43, 3.61)	1.12 (0.65, 1.93)	5.6 (-21.2, 31.8)	0.7891
Final OL/Taper Visit	Total observed, n	.	1	0				
	CGI-I	Deterioration	1 (100.0)	0	3.00 (0.02, 473.06)	1.50 (0.18, 12.46)		
		No improvement	1 (100.0)	0				

Visit		Responder	Ganaxolone N=32	Placebo N=27	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
		Improvement	0	0	0.33 (0.00, 52.56)	0.50 (0.02, 11.09)		

Responder defined as any improvement (minimally, much or very much improved) for CGI-I - Parent/Caregiver
Responder defined as any deterioration (minimally, much or very much worse) for CGI-I – Clinician

Study No. 1042-CDD-3001
Table 14.2.4.3.1 Summary of Clinical Global Impression of Improvement (CGI-I) - Patients in US

Clinical Global Impression-Improvement - by Parent/Caregiver

Visit		Responder	Ganaxolone N=18	Placebo N=24	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	17	22				
	Missing, n (%)	.	1 (5.6)	2 (8.3)				
	CGI-I	No deterioration	16 (94.1)	22 (100.0)				
		Deterioration	1 (5.9)	0	4.09 (0.16, 106.89)	3.83 (0.17, 88.62)	5.9 (-25.5, 36.0)	0.4359
		No improvement	3 (17.6)	12 (54.5)				
		Improvement	14 (82.4)	10 (45.5)	5.60 (1.25, 25.17)	1.81 (1.09, 3.01)	36.9 (5.1, 63.0)	0.0244
Visit 4 (End of Week 9)	Total observed, n	.	15	21				
	Missing, n (%)	.	3 (16.7)	3 (12.5)				
	CGI-I	No deterioration	14 (93.3)	20 (95.2)				
		Deterioration	1 (6.7)	1 (4.8)	1.43 (0.08, 24.81)	1.40 (0.09, 20.65)	1.9 (-30.6, 34.6)	1.0000
		No improvement	4 (26.7)	13 (61.9)				
		Improvement	11 (73.3)	8 (38.1)	4.47 (1.05, 18.94)	1.93 (1.03, 3.60)	35.2 (1.5, 63.0)	0.0489
Visit 5 (End of Week 17)	Total observed, n	.	14	21				
	Missing, n (%)	.	4 (22.2)	3 (12.5)				
	CGI-I	No deterioration	13 (92.9)	18 (85.7)				
		Deterioration	1 (7.1)	3 (14.3)	0.46 (0.04, 4.95)	0.50 (0.06, 4.33)	-7.1 (-40.4, 27.3)	0.6350

Visit		Responder	Ganaxolone N=18	Placebo N=24	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
		No improvement	2 (14.3)	12 (57.1)				
		Improvement	12 (85.7)	9 (42.9)	8.00 (1.42, 45.06)	2.00 (1.17, 3.43)	42.9 (8.3, 71.1)	0.0156
Final OL/Taper Visit	Total observed, n	.	2	0				
	CGI-I	No deterioration	2 (100.0)	0				
		Deterioration	0	0	0.20 (0.00, 28.47)	0.33 (0.01, 8.18)		
		No improvement	1 (50.0)	0				
		Improvement	1 (50.0)	0	1.00 (0.01, 92.42)	1.00 (0.10, 9.61)		

Responder defined as any improvement (minimally, much or very much improved) for CGI-I - Parent/Caregiver
Responder defined as any deterioration (minimally, much or very much worse) for CGI-I – Clinician

Study No. 1042-CDD-3001
Table 14.2.4.3.1 Summary of Clinical Global Impression of Improvement (CGI-I) - Females

Clinical Global Impression-Improvement - by Parent/Caregiver

Visit		Responder	Ganaxolone N=39	Placebo N=41	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	37	39				
	Missing, n (%)	.	2 (5.1)	2 (4.9)				
	CGI-I	No deterioration	35 (94.6)	39 (100.0)				
		Deterioration	2 (5.4)	0	5.56 (0.26, 119.85)	5.26 (0.26, 106.11)	5.4 (-17.3, 27.9)	0.2337
		No improvement	11 (29.7)	21 (53.8)				
		Improvement	26 (70.3)	18 (46.2)	2.76 (1.07, 7.10)	1.52 (1.02, 2.27)	24.1 (1.6, 45.4)	0.0393
Visit 4 (End of Week 9)	Total observed, n	.	35	37				
	Missing, n (%)	.	4 (10.3)	4 (9.8)				
	CGI-I	No deterioration	32 (91.4)	37 (100.0)				

Visit		Responder	Ganaxolone N=39	Placebo N=41	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
		Deterioration	3 (8.6)	0	8.08 (0.40, 162.26)	7.39 (0.40, 138.09)	8.6 (-14.7, 31.5)	0.1097
		No improvement	12 (34.3)	22 (59.5)				
		Improvement	23 (65.7)	15 (40.5)	2.81 (1.08, 7.33)	1.62 (1.03, 2.56)	25.2 (1.7, 46.9)	0.0373
Visit 5 (End of Week 17)	Total observed, n	.	33	39				
	Missing, n (%)	.	6 (15.4)	2 (4.9)				
	CGI-I	No deterioration	30 (90.9)	37 (94.9)				
		Deterioration	3 (9.1)	2 (5.1)	1.85 (0.29, 11.80)	1.77 (0.31, 9.98)	4.0 (-19.1, 26.7)	0.6551
		No improvement	12 (36.4)	20 (51.3)				
		Improvement	21 (63.6)	19 (48.7)	1.84 (0.71, 4.75)	1.31 (0.86, 1.97)	14.9 (-8.4, 37.1)	0.2398
Final OL/Taper Visit	Total observed, n	.	3	0				
	CGI-I	No deterioration	2 (66.7)	0				
		Deterioration	1 (33.3)	0	0.60 (0.01, 49.45)	0.75 (0.07, 7.73)		
		No improvement	2 (66.7)	0				
		Improvement	1 (33.3)	0	0.60 (0.01, 49.45)	0.75 (0.07, 7.73)		

Responder defined as any improvement (minimally, much or very much improved) for CGI-I - Parent/Caregiver
Responder defined as any deterioration (minimally, much or very much worse) for CGI-I – Clinician

Study No. 1042-CDD-3001
Table 14.2.4.3.1 Summary of Clinical Global Impression of Improvement (CGI-I) - Males

Clinical Global Impression-Improvement - by Parent/Caregiver

Visit		Responder	Ganaxolone N=11	Placebo N=10	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	Total observed, n	.	10	9				
	Missing, n (%)	.	1 (9.1)	1 (10.0)				

Visit		Responder	Ganaxolone N=11	Placebo N=10	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
	CGI-I	No deterioration	9 (90.0)	9 (100.0)				
		Deterioration	1 (10.0)	0	3.00 (0.11, 83.36)	2.73 (0.12, 59.57)	10.0 (-33.3, 51.4)	1.0000
		No improvement	5 (50.0)	3 (33.3)				
		Improvement	5 (50.0)	6 (66.7)	0.50 (0.08, 3.21)	0.75 (0.35, 1.62)	-16.7 (-58.9, 29.5)	0.6499
Visit 4 (End of Week 9)	Total observed, n	.	10	9				
	Missing, n (%)	.	1 (9.1)	1 (10.0)				
	CGI-I	No deterioration	8 (80.0)	7 (77.8)				
		Deterioration	2 (20.0)	2 (22.2)	0.88 (0.10, 7.95)	0.90 (0.16, 5.13)	-2.2 (-42.8, 42.5)	1.0000
		No improvement	5 (50.0)	6 (66.7)				
		Improvement	5 (50.0)	3 (33.3)	2.00 (0.31, 12.84)	1.50 (0.49, 4.56)	16.7 (-29.5, 58.9)	0.6499
Visit 5 (End of Week 17)	Total observed, n	.	10	8				
	Missing, n (%)	.	1 (9.1)	2 (20.0)				
	CGI-I	No deterioration	10 (100.0)	6 (75.0)				
		Deterioration	0	2 (25.0)	0.12 (0.01, 3.01)	0.16 (0.01, 2.99)	-25.0 (-65.3, 22.7)	0.1830
		No improvement	4 (40.0)	6 (75.0)				
		Improvement	6 (60.0)	2 (25.0)	4.50 (0.59, 34.61)	2.40 (0.65, 8.83)	35.0 (-13.5, 72.8)	0.1880

Responder defined as any improvement (minimally, much or very much improved) for CGI-I - Parent/Caregiver
Responder defined as any deterioration (minimally, much or very much worse) for CGI-I - Clinician

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are 2-6 years old

Bedtime Resistance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	26	15% responder	0 (0.0)	3 (11.5)				
	Placebo N=22	20	15% responder	0 (0.0)	4 (20.0)	0.55 (0.12, 2.53)	0.60 (0.17, 2.17)	-8.5 (-36.3, 20.8)	0.6816
Visit 4 (End of Week 9)	GNX N=30	25	15% responder	0 (0.0)	3 (12.0)				
	Placebo N=22	19	15% responder	0 (0.0)	3 (15.8)	0.73 (0.15, 3.68)	0.77 (0.20, 3.00)	-3.8 (-32.9, 25.4)	1.0000
Visit 5 (End of Week 17)	GNX N=30	23	15% responder	1 (3.3)	6 (26.1)				
	Placebo N=22	20	15% responder	0 (0.0)	7 (35.0)	0.67 (0.19, 2.38)	0.76 (0.32, 1.81)	-8.9 (-37.7, 20.9)	0.7402

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are 2-6 years old

Sleep Onset Delay Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	6 (22.2)				
	Placebo N=22	21	15% responder	0 (0.0)	5 (23.8)	0.91 (0.24, 3.54)	0.93 (0.33, 2.64)	-1.6 (-29.7, 26.8)	1.0000
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	8 (30.8)				
	Placebo N=22	21	15% responder	0 (0.0)	4 (19.0)	1.89 (0.48, 7.44)	1.62 (0.56, 4.63)	11.7 (-17.2, 38.8)	0.5047
Visit 5 (End of Week 17)	GNX N=30	25	15% responder	0 (0.0)	8 (32.0)				
	Placebo N=22	21	15% responder	0 (0.0)	6 (28.6)	1.18 (0.33, 4.17)	1.12 (0.46, 2.71)	3.4 (-25.5, 31.3)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are 2-6 years old

Sleep Duration Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	26	15% responder	1 (3.3)	9 (34.6)				
	Placebo N=22	20	15% responder	0 (0.0)	5 (25.0)	1.53 (0.44, 5.36)	1.34 (0.56, 3.24)	9.6 (-19.6, 37.4)	0.5350
Visit 4 (End of Week 9)	GNX N=30	25	15% responder	0 (0.0)	9 (36.0)				
	Placebo N=22	19	15% responder	1 (4.5)	7 (36.8)	0.96 (0.29, 3.21)	0.97 (0.46, 2.08)	-0.8 (-30.4, 28.1)	1.0000
Visit 5 (End of Week 17)	GNX N=30	24	15% responder	1 (3.3)	7 (29.2)				
	Placebo N=22	19	15% responder	1 (4.5)	9 (47.4)	0.46 (0.13, 1.61)	0.62 (0.28, 1.35)	-18.2 (-46.3, 12.2)	0.3413

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are 2-6 years old

Sleep Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	24	15% responder	1 (3.3)	4 (16.7)				
	Placebo N=22	19	15% responder	0 (0.0)	0 (0.0)	8.56 (0.43, 169.70)	7.20 (0.41, 125.97)	16.7 (-13.3, 44.7)	0.1175
Visit 4 (End of Week 9)	GNX N=30	23	15% responder	1 (3.3)	3 (13.0)				
	Placebo N=22	19	15% responder	0 (0.0)	3 (15.8)	0.80 (0.16, 4.06)	0.83 (0.21, 3.24)	-2.7 (-32.0, 27.3)	1.0000
Visit 5 (End of Week 17)	GNX N=30	23	15% responder	2 (6.7)	4 (17.4)				
	Placebo N=22	20	15% responder	0 (0.0)	3 (15.0)	1.15 (0.25, 5.38)	1.13 (0.32, 4.00)	2.4 (-27.2, 32.0)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are 2-6 years old

Night Wakings Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	22	15% responder	2 (6.7)	6 (27.3)				
	Placebo N=22	18	15% responder	0 (0.0)	4 (22.2)	1.27 (0.31, 5.12)	1.19 (0.42, 3.37)	5.1 (-25.8, 35.2)	1.0000
Visit 4 (End of Week 9)	GNX N=30	24	15% responder	0 (0.0)	6 (25.0)				
	Placebo N=22	18	15% responder	0 (0.0)	4 (22.2)	1.13 (0.28, 4.52)	1.10 (0.39, 3.12)	2.8 (-27.8, 33.0)	1.0000
Visit 5 (End of Week 17)	GNX N=30	23	15% responder	2 (6.7)	11 (47.8)				
	Placebo N=22	19	15% responder	0 (0.0)	9 (47.4)	1.02 (0.31, 3.34)	1.01 (0.54, 1.88)	0.5 (-29.8, 30.7)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are 2-6 years old

Parasomnias Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	20	15% responder	1 (3.3)	2 (10.0)				
	Placebo N=22	17	15% responder	1 (4.5)	1 (5.9)	1.78 (0.15, 21.51)	1.70 (0.17, 17.16)	4.1 (-27.2, 35.8)	1.0000
Visit 4 (End of Week 9)	GNX N=30	21	15% responder	1 (3.3)	4 (19.0)				
	Placebo N=22	17	15% responder	0 (0.0)	1 (5.9)	2.83 (0.40, 20.21)	2.45 (0.43, 13.98)	13.2 (-18.7, 42.9)	0.3551
Visit 5 (End of Week 17)	GNX N=30	21	15% responder	2 (6.7)	5 (23.8)				
	Placebo N=22	18	15% responder	1 (4.5)	4 (22.2)	1.09 (0.24, 4.89)	1.07 (0.34, 3.40)	1.6 (-30.0, 32.7)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are 2-6 years old

Sleep Disordered Breathing Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	26	15% responder	0 (0.0)	7 (26.9)				
	Placebo N=22	20	15% responder	0 (0.0)	2 (10.0)	2.85 (0.59, 13.63)	2.33 (0.63, 8.67)	16.9 (-12.1, 44.3)	0.2621
Visit 4 (End of Week 9)	GNX N=30	21	15% responder	1 (3.3)	7 (33.3)				
	Placebo N=22	18	15% responder	0 (0.0)	3 (16.7)	2.29 (0.53, 9.83)	1.85 (0.61, 5.62)	16.7 (-15.6, 46.2)	0.2898
Visit 5 (End of Week 17)	GNX N=30	24	15% responder	1 (3.3)	6 (25.0)				
	Placebo N=22	18	15% responder	0 (0.0)	4 (22.2)	1.13 (0.28, 4.52)	1.10 (0.39, 3.12)	2.8 (-27.8, 33.0)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are >6 years old

Bedtime Resistance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	16	15% responder	2 (10.0)	3 (18.8)				
	Placebo N=29	25	15% responder	1 (3.4)	5 (20.0)	0.92 (0.19, 4.54)	0.94 (0.26, 3.39)	-1.3 (-31.4, 29.5)	1.0000
Visit 4 (End of Week 9)	GNX N=20	18	15% responder	1 (5.0)	4 (22.2)				
	Placebo N=29	26	15% responder	1 (3.4)	4 (15.4)	1.57 (0.34, 7.33)	1.44 (0.41, 5.04)	6.8 (-23.3, 35.7)	0.6971
Visit 5 (End of Week 17)	GNX N=20	17	15% responder	2 (10.0)	3 (17.6)				
	Placebo N=29	25	15% responder	1 (3.4)	5 (20.0)	0.86 (0.18, 4.19)	0.88 (0.24, 3.21)	-2.4 (-32.0, 28.3)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are >6 years old

Sleep Onset Delay Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	20	15% responder	0 (0.0)	6 (30.0)				
	Placebo N=29	26	15% responder	1 (3.4)	5 (19.2)	1.75 (0.47, 6.54)	1.52 (0.57, 4.06)	10.8 (-18.3, 38.6)	0.4935
Visit 4 (End of Week 9)	GNX N=20	19	15% responder	0 (0.0)	8 (42.1)				
	Placebo N=29	26	15% responder	1 (3.4)	5 (19.2)	2.89 (0.80, 10.49)	2.09 (0.85, 5.15)	22.9 (-6.9, 49.7)	0.1114
Visit 5 (End of Week 17)	GNX N=20	19	15% responder	0 (0.0)	9 (47.4)				
	Placebo N=29	25	15% responder	1 (3.4)	2 (8.0)	8.50 (1.77, 40.95)	4.94 (1.39, 17.51)	39.4 (10.3, 64.2)	0.0045

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are >6 years old

Sleep Duration Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	20	15% responder	0 (0.0)	4 (20.0)				
	Placebo N=29	26	15% responder	1 (3.4)	4 (15.4)	1.36 (0.32, 5.83)	1.29 (0.40, 4.17)	4.6 (-24.4, 32.7)	0.7126
Visit 4 (End of Week 9)	GNX N=20	19	15% responder	0 (0.0)	6 (31.6)				
	Placebo N=29	26	15% responder	1 (3.4)	5 (19.2)	1.88 (0.50, 7.07)	1.60 (0.60, 4.24)	12.3 (-17.1, 40.2)	0.4851
Visit 5 (End of Week 17)	GNX N=20	19	15% responder	0 (0.0)	6 (31.6)				
	Placebo N=29	25	15% responder	1 (3.4)	2 (8.0)	4.53 (0.91, 22.53)	3.38 (0.89, 12.87)	23.6 (-6.0, 50.7)	0.0596

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are >6 years old

Sleep Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	14	15% responder	4 (20.0)	1 (7.1)				
	Placebo N=29	24	15% responder	2 (6.9)	4 (16.7)	0.38 (0.04, 3.84)	0.43 (0.05, 3.46)	-9.5 (-41.5, 23.4)	0.6331
Visit 4 (End of Week 9)	GNX N=20	14	15% responder	3 (15.0)	2 (14.3)				
	Placebo N=29	24	15% responder	2 (6.9)	5 (20.8)	0.63 (0.11, 3.80)	0.69 (0.15, 3.08)	-6.5 (-38.6, 26.1)	1.0000
Visit 5 (End of Week 17)	GNX N=20	14	15% responder	5 (25.0)	2 (14.3)				
	Placebo N=29	21	15% responder	3 (10.3)	3 (14.3)	1.00 (0.14, 6.91)	1.00 (0.19, 5.24)	0.0 (-33.9, 33.9)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are >6 years old

Night Wakings Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	15	15% responder	4 (20.0)	3 (20.0)				
	Placebo N=29	22	15% responder	4 (13.8)	6 (27.3)	0.67 (0.14, 3.22)	0.73 (0.22, 2.49)	-7.3 (-38.2, 25.7)	0.7111
Visit 4 (End of Week 9)	GNX N=20	14	15% responder	2 (10.0)	4 (28.6)				
	Placebo N=29	22	15% responder	4 (13.8)	8 (36.4)	0.70 (0.16, 2.98)	0.79 (0.29, 2.13)	-7.8 (-39.8, 25.4)	0.7272
Visit 5 (End of Week 17)	GNX N=20	14	15% responder	4 (20.0)	3 (21.4)				
	Placebo N=29	20	15% responder	4 (13.8)	6 (30.0)	0.64 (0.13, 3.14)	0.71 (0.21, 2.39)	-8.6 (-41.2, 25.4)	0.7041

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are >6 years old

Parasomnias Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	15	15% responder	3 (15.0)	3 (20.0)				
	Placebo N=29	21	15% responder	4 (13.8)	0 (0.0)	12.04 (0.57, 252.73)	9.63 (0.53, 173.60)	20.0 (-13.6, 50.7)	0.0637
Visit 4 (End of Week 9)	GNX N=20	14	15% responder	1 (5.0)	2 (14.3)				
	Placebo N=29	19	15% responder	3 (10.3)	4 (21.1)	0.63 (0.10, 4.01)	0.68 (0.14, 3.20)	-6.8 (-39.8, 26.9)	1.0000
Visit 5 (End of Week 17)	GNX N=20	14	15% responder	3 (15.0)	0 (0.0)				
	Placebo N=29	19	15% responder	3 (10.3)	2 (10.5)	0.24 (0.01, 5.44)	0.27 (0.01, 5.15)	-10.5 (-43.0, 23.5)	0.4962

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients who are >6 years old

Sleep Disordered Breathing Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	17	15% responder	3 (15.0)	3 (17.6)				
	Placebo N=29	23	15% responder	3 (10.3)	2 (8.7)	2.25 (0.33, 15.24)	2.03 (0.38, 10.84)	9.0 (-22.3, 38.5)	0.6340
Visit 4 (End of Week 9)	GNX N=20	16	15% responder	1 (5.0)	2 (12.5)				
	Placebo N=29	22	15% responder	3 (10.3)	4 (18.2)	0.64 (0.10, 4.03)	0.69 (0.14, 3.31)	-5.7 (-36.9, 26.2)	1.0000
Visit 5 (End of Week 17)	GNX N=20	16	15% responder	2 (10.0)	4 (25.0)				
	Placebo N=29	21	15% responder	3 (10.3)	4 (19.0)	1.42 (0.29, 6.81)	1.31 (0.39, 4.46)	6.0 (-25.8, 37.5)	0.7048

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients not in US

Bedtime Resistance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	26	15% responder	2 (6.3)	4 (15.4)				
	Placebo N=27	24	15% responder	1 (3.7)	5 (20.8)	0.69 (0.16, 2.95)	0.74 (0.22, 2.43)	-5.4 (-32.9, 21.6)	0.7210
Visit 4 (End of Week 9)	GNX N=32	28	15% responder	1 (3.1)	5 (17.9)				
	Placebo N=27	24	15% responder	1 (3.7)	5 (20.8)	0.83 (0.21, 3.29)	0.86 (0.28, 2.61)	-3.0 (-29.9, 24.4)	1.0000
Visit 5 (End of Week 17)	GNX N=32	27	15% responder	3 (9.4)	6 (22.2)				
	Placebo N=27	24	15% responder	1 (3.7)	6 (25.0)	0.86 (0.23, 3.13)	0.89 (0.33, 2.39)	-2.8 (-30.2, 24.6)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients not in US

Sleep Onset Delay Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	31	15% responder	0 (0.0)	6 (19.4)				
	Placebo N=27	25	15% responder	1 (3.7)	5 (20.0)	0.95 (0.27, 3.40)	0.96 (0.35, 2.64)	-0.6 (-26.7, 25.1)	1.0000
Visit 4 (End of Week 9)	GNX N=32	30	15% responder	0 (0.0)	9 (30.0)				
	Placebo N=27	25	15% responder	1 (3.7)	5 (20.0)	1.65 (0.49, 5.53)	1.45 (0.58, 3.61)	10.0 (-16.8, 35.8)	0.5371
Visit 5 (End of Week 17)	GNX N=32	30	15% responder	0 (0.0)	9 (30.0)				
	Placebo N=27	25	15% responder	1 (3.7)	7 (28.0)	1.09 (0.35, 3.41)	1.06 (0.48, 2.37)	2.0 (-24.6, 28.4)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients not in US

Sleep Duration Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	30	15% responder	1 (3.1)	9 (30.0)				
	Placebo N=27	24	15% responder	1 (3.7)	6 (25.0)	1.29 (0.38, 4.31)	1.20 (0.50, 2.90)	5.0 (-21.9, 31.4)	0.7659
Visit 4 (End of Week 9)	GNX N=32	29	15% responder	0 (0.0)	10 (34.5)				
	Placebo N=27	24	15% responder	2 (7.4)	9 (37.5)	0.88 (0.29, 2.65)	0.92 (0.46, 1.85)	-3.0 (-29.4, 23.8)	1.0000
Visit 5 (End of Week 17)	GNX N=32	29	15% responder	1 (3.1)	7 (24.1)				
	Placebo N=27	23	15% responder	2 (7.4)	5 (21.7)	1.15 (0.31, 4.23)	1.11 (0.40, 3.04)	2.4 (-24.4, 29.2)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients not in US

Sleep Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	24	15% responder	4 (12.5)	4 (16.7)				
	Placebo N=27	22	15% responder	2 (7.4)	1 (4.5)	4.20 (0.43, 40.87)	3.67 (0.44, 30.35)	12.1 (-17.7, 39.7)	0.3488
Visit 4 (End of Week 9)	GNX N=32	25	15% responder	3 (9.4)	5 (20.0)				
	Placebo N=27	22	15% responder	2 (7.4)	6 (27.3)	0.67 (0.17, 2.59)	0.73 (0.26, 2.07)	-7.3 (-35.3, 20.9)	0.7322
Visit 5 (End of Week 17)	GNX N=32	24	15% responder	6 (18.8)	5 (20.8)				
	Placebo N=27	21	15% responder	3 (11.1)	4 (19.0)	1.12 (0.26, 4.86)	1.09 (0.34, 3.55)	1.8 (-27.7, 30.9)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients not in US

Night Wakings Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	23	15% responder	5 (15.6)	3 (13.0)				
	Placebo N=27	20	15% responder	4 (14.8)	6 (30.0)	0.35 (0.07, 1.64)	0.43 (0.12, 1.52)	-17.0 (-45.1, 13.2)	0.2634
Visit 4 (End of Week 9)	GNX N=32	25	15% responder	1 (3.1)	4 (16.0)				
	Placebo N=27	20	15% responder	4 (14.8)	8 (40.0)	0.29 (0.07, 1.15)	0.40 (0.14, 1.14)	-24.0 (-51.0, 6.0)	0.0958
Visit 5 (End of Week 17)	GNX N=32	24	15% responder	5 (15.6)	8 (33.3)				
	Placebo N=27	20	15% responder	4 (14.8)	10 (50.0)	0.50 (0.15, 1.69)	0.67 (0.33, 1.36)	-16.7 (-44.6, 13.5)	0.3588

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients not in US

Parasomnias Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	23	15% responder	3 (9.4)	3 (13.0)				
	Placebo N=27	19	15% responder	4 (14.8)	0 (0.0)	6.66 (0.32, 137.45)	5.83 (0.32, 106.35)	13.0 (-17.4, 41.6)	0.2387
Visit 4 (End of Week 9)	GNX N=32	23	15% responder	1 (3.1)	5 (21.7)				
	Placebo N=27	18	15% responder	3 (11.1)	3 (16.7)	1.39 (0.28, 6.79)	1.30 (0.36, 4.75)	5.1 (-25.4, 34.6)	1.0000
Visit 5 (End of Week 17)	GNX N=32	23	15% responder	4 (12.5)	4 (17.4)				
	Placebo N=27	20	15% responder	3 (11.1)	3 (15.0)	1.19 (0.23, 6.11)	1.16 (0.29, 4.57)	2.4 (-27.2, 32.0)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients not in US

Sleep Disordered Breathing Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	28	15% responder	2 (6.3)	6 (21.4)				
	Placebo N=27	23	15% responder	2 (7.4)	3 (13.0)	1.82 (0.40, 8.25)	1.64 (0.46, 5.86)	8.4 (-19.2, 34.7)	0.4875
Visit 4 (End of Week 9)	GNX N=32	24	15% responder	1 (3.1)	7 (29.2)				
	Placebo N=27	20	15% responder	2 (7.4)	4 (20.0)	1.65 (0.40, 6.72)	1.46 (0.50, 4.28)	9.2 (-20.9, 38.0)	0.7279
Visit 5 (End of Week 17)	GNX N=32	27	15% responder	2 (6.3)	8 (29.6)				
	Placebo N=27	20	15% responder	3 (11.1)	5 (25.0)	1.26 (0.34, 4.66)	1.19 (0.46, 3.08)	4.6 (-23.6, 32.9)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients in US

Bedtime Resistance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	2 (12.5)				
	Placebo N=24	21	15% responder	0 (0.0)	4 (19.0)	0.61 (0.10, 3.82)	0.66 (0.14, 3.15)	-6.5 (-37.5, 25.8)	0.6796
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	2 (13.3)				
	Placebo N=24	21	15% responder	0 (0.0)	2 (9.5)	1.46 (0.18, 11.73)	1.40 (0.22, 8.86)	3.8 (-29.1, 36.4)	1.0000
Visit 5 (End of Week 17)	GNX N=18	13	15% responder	0 (0.0)	3 (23.1)				
	Placebo N=24	21	15% responder	0 (0.0)	6 (28.6)	0.75 (0.15, 3.72)	0.81 (0.24, 2.68)	-5.5 (-38.9, 28.6)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients in US

Sleep Onset Delay Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	6 (37.5)				
	Placebo N=24	22	15% responder	0 (0.0)	5 (22.7)	2.04 (0.49, 8.45)	1.65 (0.61, 4.47)	14.8 (-17.5, 44.9)	0.4712
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	7 (46.7)				
	Placebo N=24	22	15% responder	0 (0.0)	4 (18.2)	3.94 (0.89, 17.37)	2.57 (0.91, 7.25)	28.5 (-3.7, 57.8)	0.0802
Visit 5 (End of Week 17)	GNX N=18	14	15% responder	0 (0.0)	8 (57.1)				
	Placebo N=24	21	15% responder	0 (0.0)	1 (4.8)	26.67 (2.75, 258.18)	12.00 (1.68, 85.69)	52.4 (18.5, 77.8)	0.0009

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients in US

Sleep Duration Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	4 (25.0)				
	Placebo N=24	22	15% responder	0 (0.0)	3 (13.6)	2.11 (0.40, 11.13)	1.83 (0.47, 7.08)	11.4 (-20.6, 41.8)	0.4250
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	5 (33.3)				
	Placebo N=24	21	15% responder	0 (0.0)	3 (14.3)	3.00 (0.59, 15.26)	2.33 (0.66, 8.30)	19.0 (-14.8, 49.4)	0.2358
Visit 5 (End of Week 17)	GNX N=18	14	15% responder	0 (0.0)	6 (42.9)				
	Placebo N=24	21	15% responder	0 (0.0)	6 (28.6)	1.88 (0.45, 7.76)	1.50 (0.61, 3.72)	14.3 (-20.5, 46.7)	0.4769

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients in US

Sleep Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	14	15% responder	1 (5.6)	1 (7.1)				
	Placebo N=24	21	15% responder	0 (0.0)	3 (14.3)	0.59 (0.08, 4.50)	0.63 (0.10, 3.79)	-7.1 (-40.4, 27.3)	0.6350
Visit 4 (End of Week 9)	GNX N=18	12	15% responder	1 (5.6)	0 (0.0)				
	Placebo N=24	21	15% responder	0 (0.0)	2 (9.5)	0.31 (0.01, 7.05)	0.34 (0.02, 6.52)	-9.5 (-43.7, 26.4)	0.5227
Visit 5 (End of Week 17)	GNX N=18	13	15% responder	1 (5.6)	1 (7.7)				
	Placebo N=24	20	15% responder	0 (0.0)	2 (10.0)	0.89 (0.10, 7.61)	0.90 (0.13, 6.07)	-2.3 (-36.0, 31.4)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients in US

Night Wakings Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	14	15% responder	1 (5.6)	6 (42.9)				
	Placebo N=24	20	15% responder	0 (0.0)	4 (20.0)	2.80 (0.65, 12.06)	2.02 (0.74, 5.51)	22.9 (-11.2, 53.9)	0.2522
Visit 4 (End of Week 9)	GNX N=18	13	15% responder	1 (5.6)	6 (46.2)				
	Placebo N=24	20	15% responder	0 (0.0)	4 (20.0)	3.18 (0.72, 13.96)	2.17 (0.80, 5.85)	26.2 (-9.6, 57.4)	0.1393
Visit 5 (End of Week 17)	GNX N=18	13	15% responder	1 (5.6)	6 (46.2)				
	Placebo N=24	19	15% responder	0 (0.0)	5 (26.3)	2.28 (0.54, 9.62)	1.69 (0.68, 4.18)	19.8 (-15.1, 52.6)	0.2826

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients in US

Parasomnias Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	12	15% responder	1 (5.6)	2 (16.7)				
	Placebo N=24	19	15% responder	1 (4.2)	1 (5.3)	3.60 (0.29, 44.83)	3.17 (0.32, 31.24)	11.4 (-24.2, 45.5)	0.5435
Visit 4 (End of Week 9)	GNX N=18	12	15% responder	1 (5.6)	1 (8.3)				
	Placebo N=24	18	15% responder	0 (0.0)	2 (11.1)	0.86 (0.10, 7.46)	0.88 (0.13, 5.85)	-2.8 (-39.0, 34.0)	1.0000
Visit 5 (End of Week 17)	GNX N=18	12	15% responder	1 (5.6)	1 (8.3)				
	Placebo N=24	17	15% responder	1 (4.2)	3 (17.6)	0.42 (0.04, 4.66)	0.47 (0.06, 4.01)	-9.3 (-44.4, 26.8)	0.6221

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Patients in US

Sleep Disordered Breathing Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	15	15% responder	1 (5.6)	4 (26.7)				
	Placebo N=24	20	15% responder	1 (4.2)	1 (5.0)	6.91 (0.68, 69.86)	5.33 (0.66, 42.97)	21.7 (-12.6, 52.4)	0.1411
Visit 4 (End of Week 9)	GNX N=18	13	15% responder	1 (5.6)	2 (15.4)				
	Placebo N=24	20	15% responder	1 (4.2)	3 (15.0)	1.03 (0.15, 7.19)	1.03 (0.20, 5.33)	0.4 (-33.7, 33.7)	1.0000
Visit 5 (End of Week 17)	GNX N=18	13	15% responder	1 (5.6)	2 (15.4)				
	Placebo N=24	19	15% responder	0 (0.0)	3 (15.8)	1.02 (0.17, 6.13)	1.02 (0.23, 4.45)	-0.4 (-34.3, 34.3)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Females

Bedtime Resistance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	33	15% responder	2 (5.1)	3 (9.1)				
	Placebo N=41	36	15% responder	1 (2.4)	7 (19.4)	0.41 (0.10, 1.76)	0.47 (0.13, 1.66)	-10.4 (-33.6, 13.6)	0.3106
Visit 4 (End of Week 9)	GNX N=39	34	15% responder	1 (2.6)	5 (14.7)				
	Placebo N=41	36	15% responder	1 (2.4)	7 (19.4)	0.71 (0.20, 2.51)	0.76 (0.27, 2.16)	-4.7 (-27.3, 19.0)	0.7538
Visit 5 (End of Week 17)	GNX N=39	32	15% responder	2 (5.1)	7 (21.9)				
	Placebo N=41	37	15% responder	1 (2.4)	10 (27.0)	0.76 (0.25, 2.29)	0.81 (0.35, 1.88)	-5.2 (-28.3, 18.3)	0.7806

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Females

Sleep Onset Delay Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	37	15% responder	0 (0.0)	10 (27.0)				
	Placebo N=41	38	15% responder	1 (2.4)	9 (23.7)	1.19 (0.43, 3.29)	1.13 (0.53, 2.41)	3.3 (-19.7, 24.9)	0.7950
Visit 4 (End of Week 9)	GNX N=39	35	15% responder	0 (0.0)	14 (40.0)				
	Placebo N=41	38	15% responder	1 (2.4)	6 (15.8)	3.37 (1.15, 9.87)	2.42 (1.08, 5.41)	24.2 (1.5, 45.6)	0.0344
Visit 5 (End of Week 17)	GNX N=39	34	15% responder	0 (0.0)	14 (41.2)				
	Placebo N=41	38	15% responder	1 (2.4)	6 (15.8)	3.54 (1.20, 10.39)	2.49 (1.11, 5.56)	25.4 (2.3, 46.7)	0.0198

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Females

Sleep Duration Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	37	15% responder	0 (0.0)	8 (21.6)				
	Placebo N=41	37	15% responder	1 (2.4)	9 (24.3)	0.86 (0.30, 2.49)	0.89 (0.40, 2.01)	-2.7 (-26.3, 21.1)	1.0000
Visit 4 (End of Week 9)	GNX N=39	35	15% responder	0 (0.0)	10 (28.6)				
	Placebo N=41	36	15% responder	2 (4.9)	12 (33.3)	0.81 (0.30, 2.17)	0.86 (0.44, 1.70)	-4.8 (-28.3, 17.5)	0.7985
Visit 5 (End of Week 17)	GNX N=39	34	15% responder	0 (0.0)	8 (23.5)				
	Placebo N=41	36	15% responder	2 (4.9)	11 (30.6)	0.71 (0.25, 2.01)	0.78 (0.37, 1.67)	-7.0 (-30.0, 16.3)	0.5955

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Females

Sleep Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	29	15% responder	5 (12.8)	3 (10.3)				
	Placebo N=41	34	15% responder	2 (4.9)	3 (8.8)	1.19 (0.22, 6.42)	1.17 (0.26, 5.37)	1.5 (-23.1, 25.9)	1.0000
Visit 4 (End of Week 9)	GNX N=39	28	15% responder	4 (10.3)	4 (14.3)				
	Placebo N=41	34	15% responder	2 (4.9)	7 (20.6)	0.64 (0.17, 2.47)	0.69 (0.23, 2.13)	-6.3 (-30.6, 18.7)	0.7399
Visit 5 (End of Week 17)	GNX N=39	28	15% responder	6 (15.4)	5 (17.9)				
	Placebo N=41	34	15% responder	3 (7.3)	5 (14.7)	1.26 (0.33, 4.89)	1.21 (0.39, 3.77)	3.2 (-21.6, 27.7)	0.7439

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Females

Night Wakings Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	30	15% responder	5 (12.8)	6 (20.0)				
	Placebo N=41	32	15% responder	3 (7.3)	10 (31.3)	0.55 (0.17, 1.76)	0.64 (0.27, 1.54)	-11.3 (-35.2, 13.7)	0.3897
Visit 4 (End of Week 9)	GNX N=39	29	15% responder	2 (5.1)	8 (27.6)				
	Placebo N=41	32	15% responder	3 (7.3)	11 (34.4)	0.73 (0.24, 2.17)	0.80 (0.38, 1.72)	-6.8 (-31.2, 18.4)	0.5933
Visit 5 (End of Week 17)	GNX N=39	28	15% responder	5 (12.8)	10 (35.7)				
	Placebo N=41	32	15% responder	3 (7.3)	14 (43.8)	0.71 (0.25, 2.02)	0.82 (0.43, 1.54)	-8.0 (-32.8, 17.3)	0.6026

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Females

Parasomnias Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	29	15% responder	4 (10.3)	3 (10.3)				
	Placebo N=41	31	15% responder	4 (9.8)	0 (0.0)	8.32 (0.41, 168.45)	7.47 (0.40, 138.58)	10.3 (-15.1, 35.3)	0.1068
Visit 4 (End of Week 9)	GNX N=39	28	15% responder	1 (2.6)	5 (17.9)				
	Placebo N=41	30	15% responder	3 (7.3)	5 (16.7)	1.09 (0.28, 4.25)	1.07 (0.35, 3.31)	1.2 (-23.8, 27.0)	1.0000
Visit 5 (End of Week 17)	GNX N=39	27	15% responder	3 (7.7)	3 (11.1)				
	Placebo N=41	31	15% responder	4 (9.8)	6 (19.4)	0.52 (0.12, 2.32)	0.57 (0.16, 2.08)	-8.2 (-33.3, 17.4)	0.4814

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Females

Sleep Disordered Breathing Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	34	15% responder	3 (7.7)	8 (23.5)				
	Placebo N=41	35	15% responder	2 (4.9)	4 (11.4)	2.38 (0.64, 8.82)	2.06 (0.68, 6.21)	12.1 (-12.0, 34.2)	0.2182
Visit 4 (End of Week 9)	GNX N=39	29	15% responder	1 (2.6)	5 (17.2)				
	Placebo N=41	34	15% responder	2 (4.9)	6 (17.6)	0.97 (0.26, 3.59)	0.98 (0.33, 2.87)	-0.4 (-24.7, 24.0)	1.0000
Visit 5 (End of Week 17)	GNX N=39	31	15% responder	2 (5.1)	7 (22.6)				
	Placebo N=41	31	15% responder	3 (7.3)	8 (25.8)	0.84 (0.26, 2.69)	0.88 (0.36, 2.12)	-3.2 (-29.0, 22.9)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Males

Bedtime Resistance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	9	15% responder	0 (0.0)	3 (33.3)				
	Placebo N=10	9	15% responder	0 (0.0)	2 (22.2)	1.62 (0.23, 11.17)	1.40 (0.36, 5.49)	11.1 (-38.5, 56.9)	1.0000
Visit 4 (End of Week 9)	GNX N=11	9	15% responder	0 (0.0)	2 (22.2)				
	Placebo N=10	9	15% responder	0 (0.0)	0 (0.0)	6.33 (0.26, 152.86)	5.00 (0.27, 91.52)	22.2 (-28.5, 65.4)	0.4706
Visit 5 (End of Week 17)	GNX N=11	8	15% responder	1 (9.1)	2 (25.0)				
	Placebo N=10	8	15% responder	0 (0.0)	2 (25.0)	1.00 (0.13, 7.87)	1.00 (0.23, 4.44)	0.0 (-50.7, 50.7)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Males

Sleep Onset Delay Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	9	15% responder	0 (0.0)	1 (11.1)	2.00 (0.15, 26.73)	1.80 (0.19, 16.66)	8.9 (-33.3, 51.4)	1.0000
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	9	15% responder	0 (0.0)	3 (33.3)	0.50 (0.06, 4.00)	0.60 (0.13, 2.82)	-13.3 (-53.7, 33.2)	0.6285
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	3 (30.0)				
	Placebo N=10	8	15% responder	0 (0.0)	2 (25.0)	1.29 (0.16, 10.45)	1.20 (0.26, 5.53)	5.0 (-39.9, 48.7)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Males

Sleep Duration Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	9	15% responder	1 (9.1)	5 (55.6)				
	Placebo N=10	9	15% responder	0 (0.0)	0 (0.0)	23.22 (1.04, 517.93)	11.00 (0.70, 173.66)	55.6 (4.7, 87.2)	0.0294
Visit 4 (End of Week 9)	GNX N=11	9	15% responder	0 (0.0)	5 (55.6)				
	Placebo N=10	9	15% responder	0 (0.0)	0 (0.0)	23.22 (1.04, 517.93)	11.00 (0.70, 173.66)	55.6 (4.7, 87.2)	0.0294
Visit 5 (End of Week 17)	GNX N=11	9	15% responder	1 (9.1)	5 (55.6)				
	Placebo N=10	8	15% responder	0 (0.0)	0 (0.0)	20.78 (0.92, 466.97)	9.90 (0.63, 155.08)	55.6 (11.5, 86.6)	0.0294

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Males

Sleep Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	9	15% responder	0 (0.0)	2 (22.2)				
	Placebo N=10	9	15% responder	0 (0.0)	1 (11.1)	1.89 (0.20, 17.92)	1.67 (0.27, 10.33)	11.1 (-38.5, 56.9)	1.0000
Visit 4 (End of Week 9)	GNX N=11	9	15% responder	0 (0.0)	1 (11.1)				
	Placebo N=10	9	15% responder	0 (0.0)	1 (11.1)	1.00 (0.09, 11.64)	1.00 (0.12, 8.06)	0.0 (-48.0, 48.0)	1.0000
Visit 5 (End of Week 17)	GNX N=11	9	15% responder	1 (9.1)	1 (11.1)				
	Placebo N=10	7	15% responder	0 (0.0)	1 (14.3)	0.76 (0.06, 9.16)	0.80 (0.10, 6.30)	-3.2 (-49.8, 43.5)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Males

Night Wakings Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	7	15% responder	1 (9.1)	3 (42.9)				
	Placebo N=10	8	15% responder	1 (10.0)	0 (0.0)	13.22 (0.55, 316.64)	7.87 (0.48, 130.28)	42.9 (-10.6, 81.6)	0.0769
Visit 4 (End of Week 9)	GNX N=11	9	15% responder	0 (0.0)	2 (22.2)				
	Placebo N=10	8	15% responder	1 (10.0)	1 (12.5)	1.67 (0.17, 16.02)	1.50 (0.24, 9.19)	9.7 (-34.5, 54.3)	1.0000
Visit 5 (End of Week 17)	GNX N=11	9	15% responder	1 (9.1)	4 (44.4)				
	Placebo N=10	7	15% responder	1 (10.0)	1 (14.3)	4.80 (0.40, 58.01)	3.11 (0.44, 22.00)	30.2 (-19.8, 71.0)	0.3077

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Males

Parasomnias Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	6	15% responder	0 (0.0)	2 (33.3)				
	Placebo N=10	7	15% responder	1 (10.0)	1 (14.3)	2.41 (0.23, 25.35)	1.90 (0.33, 10.98)	19.0 (-37.2, 66.4)	0.5594
Visit 4 (End of Week 9)	GNX N=11	7	15% responder	1 (9.1)	1 (14.3)				
	Placebo N=10	6	15% responder	0 (0.0)	0 (0.0)	3.00 (0.10, 88.13)	2.63 (0.13, 54.64)	14.3 (-37.3, 62.0)	1.0000
Visit 5 (End of Week 17)	GNX N=11	8	15% responder	2 (18.2)	2 (25.0)				
	Placebo N=10	6	15% responder	0 (0.0)	0 (0.0)	5.00 (0.20, 125.78)	3.89 (0.22, 68.67)	25.0 (-29.8, 70.6)	0.4725

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.1 Summary of CSHQ 15 % responders - Males

Sleep Disordered Breathing Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	9	15% responder	0 (0.0)	2 (22.2)				
	Placebo N=10	8	15% responder	1 (10.0)	0 (0.0)	5.67 (0.23, 137.80)	4.50 (0.25, 81.76)	22.2 (-23.7, 63.4)	0.4706
Visit 4 (End of Week 9)	GNX N=11	8	15% responder	1 (9.1)	4 (50.0)				
	Placebo N=10	6	15% responder	1 (10.0)	1 (16.7)	5.00 (0.39, 64.39)	3.00 (0.44, 20.44)	33.3 (-21.5, 77.7)	0.3007
Visit 5 (End of Week 17)	GNX N=11	9	15% responder	1 (9.1)	3 (33.3)				
	Placebo N=10	8	15% responder	0 (0.0)	0 (0.0)	9.15 (0.40, 210.27)	6.30 (0.37, 106.01)	33.3 (-12.3, 71.8)	0.2059

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients who are 2-6 years old

Manic Hyperactive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	9 (33.3)				
	Placebo N=22	21	15% responder	0 (0.0)	6 (28.6)	1.25 (0.36, 4.32)	1.17 (0.49, 2.76)	4.8 (-23.5, 32.6)	0.7639
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	7 (26.9)				
	Placebo N=22	21	15% responder	0 (0.0)	8 (38.1)	0.60 (0.17, 2.06)	0.71 (0.31, 1.63)	-11.2 (-38.8, 17.2)	0.5327
Visit 5 (End of Week 17)	GNX N=30	24	15% responder	0 (0.0)	11 (45.8)				
	Placebo N=22	21	15% responder	0 (0.0)	3 (14.3)	5.08 (1.18, 21.91)	3.21 (1.03, 9.98)	31.5 (1.8, 57.0)	0.0284

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients who are 2-6 years old

Depressed Mood Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	3 (11.1)				
	Placebo N=22	21	15% responder	0 (0.0)	4 (19.0)	0.53 (0.11, 2.69)	0.58 (0.15, 2.33)	-7.9 (-35.7, 20.8)	0.6830
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	3 (11.5)				
	Placebo N=22	21	15% responder	0 (0.0)	5 (23.8)	0.42 (0.09, 2.00)	0.48 (0.13, 1.80)	-12.3 (-39.6, 16.2)	0.4372
Visit 5 (End of Week 17)	GNX N=30	24	15% responder	0 (0.0)	2 (8.3)				
	Placebo N=22	21	15% responder	0 (0.0)	1 (4.8)	1.82 (0.15, 21.62)	1.75 (0.17, 17.95)	3.6 (-25.4, 32.6)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients who are 2-6 years old

Social Avoidance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	5 (18.5)				
	Placebo N=22	21	15% responder	0 (0.0)	4 (19.0)	0.97 (0.22, 4.16)	0.97 (0.30, 3.18)	-0.5 (-28.8, 27.9)	1.0000
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	5 (19.2)				
	Placebo N=22	21	15% responder	0 (0.0)	5 (23.8)	0.76 (0.19, 3.09)	0.81 (0.27, 2.42)	-4.6 (-32.6, 23.6)	0.7339
Visit 5 (End of Week 17)	GNX N=30	23	15% responder	0 (0.0)	4 (17.4)				
	Placebo N=22	19	15% responder	0 (0.0)	4 (21.1)	0.79 (0.17, 3.69)	0.83 (0.24, 2.87)	-3.7 (-32.8, 26.6)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients who are 2-6 years old

General Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	5 (18.5)				
	Placebo N=22	21	15% responder	0 (0.0)	3 (14.3)	1.36 (0.29, 6.50)	1.30 (0.35, 4.82)	4.2 (-24.3, 32.3)	1.0000
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	4 (15.4)				
	Placebo N=22	21	15% responder	0 (0.0)	1 (4.8)	3.64 (0.37, 35.32)	3.23 (0.39, 26.77)	10.6 (-18.2, 38.0)	0.3623
Visit 5 (End of Week 17)	GNX N=30	24	15% responder	0 (0.0)	5 (20.8)				
	Placebo N=22	21	15% responder	0 (0.0)	3 (14.3)	1.58 (0.33, 7.59)	1.46 (0.40, 5.38)	6.5 (-23.2, 35.4)	0.7050

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients who are 2-6 years old

Obsessive Compulsive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	7 (25.9)				
	Placebo N=22	21	15% responder	0 (0.0)	3 (14.3)	2.10 (0.47, 9.36)	1.81 (0.53, 6.19)	11.6 (-17.2, 39.1)	0.4780
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	8 (30.8)				
	Placebo N=22	21	15% responder	0 (0.0)	5 (23.8)	1.42 (0.39, 5.24)	1.29 (0.50, 3.37)	7.0 (-21.8, 34.5)	0.7463
Visit 5 (End of Week 17)	GNX N=30	23	15% responder	0 (0.0)	5 (21.7)				
	Placebo N=22	20	15% responder	0 (0.0)	4 (20.0)	1.11 (0.25, 4.87)	1.09 (0.34, 3.50)	1.7 (-28.0, 31.2)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001

Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients who are >6 years old

Manic Hyperactive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	18	15% responder	0 (0.0)	9 (50.0)				
	Placebo N=29	25	15% responder	1 (3.4)	6 (24.0)	3.00 (0.85, 10.61)	2.00 (0.90, 4.47)	26.0 (-4.4, 53.4)	0.1089
Visit 4 (End of Week 9)	GNX N=20	17	15% responder	0 (0.0)	8 (47.1)				
	Placebo N=29	26	15% responder	1 (3.4)	10 (38.5)	1.41 (0.42, 4.70)	1.21 (0.62, 2.40)	8.6 (-22.4, 38.4)	0.7530
Visit 5 (End of Week 17)	GNX N=20	17	15% responder	1 (5.0)	9 (52.9)				
	Placebo N=29	24	15% responder	1 (3.4)	4 (16.7)	5.63 (1.34, 23.62)	3.18 (1.17, 8.64)	36.3 (5.3, 62.7)	0.0198

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients who are >6 years old

Depressed Mood Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	18	15% responder	0 (0.0)	4 (22.2)				
	Placebo N=29	26	15% responder	1 (3.4)	2 (7.7)	3.04 (0.57, 16.27)	2.56 (0.61, 10.69)	14.5 (-16.0, 42.6)	0.2077
Visit 4 (End of Week 9)	GNX N=20	17	15% responder	0 (0.0)	5 (29.4)				
	Placebo N=29	26	15% responder	1 (3.4)	4 (15.4)	2.20 (0.53, 9.15)	1.83 (0.61, 5.47)	14.0 (-16.8, 42.2)	0.4448
Visit 5 (End of Week 17)	GNX N=20	17	15% responder	1 (5.0)	6 (35.3)				
	Placebo N=29	25	15% responder	1 (3.4)	3 (12.0)	4.00 (0.84, 19.10)	2.94 (0.85, 10.18)	23.3 (-7.1, 51.6)	0.1242

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients who are >6 years old

Social Avoidance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	17	15% responder	1 (5.0)	7 (41.2)				
	Placebo N=29	25	15% responder	1 (3.4)	1 (4.0)	16.80 (1.82, 154.89)	10.29 (1.39, 76.27)	37.2 (7.1, 63.3)	0.0043
Visit 4 (End of Week 9)	GNX N=20	17	15% responder	0 (0.0)	6 (35.3)				
	Placebo N=29	26	15% responder	1 (3.4)	4 (15.4)	2.83 (0.70, 11.42)	2.17 (0.76, 6.15)	19.9 (-11.1, 47.5)	0.1578
Visit 5 (End of Week 17)	GNX N=20	16	15% responder	2 (10.0)	4 (25.0)				
	Placebo N=29	25	15% responder	1 (3.4)	5 (20.0)	1.33 (0.30, 5.96)	1.25 (0.39, 3.97)	5.0 (-25.7, 35.2)	0.7169

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients who are >6 years old

General Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	18	15% responder	0 (0.0)	3 (16.7)				
	Placebo N=29	26	15% responder	1 (3.4)	1 (3.8)	3.84 (0.51, 28.77)	3.32 (0.54, 20.47)	12.8 (-17.7, 40.9)	0.2890
Visit 4 (End of Week 9)	GNX N=20	17	15% responder	0 (0.0)	3 (17.6)				
	Placebo N=29	26	15% responder	1 (3.4)	2 (7.7)	2.37 (0.41, 13.59)	2.10 (0.46, 9.50)	10.0 (-20.6, 38.7)	0.3686
Visit 5 (End of Week 17)	GNX N=20	17	15% responder	1 (5.0)	1 (5.9)				
	Placebo N=29	25	15% responder	1 (3.4)	3 (12.0)	0.46 (0.04, 4.82)	0.49 (0.06, 4.33)	-6.1 (-35.7, 24.5)	0.6355

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients who are >6 years old

Obsessive Compulsive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	18	15% responder	0 (0.0)	7 (38.9)				
	Placebo N=29	25	15% responder	1 (3.4)	5 (20.0)	2.43 (0.65, 9.06)	1.87 (0.74, 4.72)	18.9 (-11.3, 46.9)	0.3014
Visit 4 (End of Week 9)	GNX N=20	17	15% responder	0 (0.0)	5 (29.4)				
	Placebo N=29	26	15% responder	1 (3.4)	7 (26.9)	1.14 (0.31, 4.24)	1.10 (0.44, 2.77)	2.5 (-27.9, 31.7)	1.0000
Visit 5 (End of Week 17)	GNX N=20	17	15% responder	1 (5.0)	7 (41.2)				
	Placebo N=29	25	15% responder	1 (3.4)	6 (24.0)	2.22 (0.58, 8.40)	1.72 (0.70, 4.22)	17.2 (-13.1, 46.4)	0.3141

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients not in US

Manic Hyperactive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	29	15% responder	0 (0.0)	11 (37.9)				
	Placebo N=27	24	15% responder	1 (3.7)	3 (12.5)	3.82 (0.99, 14.70)	2.74 (0.94, 8.00)	25.4 (-1.3, 50.0)	0.0595
Visit 4 (End of Week 9)	GNX N=32	28	15% responder	0 (0.0)	9 (32.1)				
	Placebo N=27	25	15% responder	1 (3.7)	7 (28.0)	1.20 (0.38, 3.79)	1.14 (0.51, 2.52)	4.1 (-22.9, 30.3)	0.7735
Visit 5 (End of Week 17)	GNX N=32	28	15% responder	1 (3.1)	15 (53.6)				
	Placebo N=27	24	15% responder	1 (3.7)	3 (12.5)	8.08 (1.95, 33.40)	4.29 (1.41, 13.05)	41.1 (13.8, 63.7)	0.0030

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients not in US

Depressed Mood Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	29	15% responder	0 (0.0)	5 (17.2)				
	Placebo N=27	25	15% responder	1 (3.7)	2 (8.0)	2.11 (0.43, 10.44)	1.91 (0.47, 7.73)	9.2 (-17.5, 34.9)	0.4305
Visit 4 (End of Week 9)	GNX N=32	28	15% responder	0 (0.0)	4 (14.3)				
	Placebo N=27	25	15% responder	1 (3.7)	6 (24.0)	0.55 (0.14, 2.11)	0.62 (0.21, 1.83)	-9.7 (-36.0, 17.3)	0.4878
Visit 5 (End of Week 17)	GNX N=32	28	15% responder	1 (3.1)	6 (21.4)				
	Placebo N=27	25	15% responder	1 (3.7)	4 (16.0)	1.43 (0.35, 5.80)	1.34 (0.43, 4.21)	5.4 (-21.8, 31.7)	0.7322

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients not in US

Social Avoidance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	28	15% responder	1 (3.1)	10 (35.7)				
	Placebo N=27	25	15% responder	1 (3.7)	2 (8.0)	6.39 (1.24, 32.89)	4.46 (1.08, 18.45)	27.7 (0.2, 51.8)	0.0220
Visit 4 (End of Week 9)	GNX N=32	28	15% responder	0 (0.0)	10 (35.7)				
	Placebo N=27	25	15% responder	1 (3.7)	5 (20.0)	2.12 (0.63, 7.08)	1.71 (0.71, 4.15)	15.7 (-11.7, 40.8)	0.2372
Visit 5 (End of Week 17)	GNX N=32	26	15% responder	2 (6.3)	8 (30.8)				
	Placebo N=27	24	15% responder	1 (3.7)	5 (20.8)	1.69 (0.46, 6.14)	1.48 (0.56, 3.90)	9.9 (-17.8, 36.6)	0.5260

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients not in US

General Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	29	15% responder	0 (0.0)	7 (24.1)				
	Placebo N=27	25	15% responder	1 (3.7)	3 (12.0)	2.14 (0.53, 8.66)	1.86 (0.59, 5.89)	12.1 (-14.6, 37.7)	0.3095
Visit 4 (End of Week 9)	GNX N=32	28	15% responder	0 (0.0)	6 (21.4)				
	Placebo N=27	25	15% responder	1 (3.7)	2 (8.0)	2.72 (0.57, 13.04)	2.33 (0.60, 9.07)	13.4 (-13.9, 39.3)	0.2564
Visit 5 (End of Week 17)	GNX N=32	28	15% responder	1 (3.1)	5 (17.9)				
	Placebo N=27	25	15% responder	1 (3.7)	5 (20.0)	0.87 (0.22, 3.45)	0.89 (0.29, 2.73)	-2.1 (-29.0, 24.6)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients not in US

Obsessive Compulsive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	29	15% responder	0 (0.0)	13 (44.8)				
	Placebo N=27	24	15% responder	1 (3.7)	3 (12.5)	5.03 (1.32, 19.18)	3.21 (1.13, 9.18)	32.3 (5.7, 56.1)	0.0158
Visit 4 (End of Week 9)	GNX N=32	28	15% responder	0 (0.0)	11 (39.3)				
	Placebo N=27	25	15% responder	1 (3.7)	6 (24.0)	1.97 (0.62, 6.28)	1.59 (0.71, 3.54)	15.3 (-12.0, 40.7)	0.2572
Visit 5 (End of Week 17)	GNX N=32	27	15% responder	1 (3.1)	11 (40.7)				
	Placebo N=27	25	15% responder	1 (3.7)	7 (28.0)	1.77 (0.55, 5.65)	1.46 (0.67, 3.16)	12.7 (-13.9, 39.2)	0.3917

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients in US

Manic Hyperactive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	7 (43.8)				
	Placebo N=24	22	15% responder	0 (0.0)	9 (40.9)	1.12 (0.31, 4.13)	1.07 (0.51, 2.26)	2.8 (-29.1, 34.6)	1.0000
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	6 (40.0)				
	Placebo N=24	22	15% responder	0 (0.0)	11 (50.0)	0.67 (0.18, 2.52)	0.80 (0.38, 1.69)	-10.0 (-41.2, 23.3)	0.7384
Visit 5 (End of Week 17)	GNX N=18	13	15% responder	0 (0.0)	5 (38.5)				
	Placebo N=24	21	15% responder	0 (0.0)	4 (19.0)	2.66 (0.56, 12.65)	2.02 (0.66, 6.18)	19.4 (-15.1, 51.3)	0.2538

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients in US

Depressed Mood Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	2 (12.5)				
	Placebo N=24	22	15% responder	0 (0.0)	4 (18.2)	0.64 (0.10, 4.03)	0.69 (0.14, 3.31)	-5.7 (-36.9, 26.2)	1.0000
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	4 (26.7)				
	Placebo N=24	22	15% responder	0 (0.0)	3 (13.6)	2.30 (0.43, 12.25)	1.96 (0.51, 7.51)	13.0 (-19.3, 44.2)	0.4081
Visit 5 (End of Week 17)	GNX N=18	13	15% responder	0 (0.0)	2 (15.4)				
	Placebo N=24	21	15% responder	0 (0.0)	0 (0.0)	9.35 (0.41, 211.62)	7.86 (0.41, 151.85)	15.4 (-19.3, 47.2)	0.1390

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients in US

Social Avoidance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	2 (12.5)				
	Placebo N=24	21	15% responder	0 (0.0)	3 (14.3)	0.86 (0.13, 5.85)	0.87 (0.17, 4.63)	-1.8 (-33.2, 30.2)	1.0000
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	1 (6.7)				
	Placebo N=24	22	15% responder	0 (0.0)	4 (18.2)	0.32 (0.03, 3.21)	0.37 (0.05, 2.97)	-11.5 (-42.3, 21.4)	0.6290
Visit 5 (End of Week 17)	GNX N=18	13	15% responder	0 (0.0)	0 (0.0)				
	Placebo N=24	20	15% responder	0 (0.0)	4 (20.0)	0.14 (0.01, 2.75)	0.17 (0.01, 2.86)	-20.0 (-51.6, 14.6)	0.1359

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients in US

General Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	1 (6.3)				
	Placebo N=24	22	15% responder	0 (0.0)	1 (4.5)	1.40 (0.08, 24.20)	1.38 (0.09, 20.38)	1.7 (-30.4, 33.4)	1.0000
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	1 (6.7)				
	Placebo N=24	22	15% responder	0 (0.0)	1 (4.5)	1.50 (0.09, 26.01)	1.47 (0.10, 21.67)	2.1 (-29.9, 34.1)	1.0000
Visit 5 (End of Week 17)	GNX N=18	13	15% responder	0 (0.0)	1 (7.7)				
	Placebo N=24	21	15% responder	0 (0.0)	1 (4.8)	1.67 (0.10, 29.18)	1.62 (0.11, 23.66)	2.9 (-31.0, 35.9)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Patients in US

Obsessive Compulsive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	1 (6.3)				
	Placebo N=24	22	15% responder	0 (0.0)	5 (22.7)	0.23 (0.02, 2.16)	0.28 (0.04, 2.13)	-16.5 (-46.6, 15.9)	0.3696
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	2 (13.3)				
	Placebo N=24	22	15% responder	0 (0.0)	6 (27.3)	0.41 (0.07, 2.38)	0.49 (0.11, 2.10)	-13.9 (-44.2, 19.3)	0.4310
Visit 5 (End of Week 17)	GNX N=18	13	15% responder	0 (0.0)	1 (7.7)				
	Placebo N=24	20	15% responder	0 (0.0)	3 (15.0)	0.47 (0.04, 5.11)	0.51 (0.06, 4.41)	-7.3 (-40.6, 26.7)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Females

Manic Hyperactive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	35	15% responder	0 (0.0)	15 (42.9)				
	Placebo N=41	37	15% responder	1 (2.4)	8 (21.6)	2.62 (0.96, 7.20)	1.92 (0.95, 3.88)	21.2 (-2.4, 42.4)	0.0768
Visit 4 (End of Week 9)	GNX N=39	33	15% responder	0 (0.0)	12 (36.4)				
	Placebo N=41	38	15% responder	1 (2.4)	14 (36.8)	0.98 (0.38, 2.55)	0.99 (0.54, 1.80)	-0.5 (-23.4, 22.6)	1.0000
Visit 5 (End of Week 17)	GNX N=39	31	15% responder	1 (2.6)	15 (48.4)				
	Placebo N=41	38	15% responder	1 (2.4)	7 (18.4)	4.15 (1.41, 12.24)	2.63 (1.23, 5.63)	30.0 (6.4, 51.2)	0.0102

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Females

Depressed Mood Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	35	15% responder	0 (0.0)	7 (20.0)				
	Placebo N=41	38	15% responder	1 (2.4)	3 (7.9)	2.67 (0.68, 10.41)	2.32 (0.71, 7.59)	12.1 (-11.3, 34.3)	0.1793
Visit 4 (End of Week 9)	GNX N=39	33	15% responder	0 (0.0)	6 (18.2)				
	Placebo N=41	38	15% responder	1 (2.4)	5 (13.2)	1.44 (0.42, 4.99)	1.36 (0.48, 3.83)	5.0 (-18.0, 28.0)	0.7441
Visit 5 (End of Week 17)	GNX N=39	31	15% responder	1 (2.6)	6 (19.4)				
	Placebo N=41	38	15% responder	1 (2.4)	4 (10.5)	2.04 (0.52, 8.00)	1.84 (0.57, 5.94)	8.8 (-14.9, 31.7)	0.3269

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Females

Social Avoidance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	34	15% responder	1 (2.6)	10 (29.4)				
	Placebo N=41	38	15% responder	1 (2.4)	3 (7.9)	4.86 (1.21, 19.53)	3.73 (1.12, 12.42)	21.5 (-1.9, 43.0)	0.0296
Visit 4 (End of Week 9)	GNX N=39	33	15% responder	0 (0.0)	9 (27.3)				
	Placebo N=41	38	15% responder	1 (2.4)	6 (15.8)	1.94 (0.63, 5.98)	1.68 (0.69, 4.06)	11.5 (-11.9, 33.9)	0.2600
Visit 5 (End of Week 17)	GNX N=39	29	15% responder	2 (5.1)	6 (20.7)				
	Placebo N=41	36	15% responder	1 (2.4)	8 (22.2)	0.91 (0.28, 3.01)	0.93 (0.36, 2.38)	-1.5 (-25.4, 22.8)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Females

General Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	35	15% responder	0 (0.0)	7 (20.0)				
	Placebo N=41	38	15% responder	1 (2.4)	3 (7.9)	2.67 (0.68, 10.41)	2.32 (0.71, 7.59)	12.1 (-11.3, 34.3)	0.1793
Visit 4 (End of Week 9)	GNX N=39	33	15% responder	0 (0.0)	6 (18.2)				
	Placebo N=41	38	15% responder	1 (2.4)	3 (7.9)	2.40 (0.60, 9.64)	2.13 (0.63, 7.18)	10.3 (-12.9, 33.0)	0.2867
Visit 5 (End of Week 17)	GNX N=39	31	15% responder	1 (2.6)	4 (12.9)				
	Placebo N=41	38	15% responder	1 (2.4)	4 (10.5)	1.26 (0.29, 5.50)	1.23 (0.33, 4.51)	2.4 (-21.0, 25.7)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Females

Obsessive Compulsive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	35	15% responder	0 (0.0)	12 (34.3)				
	Placebo N=41	37	15% responder	1 (2.4)	7 (18.9)	2.16 (0.75, 6.20)	1.76 (0.80, 3.85)	15.4 (-7.9, 37.3)	0.1837
Visit 4 (End of Week 9)	GNX N=39	33	15% responder	0 (0.0)	12 (36.4)				
	Placebo N=41	38	15% responder	1 (2.4)	10 (26.3)	1.58 (0.58, 4.26)	1.37 (0.69, 2.69)	10.0 (-13.3, 32.6)	0.4434
Visit 5 (End of Week 17)	GNX N=39	30	15% responder	1 (2.6)	10 (33.3)				
	Placebo N=41	37	15% responder	1 (2.4)	10 (27.0)	1.35 (0.47, 3.86)	1.23 (0.59, 2.57)	6.3 (-17.6, 29.8)	0.6019

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Males

Manic Hyperactive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	3 (30.0)				
	Placebo N=10	9	15% responder	0 (0.0)	4 (44.4)	0.54 (0.08, 3.53)	0.68 (0.20, 2.23)	-14.4 (-56.1, 32.7)	0.6499
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	3 (30.0)				
	Placebo N=10	9	15% responder	0 (0.0)	4 (44.4)	0.54 (0.08, 3.53)	0.68 (0.20, 2.23)	-14.4 (-56.1, 32.7)	0.6499
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	5 (50.0)				
	Placebo N=10	7	15% responder	0 (0.0)	0 (0.0)	15.00 (0.68, 332.00)	8.00 (0.51, 124.90)	50.0 (0.6, 82.8)	0.0441

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Males

Depressed Mood Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	0 (0.0)				
	Placebo N=10	9	15% responder	0 (0.0)	3 (33.3)	0.09 (0.00, 2.00)	0.13 (0.01, 2.22)	-33.3 (-70.0, 13.4)	0.0867
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	9	15% responder	0 (0.0)	4 (44.4)	0.31 (0.04, 2.38)	0.45 (0.11, 1.90)	-24.4 (-63.5, 23.3)	0.3498
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	8	15% responder	0 (0.0)	0 (0.0)	5.00 (0.21, 120.44)	4.09 (0.22, 74.78)	20.0 (-28.1, 61.9)	0.4771

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Males

Social Avoidance Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	8	15% responder	0 (0.0)	2 (25.0)	0.75 (0.08, 6.96)	0.80 (0.14, 4.49)	-5.0 (-48.7, 39.9)	1.0000
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	9	15% responder	0 (0.0)	3 (33.3)	0.50 (0.06, 4.00)	0.60 (0.13, 2.82)	-13.3 (-53.7, 33.2)	0.6285
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	8	15% responder	0 (0.0)	1 (12.5)	1.75 (0.13, 23.70)	1.60 (0.17, 14.63)	7.5 (-38.8, 51.4)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Males

General Anxiety Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	1 (10.0)				
	Placebo N=10	9	15% responder	0 (0.0)	1 (11.1)	0.89 (0.05, 16.66)	0.90 (0.07, 12.38)	-1.1 (-42.5, 42.5)	1.0000
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	1 (10.0)				
	Placebo N=10	9	15% responder	0 (0.0)	0 (0.0)	3.00 (0.11, 83.36)	2.73 (0.12, 59.57)	10.0 (-33.3, 51.4)	1.0000
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	8	15% responder	0 (0.0)	2 (25.0)	0.75 (0.08, 6.96)	0.80 (0.14, 4.49)	-5.0 (-48.7, 39.9)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.2 Summary of ADAMS 15 % responders - Males

Obsessive Compulsive Behavior Total

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	9	15% responder	0 (0.0)	1 (11.1)	2.00 (0.15, 26.73)	1.80 (0.19, 16.66)	8.9 (-33.3, 51.4)	1.0000
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	1 (10.0)				
	Placebo N=10	9	15% responder	0 (0.0)	2 (22.2)	0.39 (0.03, 5.21)	0.45 (0.05, 4.16)	-12.2 (-51.4, 33.3)	0.5820
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	8	15% responder	0 (0.0)	0 (0.0)	5.00 (0.21, 120.44)	4.09 (0.22, 74.78)	20.0 (-28.1, 61.9)	0.4771

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001

Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are 2-6 years old

Physical Health Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	8 (29.6)				
	Placebo N=22	20	15% responder	1 (4.5)	8 (40.0)	0.64 (0.20, 2.10)	0.75 (0.35, 1.61)	-10.4 (-38.0, 18.8)	0.5408
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	13 (50.0)				
	Placebo N=22	20	15% responder	1 (4.5)	8 (40.0)	1.47 (0.46, 4.66)	1.24 (0.65, 2.35)	10.0 (-19.5, 38.0)	0.5608
Visit 5 (End of Week 17)	GNX N=30	25	15% responder	0 (0.0)	11 (44.0)				
	Placebo N=22	20	15% responder	1 (4.5)	10 (50.0)	0.79 (0.25, 2.51)	0.88 (0.48, 1.62)	-6.0 (-34.8, 23.6)	0.7685

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are 2-6 years old

Positive Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	13 (48.1)				
	Placebo N=22	20	15% responder	1 (4.5)	6 (30.0)	2.08 (0.63, 6.80)	1.56 (0.74, 3.28)	18.1 (-11.0, 45.3)	0.2438
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	12 (46.2)				
	Placebo N=22	20	15% responder	1 (4.5)	8 (40.0)	1.27 (0.40, 4.02)	1.14 (0.59, 2.21)	6.2 (-23.1, 34.4)	0.7686
Visit 5 (End of Week 17)	GNX N=30	25	15% responder	0 (0.0)	10 (40.0)				
	Placebo N=22	20	15% responder	1 (4.5)	6 (30.0)	1.51 (0.45, 5.08)	1.30 (0.59, 2.88)	10.0 (-19.7, 38.5)	0.5444

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are 2-6 years old

Negative Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	10 (37.0)				
	Placebo N=22	19	15% responder	1 (4.5)	8 (42.1)	0.81 (0.25, 2.62)	0.88 (0.44, 1.77)	-5.1 (-33.9, 23.9)	0.7670
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	10 (38.5)				
	Placebo N=22	20	15% responder	1 (4.5)	5 (25.0)	1.79 (0.52, 6.22)	1.48 (0.63, 3.51)	13.5 (-15.9, 40.9)	0.3648
Visit 5 (End of Week 17)	GNX N=30	25	15% responder	0 (0.0)	9 (36.0)				
	Placebo N=22	19	15% responder	1 (4.5)	10 (52.6)	0.52 (0.16, 1.71)	0.70 (0.36, 1.34)	-16.6 (-44.8, 13.5)	0.3605

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are 2-6 years old

Social Interaction Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	12 (44.4)				
	Placebo N=22	20	15% responder	1 (4.5)	10 (50.0)	0.81 (0.26, 2.51)	0.89 (0.49, 1.62)	-5.6 (-34.0, 23.5)	0.7729
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	12 (46.2)				
	Placebo N=22	20	15% responder	1 (4.5)	8 (40.0)	1.27 (0.40, 4.02)	1.14 (0.59, 2.21)	6.2 (-23.1, 34.4)	0.7686
Visit 5 (End of Week 17)	GNX N=30	25	15% responder	0 (0.0)	13 (52.0)				
	Placebo N=22	19	15% responder	1 (4.5)	7 (36.8)	1.80 (0.55, 5.91)	1.38 (0.70, 2.72)	15.2 (-15.2, 43.2)	0.3715

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are 2-6 years old

Leisure and the Outdoors Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	14 (51.9)				
	Placebo N=22	20	15% responder	1 (4.5)	11 (55.0)	0.89 (0.29, 2.76)	0.95 (0.56, 1.60)	-3.1 (-31.7, 25.8)	1.0000
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	18 (69.2)				
	Placebo N=22	19	15% responder	1 (4.5)	11 (57.9)	1.61 (0.48, 5.35)	1.19 (0.76, 1.88)	11.3 (-18.4, 39.6)	0.5336
Visit 5 (End of Week 17)	GNX N=30	25	15% responder	0 (0.0)	15 (60.0)				
	Placebo N=22	19	15% responder	1 (4.5)	5 (26.3)	3.89 (1.11, 13.66)	2.17 (0.99, 4.72)	33.7 (3.5, 59.1)	0.0354

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are 2-6 years old

Independence Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=30	27	15% responder	0 (0.0)	12 (44.4)				
	Placebo N=22	20	15% responder	1 (4.5)	9 (45.0)	0.98 (0.31, 3.04)	0.99 (0.53, 1.85)	-0.6 (-29.3, 28.2)	1.0000
Visit 4 (End of Week 9)	GNX N=30	26	15% responder	0 (0.0)	16 (61.5)				
	Placebo N=22	19	15% responder	1 (4.5)	9 (47.4)	1.74 (0.54, 5.60)	1.29 (0.74, 2.23)	14.2 (-15.8, 42.4)	0.3792
Visit 5 (End of Week 17)	GNX N=30	25	15% responder	0 (0.0)	15 (60.0)				
	Placebo N=22	19	15% responder	1 (4.5)	6 (31.6)	3.07 (0.90, 10.39)	1.83 (0.91, 3.72)	28.4 (-2.0, 54.8)	0.0759

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are >6 years old

Physical Health Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	20	15% responder	0 (0.0)	2 (10.0)				
	Placebo N=29	27	15% responder	0 (0.0)	11 (40.7)	0.16 (0.03, 0.84)	0.25 (0.06, 0.99)	-30.7 (-56.1, -2.2)	0.0245
Visit 4 (End of Week 9)	GNX N=20	19	15% responder	0 (0.0)	7 (36.8)				
	Placebo N=29	26	15% responder	0 (0.0)	12 (46.2)	0.68 (0.20, 2.28)	0.80 (0.39, 1.64)	-9.3 (-37.8, 20.5)	0.5593
Visit 5 (End of Week 17)	GNX N=20	19	15% responder	0 (0.0)	7 (36.8)				
	Placebo N=29	26	15% responder	0 (0.0)	10 (38.5)	0.93 (0.28, 3.17)	0.96 (0.45, 2.06)	-1.6 (-30.7, 27.7)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are >6 years old

Positive Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	19	15% responder	0 (0.0)	6 (31.6)				
	Placebo N=29	27	15% responder	0 (0.0)	9 (33.3)	0.92 (0.26, 3.24)	0.95 (0.40, 2.22)	-1.8 (-30.5, 27.3)	1.0000
Visit 4 (End of Week 9)	GNX N=20	18	15% responder	0 (0.0)	7 (38.9)				
	Placebo N=29	27	15% responder	0 (0.0)	10 (37.0)	1.08 (0.32, 3.69)	1.05 (0.49, 2.24)	1.9 (-28.3, 31.7)	1.0000
Visit 5 (End of Week 17)	GNX N=20	17	15% responder	0 (0.0)	8 (47.1)				
	Placebo N=29	26	15% responder	0 (0.0)	9 (34.6)	1.68 (0.48, 5.85)	1.36 (0.65, 2.82)	12.4 (-18.7, 41.8)	0.5277

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are >6 years old

Negative Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	19	15% responder	0 (0.0)	8 (42.1)				
	Placebo N=29	27	15% responder	0 (0.0)	9 (33.3)	1.45 (0.43, 4.89)	1.26 (0.60, 2.67)	8.8 (-20.4, 37.3)	0.7571
Visit 4 (End of Week 9)	GNX N=20	18	15% responder	0 (0.0)	10 (55.6)				
	Placebo N=29	27	15% responder	0 (0.0)	10 (37.0)	2.13 (0.63, 7.16)	1.50 (0.79, 2.85)	18.5 (-12.2, 46.9)	0.2412
Visit 5 (End of Week 17)	GNX N=20	18	15% responder	0 (0.0)	8 (44.4)				
	Placebo N=29	26	15% responder	0 (0.0)	12 (46.2)	0.93 (0.28, 3.12)	0.96 (0.50, 1.87)	-1.7 (-31.1, 28.3)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are >6 years old

Social Interaction Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	19	15% responder	0 (0.0)	5 (26.3)				
	Placebo N=29	26	15% responder	1 (3.4)	6 (23.1)	1.20 (0.32, 4.48)	1.14 (0.43, 3.04)	3.2 (-25.8, 31.9)	1.0000
Visit 4 (End of Week 9)	GNX N=20	18	15% responder	0 (0.0)	6 (33.3)				
	Placebo N=29	25	15% responder	1 (3.4)	10 (40.0)	0.77 (0.22, 2.63)	0.85 (0.39, 1.85)	-6.7 (-35.8, 23.6)	0.7548
Visit 5 (End of Week 17)	GNX N=20	18	15% responder	0 (0.0)	5 (27.8)				
	Placebo N=29	25	15% responder	1 (3.4)	8 (32.0)	0.84 (0.23, 3.04)	0.89 (0.36, 2.17)	-4.2 (-33.3, 25.7)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are >6 years old

Leisure and the Outdoors Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	19	15% responder	0 (0.0)	7 (36.8)				
	Placebo N=29	26	15% responder	1 (3.4)	8 (30.8)	1.31 (0.39, 4.41)	1.19 (0.54, 2.63)	6.1 (-23.3, 34.7)	0.7542
Visit 4 (End of Week 9)	GNX N=20	18	15% responder	0 (0.0)	7 (38.9)				
	Placebo N=29	26	15% responder	1 (3.4)	13 (50.0)	0.65 (0.20, 2.14)	0.79 (0.40, 1.55)	-11.1 (-39.7, 19.4)	0.5467
Visit 5 (End of Week 17)	GNX N=20	18	15% responder	0 (0.0)	6 (33.3)				
	Placebo N=29	25	15% responder	1 (3.4)	9 (36.0)	0.90 (0.26, 3.12)	0.94 (0.42, 2.09)	-2.7 (-32.0, 27.3)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients who are >6 years old

Independence Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=20	20	15% responder	0 (0.0)	7 (35.0)				
	Placebo N=29	27	15% responder	0 (0.0)	10 (37.0)	0.92 (0.27, 3.06)	0.95 (0.44, 2.05)	-2.0 (-30.6, 26.5)	1.0000
Visit 4 (End of Week 9)	GNX N=20	18	15% responder	0 (0.0)	10 (55.6)				
	Placebo N=29	25	15% responder	0 (0.0)	15 (60.0)	0.83 (0.24, 2.84)	0.93 (0.55, 1.56)	-4.4 (-34.2, 25.7)	1.0000
Visit 5 (End of Week 17)	GNX N=20	18	15% responder	0 (0.0)	9 (50.0)				
	Placebo N=29	26	15% responder	0 (0.0)	8 (30.8)	2.25 (0.65, 7.81)	1.63 (0.78, 3.40)	19.2 (-10.8, 47.4)	0.2250

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients not in US

Physical Health Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	31	15% responder	0 (0.0)	8 (25.8)				
	Placebo N=27	26	15% responder	0 (0.0)	15 (57.7)	0.26 (0.08, 0.78)	0.45 (0.23, 0.88)	-31.9 (-54.9, -5.7)	0.0176
Visit 4 (End of Week 9)	GNX N=32	30	15% responder	0 (0.0)	15 (50.0)				
	Placebo N=27	25	15% responder	0 (0.0)	15 (60.0)	0.67 (0.23, 1.95)	0.83 (0.52, 1.35)	-10.0 (-35.8, 16.8)	0.5881
Visit 5 (End of Week 17)	GNX N=32	30	15% responder	0 (0.0)	14 (46.7)				
	Placebo N=27	26	15% responder	0 (0.0)	15 (57.7)	0.64 (0.22, 1.85)	0.81 (0.49, 1.34)	-11.0 (-36.6, 15.6)	0.4357

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients not in US

Positive Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	30	15% responder	0 (0.0)	17 (56.7)				
	Placebo N=27	26	15% responder	0 (0.0)	11 (42.3)	1.78 (0.62, 5.15)	1.34 (0.77, 2.31)	14.4 (-12.4, 39.6)	0.4218
Visit 4 (End of Week 9)	GNX N=32	29	15% responder	0 (0.0)	14 (48.3)				
	Placebo N=27	26	15% responder	0 (0.0)	13 (50.0)	0.93 (0.32, 2.69)	0.97 (0.56, 1.65)	-1.7 (-28.2, 25.0)	1.0000
Visit 5 (End of Week 17)	GNX N=32	29	15% responder	0 (0.0)	15 (51.7)				
	Placebo N=27	26	15% responder	0 (0.0)	12 (46.2)	1.25 (0.43, 3.61)	1.12 (0.65, 1.93)	5.6 (-21.2, 31.8)	0.7891

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients not in US

Negative Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	30	15% responder	0 (0.0)	13 (43.3)				
	Placebo N=27	26	15% responder	0 (0.0)	11 (42.3)	1.04 (0.36, 3.01)	1.02 (0.56, 1.88)	1.0 (-25.0, 27.2)	1.0000
Visit 4 (End of Week 9)	GNX N=32	29	15% responder	0 (0.0)	12 (41.4)				
	Placebo N=27	26	15% responder	0 (0.0)	10 (38.5)	1.13 (0.38, 3.33)	1.08 (0.56, 2.06)	2.9 (-23.2, 29.3)	1.0000
Visit 5 (End of Week 17)	GNX N=32	29	15% responder	0 (0.0)	13 (44.8)				
	Placebo N=27	26	15% responder	0 (0.0)	13 (50.0)	0.81 (0.28, 2.35)	0.90 (0.51, 1.57)	-5.2 (-31.4, 21.7)	0.7896

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients not in US

Social Interaction Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	30	15% responder	0 (0.0)	14 (46.7)				
	Placebo N=27	25	15% responder	1 (3.7)	10 (40.0)	1.30 (0.45, 3.72)	1.16 (0.64, 2.11)	6.7 (-19.9, 32.6)	0.7855
Visit 4 (End of Week 9)	GNX N=32	29	15% responder	0 (0.0)	15 (51.7)				
	Placebo N=27	24	15% responder	1 (3.7)	11 (45.8)	1.25 (0.43, 3.64)	1.12 (0.65, 1.94)	5.9 (-21.3, 32.5)	0.7846
Visit 5 (End of Week 17)	GNX N=32	29	15% responder	0 (0.0)	14 (48.3)				
	Placebo N=27	25	15% responder	1 (3.7)	12 (48.0)	1.01 (0.35, 2.89)	1.01 (0.58, 1.73)	0.3 (-26.4, 27.0)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients not in US

Leisure and the Outdoors Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	30	15% responder	0 (0.0)	14 (46.7)				
	Placebo N=27	25	15% responder	1 (3.7)	9 (36.0)	1.53 (0.53, 4.43)	1.28 (0.68, 2.40)	10.7 (-16.0, 36.3)	0.5837
Visit 4 (End of Week 9)	GNX N=32	29	15% responder	0 (0.0)	20 (69.0)				
	Placebo N=27	25	15% responder	1 (3.7)	13 (52.0)	2.00 (0.67, 5.93)	1.32 (0.85, 2.05)	17.0 (-10.2, 42.1)	0.2663
Visit 5 (End of Week 17)	GNX N=32	29	15% responder	0 (0.0)	16 (55.2)				
	Placebo N=27	24	15% responder	1 (3.7)	9 (37.5)	1.99 (0.68, 5.88)	1.45 (0.80, 2.63)	17.7 (-9.8, 43.2)	0.2712

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients not in US

Independence Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=32	31	15% responder	0 (0.0)	14 (45.2)				
	Placebo N=27	26	15% responder	0 (0.0)	13 (50.0)	0.82 (0.29, 2.34)	0.90 (0.52, 1.56)	-4.8 (-30.5, 21.4)	0.7931
Visit 4 (End of Week 9)	GNX N=32	29	15% responder	0 (0.0)	18 (62.1)				
	Placebo N=27	25	15% responder	0 (0.0)	14 (56.0)	1.29 (0.43, 3.82)	1.11 (0.71, 1.74)	6.1 (-20.8, 32.1)	0.7827
Visit 5 (End of Week 17)	GNX N=32	29	15% responder	0 (0.0)	16 (55.2)				
	Placebo N=27	26	15% responder	0 (0.0)	9 (34.6)	2.32 (0.78, 6.92)	1.59 (0.86, 2.97)	20.6 (-6.3, 45.6)	0.1767

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients in US

Physical Health Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	2 (12.5)				
	Placebo N=24	21	15% responder	1 (4.2)	4 (19.0)	0.67 (0.12, 3.65)	0.72 (0.18, 2.95)	-6.5 (-37.5, 25.8)	0.6796
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	5 (33.3)				
	Placebo N=24	21	15% responder	1 (4.2)	5 (23.8)	1.57 (0.38, 6.45)	1.38 (0.51, 3.70)	9.5 (-23.9, 41.1)	0.7086
Visit 5 (End of Week 17)	GNX N=18	14	15% responder	0 (0.0)	4 (28.6)				
	Placebo N=24	20	15% responder	1 (4.2)	5 (25.0)	1.21 (0.28, 5.26)	1.15 (0.40, 3.29)	3.6 (-30.0, 36.8)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients in US

Positive Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	2 (12.5)				
	Placebo N=24	21	15% responder	1 (4.2)	4 (19.0)	0.67 (0.12, 3.65)	0.72 (0.18, 2.95)	-6.5 (-37.5, 25.8)	0.6796
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	5 (33.3)				
	Placebo N=24	21	15% responder	1 (4.2)	5 (23.8)	1.57 (0.38, 6.45)	1.38 (0.51, 3.70)	9.5 (-23.9, 41.1)	0.7086
Visit 5 (End of Week 17)	GNX N=18	13	15% responder	0 (0.0)	3 (23.1)				
	Placebo N=24	20	15% responder	1 (4.2)	3 (15.0)	1.67 (0.31, 8.83)	1.50 (0.40, 5.61)	8.1 (-26.7, 40.6)	0.6588

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients in US

Negative Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	5 (31.3)				
	Placebo N=24	20	15% responder	1 (4.2)	6 (30.0)	1.07 (0.27, 4.22)	1.05 (0.41, 2.67)	1.3 (-31.5, 33.8)	1.0000
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	8 (53.3)				
	Placebo N=24	21	15% responder	1 (4.2)	5 (23.8)	3.40 (0.86, 13.47)	2.13 (0.90, 5.01)	29.5 (-3.9, 58.8)	0.0895
Visit 5 (End of Week 17)	GNX N=18	14	15% responder	0 (0.0)	4 (28.6)				
	Placebo N=24	19	15% responder	1 (4.2)	9 (47.4)	0.47 (0.12, 1.94)	0.63 (0.26, 1.55)	-18.8 (-50.6, 16.2)	0.3095

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients in US

Social Interaction Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	3 (18.8)				
	Placebo N=24	21	15% responder	1 (4.2)	6 (28.6)	0.62 (0.14, 2.74)	0.70 (0.22, 2.17)	-9.8 (-40.3, 22.8)	0.7023
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	3 (20.0)				
	Placebo N=24	21	15% responder	1 (4.2)	7 (33.3)	0.54 (0.12, 2.37)	0.64 (0.22, 1.91)	-13.3 (-44.9, 19.9)	0.4682
Visit 5 (End of Week 17)	GNX N=18	14	15% responder	0 (0.0)	4 (28.6)				
	Placebo N=24	19	15% responder	1 (4.2)	3 (15.8)	2.02 (0.41, 9.98)	1.71 (0.50, 5.84)	12.8 (-21.8, 44.5)	0.4221

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients in US

Leisure and the Outdoors Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	7 (43.8)				
	Placebo N=24	21	15% responder	1 (4.2)	10 (47.6)	0.86 (0.24, 3.08)	0.92 (0.46, 1.84)	-3.9 (-35.6, 28.7)	1.0000
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	5 (33.3)				
	Placebo N=24	20	15% responder	1 (4.2)	11 (55.0)	0.43 (0.11, 1.66)	0.63 (0.29, 1.37)	-21.7 (-52.4, 12.6)	0.3064
Visit 5 (End of Week 17)	GNX N=18	14	15% responder	0 (0.0)	5 (35.7)				
	Placebo N=24	20	15% responder	1 (4.2)	5 (25.0)	1.63 (0.39, 6.83)	1.40 (0.53, 3.73)	10.7 (-23.1, 43.4)	0.7041

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Patients in US

Independence Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=18	16	15% responder	0 (0.0)	5 (31.3)				
	Placebo N=24	21	15% responder	1 (4.2)	6 (28.6)	1.14 (0.29, 4.48)	1.10 (0.43, 2.81)	2.7 (-28.8, 34.6)	1.0000
Visit 4 (End of Week 9)	GNX N=18	15	15% responder	0 (0.0)	8 (53.3)				
	Placebo N=24	19	15% responder	1 (4.2)	10 (52.6)	1.03 (0.27, 3.83)	1.01 (0.54, 1.88)	0.7 (-33.0, 34.2)	1.0000
Visit 5 (End of Week 17)	GNX N=18	14	15% responder	0 (0.0)	8 (57.1)				
	Placebo N=24	19	15% responder	1 (4.2)	5 (26.3)	3.45 (0.84, 14.21)	2.06 (0.89, 4.76)	30.8 (-4.4, 60.9)	0.1482

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Females

Physical Health Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	37	15% responder	0 (0.0)	9 (24.3)				
	Placebo N=41	38	15% responder	1 (2.4)	14 (36.8)	0.56 (0.21, 1.50)	0.67 (0.34, 1.33)	-12.5 (-34.9, 9.3)	0.3180
Visit 4 (End of Week 9)	GNX N=39	35	15% responder	0 (0.0)	15 (42.9)				
	Placebo N=41	37	15% responder	1 (2.4)	18 (48.6)	0.80 (0.32, 1.99)	0.88 (0.54, 1.45)	-5.8 (-29.0, 17.6)	0.6442
Visit 5 (End of Week 17)	GNX N=39	34	15% responder	0 (0.0)	14 (41.2)				
	Placebo N=41	38	15% responder	1 (2.4)	18 (47.4)	0.78 (0.31, 1.97)	0.87 (0.52, 1.46)	-6.2 (-29.1, 17.0)	0.6409

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Females

Positive Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	36	15% responder	0 (0.0)	13 (36.1)				
	Placebo N=41	38	15% responder	1 (2.4)	10 (26.3)	1.56 (0.59, 4.13)	1.36 (0.69, 2.65)	9.8 (-13.4, 31.6)	0.4534
Visit 4 (End of Week 9)	GNX N=39	34	15% responder	0 (0.0)	15 (44.1)				
	Placebo N=41	38	15% responder	1 (2.4)	15 (39.5)	1.21 (0.48, 3.04)	1.11 (0.65, 1.90)	4.6 (-18.7, 27.3)	0.8116
Visit 5 (End of Week 17)	GNX N=39	32	15% responder	0 (0.0)	13 (40.6)				
	Placebo N=41	38	15% responder	1 (2.4)	12 (31.6)	1.47 (0.56, 3.86)	1.28 (0.69, 2.36)	9.0 (-14.3, 31.9)	0.4628

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Females

Negative Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	36	15% responder	0 (0.0)	16 (44.4)				
	Placebo N=41	37	15% responder	1 (2.4)	13 (35.1)	1.46 (0.58, 3.70)	1.26 (0.72, 2.20)	9.3 (-14.5, 31.5)	0.4778
Visit 4 (End of Week 9)	GNX N=39	34	15% responder	0 (0.0)	17 (50.0)				
	Placebo N=41	38	15% responder	1 (2.4)	12 (31.6)	2.12 (0.82, 5.45)	1.56 (0.89, 2.74)	18.4 (-5.1, 40.2)	0.1500
Visit 5 (End of Week 17)	GNX N=39	33	15% responder	0 (0.0)	13 (39.4)				
	Placebo N=41	37	15% responder	1 (2.4)	17 (45.9)	0.77 (0.30, 1.97)	0.86 (0.50, 1.48)	-6.6 (-29.7, 16.9)	0.6340

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Females

Social Interaction Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	36	15% responder	0 (0.0)	13 (36.1)				
	Placebo N=41	37	15% responder	2 (4.9)	11 (29.7)	1.32 (0.51, 3.47)	1.21 (0.63, 2.29)	6.4 (-17.3, 27.9)	0.6235
Visit 4 (End of Week 9)	GNX N=39	34	15% responder	0 (0.0)	14 (41.2)				
	Placebo N=41	37	15% responder	2 (4.9)	13 (35.1)	1.28 (0.50, 3.30)	1.17 (0.65, 2.09)	6.0 (-17.6, 28.6)	0.6324
Visit 5 (End of Week 17)	GNX N=39	33	15% responder	0 (0.0)	13 (39.4)				
	Placebo N=41	36	15% responder	2 (4.9)	11 (30.6)	1.46 (0.55, 3.88)	1.28 (0.68, 2.41)	8.8 (-15.0, 31.8)	0.4610

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Females

Leisure and the Outdoors Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	36	15% responder	0 (0.0)	17 (47.2)				
	Placebo N=41	37	15% responder	2 (4.9)	13 (35.1)	1.63 (0.65, 4.11)	1.33 (0.77, 2.30)	12.1 (-11.8, 34.2)	0.3461
Visit 4 (End of Week 9)	GNX N=39	34	15% responder	0 (0.0)	19 (55.9)				
	Placebo N=41	36	15% responder	2 (4.9)	19 (52.8)	1.13 (0.45, 2.86)	1.06 (0.69, 1.62)	3.1 (-20.5, 26.8)	0.8150
Visit 5 (End of Week 17)	GNX N=39	33	15% responder	0 (0.0)	17 (51.5)				
	Placebo N=41	36	15% responder	2 (4.9)	12 (33.3)	2.08 (0.80, 5.41)	1.52 (0.87, 2.66)	18.2 (-6.0, 40.5)	0.1489

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Females

Independence Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=39	37	15% responder	0 (0.0)	14 (37.8)				
	Placebo N=41	38	15% responder	1 (2.4)	13 (34.2)	1.17 (0.46, 2.95)	1.10 (0.61, 1.99)	3.6 (-19.7, 25.4)	0.8123
Visit 4 (End of Week 9)	GNX N=39	34	15% responder	0 (0.0)	21 (61.8)				
	Placebo N=41	35	15% responder	1 (2.4)	19 (54.3)	1.35 (0.52, 3.47)	1.13 (0.76, 1.69)	7.5 (-16.2, 31.3)	0.6279
Visit 5 (End of Week 17)	GNX N=39	33	15% responder	0 (0.0)	19 (57.6)				
	Placebo N=41	37	15% responder	1 (2.4)	11 (29.7)	3.10 (1.17, 8.17)	1.90 (1.08, 3.33)	27.8 (4.0, 49.2)	0.0291

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Males

Physical Health Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	1 (10.0)				
	Placebo N=10	9	15% responder	0 (0.0)	5 (55.6)	0.09 (0.01, 1.03)	0.18 (0.03, 1.26)	-45.6 (-79.3, 2.5)	0.0573
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	5 (50.0)				
	Placebo N=10	9	15% responder	0 (0.0)	2 (22.2)	3.50 (0.47, 25.90)	2.25 (0.57, 8.86)	27.8 (-18.0, 67.3)	0.3498
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	4 (40.0)				
	Placebo N=10	8	15% responder	0 (0.0)	2 (25.0)	2.00 (0.26, 15.38)	1.60 (0.39, 6.62)	15.0 (-31.5, 57.3)	0.6380

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Males

Positive Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	6 (60.0)				
	Placebo N=10	9	15% responder	0 (0.0)	5 (55.6)	1.20 (0.19, 7.44)	1.08 (0.50, 2.34)	4.4 (-41.5, 48.3)	1.0000
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	4 (40.0)				
	Placebo N=10	9	15% responder	0 (0.0)	3 (33.3)	1.33 (0.20, 8.71)	1.20 (0.36, 3.97)	6.7 (-38.0, 50.8)	1.0000
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	5 (50.0)				
	Placebo N=10	8	15% responder	0 (0.0)	3 (37.5)	1.67 (0.25, 11.07)	1.33 (0.45, 3.96)	12.5 (-34.9, 56.0)	0.6641

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Males

Negative Emotions Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	2 (20.0)				
	Placebo N=10	9	15% responder	0 (0.0)	4 (44.4)	0.31 (0.04, 2.38)	0.45 (0.11, 1.90)	-24.4 (-63.5, 23.3)	0.3498
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	3 (30.0)				
	Placebo N=10	9	15% responder	0 (0.0)	3 (33.3)	0.86 (0.12, 5.94)	0.90 (0.24, 3.38)	-3.3 (-46.0, 42.3)	1.0000
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	4 (40.0)				
	Placebo N=10	8	15% responder	0 (0.0)	5 (62.5)	0.40 (0.06, 2.70)	0.64 (0.25, 1.62)	-22.5 (-63.9, 26.0)	0.6372

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Males

Social Interaction Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	4 (40.0)				
	Placebo N=10	9	15% responder	0 (0.0)	5 (55.6)	0.53 (0.09, 3.31)	0.72 (0.28, 1.88)	-15.6 (-57.8, 31.5)	0.6563
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	4 (40.0)				
	Placebo N=10	8	15% responder	0 (0.0)	5 (62.5)	0.40 (0.06, 2.70)	0.64 (0.25, 1.62)	-22.5 (-63.9, 26.0)	0.6372
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	5 (50.0)				
	Placebo N=10	8	15% responder	0 (0.0)	4 (50.0)	1.00 (0.16, 6.42)	1.00 (0.39, 2.53)	0.0 (-45.9, 45.9)	1.0000

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Males

Leisure and the Outdoors Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	4 (40.0)				
	Placebo N=10	9	15% responder	0 (0.0)	6 (66.7)	0.33 (0.05, 2.18)	0.60 (0.25, 1.46)	-26.7 (-66.5, 20.7)	0.3698
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	6 (60.0)				
	Placebo N=10	9	15% responder	0 (0.0)	5 (55.6)	1.20 (0.19, 7.44)	1.08 (0.50, 2.34)	4.4 (-41.5, 48.3)	1.0000
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	4 (40.0)				
	Placebo N=10	8	15% responder	0 (0.0)	2 (25.0)	2.00 (0.26, 15.38)	1.60 (0.39, 6.62)	15.0 (-31.5, 57.3)	0.6380

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.2.6.5 Summary of QI-Disability % responders - Males

Independence Average

Visit	Treatment	n	Category	Missing n (%)	Responders n (%)	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
Visit 3 (End of Week 5)	GNX N=11	10	15% responder	0 (0.0)	5 (50.0)				
	Placebo N=10	9	15% responder	0 (0.0)	6 (66.7)	0.50 (0.08, 3.21)	0.75 (0.35, 1.62)	-16.7 (-58.9, 29.5)	0.6499
Visit 4 (End of Week 9)	GNX N=11	10	15% responder	0 (0.0)	5 (50.0)				
	Placebo N=10	9	15% responder	0 (0.0)	5 (55.6)	0.80 (0.13, 4.87)	0.90 (0.38, 2.11)	-5.6 (-49.9, 40.1)	1.0000
Visit 5 (End of Week 17)	GNX N=11	10	15% responder	0 (0.0)	5 (50.0)				
	Placebo N=10	8	15% responder	0 (0.0)	3 (37.5)	1.67 (0.25, 11.07)	1.33 (0.45, 3.96)	12.5 (-34.9, 56.0)	0.6641

Numbers shown in the table are p-values and are calculated using Cochran's Q for each subroup mentioned in the column headers

Study No. 1042-CDD-3001
Table 14.3.2.2.1 Overall Summary of Adverse Events - Patients who are 2-6 years old

	Placebo N=22		Ganaxolone N=30					
Category	Subjects n (%)	Events n	Subjects n (%)	Events n	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
TEAEs	21 (95.5)	85	28 (93.3)	88	0.67 (0.06, 7.85)	0.98 (0.86, 1.12)	-2.1 (-29.4, 25.3)	1.0000

Study No. 1042-CDD-3001
Table 14.3.2.2.1 Overall Summary of Adverse Events - Patients who are >6 years old

	Placebo N=29		Ganaxolone N=20					
Category	Subjects n (%)	Events n	Subjects n (%)	Events n	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
TEAEs	24 (82.8)	90	15 (75.0)	65	0.63 (0.15, 2.53)	0.91 (0.67, 1.23)	-7.8 (-35.2, 20.8)	0.7199

Study No. 1042-CDD-3001
Table 14.3.2.2.1 Overall Summary of Adverse Events - Patients not in US

	Placebo N=27		Ganaxolone N=32					
Category	Subjects n (%)	Events n	Subjects n (%)	Events n	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
TEAEs	24 (88.9)	77	26 (81.3)	81	0.54 (0.12, 2.41)	0.91 (0.74, 1.13)	-7.6 (-32.5, 17.8)	0.4877

Study No. 1042-CDD-3001
Table 14.3.2.2.1 Overall Summary of Adverse Events - Patients in US

	Placebo N=24		Ganaxolone N=18					
Category	Subjects n (%)	Events n	Subjects n (%)	Events n	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
TEAEs	21 (87.5)	98	17 (94.4)	72	2.43 (0.23, 25.51)	1.08 (0.89, 1.30)	6.9 (-23.9, 36.8)	0.6227

Study No. 1042-CDD-3001
Table 14.3.2.2.1 Overall Summary of Adverse Events - Females

	Placebo N=41		Ganaxolone N=39					
Category	**Subjects n (%)**	**Events n**	**Subjects n (%)**	**Events n**	**OR (95% CI)**	**RR (95% CI)**	**RD (95% Exact CI)**	**Fisher Exact Test p-value**
TEAEs	35 (85.4)	142	34 (87.2)	108	1.17 (0.33, 4.18)	1.02 (0.86, 1.22)	1.8 (-20.4, 22.9)	1.0000

Study No. 1042-CDD-3001
Table 14.3.2.2.1 Overall Summary of Adverse Events - Males

	Placebo N=10		Ganaxolone N=11					
Category	**Subjects n (%)**	**Events n**	**Subjects n (%)**	**Events n**	**OR (95% CI)**	**RR (95% CI)**	**RD (95% Exact CI)**	**Fisher Exact Test p-value**
TEAEs	10 (100)	33	9 (81.8)	45	0.18 (0.01, 4.27)	0.83 (0.60, 1.14)	-18.2 (-56.6, 23.4)	0.4762

Study No. 1042-CDD-3001

Table 14.3.2.2.1 Overall Summary of Adverse Event = Somnolence - Patients who are 2-6 years old

	Placebo N=22		Ganaxolone N=30					
Category	Subjects n (%)	Events n	Subjects n (%)	Events n	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
TEAEs	4 (18.2)	4	10 (33.3)	12	2.25 (0.60, 8.45)	1.83 (0.66, 5.09)	15.2 (-12.3, 41.0)	0.3439

Study No. 1042-CDD-3001

Table 14.3.2.2.1 Overall Summary of Adverse Event = Somnolence - Patients who are >6 years old

	Placebo N=29		Ganaxolone N=20					
Category	Subjects n (%)	Events n	Subjects n (%)	Events n	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
TEAEs	4 (13.8)	4	8 (40.0)	8	4.17 (1.04, 16.62)	2.90 (1.01, 8.34)	26.2 (-2.3, 52.0)	0.0480

Study No. 1042-CDD-3001

Table 14.3.2.2.1 Overall Summary of Adverse Event = Somnolence - Patients not in US

	Placebo N=27		Ganaxolone N=32					
Category	Subjects n (%)	Events n	Subjects n (%)	Events n	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
TEAEs	4 (14.8)	4	8 (25.0)	9	1.92 (0.51, 7.24)	1.69 (0.57, 5.00)	10.2 (-15.3, 34.7)	0.5177

Study No. 1042-CDD-3001

Table 14.3.2.2.1 Overall Summary of Adverse Event = Somnolence - Patients in US

	Placebo N=24		Ganaxolone N=18					
Category	Subjects n (%)	Events n	Subjects n (%)	Events n	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
TEAEs	4 (16.7)	4	10 (55.6)	11	6.25 (1.51, 25.86)	3.33 (1.24, 8.93)	38.9 (8.0, 64.4)	0.0188

Study No. 1042-CDD-3001

Table 14.3.2.2.1 Overall Summary of Adverse Event = Somnolence - Females

	Placebo N=41		Ganaxolone N=39					
Category	Subjects n (%)	Events n	Subjects n (%)	Events n	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
TEAEs	7 (17.1)	7	14 (35.9)	16	2.72 (0.96, 7.73)	2.10 (0.95, 4.65)	18.8 (-3.3, 39.4)	0.0759

Study No. 1042-CDD-3001

Table 14.3.2.2.1 Overall Summary of Adverse Event = Somnolence - Males

	Placebo N=10		Ganaxolone N=11					
Category	Subjects n (%)	Events n	Subjects n (%)	Events n	OR (95% CI)	RR (95% CI)	RD (95% Exact CI)	Fisher Exact Test p-value
TEAEs	1 (10.0)	1	4 (36.4)	4	5.14 (0.46, 56.90)	3.64 (0.48, 27.33)	26.4 (-14.3, 64.0)	0.3108

4.8 Ergebnisse aus der offenen Extensionsphase (OLE) der Marigold-Studie

4.8.1 Charakterisierung der Studienpopulation der OLE-Phase

4.8.1.1 Patientencharakteristika zu Baseline (Patienten in der OLE)

Marinus, Inc.
Study No. 1042-CDD-3001

Table 14.1.2.3
Demographics and Baseline Characteristics
(Open-Label Extension Phase, Safety Population)

Characteristic	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Age(years)			
n	45	43	88
Mean (SD)	7.67 (4.472)	6.84 (4.805)	7.26 (4.630)
Median	7.00	5.00	5.00
Q1, Q3	3.00, 11.00	3.00, 10.00	3.00, 11.00
Min, Max	2.0, 19.0	2.0, 19.0	2.0, 19.0
Sex, n(%)			
Male	8 (17.8)	10 (23.3)	18 (20.5)
Female	37 (82.2)	33 (76.7)	70 (79.5)
Ethnicity, n(%)			
Hispanic or Latino	6 (13.3)	3 (7.0)	9 (10.2)
Not-Hispanic or Latino	37 (82.2)	38 (88.4)	75 (85.2)
Unknown	1 (2.2)	1 (2.3)	2 (2.3)
Not reported	1 (2.2)	1 (2.3)	2 (2.3)
Race, n(%)			
White	41 (91.1)	39 (90.7)	80 (90.9)
Black or African American	0	0	0
Asian	3 (6.7)	2 (4.7)	5 (5.7)
American Indian or Alaska Native	0	0	0
Native Hawaiian or Other Pacific Islander	0	0	0
Unknown	0	0	0
Not reported	0	0	0
Other	1 (2.2)	2 (4.7)	3 (3.4)

OLE = open-label extension, BMI = Body mass index, N = Number of subjects in OLE Phase Safety Population, n = Number of subjects with specified category or non-missing values, % = n/N*100, SD = Standard deviation, Q1 = 25th percentile, Q3 = 75th percentile, min = minimum, max = maximum.
Percentages are based on the number of subjects in OLE Phase Safety Population.
Source Data: Listing 16.2.3.1

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-dm-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-02-03-dm-ole.rtf

Marinus, Inc.
Study No. 1042-CDD-3001

Table 14.1.2.3
Demographics and Baseline Characteristics
(Open-Label Extension Phase, Safety Population)

Characteristic	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Height(cm)			
n	45	43	88
Mean (SD)	118.60 (20.005)	114.02 (18.641)	116.36 (19.376)
Median	117.00	108.90	114.45
Q1, Q3	102.00, 132.00	101.00, 130.00	101.00, 131.20
Min, Max	82.5, 160.0	88.0, 160.0	82.5, 160.0
Weight(kg)			
n	45	43	88
Mean (SD)	21.72 (8.460)	20.25 (9.183)	21.00 (8.801)
Median	19.10	16.30	18.30
Q1, Q3	15.80, 26.10	13.20, 25.10	14.40, 25.80
Min, Max	8.7, 45.2	9.7, 42.0	8.7, 45.2
BMI(kg/m^2)			
n	45	43	88
Mean (SD)	14.96 (2.403)	14.82 (2.629)	14.89 (2.502)
Median	14.48	14.59	14.50
Q1, Q3	13.38, 16.14	12.88, 15.95	12.97, 16.05
Min, Max	11.9, 22.1	11.3, 24.0	11.3, 24.0
Country			
Australia	2 (4.4)	3 (7.0)	5 (5.7)
France	3 (6.7)	3 (7.0)	6 (6.8)
Israel	1 (2.2)	0	1 (1.1)
Italy	6 (13.3)	8 (18.6)	14 (15.9)
Poland	3 (6.7)	4 (9.3)	7 (8.0)
Russian Federation	6 (13.3)	7 (16.3)	13 (14.8)
United Kingdom	3 (6.7)	4 (9.3)	7 (8.0)
United States	21 (46.7)	14 (32.6)	35 (39.8)

OLE = open-label extension, BMI = Body mass index, N = Number of subjects in OLE Phase Safety Population, n = Number of subjects with specified category or non-missing values, % = n/N*100, SD = Standard deviation, Q1 = 25th percentile, Q3 = 75th percentile, min = minimum, max = maximum.
Percentages are based on the number of subjects in OLE Phase Safety Population.
Source Data: Listing 16.2.3.1

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-dm-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-02-03-dm-ole.rtf

Table 14.1.2.3
Demographics and Baseline Characteristics
(Open-Label Extension Phase, Safety Population)

Characteristic	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Tanner Staging, n(%)			
Stage 1	26 (57.8)	25 (58.1)	51 (58.0)
Stage 2	5 (11.1)	5 (11.6)	10 (11.4)
Stage 3	7 (15.6)	1 (2.3)	8 (9.1)
Stage 4	2 (4.4)	1 (2.3)	3 (3.4)
Stage 5	0	3 (7.0)	3 (3.4)
Missing	5 (11.1)	8 (18.6)	13 (14.8)
Number of AED Medications Taken and Stopped Prior to Treatment, n(%)			
n	44	41	85
Mean (SD)	6.75 (3.279)	7.46 (3.317)	7.09 (3.297)
Median	7.00	7.00	7.00
Q1, Q3	4.00, 9.00	5.00, 10.00	5.00, 9.00
Min, Max	1.0, 14.0	2.0, 15.0	1.0, 15.0

OLE = open-label extension, BMI = Body mass index, N = Number of subjects in OLE Phase Safety Population, n = Number of subjects with specified category or non-missing values, % = n/N*100, SD = Standard deviation, Q1 = 25th percentile, Q3 = 75th percentile, min = minimum, max = maximum.
Percentages are based on the number of subjects in OLE Phase Safety Population.
Source Data: Listing 16.2.3.1

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-dm-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-02-03-dm-ole.rtf

4.8.1.2 Medizinische Vorgeschichte (Patienten in der OLE)

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Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
Any Medical History Terms	45 (100)	43 (100)	88 (100)
CONGENITAL, FAMILIAL AND GENETIC DISORDERS	45 (100)	41 (95.3)	86 (97.7)
CDKL5 DEFICIENCY DISORDER	44 (97.8)	41 (95.3)	85 (96.6)
DEVELOPMENTAL HIP DYSPLASIA	3 (6.7)	2 (4.7)	5 (5.7)
RETT SYNDROME	2 (4.4)	1 (2.3)	3 (3.4)
ATRIAL SEPTAL DEFECT	1 (2.2)	1 (2.3)	2 (2.3)
CEREBRAL PALSY	0	2 (4.7)	2 (2.3)
BRAIN MALFORMATION	1 (2.2)	0	1 (1.1)
CLEFT LIP	1 (2.2)	0	1 (1.1)
CONGENITAL CEREBROVASCULAR ANOMALY	1 (2.2)	0	1 (1.1)
CONGENITAL NYSTAGMUS	0	1 (2.3)	1 (1.1)
CRYPTORCHISM	1 (2.2)	0	1 (1.1)
DUCTUS ARTERIOSUS PREMATURE CLOSURE	0	1 (2.3)	1 (1.1)
INTESTINAL MALROTATION	0	1 (2.3)	1 (1.1)
MICROCEPHALY	0	1 (2.3)	1 (1.1)
VESTIBULOCEREBELLAR SYNDROME	1 (2.2)	0	1 (1.1)
NERVOUS SYSTEM DISORDERS	26 (57.8)	27 (62.8)	53 (60.2)
EPILEPSY	16 (35.6)	16 (37.2)	32 (36.4)
HYPOTONIA	8 (17.8)	8 (18.6)	16 (18.2)
LENNOX-GASTAUT SYNDROME	4 (8.9)	2 (4.7)	6 (6.8)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-03-mh.rtf

Marinus, Inc.
Study No. 1042-CDD-3001

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
SEIZURE	2 (4.4)	4 (9.3)	6 (6.8)
INFANTILE SPASMS	3 (6.7)	2 (4.7)	5 (5.7)
INTELLECTUAL DISABILITY	1 (2.2)	3 (7.0)	4 (4.5)
SPEECH DISORDER DEVELOPMENTAL	2 (4.4)	2 (4.7)	4 (4.5)
DYSTONIA	2 (4.4)	1 (2.3)	3 (3.4)
ENCEPHALOPATHY	2 (4.4)	1 (2.3)	3 (3.4)
DYSKINESIA	1 (2.2)	1 (2.3)	2 (2.3)
EPILEPTIC ENCEPHALOPATHY	0	2 (4.7)	2 (2.3)
QUADRIPARESIS	0	2 (4.7)	2 (2.3)
ARACHNOID CYST	0	1 (2.3)	1 (1.1)
CHOREA	0	1 (2.3)	1 (1.1)
COGNITIVE DISORDER	0	1 (2.3)	1 (1.1)
COORDINATION ABNORMAL	0	1 (2.3)	1 (1.1)
DELAYED VISUAL MATURATION	0	1 (2.3)	1 (1.1)
DROOLING	1 (2.2)	0	1 (1.1)
EXAGGERATED STARTLE RESPONSE	1 (2.2)	0	1 (1.1)
GENERALISED TONIC-CLONIC SEIZURE	1 (2.2)	0	1 (1.1)
HYPERKINESIA	0	1 (2.3)	1 (1.1)
LANGUAGE DISORDER	0	1 (2.3)	1 (1.1)
MOTOR DEVELOPMENTAL DELAY	1 (2.2)	0	1 (1.1)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
MUSCLE SPASTICITY	1 (2.2)	0	1 (1.1)
NON-24-HOUR SLEEP-WAKE DISORDER	0	1 (2.3)	1 (1.1)
NYSTAGMUS	0	1 (2.3)	1 (1.1)
SEIZURE CLUSTER	1 (2.2)	0	1 (1.1)
SENSORY PROCESSING DISORDER	0	1 (2.3)	1 (1.1)
TONIC CONVULSION	1 (2.2)	0	1 (1.1)
UNRESPONSIVE TO STIMULI	0	1 (2.3)	1 (1.1)
GASTROINTESTINAL DISORDERS	23 (51.1)	24 (55.8)	47 (53.4)
CONSTIPATION	14 (31.1)	15 (34.9)	29 (33.0)
GASTROOESOPHAGEAL REFLUX DISEASE	12 (26.7)	10 (23.3)	22 (25.0)
DYSPHAGIA	2 (4.4)	1 (2.3)	3 (3.4)
PANCREATITIS	0	3 (7.0)	3 (3.4)
SALIVARY HYPERSECRETION	0	3 (7.0)	3 (3.4)
VOMITING	1 (2.2)	2 (4.7)	3 (3.4)
DIARRHOEA	0	2 (4.7)	2 (2.3)
FUNCTIONAL GASTROINTESTINAL DISORDER	0	1 (2.3)	1 (1.1)
GASTRIC ULCER	0	1 (2.3)	1 (1.1)
IMPAIRED GASTRIC EMPTYING	0	1 (2.3)	1 (1.1)
INTUSSUSCEPTION	0	1 (2.3)	1 (1.1)
IRRITABLE BOWEL SYNDROME	0	1 (2.3)	1 (1.1)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
MALOCCLUSION	1 (2.2)	0	1 (1.1)
PANCREATIC STEATOSIS	1 (2.2)	0	1 (1.1)
REGURGITATION	1 (2.2)	0	1 (1.1)
TOOTHACHE	1 (2.2)	0	1 (1.1)
VOLVULUS	0	1 (2.3)	1 (1.1)
EYE DISORDERS	16 (35.6)	17 (39.5)	33 (37.5)
CORTICAL VISUAL IMPAIRMENT	8 (17.8)	9 (20.9)	17 (19.3)
VISUAL IMPAIRMENT	3 (6.7)	3 (7.0)	6 (6.8)
STRABISMUS	3 (6.7)	2 (4.7)	5 (5.7)
BLINDNESS CORTICAL	2 (4.4)	1 (2.3)	3 (3.4)
ASTIGMATISM	1 (2.2)	1 (2.3)	2 (2.3)
HYPERMETROPIA	1 (2.2)	1 (2.3)	2 (2.3)
BLEPHARITIS	0	1 (2.3)	1 (1.1)
GAZE PALSY	0	1 (2.3)	1 (1.1)
GENERAL DISORDERS AND ADMINISTRATION SITE CONDITIONS	12 (26.7)	11 (25.6)	23 (26.1)
DEVELOPMENTAL DELAY	12 (26.7)	11 (25.6)	23 (26.1)
ADVERSE DRUG REACTION	0	1 (2.3)	1 (1.1)
GAIT INABILITY	1 (2.2)	0	1 (1.1)
PAIN	0	1 (2.3)	1 (1.1)
PYREXIA	1 (2.2)	0	1 (1.1)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
INFECTIONS AND INFESTATIONS	11 (24.4)	10 (23.3)	21 (23.9)
PNEUMONIA	3 (6.7)	2 (4.7)	5 (5.7)
URINARY TRACT INFECTION	1 (2.2)	2 (4.7)	3 (3.4)
BRONCHITIS	1 (2.2)	1 (2.3)	2 (2.3)
BODY TINEA	1 (2.2)	0	1 (1.1)
CHRONIC TONSILLITIS	1 (2.2)	0	1 (1.1)
LARYNGITIS	0	1 (2.3)	1 (1.1)
LOWER RESPIRATORY TRACT INFECTION	0	1 (2.3)	1 (1.1)
MEASLES	1 (2.2)	0	1 (1.1)
NASOPHARYNGITIS	1 (2.2)	0	1 (1.1)
ORAL CANDIDIASIS	0	1 (2.3)	1 (1.1)
OTITIS MEDIA	0	1 (2.3)	1 (1.1)
OTITIS MEDIA CHRONIC	1 (2.2)	0	1 (1.1)
OTOSALPINGITIS	0	1 (2.3)	1 (1.1)
PHARYNGITIS	0	1 (2.3)	1 (1.1)
RESPIRATORY TRACT INFECTION VIRAL	1 (2.2)	0	1 (1.1)
ROSEOLA	1 (2.2)	0	1 (1.1)
ROTAVIRUS INFECTION	0	1 (2.3)	1 (1.1)
TINEA CAPITIS	1 (2.2)	0	1 (1.1)
UPPER RESPIRATORY TRACT INFECTION	1 (2.2)	0	1 (1.1)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
VARICELLA	0	1 (2.3)	1 (1.1)
VIRAL INFECTION	0	1 (2.3)	1 (1.1)
RESPIRATORY, THORACIC AND MEDIASTINAL DISORDERS	7 (15.6)	12 (27.9)	19 (21.6)
CHRONIC RESPIRATORY DISEASE	3 (6.7)	2 (4.7)	5 (5.7)
SLEEP APNOEA SYNDROME	0	5 (11.6)	5 (5.7)
ASTHMA	3 (6.7)	1 (2.3)	4 (4.5)
PNEUMONIA ASPIRATION	0	2 (4.7)	2 (2.3)
RHINITIS ALLERGIC	2 (4.4)	0	2 (2.3)
CHILDHOOD ASTHMA	0	1 (2.3)	1 (1.1)
CHRONIC OBSTRUCTIVE PULMONARY DISEASE	0	1 (2.3)	1 (1.1)
DYSPNOEA	0	1 (2.3)	1 (1.1)
EPISTAXIS	0	1 (2.3)	1 (1.1)
HYPOXIA	0	1 (2.3)	1 (1.1)
LOWER RESPIRATORY TRACT CONGESTION	0	1 (2.3)	1 (1.1)
PULMONARY HYPERTENSION	0	1 (2.3)	1 (1.1)
RESPIRATORY DISORDER	1 (2.2)	0	1 (1.1)
RESPIRATORY FAILURE	0	1 (2.3)	1 (1.1)
RESTRICTIVE PULMONARY DISEASE	1 (2.2)	0	1 (1.1)
TONSILLAR HYPERTROPHY	0	1 (2.3)	1 (1.1)
SURGICAL AND MEDICAL PROCEDURES	7 (15.6)	10 (23.3)	17 (19.3)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
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Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
GASTROSTOMY	6 (13.3)	5 (11.6)	11 (12.5)
VAGAL NERVE STIMULATOR IMPLANTATION	2 (4.4)	2 (4.7)	4 (4.5)
GASTROINTESTINAL TUBE INSERTION	0	2 (4.7)	2 (2.3)
KETOGENIC DIET	1 (2.2)	1 (2.3)	2 (2.3)
TONSILLECTOMY	0	2 (4.7)	2 (2.3)
ADENOIDECTOMY	0	1 (2.3)	1 (1.1)
EAR TUBE INSERTION	0	1 (2.3)	1 (1.1)
ENTERAL NUTRITION	0	1 (2.3)	1 (1.1)
EXTERNAL VAGAL NERVE STIMULATION	0	1 (2.3)	1 (1.1)
OXYGEN THERAPY	1 (2.2)	0	1 (1.1)
SPINAL FUSION SURGERY	0	1 (2.3)	1 (1.1)
PSYCHIATRIC DISORDERS	8 (17.8)	8 (18.6)	16 (18.2)
INSOMNIA	5 (11.1)	2 (4.7)	7 (8.0)
AUTISM SPECTRUM DISORDER	1 (2.2)	2 (4.7)	3 (3.4)
AGGRESSION	0	1 (2.3)	1 (1.1)
ANXIETY	0	1 (2.3)	1 (1.1)
BRUXISM	0	1 (2.3)	1 (1.1)
EATING DISORDER	0	1 (2.3)	1 (1.1)
INTENTIONAL SELF-INJURY	0	1 (2.3)	1 (1.1)
IRRITABILITY	1 (2.2)	0	1 (1.1)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-03-mh.rtf

Marinus, Inc.
Study No. 1042-CDD-3001

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
LEARNING DISABILITY	0	1 (2.3)	1 (1.1)
ORGANIC BRAIN SYNDROME	1 (2.2)	0	1 (1.1)
SLEEP DISORDER	0	1 (2.3)	1 (1.1)
MUSCULOSKELETAL AND CONNECTIVE TISSUE DISORDERS	10 (22.2)	5 (11.6)	15 (17.0)
SCOLIOSIS	8 (17.8)	3 (7.0)	11 (12.5)
ARTHROPATHY	0	2 (4.7)	2 (2.3)
OSTEOPENIA	2 (4.4)	0	2 (2.3)
OSTEOPOROSIS	1 (2.2)	0	1 (1.1)
PATHOLOGICAL FRACTURE	1 (2.2)	0	1 (1.1)
METABOLISM AND NUTRITION DISORDERS	8 (17.8)	6 (14.0)	14 (15.9)
POOR FEEDING INFANT	1 (2.2)	2 (4.7)	3 (3.4)
FEEDING INTOLERANCE	2 (4.4)	0	2 (2.3)
VITAMIN D DEFICIENCY	1 (2.2)	1 (2.3)	2 (2.3)
WEIGHT GAIN POOR	0	2 (4.7)	2 (2.3)
CARNITINE DEFICIENCY	1 (2.2)	0	1 (1.1)
DECREASED APPETITE	1 (2.2)	0	1 (1.1)
DEHYDRATION	1 (2.2)	0	1 (1.1)
FAILURE TO THRIVE	0	1 (2.3)	1 (1.1)
HYPOVITAMINOSIS	0	1 (2.3)	1 (1.1)
MALNUTRITION	1 (2.2)	0	1 (1.1)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
MITOCHONDRIAL CYTOPATHY	0	1 (2.3)	1 (1.1)
INJURY, POISONING AND PROCEDURAL COMPLICATIONS	5 (11.1)	3 (7.0)	8 (9.1)
FEMUR FRACTURE	2 (4.4)	1 (2.3)	3 (3.4)
JOINT DISLOCATION	3 (6.7)	0	3 (3.4)
FACIAL BONES FRACTURE	0	1 (2.3)	1 (1.1)
SCAR	0	1 (2.3)	1 (1.1)
STOMA SITE HYPERGRANULATION	0	1 (2.3)	1 (1.1)
SKIN AND SUBCUTANEOUS TISSUE DISORDERS	4 (8.9)	3 (7.0)	7 (8.0)
ECZEMA	3 (6.7)	1 (2.3)	4 (4.5)
ACNE	1 (2.2)	1 (2.3)	2 (2.3)
DERMATITIS ACNEIFORM	1 (2.2)	0	1 (1.1)
DERMATITIS CONTACT	0	1 (2.3)	1 (1.1)
CARDIAC DISORDERS	3 (6.7)	3 (7.0)	6 (6.8)
CARDIAC VENTRICULAR DISORDER	1 (2.2)	2 (4.7)	3 (3.4)
BUNDLE BRANCH BLOCK RIGHT	0	1 (2.3)	1 (1.1)
MITRAL VALVE INCOMPETENCE	1 (2.2)	0	1 (1.1)
MITRAL VALVE PROLAPSE	1 (2.2)	0	1 (1.1)
RIGHT VENTRICULAR DILATATION	1 (2.2)	0	1 (1.1)
SINUS ARRHYTHMIA	1 (2.2)	0	1 (1.1)
TACHYARRHYTHMIA	0	1 (2.3)	1 (1.1)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-03-mh.rtf

Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
IMMUNE SYSTEM DISORDERS	1 (2.2)	4 (9.3)	5 (5.7)
SEASONAL ALLERGY	1 (2.2)	2 (4.7)	3 (3.4)
DRUG HYPERSENSITIVITY	0	2 (4.7)	2 (2.3)
INVESTIGATIONS	2 (4.4)	3 (7.0)	5 (5.7)
BODY MASS INDEX DECREASED	1 (2.2)	0	1 (1.1)
GAMMA-GLUTAMYLTRANSFERASE INCREASED	1 (2.2)	0	1 (1.1)
LIPASE INCREASED	1 (2.2)	0	1 (1.1)
ULTRASOUND KIDNEY ABNORMAL	0	1 (2.3)	1 (1.1)
VITAMIN D DECREASED	0	1 (2.3)	1 (1.1)
WEIGHT DECREASED	0	1 (2.3)	1 (1.1)
RENAL AND URINARY DISORDERS	2 (4.4)	3 (7.0)	5 (5.7)
METABOLIC NEPHROPATHY	0	1 (2.3)	1 (1.1)
MIXED INCONTINENCE	0	1 (2.3)	1 (1.1)
NEPHROLITHIASIS	1 (2.2)	0	1 (1.1)
RENAL CYST	1 (2.2)	0	1 (1.1)
URINARY RETENTION	0	1 (2.3)	1 (1.1)
PREGNANCY, PUERPERIUM AND PERINATAL CONDITIONS	1 (2.2)	2 (4.7)	3 (3.4)
PERINATAL BRAIN DAMAGE	1 (2.2)	2 (4.7)	3 (3.4)
PREMATURE BABY	0	1 (2.3)	1 (1.1)
VASCULAR DISORDERS	2 (4.4)	1 (2.3)	3 (3.4)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
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Table 14.1.3
Summary of Medical History
(Safety Population)

System Organ Class Preferred Term	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
CIRCULATORY COLLAPSE	0	1 (2.3)	1 (1.1)
HYPERTENSION	1 (2.2)	0	1 (1.1)
VASOCONSTRICTION	1 (2.2)	0	1 (1.1)
ENDOCRINE DISORDERS	1 (2.2)	1 (2.3)	2 (2.3)
HYPOTHYROIDISM	1 (2.2)	0	1 (1.1)
PRECOCIOUS PUBERTY	0	1 (2.3)	1 (1.1)
HEPATOBILIARY DISORDERS	0	2 (4.7)	2 (2.3)
BILIARY TRACT DISORDER	0	1 (2.3)	1 (1.1)
GALLBLADDER DISORDER	0	1 (2.3)	1 (1.1)
BLOOD AND LYMPHATIC SYSTEM DISORDERS	0	1 (2.3)	1 (1.1)
HYPOCHROMIC ANAEMIA	0	1 (2.3)	1 (1.1)
EAR AND LABYRINTH DISORDERS	0	1 (2.3)	1 (1.1)
LABYRINTHINE FISTULA	0	1 (2.3)	1 (1.1)
SOCIAL CIRCUMSTANCES	1 (2.2)	0	1 (1.1)
WALKING DISABILITY	1 (2.2)	0	1 (1.1)

Medical history terms were coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.2

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-mh.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-03-mh.rtf

4.8.1.3 Begleittherapien während der OLE-Phase

4.8.1.3.1 Nicht antikonvulsive Begleittherapien

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
Any Concomitant Non-AED Medications	39 (86.7)	34 (79.1)	73 (83.0)
ANILIDES	19 (42.2)	18 (41.9)	37 (42.0)
PARACETAMOL	18 (40.0)	18 (41.9)	36 (40.9)
IBUPROFEN;PARACETAMOL	1 (2.2)	0	1 (1.1)
OSMOTICALLY ACTING LAXATIVES	16 (35.6)	12 (27.9)	28 (31.8)
MACROGOL 3350	10 (22.2)	5 (11.6)	15 (17.0)
MACROGOL	4 (8.9)	4 (9.3)	8 (9.1)
MACROGOL 3350;POTASSIUM CHLORIDE;SODIUM BICARBONATE;SODIUM CHLORIDE	1 (2.2)	2 (4.7)	3 (3.4)
MACROGOL 4000	1 (2.2)	2 (4.7)	3 (3.4)
LACTULOSE	1 (2.2)	1 (2.3)	2 (2.3)
MACROGOL;POTASSIUM CHLORIDE;SODIUM BICARBONATE;SODIUM CHLORIDE;SODIUM SULFATE	1 (2.2)	0	1 (1.1)
PROPIONIC ACID DERIVATIVES	13 (28.9)	12 (27.9)	25 (28.4)
IBUPROFEN	11 (24.4)	12 (27.9)	23 (26.1)
KETOPROFEN	1 (2.2)	0	1 (1.1)
PROPIONIC ACID DERIVATIVES	1 (2.2)	0	1 (1.1)
PROTON PUMP INHIBITORS	14 (31.1)	11 (25.6)	25 (28.4)
LANSOPRAZOLE	9 (20.0)	5 (11.6)	14 (15.9)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cm-nonaed-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-07-02a-cm-nonaed-ole.rtf

Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
OMEPRAZOLE	5 (11.1)	4 (9.3)	9 (10.2)
ESOMEPRAZOLE	2 (4.4)	2 (4.7)	4 (4.5)
ESOMEPRAZOLE MAGNESIUM	2 (4.4)	2 (4.7)	4 (4.5)
ESOMEPRAZOLE SODIUM	2 (4.4)	1 (2.3)	3 (3.4)
VITAMIN D AND ANALOGUES	12 (26.7)	12 (27.9)	24 (27.3)
COLECALCIFEROL	8 (17.8)	6 (14.0)	14 (15.9)
VITAMIN D NOS	4 (8.9)	6 (14.0)	10 (11.4)
COMBINATIONS OF PENICILLINS, INCL. BETA-LACTAMASE INHIBITORS	7 (15.6)	10 (23.3)	17 (19.3)
AMOXICILLIN;CLAVULANIC ACID	4 (8.9)	10 (23.3)	14 (15.9)
AMOXICILLIN;CLAVULANATE POTASSIUM	1 (2.2)	1 (2.3)	2 (2.3)
AMOXICILLIN TRIHYDRATE;CLAVULANATE POTASSIUM	1 (2.2)	0	1 (1.1)
AMPICILLIN SODIUM;SULBACTAM SODIUM	1 (2.2)	0	1 (1.1)
PENICILLINS WITH EXTENDED SPECTRUM	9 (20.0)	8 (18.6)	17 (19.3)
AMOXICILLIN	9 (20.0)	4 (9.3)	13 (14.8)
AMOXICILLIN TRIHYDRATE	0	4 (9.3)	4 (4.5)
GLUCOCORTICOIDS	11 (24.4)	4 (9.3)	15 (17.0)
BUDESONIDE	4 (8.9)	1 (2.3)	5 (5.7)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
DEXAMETHASONE	3 (6.7)	1 (2.3)	4 (4.5)
BETAMETHASONE	2 (4.4)	0	2 (2.3)
FLUTICASONE PROPIONATE	1 (2.2)	1 (2.3)	2 (2.3)
BECLOMETASONE	0	1 (2.3)	1 (1.1)
FLUTICASONE	1 (2.2)	0	1 (1.1)
MOMETASONE FUROATE	1 (2.2)	0	1 (1.1)
PREDNISONE	0	1 (2.3)	1 (1.1)
TRIAMCINOLONE ACETONIDE	1 (2.2)	0	1 (1.1)
THIRD-GENERATION CEPHALOSPORINS	11 (24.4)	4 (9.3)	15 (17.0)
CEFTRIAXONE	8 (17.8)	4 (9.3)	12 (13.6)
CEFDINIR	1 (2.2)	2 (4.7)	3 (3.4)
CEFIXIME	1 (2.2)	0	1 (1.1)
CEFOTAXIME SODIUM	1 (2.2)	0	1 (1.1)
CEFPODOXIME PROXETIL	1 (2.2)	0	1 (1.1)
CEFTAZIDIME	1 (2.2)	0	1 (1.1)
MELATONIN RECEPTOR AGONISTS	5 (11.1)	9 (20.9)	14 (15.9)
MELATONIN	5 (11.1)	9 (20.9)	14 (15.9)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
SELECTIVE BETA-2-ADRENORECEPTOR AGONISTS	7 (15.6)	7 (16.3)	14 (15.9)
SALBUTAMOL	6 (13.3)	6 (14.0)	12 (13.6)
SALBUTAMOL SULFATE	1 (2.2)	1 (2.3)	2 (2.3)
TERBUTALINE SULFATE	0	1 (2.3)	1 (1.1)
MULTIVITAMINS, PLAIN	9 (20.0)	4 (9.3)	13 (14.8)
MULTIVITAMINS, PLAIN	6 (13.3)	3 (7.0)	9 (10.2)
VITAMINS NOS	3 (6.7)	1 (2.3)	4 (4.5)
SOLUTIONS AFFECTING THE ELECTROLYTE BALANCE	10 (22.2)	2 (4.7)	12 (13.6)
SODIUM CHLORIDE	7 (15.6)	2 (4.7)	9 (10.2)
GLUCOSE;SODIUM CHLORIDE	2 (4.4)	0	2 (2.3)
CALCIUM CHLORIDE;GLUCOSE;POTASSIUM CHLORIDE;SODIUM LACTATE	1 (2.2)	0	1 (1.1)
CALCIUM CHLORIDE;POTASSIUM CHLORIDE;SODIUM LACTATE	1 (2.2)	0	1 (1.1)
GLUCOSE;POTASSIUM CHLORIDE;SODIUM CHLORIDE	1 (2.2)	0	1 (1.1)
SOLUTIONS AFFECTING THE ELECTROLYTE BALANCE	1 (2.2)	0	1 (1.1)
AMINO ACIDS AND DERIVATIVES	7 (15.6)	4 (9.3)	11 (12.5)
LEVOCARNITINE	5 (11.1)	4 (9.3)	9 (10.2)
CARNITINE	1 (2.2)	0	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
LEVOGLUTAMIDE	1 (2.2)	0	1 (1.1)
ANTIDIARRHEAL MICROORGANISMS	8 (17.8)	3 (7.0)	11 (12.5)
LACTOBACILLUS ACIDOPHILUS	3 (6.7)	1 (2.3)	4 (4.5)
LACTOBACILLUS NOS	1 (2.2)	2 (4.7)	3 (3.4)
ANTIDIARRHEAL MICROORGANISMS	2 (4.4)	0	2 (2.3)
PROBIOTICS NOS	2 (4.4)	0	2 (2.3)
BIFIDOBACTERIUM BIFIDUM;BIFIDOBACTERIUM BREVE;LACTOBACILLUS ACIDOPHILUS;LACTOBACILLUS CASEI;LACTOBACILLUS PLANTARUM;LACTOBACILLUS RHAMNOSUS;PEDIOCOCCUS ACIDILACTICI;STREPTOCOCCUS LACTIS	1 (2.2)	0	1 (1.1)
MACROLIDES	7 (15.6)	3 (7.0)	10 (11.4)
AZITHROMYCIN	5 (11.1)	3 (7.0)	8 (9.1)
ERYTHROMYCIN	2 (4.4)	0	2 (2.3)
ERYTHROMYCIN ETHYLSUCCINATE	1 (2.2)	0	1 (1.1)
OTHER VIRAL VACCINES	3 (6.7)	7 (16.3)	10 (11.4)
OTHER VIRAL VACCINES	3 (6.7)	7 (16.3)	10 (11.4)
PIPERAZINE DERIVATIVES	7 (15.6)	3 (7.0)	10 (11.4)
CETIRIZINE HYDROCHLORIDE	4 (8.9)	2 (4.7)	6 (6.8)
CETIRIZINE	2 (4.4)	0	2 (2.3)
HYDROXYZINE	0	1 (2.3)	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
HYDROXYZINE HYDROCHLORIDE	1 (2.2)	0	1 (1.1)
H2-RECEPTOR ANTAGONISTS	6 (13.3)	3 (7.0)	9 (10.2)
FAMOTIDINE	2 (4.4)	3 (7.0)	5 (5.7)
RANITIDINE HYDROCHLORIDE	2 (4.4)	2 (4.7)	4 (4.5)
RANITIDINE	3 (6.7)	0	3 (3.4)
CONTACT LAXATIVES	5 (11.1)	2 (4.7)	7 (8.0)
SENNA ALEXANDRINA	3 (6.7)	2 (4.7)	5 (5.7)
SENNOSIDE A+B	2 (4.4)	0	2 (2.3)
CORTICOSTEROIDS	3 (6.7)	4 (9.3)	7 (8.0)
FLUTICASONE	2 (4.4)	2 (4.7)	4 (4.5)
BUDESONIDE	1 (2.2)	0	1 (1.1)
DEXAMETHASONE SODIUM METASULFOBENZOATE;NEOMYCIN SULFATE;PHENYLEPHRINE HYDROCHLORIDE;POLYMYXIN B SULFATE	0	1 (2.3)	1 (1.1)
FLUTICASONE PROPIONATE	0	1 (2.3)	1 (1.1)
TIXOCORTOL PIVALATE	1 (2.2)	0	1 (1.1)
OTHER ANTIHISTAMINES FOR SYSTEMIC USE	3 (6.7)	4 (9.3)	7 (8.0)
LORATADINE	1 (2.2)	3 (7.0)	4 (4.5)
CYPROHEPTADINE HYDROCHLORIDE	2 (4.4)	0	2 (2.3)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
FEXOFENADINE HYDROCHLORIDE	0	1 (2.3)	1 (1.1)
SYNTHETIC ANTICHOLINERGICS, QUATERNARY AMMONIUM COMPOUNDS	4 (8.9)	3 (7.0)	7 (8.0)
GLYCOPYRRONIUM	3 (6.7)	3 (7.0)	6 (6.8)
GLYCOPYRRONIUM BROMIDE	1 (2.2)	0	1 (1.1)
UNSPECIFIED HERBAL AND TRADITIONAL MEDICINE	5 (11.1)	2 (4.7)	7 (8.0)
SENNA ALEXANDRINA	2 (4.4)	1 (2.3)	3 (3.4)
ANGELICA ARCHANGELICA ROOT;CARUM CARVI FRUIT;CHELIDONIUM MAJUS HERB;GLYCYRRHIZA GLABRA ROOT;IBERIS AMARA;MATRICARIA RECUTITA FLOWER;MELISSA OFFICINALIS LEAF;MENTHA X PIPERITA LEAF;SILYBUM MARIANUM F	0	1 (2.3)	1 (1.1)
ENZYMES NOS;HONEY;PRUNUS DOMESTICA	1 (2.2)	0	1 (1.1)
UNSPECIFIED HERBAL	1 (2.2)	0	1 (1.1)
UNSPECIFIED HERBAL AND TRADITIONAL MEDICINE	1 (2.2)	0	1 (1.1)
ENEMAS	5 (11.1)	1 (2.3)	6 (6.8)
GLYCEROL	5 (11.1)	0	5 (5.7)
SODIUM PHOSPHATE	0	1 (2.3)	1 (1.1)
GENERAL NUTRIENTS	3 (6.7)	3 (7.0)	6 (6.8)
NUTRIENTS NOS	3 (6.7)	2 (4.7)	5 (5.7)
GENERAL NUTRIENTS	0	1 (2.3)	1 (1.1)
INFLUENZA VACCINES	1 (2.2)	5 (11.6)	6 (6.8)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
INFLUENZA VACCINE	1 (2.2)	5 (11.6)	6 (6.8)
OTHER COMBINATIONS OF NUTRIENTS	3 (6.7)	3 (7.0)	6 (6.8)
FISH OIL	2 (4.4)	2 (4.7)	4 (4.5)
CALCIUM PHOSPHATE;FUMARIC ACID;POTASSIUM CHLORIDE	1 (2.2)	0	1 (1.1)
CREATINE	0	1 (2.3)	1 (1.1)
OTHER COMBINATIONS OF NUTRIENTS	0	1 (2.3)	1 (1.1)
SEROTONIN (5HT3) ANTAGONISTS	3 (6.7)	3 (7.0)	6 (6.8)
ONDANSETRON	3 (6.7)	3 (7.0)	6 (6.8)
BENZODIAZEPINE DERIVATIVES	3 (6.7)	2 (4.7)	5 (5.7)
CLONAZEPAM	2 (4.4)	1 (2.3)	3 (3.4)
CLOBAZAM	1 (2.2)	0	1 (1.1)
LORAZEPAM	1 (2.2)	0	1 (1.1)
MIDAZOLAM	0	1 (2.3)	1 (1.1)
CENTRALLY ACTING SYMPATHOMIMETICS	4 (8.9)	1 (2.3)	5 (5.7)
CLONIDINE	4 (8.9)	1 (2.3)	5 (5.7)
OTHER NERVOUS SYSTEM DRUGS	3 (6.7)	2 (4.7)	5 (5.7)
GABAPENTIN	1 (2.2)	2 (4.7)	3 (3.4)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
SILVER NITRATE	1 (2.2)	0	1 (1.1)
TETRABENAZINE	1 (2.2)	0	1 (1.1)
THIOCTIC ACID	0	1 (2.3)	1 (1.1)
OTHER PLAIN VITAMIN PREPARATIONS	3 (6.7)	2 (4.7)	5 (5.7)
PYRIDOXINE	1 (2.2)	1 (2.3)	2 (2.3)
BIOTIN	1 (2.2)	0	1 (1.1)
INOSITOL	0	1 (2.3)	1 (1.1)
PYRIDOXINE HYDROCHLORIDE	1 (2.2)	0	1 (1.1)
PROGESTOGENS	1 (2.2)	4 (9.3)	5 (5.7)
MEDROXYPROGESTERONE ACETATE	0	2 (4.7)	2 (2.3)
NORETHISTERONE	1 (2.2)	1 (2.3)	2 (2.3)
DESOGESTREL	0	1 (2.3)	1 (1.1)
AMINOALKYL ETHERS	1 (2.2)	3 (7.0)	4 (4.5)
DIPHENHYDRAMINE	0	2 (4.7)	2 (2.3)
DIPHENHYDRAMINE HYDROCHLORIDE	1 (2.2)	1 (2.3)	2 (2.3)
CALCIUM	3 (6.7)	1 (2.3)	4 (4.5)
CALCIUM	3 (6.7)	1 (2.3)	4 (4.5)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
CALCIUM, COMBINATIONS WITH VITAMIN D AND/OR OTHER DRUGS	3 (6.7)	1 (2.3)	4 (4.5)
CALCIUM;COLECALCIFEROL	1 (2.2)	0	1 (1.1)
CALCIUM;COLECALCIFEROL;MAGNESIUM	0	1 (2.3)	1 (1.1)
CALCIUM;MAGNESIUM	1 (2.2)	0	1 (1.1)
CALCIUM;VITAMIN D NOS	1 (2.2)	0	1 (1.1)
ELECTROLYTE SOLUTIONS	2 (4.4)	2 (4.7)	4 (4.5)
POTASSIUM CHLORIDE	2 (4.4)	2 (4.7)	4 (4.5)
CALCIUM GLUCONATE	1 (2.2)	0	1 (1.1)
FIRST-GENERATION CEPHALOSPORINS	4 (8.9)	0	4 (4.5)
CEFALEXIN	3 (6.7)	0	3 (3.4)
CEFAZOLIN	1 (2.2)	0	1 (1.1)
CEFAZOLIN SODIUM	1 (2.2)	0	1 (1.1)
MULTIVITAMINS WITH MINERALS	4 (8.9)	0	4 (4.5)
ASCORBIC ACID;BIOTIN;CALCIUM;CHROMIUM;COLECALCIFEROL;COPPER;CYANOCOBALAMIN;FOLATE SODIUM;IODINE;IRON;MAGNESIUM;MANGANESE;MENADIONE;MOLYBDENUM;NICOTINIC ACID;PANTOTHENIC ACID;PHOSPHORUS;POTASSIUM;PYR	1 (2.2)	0	1 (1.1)
ASCORBIC ACID;BIOTIN;CALCIUM;COLECALCIFEROL;COPPER;CYANOCOBALAMIN;FOLIC ACID;IODINE;IRON;NICOTINIC ACID;PANTOTHENIC ACID;PHYTOMENADIONE;PYRIDOXINE HYDROCHLORIDE;RETINOL;RIBOFLAVIN;SODIUM;THIAMINE;TO	1 (2.2)	0	1 (1.1)
MINERALS NOS;VITAMINS NOS	1 (2.2)	0	1 (1.1)
MULTIVITAMINS WITH MINERALS [UMBRELLA TERM]	1 (2.2)	0	1 (1.1)

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Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
NEURAMINIDASE INHIBITORS	3 (6.7)	1 (2.3)	4 (4.5)
OSELTAMIVIR PHOSPHATE	3 (6.7)	1 (2.3)	4 (4.5)
OTHER ANTIEPILEPTICS	3 (6.7)	1 (2.3)	4 (4.5)
ACETAZOLAMIDE	0	1 (2.3)	1 (1.1)
CANNABIDIOL	1 (2.2)	0	1 (1.1)
LAMOTRIGINE	1 (2.2)	0	1 (1.1)
LEVETIRACETAM	1 (2.2)	0	1 (1.1)
OTHER MUSCLE RELAXANTS, PERIPHERALLY ACTING AGENTS	2 (4.4)	2 (4.7)	4 (4.5)
BOTULINUM TOXIN TYPE A	2 (4.4)	2 (4.7)	4 (4.5)
POTASSIUM	1 (2.2)	3 (7.0)	4 (4.5)
POTASSIUM CITRATE	1 (2.2)	2 (4.7)	3 (3.4)
CITRIC ACID;POTASSIUM BICARBONATE	0	1 (2.3)	1 (1.1)
SALT SOLUTIONS	2 (4.4)	2 (4.7)	4 (4.5)
SODIUM CHLORIDE	1 (2.2)	1 (2.3)	2 (2.3)
SODIUM BICARBONATE	0	1 (2.3)	1 (1.1)
SODIUM CITRATE	1 (2.2)	0	1 (1.1)
ANTACIDS WITH SODIUM BICARBONATE	1 (2.2)	2 (4.7)	3 (3.4)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
SODIUM ALGINATE;SODIUM BICARBONATE	0	2 (4.7)	2 (2.3)
SODIUM BICARBONATE	1 (2.2)	0	1 (1.1)
BETA-LACTAMASE RESISTANT PENICILLINS	2 (4.4)	1 (2.3)	3 (3.4)
FLUCLOXACILLIN	2 (4.4)	1 (2.3)	3 (3.4)
BETA-LACTAMASE SENSITIVE PENICILLINS	2 (4.4)	1 (2.3)	3 (3.4)
BENZYLPENICILLIN	1 (2.2)	1 (2.3)	2 (2.3)
PHENOXYMETHYLPENICILLIN	1 (2.2)	0	1 (1.1)
CORTICOSTEROIDS, WEAK (GROUP I)	2 (4.4)	1 (2.3)	3 (3.4)
HYDROCORTISONE	2 (4.4)	1 (2.3)	3 (3.4)
GLYCOPEPTIDE ANTIBACTERIALS	1 (2.2)	2 (4.7)	3 (3.4)
VANCOMYCIN HYDROCHLORIDE	1 (2.2)	1 (2.3)	2 (2.3)
VANCOMYCIN	0	1 (2.3)	1 (1.1)
IMIDAZOLE DERIVATIVES	2 (4.4)	1 (2.3)	3 (3.4)
METRONIDAZOLE	2 (4.4)	1 (2.3)	3 (3.4)
IRON BIVALENT, ORAL PREPARATIONS	2 (4.4)	1 (2.3)	3 (3.4)
FERROUS SULFATE	2 (4.4)	1 (2.3)	3 (3.4)
LEUKOTRIENE RECEPTOR ANTAGONISTS	2 (4.4)	1 (2.3)	3 (3.4)

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
MONTELUKAST SODIUM	2 (4.4)	0	2 (2.3)
MONTELUKAST	0	1 (2.3)	1 (1.1)
LINCOSAMIDES	2 (4.4)	1 (2.3)	3 (3.4)
CLINDAMYCIN	2 (4.4)	1 (2.3)	3 (3.4)
MAGNESIUM	2 (4.4)	1 (2.3)	3 (3.4)
MAGNESIUM CARBONATE	1 (2.2)	0	1 (1.1)
MAGNESIUM HYDROXIDE	0	1 (2.3)	1 (1.1)
MAGNESIUM OXIDE	1 (2.2)	0	1 (1.1)
OTHER ANTIVIRALS	1 (2.2)	2 (4.7)	3 (3.4)
IMIDAZOLYL ETHANAMIDE PENTANDIOIC ACID	1 (2.2)	0	1 (1.1)
INOSINE PRANOBEX	0	1 (2.3)	1 (1.1)
UMIFENOVIR	0	1 (2.3)	1 (1.1)
OTHER DRUGS FOR FUNCTIONAL GASTROINTESTINAL DISORDERS	2 (4.4)	1 (2.3)	3 (3.4)
SIMETICONE	2 (4.4)	0	2 (2.3)
DIMETICONE	0	1 (2.3)	1 (1.1)
OTHER NASAL PREPARATIONS	1 (2.2)	2 (4.7)	3 (3.4)
SODIUM CHLORIDE	1 (2.2)	2 (4.7)	3 (3.4)

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
PROPULSIVES	3 (6.7)	0	3 (3.4)
DOMPERIDONE	3 (6.7)	0	3 (3.4)
SECOND-GENERATION CEPHALOSPORINS	2 (4.4)	1 (2.3)	3 (3.4)
CEFUROXIME	2 (4.4)	0	2 (2.3)
CEFUROXIME AXETIL	0	1 (2.3)	1 (1.1)
SOFTENERS, EMOLLIENTS	2 (4.4)	1 (2.3)	3 (3.4)
DOCUSATE	1 (2.2)	0	1 (1.1)
PARAFFIN	0	1 (2.3)	1 (1.1)
PARAFFIN, LIQUID	1 (2.2)	0	1 (1.1)
ADRENERGIC AND DOPAMINERGIC AGENTS	1 (2.2)	1 (2.3)	2 (2.3)
EPINEPHRINE	1 (2.2)	0	1 (1.1)
NOREPINEPHRINE BITARTRATE	0	1 (2.3)	1 (1.1)
ALDEHYDES AND DERIVATIVES	1 (2.2)	1 (2.3)	2 (2.3)
CHLORAL HYDRATE	1 (2.2)	1 (2.3)	2 (2.3)
ANTICHOLINERGICS	1 (2.2)	1 (2.3)	2 (2.3)
GLYCOPYRRONIUM	0	1 (2.3)	1 (1.1)
IPRATROPIUM BROMIDE	1 (2.2)	0	1 (1.1)

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
ASCORBIC ACID (VITAMIN C), PLAIN	2 (4.4)	0	2 (2.3)
ASCORBIC ACID	2 (4.4)	0	2 (2.3)
BELLADONNA ALKALOIDS, TERTIARY AMINES	0	2 (4.7)	2 (2.3)
ATROPINE	0	1 (2.3)	1 (1.1)
HYOSCYAMINE SULFATE	0	1 (2.3)	1 (1.1)
CALCIUM COMPOUNDS	2 (4.4)	0	2 (2.3)
CALCIUM CARBONATE	2 (4.4)	0	2 (2.3)
COMBINATIONS OF SULFONAMIDES AND TRIMETHOPRIM, INCL. DERIVATIVES	2 (4.4)	0	2 (2.3)
SULFAMETHOXAZOLE;TRIMETHOPRIM	2 (4.4)	0	2 (2.3)
CORTICOSTEROIDS, MODERATELY POTENT (GROUP II)	1 (2.2)	1 (2.3)	2 (2.3)
TRIAMCINOLONE	0	1 (2.3)	1 (1.1)
TRIAMCINOLONE ACETONIDE	1 (2.2)	0	1 (1.1)
DIAZEPINES, OXAZEPINES, THIAZEPINES AND OXEPINES	1 (2.2)	1 (2.3)	2 (2.3)
OLANZAPINE	0	1 (2.3)	1 (1.1)
QUETIAPINE	1 (2.2)	0	1 (1.1)
EXPECTORANTS	1 (2.2)	1 (2.3)	2 (2.3)
GUAIFENESIN	1 (2.2)	1 (2.3)	2 (2.3)

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
FAT/CARBOHYDRATES/PROTEINS/MINERALS/VITAMINS, COMBINATIONS	1 (2.2)	1 (2.3)	2 (2.3)
CARNITINE;CAROTENOIDS NOS;CHOLINE;FIBRE INSOLUBLE;FIBRE SOLUBLE;FISH OIL;HERBAL OIL NOS;MALTODEXTRIN;MINERALS NOS;PROTEINS NOS;TAURINE;VITAMINS NOS	1 (2.2)	0	1 (1.1)
CARNITINE;CAROTENOIDS NOS;CHOLINE;FISH OIL;HERBAL OIL NOS;MALTODEXTRIN;MINERALS NOS;PROTEINS NOS;TAURINE;VITAMINS NOS	0	1 (2.3)	1 (1.1)
FATTY ACID DERIVATIVES	0	2 (4.7)	2 (2.3)
VIGABATRIN	0	2 (4.7)	2 (2.3)
FOURTH-GENERATION CEPHALOSPORINS	2 (4.4)	0	2 (2.3)
CEFEPIME	1 (2.2)	0	1 (1.1)
CEFEPIME HYDROCHLORIDE	1 (2.2)	0	1 (1.1)
MEDICAL GASES	1 (2.2)	1 (2.3)	2 (2.3)
OXYGEN	1 (2.2)	1 (2.3)	2 (2.3)
MUCOLYTICS	1 (2.2)	1 (2.3)	2 (2.3)
ACETYLCYSTEINE	1 (2.2)	0	1 (1.1)
SODIUM CHLORIDE	0	1 (2.3)	1 (1.1)
MULTIVITAMINS, OTHER COMBINATIONS	1 (2.2)	1 (2.3)	2 (2.3)
ASCORBIC ACID;BETACAROTENE;BIOTIN;CALCIUM CARBONATE;CALCIUM PANTOTHENATE;CHROMIC CHLORIDE;COLECALCIFEROL;COPPER SULFATE;CYANOCOBALAMIN;FERROUS FUMARATE;FOLIC ACID;LYCOPENE;MAGNESIUM OXIDE;MANGANESE	0	1 (2.3)	1 (1.1)
MINERALS NOS;VITAMINS NOS	1 (2.2)	0	1 (1.1)
NATURAL OPIUM ALKALOIDS	1 (2.2)	1 (2.3)	2 (2.3)

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
HYDROMORPHONE HYDROCHLORIDE	1 (2.2)	0	1 (1.1)
OXYCODONE	1 (2.2)	0	1 (1.1)
OXYCODONE HYDROCHLORIDE	0	1 (2.3)	1 (1.1)
OPIUM DERIVATIVES AND EXPECTORANTS	1 (2.2)	1 (2.3)	2 (2.3)
CHLORPHENAMINE MALEATE;DEXTROMETHORPHAN HYDROBROMIDE;GUAIFENESIN;PHENYLEPHRINE	1 (2.2)	0	1 (1.1)
DEXTROMETHORPHAN HYDROBROMIDE;GUAIFENESIN	0	1 (2.3)	1 (1.1)
OTHER ANALGESICS AND ANTIPYRETICS	1 (2.2)	1 (2.3)	2 (2.3)
CLONIDINE	1 (2.2)	1 (2.3)	2 (2.3)
OTHER ANTIDEPRESSANTS	1 (2.2)	1 (2.3)	2 (2.3)
TRAZODONE	1 (2.2)	1 (2.3)	2 (2.3)
OTHER COUGH SUPPRESSANTS	1 (2.2)	1 (2.3)	2 (2.3)
BUTAMIRATE CITRATE	1 (2.2)	0	1 (1.1)
CLOPERASTINE	0	1 (2.3)	1 (1.1)
OTHER HYPNOTICS AND SEDATIVES	1 (2.2)	1 (2.3)	2 (2.3)
HYOSCINE	1 (2.2)	0	1 (1.1)
NIAPRAZINE	0	1 (2.3)	1 (1.1)
OTHER MINERAL PRODUCTS	1 (2.2)	1 (2.3)	2 (2.3)

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
ECHINACEA ANGUSTIFOLIA;MAGNESIUM AMINO ACID CHELATE;MANGANESE AMINO ACID CHELATE;PYRIDOXINE HYDROCHLORIDE;RETINOL;SMILAX ARISTOLOCHIIFOLIA;ZINC AMINO ACID CHELATE	0	1 (2.3)	1 (1.1)
POTASSIUM PHOSPHATE DIBASIC;POTASSIUM PHOSPHATE MONOBASIC;SODIUM PHOSPHATE DIBASIC;SODIUM PHOSPHATE MONOBASIC	1 (2.2)	0	1 (1.1)
OTHER THROAT PREPARATIONS	1 (2.2)	1 (2.3)	2 (2.3)
BENZYDAMINE HYDROCHLORIDE	0	1 (2.3)	1 (1.1)
PROPOLIS	1 (2.2)	0	1 (1.1)
SELENIUM	1 (2.2)	1 (2.3)	2 (2.3)
SELENIUM	1 (2.2)	1 (2.3)	2 (2.3)
SILVER COMPOUNDS	2 (4.4)	0	2 (2.3)
SILVER NITRATE	2 (4.4)	0	2 (2.3)
SULFONAMIDES, PLAIN	1 (2.2)	1 (2.3)	2 (2.3)
FUROSEMIDE	1 (2.2)	1 (2.3)	2 (2.3)
VARIOUS ALIMENTARY TRACT AND METABOLISM PRODUCTS	2 (4.4)	0	2 (2.3)
CITRIC ACID;SODIUM CITRATE	1 (2.2)	0	1 (1.1)
PHOSPHORUS	1 (2.2)	0	1 (1.1)
ACETIC ACID DERIVATIVES AND RELATED SUBSTANCES	1 (2.2)	0	1 (1.1)
KETOROLAC TROMETHAMINE	1 (2.2)	0	1 (1.1)
AMIDES	1 (2.2)	0	1 (1.1)

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Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
BUPIVACAINE	1 (2.2)	0	1 (1.1)
LIDOCAINE	1 (2.2)	0	1 (1.1)
ROPIVACAINE	1 (2.2)	0	1 (1.1)
AMINO ACIDS	1 (2.2)	0	1 (1.1)
TRANEXAMIC ACID	1 (2.2)	0	1 (1.1)
ANTIBIOTICS	0	1 (2.3)	1 (1.1)
NYSTATIN	0	1 (2.3)	1 (1.1)
ANTIDOTES	1 (2.2)	0	1 (1.1)
NALOXONE HYDROCHLORIDE	1 (2.2)	0	1 (1.1)
ANTIFUNGALS FOR SYSTEMIC USE	1 (2.2)	0	1 (1.1)
GRISEOFULVIN	1 (2.2)	0	1 (1.1)
TERBINAFINE	1 (2.2)	0	1 (1.1)
ANTIINFECTIVES	1 (2.2)	0	1 (1.1)
CIPROFLOXACIN	1 (2.2)	0	1 (1.1)
ANTIINFECTIVES AND ANTISEPTICS FOR LOCAL ORAL TREATMENT	0	1 (2.3)	1 (1.1)
NYSTATIN	0	1 (2.3)	1 (1.1)
APPETITE STIMULANTS	1 (2.2)	0	1 (1.1)

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Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
CYPROHEPTADINE HYDROCHLORIDE	1 (2.2)	0	1 (1.1)
BACTERIAL AND VIRAL VACCINES, COMBINED	0	1 (2.3)	1 (1.1)
DIPHTHERIA VACCINE TOXOID;PERTUSSIS VACCINE ACELLULAR 3-COMPONENT;POLIO VACCINE INACT 3V (VERO);TETANUS VACCINE TOXOID	0	1 (2.3)	1 (1.1)
BELLADONNA ALKALOIDS, SEMISYNTHETIC, QUATERNARY AMMONIUM COMPOUNDS	1 (2.2)	0	1 (1.1)
HYOSCINE BUTYLBROMIDE	1 (2.2)	0	1 (1.1)
BLOOD SUBSTITUTES AND PLASMA PROTEIN FRACTIONS	1 (2.2)	0	1 (1.1)
ALBUMIN HUMAN	1 (2.2)	0	1 (1.1)
CORTICOSTEROIDS AND ANTIINFECTIVES IN COMBINATION	1 (2.2)	0	1 (1.1)
CORTICOSTEROIDS AND ANTIINFECTIVES IN COMBINATION	1 (2.2)	0	1 (1.1)
CORTICOSTEROIDS, POTENT (GROUP III)	0	1 (2.3)	1 (1.1)
BECLOMETASONE DIPROPIONATE	0	1 (2.3)	1 (1.1)
COUGH AND COLD PREPARATIONS	1 (2.2)	0	1 (1.1)
COUGH AND COLD PREPARATIONS	1 (2.2)	0	1 (1.1)
COXIBS	0	1 (2.3)	1 (1.1)
CELECOXIB	0	1 (2.3)	1 (1.1)
DIHYDROPYRIDINE DERIVATIVES	1 (2.2)	0	1 (1.1)
AMLODIPINE	1 (2.2)	0	1 (1.1)

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
DRUGS FOR TREATMENT OF HYPERKALEMIA AND HYPERPHOSPHATEMIA	1 (2.2)	0	1 (1.1)
CALCIUM;MAGNESIUM	1 (2.2)	0	1 (1.1)
ESTERS OF AMINOBENZOIC ACID	1 (2.2)	0	1 (1.1)
BENZOCAINE	1 (2.2)	0	1 (1.1)
ESTERS OF BENZOIC ACID	1 (2.2)	0	1 (1.1)
AMYLOCAINE HYDROCHLORIDE;SODIUM BENZOATE	1 (2.2)	0	1 (1.1)
ESTREN DERIVATIVES	0	1 (2.3)	1 (1.1)
NORETHISTERONE	0	1 (2.3)	1 (1.1)
ETHERS OF TROPINE OR TROPINE DERIVATIVES	0	1 (2.3)	1 (1.1)
BENZATROPINE MESILATE	0	1 (2.3)	1 (1.1)
FLUOROQUINOLONES	1 (2.2)	0	1 (1.1)
CIPROFLOXACIN	1 (2.2)	0	1 (1.1)
FOLIC ACID AND DERIVATIVES	1 (2.2)	0	1 (1.1)
FOLIC ACID	1 (2.2)	0	1 (1.1)
HOMEOPATHIC PREPARATION	1 (2.2)	0	1 (1.1)
ARNICA MONTANA	1 (2.2)	0	1 (1.1)
HYPNOTICS AND SEDATIVES IN COMBINATION, EXCL. BARBITURATES	0	1 (2.3)	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cm-nonaed-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-07-02a-cm-nonaed-ole.rtf

Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
ALPHA-CASOZEPINE ENRICHED HYDROLYSED MILK PRO;CALCIUM PHOSPHATE DIHYDRATE;CHOLINE BITARTRATE;GLYCINE;INOSITOL;MAGNESIUM AMINO ACID CHELATE;PRUNUS CERASUS FRUIT;SODIUM PHOSPHATE DIBASIC;TOCOPHERYL AC	0	1 (2.3)	1 (1.1)
IMIDAZOLE AND TRIAZOLE DERIVATIVES	1 (2.2)	0	1 (1.1)
CLOTRIMAZOLE	1 (2.2)	0	1 (1.1)
KETOCONAZOLE	1 (2.2)	0	1 (1.1)
IMIDAZOLINE RECEPTOR AGONISTS	1 (2.2)	0	1 (1.1)
CLONIDINE	1 (2.2)	0	1 (1.1)
INSULINS AND ANALOGUES FOR INJECTION, FAST-ACTING	1 (2.2)	0	1 (1.1)
INSULIN	1 (2.2)	0	1 (1.1)
INTERFERONS	0	1 (2.3)	1 (1.1)
IMMUNOGLOBULIN HUMAN NORMAL;INTERFERON ALFA-2B	0	1 (2.3)	1 (1.1)
IRON TRIVALENT, ORAL PREPARATIONS	1 (2.2)	0	1 (1.1)
FERRIC HYDROXIDE POLYMALTOSE COMPLEX	1 (2.2)	0	1 (1.1)
LIVER THERAPY	0	1 (2.3)	1 (1.1)
CHOLINE BITARTRATE	0	1 (2.3)	1 (1.1)
CHOLINE BITARTRATE;INOSITOL;METHIONINE	0	1 (2.3)	1 (1.1)
MEASLES VACCINES	0	1 (2.3)	1 (1.1)
MEASLES VACCINE LIVE (ENDERS-EDMONSTON);MUMPS VACCINE LIVE (JERYL LYNN);RUBELLA VACCINE LIVE (WISTAR RA 27/3);VARICELLA ZOSTER VACCINE LIVE (OKA/MERCK)	0	1 (2.3)	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
NUCLEOSIDES AND NUCLEOTIDES EXCL. REVERSE TRANSCRIPTASE INHIBITORS	1 (2.2)	0	1 (1.1)
REMDESIVIR	1 (2.2)	0	1 (1.1)
OPIOID ANESTHETICS	1 (2.2)	0	1 (1.1)
FENTANYL	1 (2.2)	0	1 (1.1)
OTHER AMINOGLYCOSIDES	1 (2.2)	0	1 (1.1)
TOBRAMYCIN	1 (2.2)	0	1 (1.1)
OTHER ANTIBACTERIALS	0	1 (2.3)	1 (1.1)
LINEZOLID	0	1 (2.3)	1 (1.1)
OTHER ANTIFUNGALS FOR TOPICAL USE	0	1 (2.3)	1 (1.1)
CICLOPIROX	0	1 (2.3)	1 (1.1)
OTHER ANTIPSYCHOTICS	1 (2.2)	0	1 (1.1)
RISPERIDONE	1 (2.2)	0	1 (1.1)
OTHER ANXIOLYTICS	0	1 (2.3)	1 (1.1)
ESCITALOPRAM OXALATE	0	1 (2.3)	1 (1.1)
OTHER CARDIAC PREPARATIONS	0	1 (2.3)	1 (1.1)
UBIDECARENONE	0	1 (2.3)	1 (1.1)
OTHER CARDIAC STIMULANTS	1 (2.2)	0	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cm-nonaed-ole.sas
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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
IPRATROPIUM BROMIDE	1 (2.2)	0	1 (1.1)
OTHER CENTRALLY ACTING AGENTS	1 (2.2)	0	1 (1.1)
CYCLOBENZAPRINE	1 (2.2)	0	1 (1.1)
OTHER COLD PREPARATIONS	1 (2.2)	0	1 (1.1)
CAMPHOR;EUCALYPTUS GLOBULUS OIL;LEVOMENTHOL;PINUS PINASTER OIL;THYMOL	1 (2.2)	0	1 (1.1)
OTHER DRUGS FOR CONSTIPATION	1 (2.2)	0	1 (1.1)
GLYCEROL	1 (2.2)	0	1 (1.1)
OTHER DRUGS FOR PEPTIC ULCER AND GASTRO-OESOPHAGEAL REFLUX DISEASE (GORD)	1 (2.2)	0	1 (1.1)
CALCIUM CARBONATE;SODIUM ALGINATE;SODIUM BICARBONATE	1 (2.2)	0	1 (1.1)
OTHER ECTOPARASITICIDES, INCL. SCABICIDES	0	1 (2.3)	1 (1.1)
DIMETICONE	0	1 (2.3)	1 (1.1)
OTHER GENERAL ANESTHETICS	1 (2.2)	0	1 (1.1)
PROPOFOL	1 (2.2)	0	1 (1.1)
OTHER LIPID MODIFYING AGENTS	1 (2.2)	0	1 (1.1)
TRIGLYCERIDES	1 (2.2)	0	1 (1.1)
OTHER OPHTHALMOLOGICALS	0	1 (2.3)	1 (1.1)
OTHER OPHTHALMOLOGICALS	0	1 (2.3)	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
OTHER PSYCHOSTIMULANTS AND NOOTROPICS	1 (2.2)	0	1 (1.1)
INOSINE;NICOTINAMIDE;RIBOFLAVIN;SUCCINIC ACID	1 (2.2)	0	1 (1.1)
OTHER QUATERNARY AMMONIUM COMPOUNDS	1 (2.2)	0	1 (1.1)
ROCURONIUM	1 (2.2)	0	1 (1.1)
OTHER THERAPEUTIC PRODUCTS	1 (2.2)	0	1 (1.1)
TAURINE	1 (2.2)	0	1 (1.1)
OTHER UROLOGICALS	1 (2.2)	0	1 (1.1)
CITRIC ACID;POTASSIUM CITRATE	1 (2.2)	0	1 (1.1)
OXICAMS	1 (2.2)	0	1 (1.1)
PIROXICAM	1 (2.2)	0	1 (1.1)
PERTUSSIS VACCINES	1 (2.2)	0	1 (1.1)
DIPHTHERIA VACCINE TOXOID;PERTUSSIS VACCINE ACELLULAR;TETANUS VACCINE TOXOID	1 (2.2)	0	1 (1.1)
PHENYLPIPERIDINE DERIVATIVES	1 (2.2)	0	1 (1.1)
FENTANYL	1 (2.2)	0	1 (1.1)
PLATELET AGGREGATION INHIBITORS EXCL. HEPARIN	1 (2.2)	0	1 (1.1)
ACETYLSALICYLIC ACID	1 (2.2)	0	1 (1.1)
PNEUMOCOCCAL VACCINES	1 (2.2)	0	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
PNEUMOCOCCAL VACCINE POLYSACCH 23V	1 (2.2)	0	1 (1.1)
PREGNEN (4) DERIVATIVES	0	1 (2.3)	1 (1.1)
MEDROXYPROGESTERONE ACETATE	0	1 (2.3)	1 (1.1)
SOLUTIONS FOR PARENTERAL NUTRITION	1 (2.2)	0	1 (1.1)
GLUCOSE	1 (2.2)	0	1 (1.1)
THYROID HORMONES	1 (2.2)	0	1 (1.1)
LEVOTHYROXINE SODIUM	1 (2.2)	0	1 (1.1)
URINARY CONCREMENT SOLVENTS	1 (2.2)	0	1 (1.1)
CITRIC ACID;POTASSIUM CITRATE;SODIUM CITRATE ACID	1 (2.2)	0	1 (1.1)
VARICELLA ZOSTER VACCINES	1 (2.2)	0	1 (1.1)
VARICELLA ZOSTER VACCINE	1 (2.2)	0	1 (1.1)
VITAMINS, OTHER COMBINATIONS	1 (2.2)	0	1 (1.1)
VITAMINS, OTHER COMBINATIONS	1 (2.2)	0	1 (1.1)
WATERSOLUBLE, NEPHROTROPIC, LOW OSMOLAR X-RAY CONTRAST MEDIA	1 (2.2)	0	1 (1.1)
IOHEXOL	1 (2.2)	0	1 (1.1)
XANTHINE DERIVATIVES	0	1 (2.3)	1 (1.1)
CAFFEINE	0	1 (2.3)	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cm-nonaed-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-07-02a-cm-nonaed-ole.rtf

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Table 14.1.7.2A
Summary of Concomitant Non-AED Medications
(Open-Label Extension Phase, Safety Population)

	Treatment Group during Double-Blind Phase		
	Placebo	Ganaxolone	Total
WHO Drug Class Preferred Term [1]	(N=45) n (%)	(N=43) n (%)	(N=88) n (%)
ZINC	1 (2.2)	0	1 (1.1)
ZINC	1 (2.2)	0	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cm-nonaed-ole.sas
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4.8.1.3.2 Antikonvulsive Begleittherapien

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Table 14.1.7.3A
Summary of Concomitant AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
Any Concomitant AED Medications	43 (95.6)	42 (97.7)	85 (96.6)
OTHER ANTIEPILEPTICS	34 (75.6)	30 (69.8)	64 (72.7)
LEVETIRACETAM	12 (26.7)	11 (25.6)	23 (26.1)
ZONISAMIDE	8 (17.8)	6 (14.0)	14 (15.9)
LAMOTRIGINE	8 (17.8)	5 (11.6)	13 (14.8)
TOPIRAMATE	8 (17.8)	5 (11.6)	13 (14.8)
CANNABIDIOL	5 (11.1)	4 (9.3)	9 (10.2)
LACOSAMIDE	3 (6.7)	3 (7.0)	6 (6.8)
PERAMPANEL	3 (6.7)	3 (7.0)	6 (6.8)
FELBAMATE	3 (6.7)	1 (2.3)	4 (4.5)
GABAPENTIN	1 (2.2)	3 (7.0)	4 (4.5)
BRIVARACETAM	0	2 (4.7)	2 (2.3)
SULTIAME	1 (2.2)	1 (2.3)	2 (2.3)
CANNABINOIDS NOS	0	1 (2.3)	1 (1.1)
STIRIPENTOL	1 (2.2)	0	1 (1.1)
BENZODIAZEPINE DERIVATIVES	25 (55.6)	26 (60.5)	51 (58.0)
CLOBAZAM	17 (37.8)	13 (30.2)	30 (34.1)
CLONAZEPAM	5 (11.1)	6 (14.0)	11 (12.5)
DIAZEPAM	2 (4.4)	4 (9.3)	6 (6.8)
NITRAZEPAM	2 (4.4)	3 (7.0)	5 (5.7)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.2A

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Table 14.1.7.3A
Summary of Concomitant AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
MIDAZOLAM	1 (2.2)	3 (7.0)	4 (4.5)
MIDAZOLAM HYDROCHLORIDE	2 (4.4)	2 (4.7)	4 (4.5)
FATTY ACID DERIVATIVES	23 (51.1)	25 (58.1)	48 (54.5)
VALPROATE SEMISODIUM	16 (35.6)	17 (39.5)	33 (37.5)
VIGABATRIN	11 (24.4)	10 (23.3)	21 (23.9)
CARBOXAMIDE DERIVATIVES	7 (15.6)	9 (20.9)	16 (18.2)
RUFINAMIDE	6 (13.3)	7 (16.3)	13 (14.8)
CARBAMAZEPINE	1 (2.2)	1 (2.3)	2 (2.3)
OXCARBAZEPINE	0	2 (4.7)	2 (2.3)
BARBITURATES AND DERIVATIVES	3 (6.7)	3 (7.0)	6 (6.8)
PHENOBARBITAL	3 (6.7)	3 (7.0)	6 (6.8)
HYDANTOIN DERIVATIVES	2 (4.4)	2 (4.7)	4 (4.5)
PHENYTOIN	2 (4.4)	1 (2.3)	3 (3.4)
FOSPHENYTOIN	0	1 (2.3)	1 (1.1)
SUCCINIMIDE DERIVATIVES	1 (2.2)	2 (4.7)	3 (3.4)
ETHOSUXIMIDE	1 (2.2)	2 (4.7)	3 (3.4)
ALDEHYDES AND DERIVATIVES	0	1 (2.3)	1 (1.1)
CHLORAL HYDRATE	0	1 (2.3)	1 (1.1)
GLUCOCORTICOIDS	1 (2.2)	0	1 (1.1)
HYDROCORTISONE	1 (2.2)	0	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.2A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-07-03a-cm-aed-ole.rtf

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Table 14.1.7.3A
Summary of Concomitant AED Medications
(Open-Label Extension Phase, Safety Population)

WHO Drug Class Preferred Term [1]	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
IMMUNOGLOBULINS	0	1 (2.3)	1 (1.1)
IMMUNOGLOBULINS NOS	0	1 (2.3)	1 (1.1)
OTHER PLAIN VITAMIN PREPARATIONS	0	1 (2.3)	1 (1.1)
PYRIDOXINE	0	1 (2.3)	1 (1.1)
PROGESTOGENS	1 (2.2)	0	1 (1.1)
MEDROXYPROGESTERONE ACETATE	1 (2.2)	0	1 (1.1)

Note: concomitant medications are defined as medications (other than the study drug) administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]WHO Drug Dictionary Version 3 (March 2020).
Source Data: Listing 16.2.3.7.2A

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4.8.1.3.3 Nicht-medikamentöse Begleittherapien

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Table 14.1.7.4A
Summary of Concomitant Therapies
(Open-Label Extension Phase, Safety Population)

	Treatment Group during Double-Blind Phase		
Therapy [1]	Placebo (N=45) n (%)	Ganaxolone (N=43) n (%)	Total (N=88) n (%)
Concomitant Therapies	26 (57.8)	26 (60.5)	52 (59.1)
Physiotherapy	21 (46.7)	18 (41.9)	39 (44.3)
Speech Rehabilitation	18 (40.0)	14 (32.6)	32 (36.4)
Occupational Therapy	15 (33.3)	11 (25.6)	26 (29.5)
Therapeutic Procedure	6 (13.3)	7 (16.3)	13 (14.8)
Vagal Nerve Stimulator Implantation	6 (13.3)	6 (14.0)	12 (13.6)
Ketogenic Diet	6 (13.3)	3 (7.0)	9 (10.2)
Gastrostomy	4 (8.9)	2 (4.7)	6 (6.8)
Hydrotherapy	4 (8.9)	1 (2.3)	5 (5.7)
Oxygen Therapy	3 (6.7)	2 (4.7)	5 (5.7)
Psychotherapy	1 (2.2)	3 (7.0)	4 (4.5)
Airway Secretion Clearance Therapy	2 (4.4)	1 (2.3)	3 (3.4)
Hip Surgery	2 (4.4)	0	2 (2.3)
Acupuncture	0	1 (2.3)	1 (1.1)
Adenotonsillectomy	0	1 (2.3)	1 (1.1)
Cardiac Pacemaker Replacement	1 (2.2)	0	1 (1.1)
External Vagal Nerve Stimulation	0	1 (2.3)	1 (1.1)
Eye Exercises	0	1 (2.3)	1 (1.1)
Eyeglasses Therapy	1 (2.2)	0	1 (1.1)
Gastroenterostomy	1 (2.2)	0	1 (1.1)
Gastrointestinal Tube Insertion	0	1 (2.3)	1 (1.1)
Jejunostomy	1 (2.2)	0	1 (1.1)
Orthosis User	1 (2.2)	0	1 (1.1)

Note: concomitant therapies are defined as therapies administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]Therapies are coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.7.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-prct-ole.sas
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Table 14.1.7.4A
Summary of Concomitant Therapies
(Open-Label Extension Phase, Safety Population)

	Treatment Group during Double-Blind Phase		
	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Therapy [1]	n (%)	n (%)	n (%)
Physiotherapy Chest	1 (2.2)	0	1 (1.1)
Spinal Fusion Surgery	0	1 (2.3)	1 (1.1)
Therapeutic Aspiration	0	1 (2.3)	1 (1.1)
Tooth Repair	1 (2.2)	0	1 (1.1)

Note: concomitant therapies are defined as therapies administered on or after the first dosing day of the open-label extension phase.
Subjects are grouped by the treatment received in DB phase.
[1]Therapies are coded using MedDRA version 23.0.
Source Data: Listing 16.2.3.7.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-prct-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-01-07-04a-prct-ole.rtf

4.8.2 Anfallstagebuch-Compliance während der OLE

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Table 14.2.1A
Summary of Seizure Diary Compliance (%)
(Open-Label Extension Phase, Intent-to-Treat Population)

Category	Treatment Group during Double-Blind Phase		
Statistics	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Seizure Diary Entries			
Baseline (6 weeks prior to the first dose)			
n	45	42	87
Mean (SD)	94.31 (12.602)	97.87 (5.217)	96.03 (9.871)
Median	100.00	100.00	100.00
Q1, Q3	95.24, 100.00	97.62, 100.00	95.24, 100.00
Min, Max	28.6, 100.0	73.8, 100.0	28.6, 100.0
At Least 80% Compliant, n(%)	41 (91.1)	41 (97.6)	82 (94.3)
Visit 5 (Week 17) to Visit 6 (Week 19)			
n	43	42	85
Mean (SD)	95.44 (10.637)	95.35 (10.465)	95.39 (10.489)
Median	100.00	100.00	100.00
Q1, Q3	94.74, 100.00	93.33, 100.00	94.12, 100.00
Min, Max	52.9, 100.0	47.1, 100.0	47.1, 100.0
At Least 80% Compliant, n(%)	40 (93.0)	39 (92.9)	79 (92.9)

Diary compliance: 100 x (Number of days with available seizure data within the interval) / (Number of days within the interval), with the days within the interval defined as the day of the start of the interval up to the day before the end of the interval (the Baseline interval ends the day before the first dose).
The Entire OLE Phase interval consists of days considered for the seizure analyses in that interval (See Table 14.2.2.1A).
The percentage of subjects at least 80% compliant is based on the calculated compliances and the denominator is the number of subjects with data in the interval (n).
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.1.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-sdc-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-01a-sdc-ole.rtf

Table 14.2.1A
Summary of Seizure Diary Compliance (%)
(Open-Label Extension Phase, Intent-to-Treat Population)

Category Statistics	Treatment Group during Double-Blind Phase Placebo (N=45)	 Ganaxolone (N=43)	 Total (N=88)
Visit 6 (Week 19) to Visit 7 (Week 21)			
n	40	42	82
Mean (SD)	94.53 (13.034)	95.99 (9.916)	95.28 (11.493)
Median	100.00	100.00	100.00
Q1, Q3	100.00, 100.00	100.00, 100.00	100.00, 100.00
Min, Max	37.9, 100.0	44.4, 100.0	37.9, 100.0
At Least 80% Compliant, n(%)	35 (87.5)	41 (97.6)	76 (92.7)
Visit 7 (Week 21) to Visit 8 (Week 34)			
n	36	41	77
Mean (SD)	94.53 (13.152)	90.30 (18.159)	92.28 (16.053)
Median	100.00	98.96	100.00
Q1, Q3	97.83, 100.00	93.26, 100.00	93.88, 100.00
Min, Max	42.9, 100.0	31.5, 100.0	31.5, 100.0
At Least 80% Compliant, n(%)	33 (91.7)	35 (85.4)	68 (88.3)

Diary compliance: 100 x (Number of days with available seizure data within the interval) / (Number of days within the interval), with the days within the interval defined as the day of the start of the interval up to the day before the end of the interval (the Baseline interval ends the day before the first dose).
The Entire OLE Phase interval consists of days considered for the seizure analyses in that interval (See Table 14.2.2.1A).
The percentage of subjects at least 80% compliant is based on the calculated compliances and the denominator is the number of subjects with data in the interval (n).
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.1.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-sdc-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-01a-sdc-ole.rtf

Table 14.2.1A
Summary of Seizure Diary Compliance (%)
(Open-Label Extension Phase, Intent-to-Treat Population)

Category Statistics	Treatment Group during Double-Blind Phase Placebo (N=45)	 Ganaxolone (N=43)	 Total (N=88)
Visit 8 (Week 34) to Visit 9 (Week 52)			
n	33	32	65
Mean (SD)	87.44 (25.160)	94.29 (11.069)	90.81 (19.692)
Median	98.50	99.26	99.21
Q1, Q3	90.76, 100.00	93.25, 100.00	92.06, 100.00
Min, Max	4.0, 100.0	55.6, 100.0	4.0, 100.0
At Least 80% Compliant, n(%)	28 (84.8)	30 (93.8)	58 (89.2)
Visit 9 (Week 52) to Visit 10 (Week 68)			
n	30	29	59
Mean (SD)	91.47 (17.645)	89.51 (19.435)	90.51 (18.412)
Median	98.70	97.32	98.23
Q1, Q3	95.41, 100.00	93.33, 100.00	93.75, 100.00
Min, Max	28.7, 100.0	17.5, 100.0	17.5, 100.0
At Least 80% Compliant, n(%)	25 (83.3)	24 (82.8)	49 (83.1)

Diary compliance: 100 x (Number of days with available seizure data within the interval) / (Number of days within the interval), with the days within the interval defined as the day of the start of the interval up to the day before the end of the interval (the Baseline interval ends the day before the first dose).
The Entire OLE Phase interval consists of days considered for the seizure analyses in that interval (See Table 14.2.2.1A).
The percentage of subjects at least 80% compliant is based on the calculated compliances and the denominator is the number of subjects with data in the interval (n).
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.1.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-sdc-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-01a-sdc-ole.rtf

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Table 14.2.1A
Summary of Seizure Diary Compliance (%)
(Open-Label Extension Phase, Intent-to-Treat Population)

Category Statistics	Treatment Group during Double-Blind Phase Placebo (N=45)	 Ganaxolone (N=43)	 Total (N=88)
Visit 10 (Week 68) to Visit 11 (Week 84)			
n	25	28	53
Mean (SD)	89.87 (20.887)	79.08 (27.137)	84.17 (24.764)
Median	99.11	94.20	99.11
Q1, Q3	95.58, 100.00	63.01, 100.00	70.00, 100.00
Min, Max	17.0, 100.0	12.3, 100.0	12.3, 100.0
At Least 80% Compliant, n(%)	21 (84.0)	17 (60.7)	38 (71.7)
Visit 11 (Week 84) to Visit 12 (Week 100)			
n	23	25	48
Mean (SD)	89.40 (21.363)	80.91 (26.037)	84.98 (24.046)
Median	100.00	89.09	97.77
Q1, Q3	91.34, 100.00	81.82, 100.00	85.59, 100.00
Min, Max	31.1, 100.0	16.8, 100.0	16.8, 100.0
At Least 80% Compliant, n(%)	19 (82.6)	19 (76.0)	38 (79.2)

Diary compliance: 100 x (Number of days with available seizure data within the interval) / (Number of days within the interval), with the days within the interval defined as the day of the start of the interval up to the day before the end of the interval (the Baseline interval ends the day before the first dose).
The Entire OLE Phase interval consists of days considered for the seizure analyses in that interval (See Table 14.2.2.1A).
The percentage of subjects at least 80% compliant is based on the calculated compliances and the denominator is the number of subjects with data in the interval (n).
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.1.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-sdc-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-01a-sdc-ole.rtf

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Table 14.2.1A
Summary of Seizure Diary Compliance (%)
(Open-Label Extension Phase, Intent-to-Treat Population)

Category Statistics	Treatment Group during Double-Blind Phase Placebo (N=45)	 Ganaxolone (N=43)	 Total (N=88)
Visit 12 (Week 100) to Visit 13 (Week 116)			
n	23	25	48
Mean (SD)	92.28 (20.809)	84.99 (22.863)	88.48 (21.980)
Median	99.16	96.43	98.63
Q1, Q3	96.12, 100.00	76.22, 100.00	88.39, 100.00
Min, Max	10.1, 100.0	10.2, 100.0	10.1, 100.0
At Least 80% Compliant, n(%)	21 (91.3)	18 (72.0)	39 (81.3)
Visit 13 (Week 116) to Visit 14 (Week 132)			
n	20	20	40
Mean (SD)	88.51 (22.337)	83.76 (26.087)	86.14 (24.092)
Median	96.67	98.39	96.96
Q1, Q3	87.93, 100.00	76.04, 100.00	83.84, 100.00
Min, Max	3.8, 100.0	14.3, 100.0	3.8, 100.0
At Least 80% Compliant, n(%)	17 (85.0)	14 (70.0)	31 (77.5)

Diary compliance: 100 x (Number of days with available seizure data within the interval) / (Number of days within the interval), with the days within the interval defined as the day of the start of the interval up to the day before the end of the interval (the Baseline interval ends the day before the first dose).
The Entire OLE Phase interval consists of days considered for the seizure analyses in that interval (See Table 14.2.2.1A).
The percentage of subjects at least 80% compliant is based on the calculated compliances and the denominator is the number of subjects with data in the interval (n).
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.1.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-sdc-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-01a-sdc-ole.rtf

Marinus, Inc.
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Table 14.2.1A
Summary of Seizure Diary Compliance (%)
(Open-Label Extension Phase, Intent-to-Treat Population)

Category Statistics	Treatment Group during Double-Blind Phase Placebo (N=45)	 Ganaxolone (N=43)	 Total (N=88)
Visit 14 (Week 132) to Visit 15 (Week 148)			
n	12	9	21
Mean (SD)	91.72 (14.720)	82.71 (19.309)	87.86 (17.004)
Median	98.75	90.44	95.87
Q1, Q3	91.68, 100.00	78.43, 94.64	85.09, 99.17
Min, Max	59.5, 100.0	38.3, 99.2	38.3, 100.0
At Least 80% Compliant, n(%)	10 (83.3)	6 (66.7)	16 (76.2)
Visit 15 (Week 148) to Visit 16 (Week 164)			
n	11	7	18
Mean (SD)	88.03 (21.555)	89.24 (13.723)	88.50 (18.443)
Median	98.99	93.60	97.28
Q1, Q3	89.29, 100.00	73.91, 100.00	89.29, 100.00
Min, Max	42.4, 100.0	66.0, 100.0	42.4, 100.0
At Least 80% Compliant, n(%)	9 (81.8)	5 (71.4)	14 (77.8)

Diary compliance: 100 x (Number of days with available seizure data within the interval) / (Number of days within the interval), with the days within the interval defined as the day of the start of the interval up to the day before the end of the interval (the Baseline interval ends the day before the first dose).
The Entire OLE Phase interval consists of days considered for the seizure analyses in that interval (See Table 14.2.2.1A).
The percentage of subjects at least 80% compliant is based on the calculated compliances and the denominator is the number of subjects with data in the interval (n).
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.1.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-sdc-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-01a-sdc-ole.rtf

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Table 14.2.1A
Summary of Seizure Diary Compliance (%)
(Open-Label Extension Phase, Intent-to-Treat Population)

Category Statistics	Treatment Group during Double-Blind Phase Placebo (N=45)	 Ganaxolone (N=43)	 Total (N=88)
Visit 16 (Week 164) to Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	57.64 (50.971)	36.97 (N/A)	50.75 (37.966)
Median	57.64	36.97	36.97
Q1, Q3	21.60, 93.68	36.97, 36.97	21.60, 93.68
Min, Max	21.6, 93.7	37.0, 37.0	21.6, 93.7
At Least 80% Compliant, n(%)	1 (50.0)	0	1 (33.3)
Entire OLE Post-Baseline Phase			
n	45	43	88
Mean (SD)	89.05 (14.075)	88.37 (15.187)	88.72 (14.548)
Median	94.51	94.88	94.76
Q1, Q3	84.89, 99.51	83.47, 99.36	83.78, 99.44
Min, Max	41.9, 100.0	24.0, 100.0	24.0, 100.0
At Least 80% Compliant, n(%)	36 (80.0)	33 (76.7)	69 (78.4)

Diary compliance: 100 x (Number of days with available seizure data within the interval) / (Number of days within the interval), with the days within the interval defined as the day of the start of the interval up to the day before the end of the interval (the Baseline interval ends the day before the first dose).
The Entire OLE Phase interval consists of days considered for the seizure analyses in that interval (See Table 14.2.2.1A).
The percentage of subjects at least 80% compliant is based on the calculated compliances and the denominator is the number of subjects with data in the interval (n).
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.1.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-sdc-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-01a-sdc-ole.rtf

4.8.3 Ergebnisse zum Endpunkt Morbidität – Veränderung der Anfallshäufigkeit während der OLE

4.8.3.1 Prozent an Tagen ohne schwere motorische Anfälle während der OLE

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Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline			
n	45	42	87
Mean (SD)	31.74 (27.091)	21.08 (26.235)	26.60 (27.062)
Median (95% Distribution-Free CI)	27.27 (16.22, 40.48)	8.01 (0.00, 29.27)	19.05 (9.52, 30.95)
Q1, Q3	9.52, 54.76	0.00, 34.15	0.00, 45.24
Min, Max	0.0, 97.5	0.0, 82.9	0.0, 97.5

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
The First 4 Weeks of the OLE Phase			
n	45	43	88
Mean (SD)	43.00 (35.609)	30.96 (31.566)	37.12 (34.043)
Median (95% Distribution-Free CI)	31.25 (23.08, 70.37)	19.23 (10.71, 42.86)	28.29 (19.23, 42.86)
Q1, Q3	7.14, 75.00	3.57, 55.56	3.64, 67.95
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0
Arithmetic Change from Baseline			
n	45	42	87
Mean (SD)	11.26 (22.260)	10.16 (28.348)	10.73 (25.238)
Median (95% Distribution-Free CI)	10.58 (1.19, 15.03)	0.74 (0.00, 7.86)	4.36 (0.00, 10.71)
Q1, Q3	0.00, 19.05	0.00, 11.90	0.00, 18.52
Min, Max	-48.8, 63.1	-36.4, 100.0	-48.8, 100.0

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

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Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
The First 17 Weeks of the OLE Phase			
n	41	43	84
Mean (SD)	48.36 (33.269)	31.84 (32.076)	39.90 (33.511)
Median (95% Distribution-Free CI)	50.00 (33.93, 70.34)	17.70 (9.84, 44.54)	38.30 (17.70, 50.00)
Q1, Q3	15.22, 78.99	5.88, 56.78	8.33, 64.00
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0
Arithmetic Change from Baseline			
n	41	42	83
Mean (SD)	16.19 (20.740)	11.24 (27.507)	13.68 (24.379)
Median (95% Distribution-Free CI)	10.30 (5.69, 25.02)	4.74 (0.84, 9.71)	6.72 (3.50, 13.14)
Q1, Q3	2.24, 30.23	0.00, 16.54	0.00, 25.83
Min, Max	-23.1, 72.9	-36.3, 100.0	-36.3, 100.0

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
The First 35 Weeks of the OLE Phase			
n	38	39	77
Mean (SD)	47.14 (33.333)	33.31 (32.605)	40.14 (33.479)
Median (95% Distribution-Free CI)	49.20 (25.00, 66.39)	20.16 (8.16, 45.68)	32.00 (20.16, 53.17)
Q1, Q3	17.24, 76.05	4.59, 61.73	7.79, 66.39
Min, Max	0.0, 98.6	0.0, 100.0	0.0, 100.0
Arithmetic Change from Baseline			
n	38	39	77
Mean (SD)	13.57 (20.436)	11.04 (29.322)	12.29 (25.196)
Median (95% Distribution-Free CI)	10.66 (0.17, 21.13)	4.88 (2.04, 12.79)	7.62 (4.20, 12.79)
Q1, Q3	0.00, 30.05	0.27, 14.44	0.00, 22.56
Min, Max	-27.3, 63.4	-41.9, 100.0	-41.9, 100.0

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
The First 51 Weeks of the OLE Phase			
n	34	33	67
Mean (SD)	51.26 (32.276)	34.16 (34.384)	42.84 (34.180)
Median (95% Distribution-Free CI)	54.42 (33.71, 75.43)	17.77 (7.32, 51.61)	40.21 (20.38, 58.71)
Q1, Q3	20.38, 79.44	5.90, 65.88	9.97, 72.93
Min, Max	0.0, 98.8	0.0, 100.0	0.0, 100.0
Arithmetic Change from Baseline			
n	34	33	67
Mean (SD)	15.26 (19.799)	13.06 (28.375)	14.18 (24.241)
Median (95% Distribution-Free CI)	11.63 (8.63, 29.18)	5.90 (1.11, 12.93)	9.44 (4.94, 13.21)
Q1, Q3	1.12, 31.61	0.18, 15.88	0.18, 29.18
Min, Max	-27.0, 61.7	-30.3, 100.0	-30.3, 100.0

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
The First 67 Weeks of the OLE Phase			
n	30	29	59
Mean (SD)	52.33 (32.615)	33.99 (31.955)	43.32 (33.323)
Median (95% Distribution-Free CI)	53.36 (31.77, 75.92)	22.88 (7.71, 53.92)	42.26 (22.88, 60.78)
Q1, Q3	26.07, 79.74	5.06, 59.78	10.30, 75.92
Min, Max	0.0, 99.1	0.0, 93.9	0.0, 99.1
Arithmetic Change from Baseline			
n	30	29	59
Mean (SD)	16.17 (18.253)	10.39 (24.625)	13.33 (21.629)
Median (95% Distribution-Free CI)	13.08 (6.81, 26.07)	5.33 (0.65, 18.57)	9.46 (3.27, 17.24)
Q1, Q3	2.02, 29.94	0.00, 20.10	0.21, 27.93
Min, Max	-31.5, 51.8	-32.4, 93.9	-32.4, 93.9

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
The First 83 Weeks of the OLE Phase			
n	25	27	52
Mean (SD)	54.15 (34.598)	34.75 (31.838)	44.08 (34.290)
Median (95% Distribution-Free CI)	62.99 (34.08, 80.00)	29.26 (7.24, 52.24)	42.48 (21.71, 63.95)
Q1, Q3	22.07, 87.24	4.07, 69.71	8.48, 73.91
Min, Max	0.0, 99.1	0.0, 94.9	0.0, 99.1
Arithmetic Change from Baseline			
n	25	27	52
Mean (SD)	16.56 (19.373)	9.71 (24.833)	13.01 (22.427)
Median (95% Distribution-Free CI)	14.01 (3.72, 27.85)	4.45 (0.00, 20.75)	9.73 (3.04, 17.76)
Q1, Q3	1.90, 28.65	0.00, 20.83	0.00, 24.82
Min, Max	-31.8, 52.4	-33.5, 94.9	-33.5, 94.9

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
The First 99 Weeks of the OLE Phase			
n	24	27	51
Mean (SD)	54.18 (34.921)	35.12 (31.400)	44.09 (34.147)
Median (95% Distribution-Free CI)	63.24 (33.43, 84.84)	31.07 (10.53, 53.75)	40.37 (22.81, 62.97)
Q1, Q3	26.86, 85.99	4.27, 62.97	10.53, 73.15
Min, Max	0.0, 99.3	0.0, 95.7	0.0, 99.3
Arithmetic Change from Baseline			
n	24	27	51
Mean (SD)	15.99 (20.295)	10.08 (25.254)	12.86 (23.021)
Median (95% Distribution-Free CI)	12.67 (4.21, 33.43)	9.81 (0.00, 17.19)	10.94 (2.89, 16.40)
Q1, Q3	0.70, 33.95	0.00, 20.31	0.00, 27.44
Min, Max	-40.7, 51.8	-33.2, 95.7	-40.7, 95.7

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
The First 115 Weeks of the OLE Phase			
n	21	23	44
Mean (SD)	58.57 (32.160)	38.54 (31.465)	48.10 (33.016)
Median (95% Distribution-Free CI)	62.98 (35.99, 87.56)	36.59 (10.94, 55.23)	45.03 (31.35, 68.48)
Q1, Q3	35.99, 87.56	7.47, 68.48	19.18, 76.87
Min, Max	0.0, 99.5	0.0, 96.0	0.0, 99.5
Arithmetic Change from Baseline			
n	21	23	44
Mean (SD)	18.90 (21.544)	13.63 (26.366)	16.15 (24.055)
Median (95% Distribution-Free CI)	17.65 (8.16, 34.79)	11.42 (0.00, 20.91)	14.62 (8.16, 20.91)
Q1, Q3	8.16, 34.79	0.00, 25.33	0.26, 31.65
Min, Max	-44.6, 51.9	-32.5, 96.0	-44.6, 96.0

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
The First 131 Weeks of the OLE Phase			
n	19	19	38
Mean (SD)	62.85 (30.975)	38.87 (30.000)	50.86 (32.437)
Median (95% Distribution-Free CI)	69.58 (45.83, 87.41)	36.63 (10.40, 64.44)	49.23 (34.65, 70.11)
Q1, Q3	41.32, 87.41	7.00, 64.44	26.72, 80.66
Min, Max	0.0, 99.5	0.1, 93.0	0.0, 99.5
Arithmetic Change from Baseline			
n	19	19	38
Mean (SD)	23.78 (16.305)	8.46 (23.113)	16.12 (21.202)
Median (95% Distribution-Free CI)	18.45 (11.14, 38.99)	10.40 (-5.20, 28.99)	16.00 (7.63, 28.99)
Q1, Q3	10.84, 38.99	-8.39, 28.99	0.41, 33.87
Min, Max	0.0, 51.7	-32.7, 50.1	-32.7, 51.7

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

	Treatment Group during Double-Blind Phase		
Interval	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
The First 147 Weeks of the OLE Phase			
n	16	13	29
Mean (SD)	64.28 (28.707)	38.64 (32.439)	52.79 (32.570)
Median (95% Distribution-Free CI)	66.70 (43.05, 88.18)	44.52 (3.27, 73.81)	58.20 (42.61, 74.89)
Q1, Q3	42.83, 87.87	4.93, 60.03	34.97, 80.86
Min, Max	2.7, 99.6	0.1, 91.1	0.1, 99.6
Arithmetic Change from Baseline			
n	16	13	29
Mean (SD)	23.67 (14.978)	6.46 (20.302)	15.95 (19.305)
Median (95% Distribution-Free CI)	19.40 (11.36, 39.01)	4.93 (-8.04, 23.81)	15.01 (7.61, 29.56)
Q1, Q3	11.26, 36.90	-5.43, 19.43	4.93, 34.32
Min, Max	0.3, 49.6	-30.9, 40.2	-30.9, 49.6

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
The First 163 Weeks of the OLE Phase			
n	11	8	19
Mean (SD)	63.38 (28.638)	31.66 (31.833)	50.02 (33.297)
Median (95% Distribution-Free CI)	61.08 (44.98, 97.84)	26.85 (0.27, 76.63)	58.55 (42.38, 76.63)
Q1, Q3	44.98, 87.43	2.55, 58.86	6.31, 76.63
Min, Max	2.7, 99.6	0.1, 76.6	0.1, 99.6
Arithmetic Change from Baseline			
n	11	8	19
Mean (SD)	23.42 (16.762)	13.99 (17.886)	19.45 (17.418)
Median (95% Distribution-Free CI)	15.84 (9.98, 42.43)	6.99 (0.10, 39.70)	15.06 (5.43, 37.12)
Q1, Q3	9.98, 39.94	0.19, 31.88	4.82, 37.12
Min, Max	0.3, 48.1	-5.9, 39.7	-5.9, 48.1

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

Table 14.2.3.1.1A
Summary of the Percentage of Seizure-Free Days for Primary Seizure Types
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
The First 179 Weeks of the OLE Phase			
n	4	2	6
Mean (SD)	47.32 (39.875)	23.62 (33.276)	39.42 (36.403)
Median (95% Distribution-Free CI)	43.60 (2.52, 99.57)	23.62 (0.09, 47.15)	43.60 (0.09, 99.57)
Q1, Q3	22.29, 72.35	0.09, 47.15	2.52, 47.15
Min, Max	2.5, 99.6	0.1, 47.2	0.1, 99.6
Arithmetic Change from Baseline			
n	4	2	6
Mean (SD)	23.08 (21.150)	19.78 (27.837)	21.98 (20.647)
Median (95% Distribution-Free CI)	24.88 (0.14, 42.43)	19.78 (0.09, 39.46)	24.80 (0.09, 42.43)
Q1, Q3	5.13, 41.02	0.09, 39.46	0.14, 39.62
Min, Max	0.1, 42.4	0.1, 39.5	0.1, 42.4

Abbreviations: CI=Confidence interval.
Notes: Summaries are based on the individual seizures, the countable seizures, and the clusters with uncountable seizures.
The primary seizure types include bilateral tonic (sustained motor activity >= 3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Within the baseline and post baseline intervals, the percentage of seizure-free days is the number of days in the interval with zero seizures divided by the number of days with seizure data in the interval, multiplied by 100.
The baseline interval consists of the 6 weeks prior to the first dose of the DB phase.
The open-label extension phase starting from the first dosing day of OLE treatment.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.6.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-scprim-pchg-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-01-01a-scprim-pchg-ol.rtf

4.8.3.2 Anzahl der Patienten mit einer Reduktion der Gesamtanfallshäufigkeit pro 28 Tage um ≥ 25 %, ≥ 50 % und ≥ 75 % während der OLE

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Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 4 Weeks of the OLE Phase			
Primary [1]			
n	45	42	87
25% Responder	22 (48.9)	23 (54.8)	45 (51.7)
50% Responder	9 (20.0)	12 (28.6)	21 (24.1)
75% Responder	5 (11.1)	5 (11.9)	10 (11.5)
All			
n	45	42	87
25% Responder	22 (48.9)	23 (54.8)	45 (51.7)
50% Responder	11 (24.4)	13 (31.0)	24 (27.6)
75% Responder	3 (6.7)	6 (14.3)	9 (10.3)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 17 Weeks of the OLE Phase			
Primary [1]			
n	41	42	83
25% Responder	19 (46.3)	28 (66.7)	47 (56.6)
50% Responder	11 (26.8)	13 (31.0)	24 (28.9)
75% Responder	7 (17.1)	4 (9.5)	11 (13.3)
All			
n	41	42	83
25% Responder	17 (41.5)	26 (61.9)	43 (51.8)
50% Responder	10 (24.4)	10 (23.8)	20 (24.1)
75% Responder	4 (9.8)	5 (11.9)	9 (10.8)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 35 Weeks of the OLE Phase			
Primary [1]			
n	38	39	77
25% Responder	20 (52.6)	22 (56.4)	42 (54.5)
50% Responder	11 (28.9)	10 (25.6)	21 (27.3)
75% Responder	5 (13.2)	5 (12.8)	10 (13.0)
All			
n	38	39	77
25% Responder	18 (47.4)	23 (59.0)	41 (53.2)
50% Responder	8 (21.1)	11 (28.2)	19 (24.7)
75% Responder	1 (2.6)	5 (12.8)	6 (7.8)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 51 Weeks of the OLE Phase			
Primary [1]			
n	34	33	67
25% Responder	18 (52.9)	19 (57.6)	37 (55.2)
50% Responder	11 (32.4)	15 (45.5)	26 (38.8)
75% Responder	6 (17.6)	4 (12.1)	10 (14.9)
All			
n	34	33	67
25% Responder	18 (52.9)	21 (63.6)	39 (58.2)
50% Responder	9 (26.5)	13 (39.4)	22 (32.8)
75% Responder	2 (5.9)	3 (9.1)	5 (7.5)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 67 Weeks of the OLE Phase			
Primary [1]			
n	30	29	59
25% Responder	19 (63.3)	16 (55.2)	35 (59.3)
50% Responder	11 (36.7)	13 (44.8)	24 (40.7)
75% Responder	6 (20.0)	3 (10.3)	9 (15.3)
All			
n	30	29	59
25% Responder	18 (60.0)	17 (58.6)	35 (59.3)
50% Responder	10 (33.3)	12 (41.4)	22 (37.3)
75% Responder	3 (10.0)	2 (6.9)	5 (8.5)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 83 Weeks of the OLE Phase			
Primary [1]			
n	25	27	52
25% Responder	18 (72.0)	15 (55.6)	33 (63.5)
50% Responder	9 (36.0)	12 (44.4)	21 (40.4)
75% Responder	6 (24.0)	3 (11.1)	9 (17.3)
All			
n	25	27	52
25% Responder	16 (64.0)	16 (59.3)	32 (61.5)
50% Responder	9 (36.0)	12 (44.4)	21 (40.4)
75% Responder	3 (12.0)	2 (7.4)	5 (9.6)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

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Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 99 Weeks of the OLE Phase			
Primary [1]			
n	24	27	51
25% Responder	17 (70.8)	15 (55.6)	32 (62.7)
50% Responder	9 (37.5)	12 (44.4)	21 (41.2)
75% Responder	6 (25.0)	3 (11.1)	9 (17.6)
All			
n	24	27	51
25% Responder	16 (66.7)	17 (63.0)	33 (64.7)
50% Responder	9 (37.5)	12 (44.4)	21 (41.2)
75% Responder	3 (12.5)	3 (11.1)	6 (11.8)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 115 Weeks of the OLE Phase			
Primary [1]			
n	21	23	44
25% Responder	17 (81.0)	12 (52.2)	29 (65.9)
50% Responder	9 (42.9)	10 (43.5)	19 (43.2)
75% Responder	5 (23.8)	3 (13.0)	8 (18.2)
All			
n	21	23	44
25% Responder	16 (76.2)	15 (65.2)	31 (70.5)
50% Responder	9 (42.9)	10 (43.5)	19 (43.2)
75% Responder	3 (14.3)	3 (13.0)	6 (13.6)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

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Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 131 Weeks of the OLE Phase			
Primary [1]			
n	19	19	38
25% Responder	16 (84.2)	9 (47.4)	25 (65.8)
50% Responder	10 (52.6)	7 (36.8)	17 (44.7)
75% Responder	5 (26.3)	2 (10.5)	7 (18.4)
All			
n	19	19	38
25% Responder	15 (78.9)	12 (63.2)	27 (71.1)
50% Responder	12 (63.2)	6 (31.6)	18 (47.4)
75% Responder	3 (15.8)	2 (10.5)	5 (13.2)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 147 Weeks of the OLE Phase			
Primary [1]			
n	16	13	29
25% Responder	13 (81.3)	7 (53.8)	20 (69.0)
50% Responder	9 (56.3)	5 (38.5)	14 (48.3)
75% Responder	4 (25.0)	1 (7.7)	5 (17.2)
All			
n	16	13	29
25% Responder	13 (81.3)	8 (61.5)	21 (72.4)
50% Responder	10 (62.5)	4 (30.8)	14 (48.3)
75% Responder	2 (12.5)	1 (7.7)	3 (10.3)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

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Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 163 Weeks of the OLE Phase			
Primary [1]			
n	11	8	19
25% Responder	8 (72.7)	4 (50.0)	12 (63.2)
50% Responder	5 (45.5)	3 (37.5)	8 (42.1)
75% Responder	3 (27.3)	1 (12.5)	4 (21.1)
All			
n	11	8	19
25% Responder	9 (81.8)	4 (50.0)	13 (68.4)
50% Responder	6 (54.5)	2 (25.0)	8 (42.1)
75% Responder	1 (9.1)	1 (12.5)	2 (10.5)

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

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Table 14.2.3.2.1A
Summary of Notable Reduction Rates in 28-Day Seizure Frequency
(Open-Label Extension Phase, Intent-to-Treat Population)

Interval Seizure Type Category	Treatment Group during Double-Blind Phase Placebo (N=45) n (%)	 Ganaxolone (N=43) n (%)	 Total (N=88) n (%)
The First 179 Weeks of the OLE Phase			
Primary [1]			
n	4	2	6
25% Responder	2 (50.0)	1 (50.0)	3 (50.0)
50% Responder	2 (50.0)	1 (50.0)	3 (50.0)
75% Responder	1 (25.0)	0	1 (16.7)
All			
n	4	2	6
25% Responder	3 (75.0)	1 (50.0)	4 (66.7)
50% Responder	2 (50.0)	1 (50.0)	3 (50.0)
75% Responder	0	0	0

Notes: A responder is a subject whose percent change from baseline in 28-day seizure frequency indicates a reduction greater than or equal to the threshold, i.e. 25%, 50% and 75%. The seizure frequency is based on the sum of the individual seizures, the countable seizures, and the clusters with uncountable seizures (each cluster with uncountable seizures counts as 1 seizure).
The percentages are based on the number of subjects with percent changes (n).
[1]The primary seizure types include bilateral tonic (sustained motor activity >=3 seconds), generalized tonic-clonic, atonic/drop, bilateral clonic, and focal to bilateral tonic-clonic.
Subjects are grouped by the treatment received in DB phase.
Duplicate seizure diary entries are not used in the analysis.
Source Data: Listing 16.2.5.3.1a.

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-rr-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-02-01a-rr-ole.rtf

4.8.4 Ergebnisse zum Endpunkt Morbidität – Globaler Betreuereindruck der Veränderung (CGI) während der OLE

4.8.4.1 Veränderung der Anfallsintensität und -dauer (CGI-CSID) während der OLE

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Table 14.2.3.3.1A
Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
Total Observed, n	41	42	83
Very Much Improved	5 (12.2)	3 (7.1)	8 (9.6)
Much Improved	8 (19.5)	13 (31.0)	21 (25.3)
Minimally Improved	15 (36.6)	10 (23.8)	25 (30.1)
No Change	9 (22.0)	9 (21.4)	18 (21.7)
Minimally Worse	2 (4.9)	6 (14.3)	8 (9.6)
Much Worse	2 (4.9)	1 (2.4)	3 (3.6)
Very Much Worse	0	0	0
Visit 8 (Week 34)			
Total Observed, n	38	40	78
Very Much Improved	6 (15.8)	2 (5.0)	8 (10.3)
Much Improved	11 (28.9)	9 (22.5)	20 (25.6)
Minimally Improved	11 (28.9)	14 (35.0)	25 (32.1)
No Change	8 (21.1)	10 (25.0)	18 (23.1)
Minimally Worse	2 (5.3)	1 (2.5)	3 (3.8)
Much Worse	0	4 (10.0)	4 (5.1)
Very Much Worse	0	0	0
Visit 9 (Week 52)			
Total Observed, n	33	34	67
Very Much Improved	4 (12.1)	4 (11.8)	8 (11.9)
Much Improved	9 (27.3)	9 (26.5)	18 (26.9)
Minimally Improved	7 (21.2)	11 (32.4)	18 (26.9)
No Change	11 (33.3)	6 (17.6)	17 (25.4)
Minimally Worse	0	3 (8.8)	3 (4.5)
Much Worse	2 (6.1)	0	2 (3.0)
Very Much Worse	0	1 (2.9)	1 (1.5)

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.6.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgicsid-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-03-01a-cgicsid-ole.rtf

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Table 14.2.3.3.1A
Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
Total Observed, n	30	30	60
Very Much Improved	5 (16.7)	1 (3.3)	6 (10.0)
Much Improved	7 (23.3)	12 (40.0)	19 (31.7)
Minimally Improved	11 (36.7)	10 (33.3)	21 (35.0)
No Change	5 (16.7)	3 (10.0)	8 (13.3)
Minimally Worse	2 (6.7)	3 (10.0)	5 (8.3)
Much Worse	0	1 (3.3)	1 (1.7)
Very Much Worse	0	0	0
Visit 11 (Week 84)			
Total Observed, n	25	28	53
Very Much Improved	4 (16.0)	3 (10.7)	7 (13.2)
Much Improved	5 (20.0)	7 (25.0)	12 (22.6)
Minimally Improved	15 (60.0)	8 (28.6)	23 (43.4)
No Change	0	5 (17.9)	5 (9.4)
Minimally Worse	0	5 (17.9)	5 (9.4)
Much Worse	1 (4.0)	0	1 (1.9)
Very Much Worse	0	0	0
Visit 12 (Week 100)			
Total Observed, n	23	27	50
Very Much Improved	5 (21.7)	1 (3.7)	6 (12.0)
Much Improved	8 (34.8)	9 (33.3)	17 (34.0)
Minimally Improved	5 (21.7)	9 (33.3)	14 (28.0)
No Change	4 (17.4)	5 (18.5)	9 (18.0)
Minimally Worse	1 (4.3)	1 (3.7)	2 (4.0)
Much Worse	0	2 (7.4)	2 (4.0)
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.6.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgicsid-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-03-01a-cgicsid-ole.rtf

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Table 14.2.3.3.1A
Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
Total Observed, n	23	26	49
Very Much Improved	6 (26.1)	1 (3.8)	7 (14.3)
Much Improved	7 (30.4)	11 (42.3)	18 (36.7)
Minimally Improved	7 (30.4)	8 (30.8)	15 (30.6)
No Change	3 (13.0)	5 (19.2)	8 (16.3)
Minimally Worse	0	0	0
Much Worse	0	1 (3.8)	1 (2.0)
Very Much Worse	0	0	0
Visit 14 (Week 132)			
Total Observed, n	22	22	44
Very Much Improved	6 (27.3)	2 (9.1)	8 (18.2)
Much Improved	7 (31.8)	8 (36.4)	15 (34.1)
Minimally Improved	4 (18.2)	8 (36.4)	12 (27.3)
No Change	5 (22.7)	3 (13.6)	8 (18.2)
Minimally Worse	0	1 (4.5)	1 (2.3)
Much Worse	0	0	0
Very Much Worse	0	0	0
Visit 15 (Week 148)			
Total Observed, n	17	18	35
Very Much Improved	6 (35.3)	0	6 (17.1)
Much Improved	5 (29.4)	9 (50.0)	14 (40.0)
Minimally Improved	4 (23.5)	5 (27.8)	9 (25.7)
No Change	2 (11.8)	3 (16.7)	5 (14.3)
Minimally Worse	0	1 (5.6)	1 (2.9)
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.6.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgicsid-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-03-01a-cgicsid-ole.rtf

Table 14.2.3.3.1A
Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
Total Observed, n	11	9	20
Very Much Improved	4 (36.4)	0	4 (20.0)
Much Improved	5 (45.5)	3 (33.3)	8 (40.0)
Minimally Improved	1 (9.1)	5 (55.6)	6 (30.0)
No Change	1 (9.1)	1 (11.1)	2 (10.0)
Minimally Worse	0	0	0
Much Worse	0	0	0
Very Much Worse	0	0	0
Visit 17 (Week 180)			
Total Observed, n	2	1	3
Very Much Improved	0	0	0
Much Improved	0	0	0
Minimally Improved	1 (50.0)	1 (100)	2 (66.7)
No Change	1 (50.0)	0	1 (33.3)
Minimally Worse	0	0	0
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.6.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgicsid-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-03-01a-cgicsid-ole.rtf

Table 14.2.3.3.1A
Summary of Caregiver Global Impression of Change in Seizure Intensity/Duration (CGI-CSID)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [1]			
Total Observed, n	17	12	29
Very Much Improved	4 (23.5)	1 (8.3)	5 (17.2)
Much Improved	8 (47.1)	4 (33.3)	12 (41.4)
Minimally Improved	4 (23.5)	4 (33.3)	8 (27.6)
No Change	1 (5.9)	1 (8.3)	2 (6.9)
Minimally Worse	0	1 (8.3)	1 (3.4)
Much Worse	0	1 (8.3)	1 (3.4)
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.6.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgicsid-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-03-03-01a-cgicsid-ole.rtf

4.8.4.2 Veränderung der Aufmerksamkeit (CGICA) während der OLE

Marinus, Inc.
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Table 14.2.4.1.1A
Summary of Caregiver Global Impression of Change in Attention (CGICA)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
Total Observed, n	42	43	85
Very Much Improved	3 (7.1)	0	3 (3.5)
Much Improved	9 (21.4)	11 (25.6)	20 (23.5)
Minimally Improved	11 (26.2)	14 (32.6)	25 (29.4)
No Change	16 (38.1)	17 (39.5)	33 (38.8)
Minimally Worse	3 (7.1)	0	3 (3.5)
Much Worse	0	1 (2.3)	1 (1.2)
Very Much Worse	0	0	0
Visit 8 (Week 34)			
Total Observed, n	38	41	79
Very Much Improved	2 (5.3)	2 (4.9)	4 (5.1)
Much Improved	8 (21.1)	11 (26.8)	19 (24.1)
Minimally Improved	15 (39.5)	11 (26.8)	26 (32.9)
No Change	13 (34.2)	16 (39.0)	29 (36.7)
Minimally Worse	0	0	0
Much Worse	0	1 (2.4)	1 (1.3)
Very Much Worse	0	0	0
Visit 9 (Week 52)			
Total Observed, n	33	34	67
Very Much Improved	1 (3.0)	1 (2.9)	2 (3.0)
Much Improved	12 (36.4)	12 (35.3)	24 (35.8)
Minimally Improved	7 (21.2)	11 (32.4)	18 (26.9)
No Change	13 (39.4)	8 (23.5)	21 (31.3)
Minimally Worse	0	1 (2.9)	1 (1.5)
Much Worse	0	1 (2.9)	1 (1.5)
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgica-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-01-01a-cgica-ole.rtf

Marinus, Inc.
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Table 14.2.4.1.1A
Summary of Caregiver Global Impression of Change in Attention (CGICA)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
Total Observed, n	30	30	60
Very Much Improved	2 (6.7)	1 (3.3)	3 (5.0)
Much Improved	8 (26.7)	9 (30.0)	17 (28.3)
Minimally Improved	10 (33.3)	11 (36.7)	21 (35.0)
No Change	10 (33.3)	9 (30.0)	19 (31.7)
Minimally Worse	0	0	0
Much Worse	0	0	0
Very Much Worse	0	0	0
Visit 11 (Week 84)			
Total Observed, n	25	28	53
Very Much Improved	2 (8.0)	4 (14.3)	6 (11.3)
Much Improved	9 (36.0)	10 (35.7)	19 (35.8)
Minimally Improved	8 (32.0)	7 (25.0)	15 (28.3)
No Change	6 (24.0)	7 (25.0)	13 (24.5)
Minimally Worse	0	0	0
Much Worse	0	0	0
Very Much Worse	0	0	0
Visit 12 (Week 100)			
Total Observed, n	23	27	50
Very Much Improved	2 (8.7)	3 (11.1)	5 (10.0)
Much Improved	9 (39.1)	9 (33.3)	18 (36.0)
Minimally Improved	7 (30.4)	9 (33.3)	16 (32.0)
No Change	5 (21.7)	5 (18.5)	10 (20.0)
Minimally Worse	0	1 (3.7)	1 (2.0)
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgica-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-01-01a-cgica-ole.rtf

Marinus, Inc.
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Table 14.2.4.1.1A
Summary of Caregiver Global Impression of Change in Attention (CGICA)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
Total Observed, n	23	25	48
Very Much Improved	3 (13.0)	3 (12.0)	6 (12.5)
Much Improved	8 (34.8)	8 (32.0)	16 (33.3)
Minimally Improved	7 (30.4)	7 (28.0)	14 (29.2)
No Change	5 (21.7)	6 (24.0)	11 (22.9)
Minimally Worse	0	0	0
Much Worse	0	1 (4.0)	1 (2.1)
Very Much Worse	0	0	0
Visit 14 (Week 132)			
Total Observed, n	22	22	44
Very Much Improved	4 (18.2)	3 (13.6)	7 (15.9)
Much Improved	7 (31.8)	6 (27.3)	13 (29.5)
Minimally Improved	4 (18.2)	6 (27.3)	10 (22.7)
No Change	7 (31.8)	5 (22.7)	12 (27.3)
Minimally Worse	0	2 (9.1)	2 (4.5)
Much Worse	0	0	0
Very Much Worse	0	0	0
Visit 15 (Week 148)			
Total Observed, n	17	18	35
Very Much Improved	3 (17.6)	3 (16.7)	6 (17.1)
Much Improved	6 (35.3)	5 (27.8)	11 (31.4)
Minimally Improved	3 (17.6)	2 (11.1)	5 (14.3)
No Change	5 (29.4)	8 (44.4)	13 (37.1)
Minimally Worse	0	0	0
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgica-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-01-01a-cgica-ole.rtf

Table 14.2.4.1.1A
Summary of Caregiver Global Impression of Change in Attention (CGICA)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
Total Observed, n	11	9	20
Very Much Improved	4 (36.4)	1 (11.1)	5 (25.0)
Much Improved	3 (27.3)	0	3 (15.0)
Minimally Improved	1 (9.1)	5 (55.6)	6 (30.0)
No Change	3 (27.3)	3 (33.3)	6 (30.0)
Minimally Worse	0	0	0
Much Worse	0	0	0
Very Much Worse	0	0	0
Visit 17 (Week 180)			
Total Observed, n	2	1	3
Very Much Improved	1 (50.0)	0	1 (33.3)
Much Improved	1 (50.0)	1 (100)	2 (66.7)
Minimally Improved	0	0	0
No Change	0	0	0
Minimally Worse	0	0	0
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgica-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-01-01a-cgica-ole.rtf

Table 14.2.4.1.1A
Summary of Caregiver Global Impression of Change in Attention (CGICA)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Taper Visit [1]			
Total Observed, n	17	12	29
Very Much Improved	3 (17.6)	1 (8.3)	4 (13.8)
Much Improved	3 (17.6)	5 (41.7)	8 (27.6)
Minimally Improved	7 (41.2)	3 (25.0)	10 (34.5)
No Change	4 (23.5)	2 (16.7)	6 (20.7)
Minimally Worse	0	1 (8.3)	1 (3.4)
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgica-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-01-01a-cgica-ole.rtf

4.8.4.3 Veränderung des Verhaltens (CGI-C – Zielverhalten) während der OLE

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Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
Chosen Target Behavior:			
Total Observed, n	40	40	80
Sociabilty	11 (27.5)	11 (27.5)	22 (27.5)
Communication	16 (40.0)	12 (30.0)	28 (35.0)
Irritability	5 (12.5)	12 (30.0)	17 (21.3)
Hyperactivity	8 (20.0)	5 (12.5)	13 (16.3)
Score:			
Total Observed, n	42	43	85
Very Much Improved	3 (7.1)	0	3 (3.5)
Much Improved	5 (11.9)	8 (18.6)	13 (15.3)
Minimally Improved	9 (21.4)	16 (37.2)	25 (29.4)
No Change	23 (54.8)	16 (37.2)	39 (45.9)
Minimally Worse	2 (4.8)	2 (4.7)	4 (4.7)
Much Worse	0	1 (2.3)	1 (1.2)
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
Chosen Target Behavior:			
Total Observed, n	36	38	74
Sociabilty	13 (36.1)	10 (26.3)	23 (31.1)
Communication	13 (36.1)	12 (31.6)	25 (33.8)
Irritability	6 (16.7)	11 (28.9)	17 (23.0)
Hyperactivity	4 (11.1)	5 (13.2)	9 (12.2)
Score:			
Total Observed, n	38	41	79
Very Much Improved	2 (5.3)	3 (7.3)	5 (6.3)
Much Improved	7 (18.4)	10 (24.4)	17 (21.5)
Minimally Improved	12 (31.6)	12 (29.3)	24 (30.4)
No Change	17 (44.7)	13 (31.7)	30 (38.0)
Minimally Worse	0	3 (7.3)	3 (3.8)
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase Placebo (N=45)	 Ganaxolone (N=43)	 Total (N=88)
Visit 9 (Week 52)			
Chosen Target Behavior:			
Total Observed, n	31	29	60
Sociabilty	10 (32.3)	8 (27.6)	18 (30.0)
Communication	13 (41.9)	9 (31.0)	22 (36.7)
Irritability	4 (12.9)	8 (27.6)	12 (20.0)
Hyperactivity	4 (12.9)	4 (13.8)	8 (13.3)
Score:			
Total Observed, n	33	34	67
Very Much Improved	2 (6.1)	1 (2.9)	3 (4.5)
Much Improved	4 (12.1)	7 (20.6)	11 (16.4)
Minimally Improved	13 (39.4)	14 (41.2)	27 (40.3)
No Change	12 (36.4)	9 (26.5)	21 (31.3)
Minimally Worse	1 (3.0)	3 (8.8)	4 (6.0)
Much Worse	1 (3.0)	0	1 (1.5)
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
Chosen Target Behavior:			
Total Observed, n	28	30	58
Sociabilty	10 (35.7)	7 (23.3)	17 (29.3)
Communication	10 (35.7)	12 (40.0)	22 (37.9)
Irritability	3 (10.7)	6 (20.0)	9 (15.5)
Hyperactivity	5 (17.9)	5 (16.7)	10 (17.2)
Score:			
Total Observed, n	29	30	59
Very Much Improved	2 (6.9)	1 (3.3)	3 (5.1)
Much Improved	5 (17.2)	7 (23.3)	12 (20.3)
Minimally Improved	11 (37.9)	12 (40.0)	23 (39.0)
No Change	11 (37.9)	7 (23.3)	18 (30.5)
Minimally Worse	0	2 (6.7)	2 (3.4)
Much Worse	0	1 (3.3)	1 (1.7)
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
Chosen Target Behavior:			
Total Observed, n	24	27	51
Sociabilty	11 (45.8)	8 (29.6)	19 (37.3)
Communication	7 (29.2)	9 (33.3)	16 (31.4)
Irritability	2 (8.3)	5 (18.5)	7 (13.7)
Hyperactivity	4 (16.7)	5 (18.5)	9 (17.6)
Score:			
Total Observed, n	25	28	53
Very Much Improved	2 (8.0)	1 (3.6)	3 (5.7)
Much Improved	6 (24.0)	11 (39.3)	17 (32.1)
Minimally Improved	8 (32.0)	6 (21.4)	14 (26.4)
No Change	7 (28.0)	9 (32.1)	16 (30.2)
Minimally Worse	1 (4.0)	1 (3.6)	2 (3.8)
Much Worse	1 (4.0)	0	1 (1.9)
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
Chosen Target Behavior:			
Total Observed, n	22	26	48
Sociabilty	8 (36.4)	8 (30.8)	16 (33.3)
Communication	9 (40.9)	9 (34.6)	18 (37.5)
Irritability	2 (9.1)	4 (15.4)	6 (12.5)
Hyperactivity	3 (13.6)	5 (19.2)	8 (16.7)
Score:			
Total Observed, n	23	26	49
Very Much Improved	2 (8.7)	1 (3.8)	3 (6.1)
Much Improved	7 (30.4)	9 (34.6)	16 (32.7)
Minimally Improved	7 (30.4)	7 (26.9)	14 (28.6)
No Change	6 (26.1)	8 (30.8)	14 (28.6)
Minimally Worse	0	0	0
Much Worse	1 (4.3)	1 (3.8)	2 (4.1)
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase Placebo (N=45)	 Ganaxolone (N=43)	 Total (N=88)
Visit 13 (Week 116)			
Chosen Target Behavior:			
Total Observed, n	22	25	47
Sociabilty	7 (31.8)	7 (28.0)	14 (29.8)
Communication	10 (45.5)	8 (32.0)	18 (38.3)
Irritability	2 (9.1)	5 (20.0)	7 (14.9)
Hyperactivity	3 (13.6)	5 (20.0)	8 (17.0)
Score:			
Total Observed, n	23	25	48
Very Much Improved	3 (13.0)	1 (4.0)	4 (8.3)
Much Improved	5 (21.7)	6 (24.0)	11 (22.9)
Minimally Improved	6 (26.1)	7 (28.0)	13 (27.1)
No Change	7 (30.4)	8 (32.0)	15 (31.3)
Minimally Worse	2 (8.7)	1 (4.0)	3 (6.3)
Much Worse	0	2 (8.0)	2 (4.2)
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
Chosen Target Behavior:			
Total Observed, n	21	22	43
Sociabilty	6 (28.6)	6 (27.3)	12 (27.9)
Communication	10 (47.6)	8 (36.4)	18 (41.9)
Irritability	2 (9.5)	3 (13.6)	5 (11.6)
Hyperactivity	3 (14.3)	5 (22.7)	8 (18.6)
Score:			
Total Observed, n	22	22	44
Very Much Improved	4 (18.2)	1 (4.5)	5 (11.4)
Much Improved	5 (22.7)	8 (36.4)	13 (29.5)
Minimally Improved	6 (27.3)	3 (13.6)	9 (20.5)
No Change	6 (27.3)	9 (40.9)	15 (34.1)
Minimally Worse	0	1 (4.5)	1 (2.3)
Much Worse	1 (4.5)	0	1 (2.3)
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
Chosen Target Behavior:			
Total Observed, n	16	18	34
Sociabilty	5 (31.3)	7 (38.9)	12 (35.3)
Communication	8 (50.0)	4 (22.2)	12 (35.3)
Irritability	1 (6.3)	3 (16.7)	4 (11.8)
Hyperactivity	2 (12.5)	4 (22.2)	6 (17.6)
Score:			
Total Observed, n	17	18	35
Very Much Improved	3 (17.6)	1 (5.6)	4 (11.4)
Much Improved	5 (29.4)	4 (22.2)	9 (25.7)
Minimally Improved	4 (23.5)	2 (11.1)	6 (17.1)
No Change	5 (29.4)	10 (55.6)	15 (42.9)
Minimally Worse	0	1 (5.6)	1 (2.9)
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
Chosen Target Behavior:			
Total Observed, n	11	9	20
Sociabilty	2 (18.2)	4 (44.4)	6 (30.0)
Communication	8 (72.7)	2 (22.2)	10 (50.0)
Irritability	1 (9.1)	2 (22.2)	3 (15.0)
Hyperactivity	0	1 (11.1)	1 (5.0)
Score:			
Total Observed, n	11	9	20
Very Much Improved	4 (36.4)	1 (11.1)	5 (25.0)
Much Improved	2 (18.2)	1 (11.1)	3 (15.0)
Minimally Improved	2 (18.2)	3 (33.3)	5 (25.0)
No Change	3 (27.3)	4 (44.4)	7 (35.0)
Minimally Worse	0	0	0
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
Chosen Target Behavior:			
Total Observed, n	2	1	3
Sociabilty	0	0	0
Communication	2 (100)	1 (100)	3 (100)
Irritability	0	0	0
Hyperactivity	0	0	0
Score:			
Total Observed, n	2	1	3
Very Much Improved	1 (50.0)	0	1 (33.3)
Much Improved	1 (50.0)	1 (100)	2 (66.7)
Minimally Improved	0	0	0
No Change	0	0	0
Minimally Worse	0	0	0
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

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Table 14.2.4.2.1A
Summary of Caregiver Global Impression of Change in Target Behavior (CGI-C)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Treatment Group during Double-Blind Phase: Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [1]			
Chosen Target Behavior:			
Total Observed, n	17	11	28
Sociabilty	6 (35.3)	4 (36.4)	10 (35.7)
Communication	8 (47.1)	4 (36.4)	12 (42.9)
Irritability	1 (5.9)	2 (18.2)	3 (10.7)
Hyperactivity	2 (11.8)	1 (9.1)	3 (10.7)
Score:			
Total Observed, n	17	11	28
Very Much Improved	3 (17.6)	1 (9.1)	4 (14.3)
Much Improved	4 (23.5)	3 (27.3)	7 (25.0)
Minimally Improved	7 (41.2)	3 (27.3)	10 (35.7)
No Change	2 (11.8)	3 (27.3)	5 (17.9)
Minimally Worse	1 (5.9)	1 (9.1)	2 (7.1)
Much Worse	0	0	0
Very Much Worse	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgic-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-02-01a-cgic-ole.rtf

4.8.4.4 Globaler klinischer Eindruck der Verbesserung (CGI-I – Eltern/Betreuer) während der OLE

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Table 14.2.4.3.1A
Summary of Clinical Global Impression of Improvement (CGI-I)
(Open-Label Extension Phase, Intent-to-Treat Population)

	Parent/Caregiver			Clinician		
	Treatment Group during Double-Blind Phase			Treatment Group during Double-Blind Phase		
Visit Category	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 5 (Week 17)						
Total Observed, n	0	1	1	0	0	0
Very Much Improved	0	0	0	0	0	0
Much Improved	0	0	0	0	0	0
Minimally Improved	0	1 (100)	1 (100)	0	0	0
No Change	0	0	0	0	0	0
Minimally Worse	0	0	0	0	0	0
Much Worse	0	0	0	0	0	0
Very Much Worse	0	0	0	0	0	0
Visit 6 (Week 19)						
Total Observed, n	44	39	83	44	40	84
Very Much Improved	5 (11.4)	1 (2.6)	6 (7.2)	2 (4.5)	0	2 (2.4)
Much Improved	5 (11.4)	6 (15.4)	11 (13.3)	11 (25.0)	8 (20.0)	19 (22.6)
Minimally Improved	21 (47.7)	14 (35.9)	35 (42.2)	16 (36.4)	13 (32.5)	29 (34.5)
No Change	11 (25.0)	16 (41.0)	27 (32.5)	12 (27.3)	18 (45.0)	30 (35.7)
Minimally Worse	2 (4.5)	1 (2.6)	3 (3.6)	3 (6.8)	0	3 (3.6)
Much Worse	0	1 (2.6)	1 (1.2)	0	1 (2.5)	1 (1.2)
Very Much Worse	0	0	0	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgii-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-03-01a-cgii-ole.rtf

Marinus, Inc.
Study No. 1042-CDD-3001

Table 14.2.4.3.1A
Summary of Clinical Global Impression of Improvement (CGI-I)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Parent/Caregiver: Treatment Group during Double-Blind Phase: Placebo (N=45)	Parent/Caregiver: Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Parent/Caregiver: Total (N=88)	Clinician: Treatment Group during Double-Blind Phase: Placebo (N=45)	Clinician: Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Clinician: Total (N=88)
Visit 7 (Week 21)						
Total Observed, n	39	43	82	42	41	83
Very Much Improved	4 (10.3)	0	4 (4.9)	2 (4.8)	0	2 (2.4)
Much Improved	6 (15.4)	11 (25.6)	17 (20.7)	9 (21.4)	11 (26.8)	20 (24.1)
Minimally Improved	16 (41.0)	19 (44.2)	35 (42.7)	16 (38.1)	16 (39.0)	32 (38.6)
No Change	11 (28.2)	10 (23.3)	21 (25.6)	13 (31.0)	13 (31.7)	26 (31.3)
Minimally Worse	2 (5.1)	2 (4.7)	4 (4.9)	1 (2.4)	1 (2.4)	2 (2.4)
Much Worse	0	1 (2.3)	1 (1.2)	1 (2.4)	0	1 (1.2)
Very Much Worse	0	0	0	0	0	0
Visit 8 (Week 34)						
Total Observed, n	38	39	77	38	41	79
Very Much Improved	4 (10.5)	2 (5.1)	6 (7.8)	1 (2.6)	0	1 (1.3)
Much Improved	13 (34.2)	10 (25.6)	23 (29.9)	10 (26.3)	11 (26.8)	21 (26.6)
Minimally Improved	14 (36.8)	14 (35.9)	28 (36.4)	19 (50.0)	14 (34.1)	33 (41.8)
No Change	7 (18.4)	11 (28.2)	18 (23.4)	7 (18.4)	14 (34.1)	21 (26.6)
Minimally Worse	0	1 (2.6)	1 (1.3)	1 (2.6)	1 (2.4)	2 (2.5)
Much Worse	0	1 (2.6)	1 (1.3)	0	1 (2.4)	1 (1.3)
Very Much Worse	0	0	0	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgii-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-03-01a-cgii-ole.rtf

Table 14.2.4.3.1A
Summary of Clinical Global Impression of Improvement (CGI-I)
(Open-Label Extension Phase, Intent-to-Treat Population)

	Parent/Caregiver			Clinician		
	Treatment Group during Double-Blind Phase			Treatment Group during Double-Blind Phase		
Visit Category	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)						
Total Observed, n	32	32	64	34	33	67
Very Much Improved	2 (6.3)	2 (6.3)	4 (6.3)	2 (5.9)	1 (3.0)	3 (4.5)
Much Improved	11 (34.4)	9 (28.1)	20 (31.3)	11 (32.4)	9 (27.3)	20 (29.9)
Minimally Improved	9 (28.1)	10 (31.3)	19 (29.7)	12 (35.3)	11 (33.3)	23 (34.3)
No Change	7 (21.9)	9 (28.1)	16 (25.0)	8 (23.5)	9 (27.3)	17 (25.4)
Minimally Worse	2 (6.3)	2 (6.3)	4 (6.3)	1 (2.9)	3 (9.1)	4 (6.0)
Much Worse	1 (3.1)	0	1 (1.6)	0	0	0
Very Much Worse	0	0	0	0	0	0
Visit 10 (Week 68)						
Total Observed, n	29	29	58	31	30	61
Very Much Improved	3 (10.3)	0	3 (5.2)	1 (3.2)	0	1 (1.6)
Much Improved	7 (24.1)	12 (41.4)	19 (32.8)	11 (35.5)	9 (30.0)	20 (32.8)
Minimally Improved	11 (37.9)	9 (31.0)	20 (34.5)	8 (25.8)	8 (26.7)	16 (26.2)
No Change	7 (24.1)	4 (13.8)	11 (19.0)	8 (25.8)	8 (26.7)	16 (26.2)
Minimally Worse	1 (3.4)	4 (13.8)	5 (8.6)	3 (9.7)	4 (13.3)	7 (11.5)
Much Worse	0	0	0	0	1 (3.3)	1 (1.6)
Very Much Worse	0	0	0	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgii-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-03-01a-cgii-ole.rtf

Table 14.2.4.3.1A
Summary of Clinical Global Impression of Improvement (CGI-I)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Parent/Caregiver			Clinician		
	Treatment Group during Double-Blind Phase			Treatment Group during Double-Blind Phase		
	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)						
Total Observed, n	25	26	51	26	27	53
Very Much Improved	3 (12.0)	2 (7.7)	5 (9.8)	2 (7.7)	0	2 (3.8)
Much Improved	7 (28.0)	9 (34.6)	16 (31.4)	9 (34.6)	7 (25.9)	16 (30.2)
Minimally Improved	13 (52.0)	7 (26.9)	20 (39.2)	8 (30.8)	10 (37.0)	18 (34.0)
No Change	2 (8.0)	6 (23.1)	8 (15.7)	6 (23.1)	9 (33.3)	15 (28.3)
Minimally Worse	0	2 (7.7)	2 (3.9)	1 (3.8)	1 (3.7)	2 (3.8)
Much Worse	0	0	0	0	0	0
Very Much Worse	0	0	0	0	0	0
Visit 12 (Week 100)						
Total Observed, n	23	26	49	24	26	50
Very Much Improved	3 (13.0)	1 (3.8)	4 (8.2)	2 (8.3)	1 (3.8)	3 (6.0)
Much Improved	8 (34.8)	7 (26.9)	15 (30.6)	8 (33.3)	4 (15.4)	12 (24.0)
Minimally Improved	8 (34.8)	13 (50.0)	21 (42.9)	8 (33.3)	8 (30.8)	16 (32.0)
No Change	4 (17.4)	4 (15.4)	8 (16.3)	6 (25.0)	11 (42.3)	17 (34.0)
Minimally Worse	0	1 (3.8)	1 (2.0)	0	2 (7.7)	2 (4.0)
Much Worse	0	0	0	0	0	0
Very Much Worse	0	0	0	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgii-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-03-01a-cgii-ole.rtf

Marinus, Inc.
Study No. 1042-CDD-3001

01SEP2023 04:21

Table 14.2.4.3.1A
Summary of Clinical Global Impression of Improvement (CGI-I)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Parent/Caregiver: Treatment Group during Double-Blind Phase: Placebo (N=45)	Parent/Caregiver: Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Parent/Caregiver: Total (N=88)	Clinician: Treatment Group during Double-Blind Phase: Placebo (N=45)	Clinician: Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Clinician: Total (N=88)
Visit 13 (Week 116)						
Total Observed, n	23	25	48	23	26	49
Very Much Improved	5 (21.7)	3 (12.0)	8 (16.7)	2 (8.7)	1 (3.8)	3 (6.1)
Much Improved	8 (34.8)	7 (28.0)	15 (31.3)	7 (30.4)	5 (19.2)	12 (24.5)
Minimally Improved	5 (21.7)	7 (28.0)	12 (25.0)	8 (34.8)	10 (38.5)	18 (36.7)
No Change	5 (21.7)	5 (20.0)	10 (20.8)	6 (26.1)	8 (30.8)	14 (28.6)
Minimally Worse	0	3 (12.0)	3 (6.3)	0	2 (7.7)	2 (4.1)
Much Worse	0	0	0	0	0	0
Very Much Worse	0	0	0	0	0	0
Visit 14 (Week 132)						
Total Observed, n	22	23	45	22	23	45
Very Much Improved	6 (27.3)	3 (13.0)	9 (20.0)	2 (9.1)	2 (8.7)	4 (8.9)
Much Improved	8 (36.4)	9 (39.1)	17 (37.8)	6 (27.3)	5 (21.7)	11 (24.4)
Minimally Improved	4 (18.2)	7 (30.4)	11 (24.4)	6 (27.3)	9 (39.1)	15 (33.3)
No Change	3 (13.6)	3 (13.0)	6 (13.3)	7 (31.8)	5 (21.7)	12 (26.7)
Minimally Worse	1 (4.5)	1 (4.3)	2 (4.4)	1 (4.5)	2 (8.7)	3 (6.7)
Much Worse	0	0	0	0	0	0
Very Much Worse	0	0	0	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgii-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-03-01a-cgii-ole.rtf

Table 14.2.4.3.1A
Summary of Clinical Global Impression of Improvement (CGI-I)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Parent/Caregiver			Clinician		
	Treatment Group during Double-Blind Phase			Treatment Group during Double-Blind Phase		
	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)						
Total Observed, n	17	18	35	17	18	35
Very Much Improved	3 (17.6)	1 (5.6)	4 (11.4)	1 (5.9)	1 (5.6)	2 (5.7)
Much Improved	8 (47.1)	8 (44.4)	16 (45.7)	8 (47.1)	6 (33.3)	14 (40.0)
Minimally Improved	3 (17.6)	4 (22.2)	7 (20.0)	3 (17.6)	5 (27.8)	8 (22.9)
No Change	3 (17.6)	4 (22.2)	7 (20.0)	4 (23.5)	6 (33.3)	10 (28.6)
Minimally Worse	0	1 (5.6)	1 (2.9)	1 (5.9)	0	1 (2.9)
Much Worse	0	0	0	0	0	0
Very Much Worse	0	0	0	0	0	0
Taper Visit [1]						
Total Observed, n	15	12	27	17	12	29
Very Much Improved	3 (20.0)	0	3 (11.1)	1 (5.9)	0	1 (3.4)
Much Improved	4 (26.7)	4 (33.3)	8 (29.6)	8 (47.1)	3 (25.0)	11 (37.9)
Minimally Improved	4 (26.7)	5 (41.7)	9 (33.3)	6 (35.3)	5 (41.7)	11 (37.9)
No Change	3 (20.0)	2 (16.7)	5 (18.5)	2 (11.8)	3 (25.0)	5 (17.2)
Minimally Worse	1 (6.7)	1 (8.3)	2 (7.4)	0	1 (8.3)	1 (3.4)
Much Worse	0	0	0	0	0	0
Very Much Worse	0	0	0	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgii-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-03-01a-cgii-ole.rtf

Marinus, Inc.
Study No. 1042-CDD-3001

Table 14.2.4.3.1A
Summary of Clinical Global Impression of Improvement (CGI-I)
(Open-Label Extension Phase, Intent-to-Treat Population)

Visit Category	Parent/Caregiver: Treatment Group during Double-Blind Phase: Placebo (N=45)	Parent/Caregiver: Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Parent/Caregiver: Total (N=88)	Clinician: Treatment Group during Double-Blind Phase: Placebo (N=45)	Clinician: Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Clinician: Total (N=88)
Visit 16 (Week 164)						
Total Observed, n	11	9	20	11	9	20
Very Much Improved	5 (45.5)	0	5 (25.0)	0	0	0
Much Improved	4 (36.4)	4 (44.4)	8 (40.0)	8 (72.7)	3 (33.3)	11 (55.0)
Minimally Improved	1 (9.1)	4 (44.4)	5 (25.0)	1 (9.1)	4 (44.4)	5 (25.0)
No Change	1 (9.1)	1 (11.1)	2 (10.0)	1 (9.1)	1 (11.1)	2 (10.0)
Minimally Worse	0	0	0	1 (9.1)	1 (11.1)	2 (10.0)
Much Worse	0	0	0	0	0	0
Very Much Worse	0	0	0	0	0	0
Visit 17 (Week 180)						
Total Observed, n	2	1	3	2	1	3
Very Much Improved	1 (50.0)	0	1 (33.3)	0	0	0
Much Improved	1 (50.0)	1 (100)	2 (66.7)	1 (50.0)	0	1 (33.3)
Minimally Improved	0	0	0	0	1 (100)	1 (33.3)
No Change	0	0	0	1 (50.0)	0	1 (33.3)
Minimally Worse	0	0	0	0	0	0
Much Worse	0	0	0	0	0	0
Very Much Worse	0	0	0	0	0	0

Note: Percentages are based on the number observed.
[1]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.7.3A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cgii-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-04-03-01a-cgii-ole.rtf

4.8.5 Ergebnisse zum Endpunkt Morbidität – Veränderung des CSHQ gegenüber Baseline während der OLE

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	45	41	86
Mean (SD)	8.6 (2.76)	8.3 (2.55)	8.5 (2.65)
95% Confidence Interval of Mean	7.7, 9.4	7.5, 9.1	7.9, 9.0
Median	8.0	8.0	8.0
Q1, Q3	6.0, 10.0	6.0, 10.0	6.0, 10.0
Min, Max	6, 15	6, 15	6, 15

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	40	40	80
Mean (SD)	8.1 (2.45)	7.9 (2.38)	8.0 (2.41)
95% Confidence Interval of Mean	7.3, 8.9	7.1, 8.7	7.5, 8.5
Median	7.5	7.0	7.0
Q1, Q3	6.0, 9.5	6.0, 9.5	6.0, 9.5
Min, Max	6, 15	6, 14	6, 15
Change from Baseline			
n	40	39	79
Mean (SD)	-0.6 (1.68)	-0.6 (1.71)	-0.6 (1.68)
95% Confidence Interval of Mean	-1.1, -0.1	-1.1, -0.0	-1.0, -0.2
Median	0.0	0.0	0.0
Q1, Q3	-2.0, 0.0	-1.0, 0.0	-2.0, 0.0
Min, Max	-4, 4	-5, 5	-5, 5

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	35	39	74
Mean (SD)	7.5 (2.29)	7.7 (2.16)	7.6 (2.21)
95% Confidence Interval of Mean	6.7, 8.2	7.0, 8.4	7.1, 8.1
Median	6.0	7.0	7.0
Q1, Q3	6.0, 8.0	6.0, 9.0	6.0, 8.0
Min, Max	6, 14	6, 13	6, 14
Change from Baseline			
n	35	38	73
Mean (SD)	-1.2 (1.73)	-0.7 (1.83)	-1.0 (1.78)
95% Confidence Interval of Mean	-1.8, -0.6	-1.3, -0.1	-1.4, -0.5
Median	-1.0	-1.0	-1.0
Q1, Q3	-3.0, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-5, 2	-5, 6	-5, 6

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	31	64
Mean (SD)	8.0 (2.46)	7.5 (2.45)	7.8 (2.45)
95% Confidence Interval of Mean	7.1, 8.9	6.7, 8.4	7.2, 8.4
Median	8.0	6.0	7.0
Q1, Q3	6.0, 8.0	6.0, 8.0	6.0, 8.0
Min, Max	6, 15	6, 15	6, 15
Change from Baseline			
n	33	31	64
Mean (SD)	-0.7 (2.00)	-0.9 (1.73)	-0.8 (1.86)
95% Confidence Interval of Mean	-1.4, -0.0	-1.6, -0.3	-1.3, -0.4
Median	0.0	-1.0	0.0
Q1, Q3	-2.0, 0.0	-1.0, 0.0	-2.0, 0.0
Min, Max	-5, 3	-5, 2	-5, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	30	60
Mean (SD)	7.9 (2.47)	7.9 (2.32)	7.9 (2.38)
95% Confidence Interval of Mean	6.9, 8.8	7.1, 8.8	7.3, 8.5
Median	7.0	7.0	7.0
Q1, Q3	6.0, 9.0	6.0, 9.0	6.0, 9.0
Min, Max	6, 14	6, 13	6, 14
Change from Baseline			
n	30	29	59
Mean (SD)	-0.5 (1.33)	-0.6 (1.50)	-0.5 (1.41)
95% Confidence Interval of Mean	-1.0, -0.0	-1.1, 0.0	-0.9, -0.2
Median	0.0	0.0	0.0
Q1, Q3	-2.0, 0.0	-1.0, 0.0	-2.0, 0.0
Min, Max	-3, 2	-4, 2	-4, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	28	53
Mean (SD)	8.3 (2.76)	7.9 (2.18)	8.1 (2.45)
95% Confidence Interval of Mean	7.2, 9.5	7.1, 8.8	7.4, 8.8
Median	8.0	7.5	8.0
Q1, Q3	6.0, 9.0	6.0, 9.0	6.0, 9.0
Min, Max	6, 14	6, 13	6, 14
Change from Baseline			
n	25	27	52
Mean (SD)	-0.6 (1.69)	-0.6 (1.91)	-0.6 (1.79)
95% Confidence Interval of Mean	-1.3, 0.1	-1.3, 0.2	-1.1, -0.1
Median	0.0	-1.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-4, 4	-4, 5	-4, 5

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	26	49
Mean (SD)	7.5 (2.21)	7.5 (1.94)	7.5 (2.05)
95% Confidence Interval of Mean	6.6, 8.5	6.7, 8.2	6.9, 8.1
Median	7.0	7.0	7.0
Q1, Q3	6.0, 8.0	6.0, 8.0	6.0, 8.0
Min, Max	6, 14	6, 12	6, 14
Change from Baseline			
n	23	25	48
Mean (SD)	-1.3 (2.10)	-1.2 (1.68)	-1.3 (1.87)
95% Confidence Interval of Mean	-2.2, -0.4	-1.9, -0.5	-1.8, -0.7
Median	0.0	-1.0	-1.0
Q1, Q3	-2.0, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-7, 1	-5, 2	-7, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	22	26	48
Mean (SD)	7.0 (1.84)	7.6 (1.98)	7.3 (1.92)
95% Confidence Interval of Mean	6.2, 7.9	6.8, 8.4	6.8, 7.9
Median	6.0	6.5	6.0
Q1, Q3	6.0, 7.0	6.0, 9.0	6.0, 8.0
Min, Max	6, 13	6, 12	6, 13
Change from Baseline			
n	22	25	47
Mean (SD)	-1.4 (2.04)	-1.2 (1.78)	-1.3 (1.89)
95% Confidence Interval of Mean	-2.3, -0.5	-1.9, -0.5	-1.9, -0.7
Median	0.0	-1.0	0.0
Q1, Q3	-3.0, 0.0	-2.0, 0.0	-3.0, 0.0
Min, Max	-7, 0	-5, 2	-7, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	21	22	43
Mean (SD)	7.3 (2.03)	7.7 (1.96)	7.5 (1.98)
95% Confidence Interval of Mean	6.4, 8.2	6.8, 8.6	6.9, 8.1
Median	6.0	7.0	7.0
Q1, Q3	6.0, 8.0	6.0, 9.0	6.0, 8.0
Min, Max	6, 14	6, 12	6, 14
Change from Baseline			
n	21	22	43
Mean (SD)	-1.0 (2.01)	-1.0 (2.12)	-1.0 (2.04)
95% Confidence Interval of Mean	-2.0, -0.1	-1.9, -0.1	-1.7, -0.4
Median	0.0	-1.0	-1.0
Q1, Q3	-2.0, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-7, 1	-5, 5	-7, 5

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	7.6 (2.44)	7.6 (1.96)	7.6 (2.17)
95% Confidence Interval of Mean	6.2, 9.0	6.6, 8.7	6.8, 8.4
Median	6.0	7.0	6.0
Q1, Q3	6.0, 9.0	6.0, 8.5	6.0, 9.0
Min, Max	6, 14	6, 12	6, 14
Change from Baseline			
n	15	16	31
Mean (SD)	-1.2 (2.70)	-1.1 (2.35)	-1.1 (2.49)
95% Confidence Interval of Mean	-2.7, 0.3	-2.3, 0.2	-2.0, -0.2
Median	0.0	-0.5	0.0
Q1, Q3	-3.0, 0.0	-2.0, 0.0	-3.0, 0.0
Min, Max	-7, 3	-6, 3	-7, 3

Abbreviation: N/A=Not applicable.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	7.5 (2.81)	8.9 (2.15)	8.2 (2.56)
95% Confidence Interval of Mean	5.7, 9.4	7.2, 10.5	7.0, 9.3
Median	6.0	9.0	7.5
Q1, Q3	6.0, 8.0	7.0, 10.0	6.0, 10.0
Min, Max	6, 15	6, 12	6, 15
Change from Baseline			
n	11	9	20
Mean (SD)	-1.5 (2.54)	-0.4 (2.35)	-1.0 (2.45)
95% Confidence Interval of Mean	-3.2, 0.3	-2.3, 1.4	-2.1, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-3.0, 0.0	-3.0, 1.0	-3.0, 0.5
Min, Max	-7, 1	-4, 3	-7, 3

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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	10.0 (1.41)	8.0 (N/A)	9.3 (1.53)
95% Confidence Interval of Mean	-2.7, 22.7	N/A, N/A	5.5, 13.1
Median	10.0	8.0	9.0
Q1, Q3	9.0, 11.0	8.0, 8.0	8.0, 11.0
Min, Max	9, 11	8, 8	8, 11
Change from Baseline			
n	2	1	3
Mean (SD)	-3.0 (1.41)	-5.0 (N/A)	-3.7 (1.53)
95% Confidence Interval of Mean	-15.7, 9.7	N/A, N/A	-7.5, 0.1
Median	-3.0	-5.0	-4.0
Q1, Q3	-4.0, -2.0	-5.0, -5.0	-5.0, -2.0
Min, Max	-4, -2	-5, -5	-5, -2

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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Bedtime Resistance (Questions 1, 3, 4, 5, 6, 8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	7.4 (2.29)	8.1 (2.81)	7.7 (2.50)
95% Confidence Interval of Mean	6.2, 8.5	6.3, 9.9	6.7, 8.6
Median	6.0	7.5	7.0
Q1, Q3	6.0, 8.0	6.0, 8.5	6.0, 8.0
Min, Max	6, 14	6, 16	6, 16
Change from Baseline			
n	17	11	28
Mean (SD)	-1.4 (2.43)	-0.7 (3.07)	-1.1 (2.66)
95% Confidence Interval of Mean	-2.7, -0.2	-2.8, 1.3	-2.2, -0.1
Median	0.0	-1.0	-1.0
Q1, Q3	-3.0, 0.0	-2.0, 0.0	-2.5, 0.0
Min, Max	-6, 3	-5, 7	-6, 7

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	45	43	88
Mean (SD)	1.9 (0.75)	1.8 (0.80)	1.8 (0.77)
95% Confidence Interval of Mean	1.7, 2.1	1.5, 2.0	1.7, 2.0
Median	2.0	2.0	2.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	41	42	83
Mean (SD)	1.7 (0.79)	1.5 (0.59)	1.6 (0.70)
95% Confidence Interval of Mean	1.4, 1.9	1.3, 1.7	1.4, 1.7
Median	1.0	1.0	1.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	41	42	83
Mean (SD)	-0.2 (0.65)	-0.3 (0.84)	-0.3 (0.75)
95% Confidence Interval of Mean	-0.4, -0.0	-0.6, -0.0	-0.4, -0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-2, 1	-2, 1	-2, 1

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	37	40	77
Mean (SD)	1.6 (0.72)	1.6 (0.68)	1.6 (0.69)
95% Confidence Interval of Mean	1.4, 1.9	1.4, 1.8	1.4, 1.8
Median	1.0	1.0	1.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	37	40	77
Mean (SD)	-0.2 (0.46)	-0.2 (0.92)	-0.2 (0.73)
95% Confidence Interval of Mean	-0.3, -0.0	-0.5, 0.1	-0.4, -0.0
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-1, 1	-2, 2	-2, 2

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	33	66
Mean (SD)	1.6 (0.75)	1.6 (0.75)	1.6 (0.74)
95% Confidence Interval of Mean	1.3, 1.9	1.3, 1.8	1.4, 1.8
Median	1.0	1.0	1.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	33	33	66
Mean (SD)	-0.2 (0.68)	-0.2 (0.98)	-0.2 (0.84)
95% Confidence Interval of Mean	-0.4, 0.1	-0.5, 0.2	-0.4, 0.0
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-1, 2	-2, 2	-2, 2

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	30	60
Mean (SD)	1.6 (0.73)	1.6 (0.72)	1.6 (0.72)
95% Confidence Interval of Mean	1.3, 1.8	1.3, 1.9	1.4, 1.8
Median	1.0	1.0	1.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	30	30	60
Mean (SD)	-0.2 (0.50)	-0.2 (0.91)	-0.2 (0.73)
95% Confidence Interval of Mean	-0.4, -0.0	-0.5, 0.2	-0.4, -0.0
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-1, 1	-2, 2	-2, 2

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	28	53
Mean (SD)	1.7 (0.75)	1.7 (0.67)	1.7 (0.70)
95% Confidence Interval of Mean	1.4, 2.0	1.4, 1.9	1.5, 1.9
Median	2.0	2.0	2.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	25	28	53
Mean (SD)	-0.1 (0.53)	-0.1 (0.92)	-0.1 (0.75)
95% Confidence Interval of Mean	-0.3, 0.1	-0.5, 0.2	-0.3, 0.1
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-1, 1	-2, 2	-2, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	27	50
Mean (SD)	1.6 (0.73)	1.6 (0.75)	1.6 (0.73)
95% Confidence Interval of Mean	1.3, 1.9	1.3, 1.9	1.4, 1.8
Median	1.0	1.0	1.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	23	27	50
Mean (SD)	-0.3 (0.57)	-0.1 (0.82)	-0.2 (0.72)
95% Confidence Interval of Mean	-0.6, -0.1	-0.5, 0.2	-0.4, -0.0
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-1, 1	-2, 2	-2, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	22	26	48
Mean (SD)	1.6 (0.73)	1.7 (0.69)	1.6 (0.70)
95% Confidence Interval of Mean	1.3, 2.0	1.4, 1.9	1.4, 1.8
Median	1.5	2.0	2.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	22	26	48
Mean (SD)	-0.2 (0.61)	-0.2 (1.10)	-0.2 (0.90)
95% Confidence Interval of Mean	-0.5, 0.0	-0.6, 0.3	-0.5, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-1, 1	-2, 2	-2, 2

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Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	23	45
Mean (SD)	1.6 (0.79)	1.7 (0.57)	1.6 (0.68)
95% Confidence Interval of Mean	1.3, 2.0	1.4, 1.9	1.4, 1.8
Median	1.0	2.0	2.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	22	23	45
Mean (SD)	-0.3 (0.63)	-0.3 (0.81)	-0.3 (0.72)
95% Confidence Interval of Mean	-0.6, 0.0	-0.6, 0.1	-0.5, -0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-1, 1	-2, 1	-2, 1

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	1.4 (0.74)	1.7 (0.79)	1.5 (0.77)
95% Confidence Interval of Mean	1.0, 1.8	1.3, 2.1	1.3, 1.8
Median	1.0	1.5	1.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	15	16	31
Mean (SD)	-0.3 (0.62)	-0.3 (1.06)	-0.3 (0.86)
95% Confidence Interval of Mean	-0.7, 0.0	-0.8, 0.3	-0.6, 0.0
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-1, 1	-2, 2	-2, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	1.4 (0.67)	1.9 (0.78)	1.6 (0.75)
95% Confidence Interval of Mean	0.9, 1.8	1.3, 2.5	1.2, 2.0
Median	1.0	2.0	1.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	11	9	20
Mean (SD)	-0.4 (0.50)	0.0 (1.00)	-0.2 (0.77)
95% Confidence Interval of Mean	-0.7, -0.0	-0.8, 0.8	-0.6, 0.2
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-1, 0	-1, 2	-1, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	3.0 (0.00)	2.0 (N/A)	2.7 (0.58)
95% Confidence Interval of Mean	3.0, 3.0	N/A, N/A	1.2, 4.1
Median	3.0	2.0	3.0
Q1, Q3	3.0, 3.0	2.0, 2.0	2.0, 3.0
Min, Max	3, 3	2, 2	2, 3
Change from Baseline			
n	2	1	3
Mean (SD)	0.5 (0.71)	-1.0 (N/A)	0.0 (1.00)
95% Confidence Interval of Mean	-5.9, 6.9	N/A, N/A	-2.5, 2.5
Median	0.5	-1.0	0.0
Q1, Q3	0.0, 1.0	-1.0, -1.0	-1.0, 1.0
Min, Max	0, 1	-1, -1	-1, 1

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Onset Delay (Question 2)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	1.5 (0.80)	1.7 (0.65)	1.6 (0.74)
95% Confidence Interval of Mean	1.1, 1.9	1.3, 2.1	1.3, 1.8
Median	1.0	2.0	1.0
Q1, Q3	1.0, 2.0	1.0, 2.0	1.0, 2.0
Min, Max	1, 3	1, 3	1, 3
Change from Baseline			
n	17	12	29
Mean (SD)	-0.3 (0.69)	-0.1 (0.67)	-0.2 (0.68)
95% Confidence Interval of Mean	-0.6, 0.1	-0.5, 0.3	-0.5, 0.0
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-0.5, 0.0	-1.0, 0.0
Min, Max	-1, 1	-1, 1	-1, 1

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	45	42	87
Mean (SD)	4.6 (1.83)	4.6 (1.80)	4.6 (1.80)
95% Confidence Interval of Mean	4.1, 5.1	4.0, 5.1	4.2, 5.0
Median	4.0	4.0	4.0
Q1, Q3	3.0, 6.0	3.0, 6.0	3.0, 6.0
Min, Max	3, 9	3, 8	3, 9

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	41	42	83
Mean (SD)	4.5 (1.72)	4.3 (1.44)	4.4 (1.58)
95% Confidence Interval of Mean	4.0, 5.1	3.9, 4.8	4.1, 4.8
Median	4.0	4.0	4.0
Q1, Q3	3.0, 6.0	3.0, 5.0	3.0, 5.0
Min, Max	3, 9	3, 8	3, 9
Change from Baseline			
n	41	41	82
Mean (SD)	-0.1 (1.59)	-0.3 (1.85)	-0.2 (1.72)
95% Confidence Interval of Mean	-0.6, 0.4	-0.9, 0.2	-0.6, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-2.0, 1.0	-1.0, 1.0
Min, Max	-5, 4	-5, 4	-5, 4

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	37	39	76
Mean (SD)	4.6 (1.72)	4.6 (1.68)	4.6 (1.69)
95% Confidence Interval of Mean	4.0, 5.2	4.1, 5.2	4.2, 5.0
Median	4.0	4.0	4.0
Q1, Q3	3.0, 6.0	3.0, 6.0	3.0, 6.0
Min, Max	3, 9	3, 8	3, 9
Change from Baseline			
n	37	38	75
Mean (SD)	0.1 (1.35)	0.1 (1.89)	0.1 (1.63)
95% Confidence Interval of Mean	-0.4, 0.5	-0.6, 0.7	-0.3, 0.4
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 1.0	-1.0, 1.0	-1.0, 1.0
Min, Max	-3, 3	-4, 5	-4, 5

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	33	66
Mean (SD)	4.5 (1.46)	4.3 (1.55)	4.4 (1.50)
95% Confidence Interval of Mean	4.0, 5.1	3.8, 4.9	4.1, 4.8
Median	5.0	4.0	4.0
Q1, Q3	3.0, 6.0	3.0, 6.0	3.0, 6.0
Min, Max	3, 8	3, 7	3, 8
Change from Baseline			
n	33	33	66
Mean (SD)	-0.1 (1.68)	-0.1 (1.70)	-0.1 (1.68)
95% Confidence Interval of Mean	-0.7, 0.5	-0.7, 0.5	-0.5, 0.3
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 1.0	-1.0, 0.0	-1.0, 1.0
Min, Max	-4, 4	-4, 4	-4, 4

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	30	60
Mean (SD)	4.6 (1.83)	4.2 (1.39)	4.4 (1.63)
95% Confidence Interval of Mean	3.9, 5.3	3.6, 4.7	3.9, 4.8
Median	4.0	4.0	4.0
Q1, Q3	3.0, 6.0	3.0, 5.0	3.0, 5.5
Min, Max	3, 9	3, 7	3, 9
Change from Baseline			
n	30	30	60
Mean (SD)	0.1 (1.26)	-0.3 (1.51)	-0.1 (1.39)
95% Confidence Interval of Mean	-0.4, 0.5	-0.8, 0.3	-0.5, 0.3
Median	0.0	0.0	0.0
Q1, Q3	0.0, 1.0	-1.0, 1.0	-1.0, 1.0
Min, Max	-3, 3	-4, 3	-4, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	28	53
Mean (SD)	4.8 (1.72)	4.3 (1.47)	4.6 (1.60)
95% Confidence Interval of Mean	4.1, 5.6	3.8, 4.9	4.1, 5.0
Median	4.0	4.0	4.0
Q1, Q3	3.0, 6.0	3.0, 5.0	3.0, 6.0
Min, Max	3, 8	3, 7	3, 8
Change from Baseline			
n	25	28	53
Mean (SD)	0.2 (1.18)	-0.1 (1.51)	0.0 (1.36)
95% Confidence Interval of Mean	-0.3, 0.6	-0.7, 0.4	-0.4, 0.4
Median	0.0	0.0	0.0
Q1, Q3	0.0, 1.0	-1.0, 0.5	-1.0, 1.0
Min, Max	-2, 3	-4, 3	-4, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	27	50
Mean (SD)	4.6 (1.53)	3.9 (1.27)	4.2 (1.42)
95% Confidence Interval of Mean	3.9, 5.3	3.4, 4.4	3.8, 4.6
Median	4.0	3.0	4.0
Q1, Q3	3.0, 6.0	3.0, 5.0	3.0, 6.0
Min, Max	3, 8	3, 6	3, 8
Change from Baseline			
n	23	27	50
Mean (SD)	-0.2 (1.38)	-0.4 (1.50)	-0.3 (1.43)
95% Confidence Interval of Mean	-0.8, 0.4	-1.0, 0.2	-0.7, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-3, 2	-4, 3	-4, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	22	26	48
Mean (SD)	4.5 (1.44)	4.4 (1.72)	4.4 (1.58)
95% Confidence Interval of Mean	3.8, 5.1	3.7, 5.1	4.0, 4.9
Median	4.5	3.0	4.0
Q1, Q3	3.0, 6.0	3.0, 6.0	3.0, 6.0
Min, Max	3, 7	3, 7	3, 7
Change from Baseline			
n	22	26	48
Mean (SD)	-0.1 (1.57)	-0.2 (1.83)	-0.1 (1.70)
95% Confidence Interval of Mean	-0.8, 0.6	-0.9, 0.6	-0.6, 0.4
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 1.0	-1.0, 0.0	-1.0, 0.5
Min, Max	-5, 2	-4, 4	-5, 4

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	21	23	44
Mean (SD)	4.3 (1.23)	4.3 (1.64)	4.3 (1.44)
95% Confidence Interval of Mean	3.7, 4.8	3.6, 5.0	3.9, 4.7
Median	4.0	3.0	4.0
Q1, Q3	3.0, 5.0	3.0, 6.0	3.0, 6.0
Min, Max	3, 6	3, 8	3, 8
Change from Baseline			
n	21	23	44
Mean (SD)	-0.3 (1.62)	-0.2 (1.75)	-0.2 (1.67)
95% Confidence Interval of Mean	-1.0, 0.5	-0.9, 0.6	-0.7, 0.3
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-4, 3	-4, 3	-4, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	4.4 (1.50)	4.6 (1.79)	4.5 (1.63)
95% Confidence Interval of Mean	3.6, 5.2	3.6, 5.5	3.9, 5.1
Median	4.0	4.0	4.0
Q1, Q3	3.0, 5.0	3.0, 6.0	3.0, 6.0
Min, Max	3, 8	3, 8	3, 8
Change from Baseline			
n	15	16	31
Mean (SD)	0.4 (1.68)	0.3 (1.40)	0.4 (1.52)
95% Confidence Interval of Mean	-0.5, 1.3	-0.4, 1.1	-0.2, 0.9
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 1.0	0.0, 1.0	0.0, 1.0
Min, Max	-2, 5	-3, 3	-3, 5

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	4.3 (1.19)	4.1 (1.27)	4.2 (1.20)
95% Confidence Interval of Mean	3.5, 5.1	3.1, 5.1	3.6, 4.8
Median	4.0	4.0	4.0
Q1, Q3	3.0, 5.0	3.0, 5.0	3.0, 5.0
Min, Max	3, 6	3, 6	3, 6
Change from Baseline			
n	11	9	20
Mean (SD)	0.1 (0.83)	-0.1 (1.36)	0.0 (1.08)
95% Confidence Interval of Mean	-0.5, 0.6	-1.2, 0.9	-0.5, 0.5
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	0.0, 0.0	0.0, 0.0
Min, Max	-1, 2	-3, 2	-3, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	5.0 (1.41)	3.0 (N/A)	4.3 (1.53)
95% Confidence Interval of Mean	-7.7, 17.7	N/A, N/A	0.5, 8.1
Median	5.0	3.0	4.0
Q1, Q3	4.0, 6.0	3.0, 3.0	3.0, 6.0
Min, Max	4, 6	3, 3	3, 6
Change from Baseline			
n	2	1	3
Mean (SD)	0.0 (0.00)	0.0 (N/A)	0.0 (0.00)
95% Confidence Interval of Mean	0.0, 0.0	N/A, N/A	0.0, 0.0
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	0.0, 0.0	0.0, 0.0
Min, Max	0, 0	0, 0	0, 0

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Duration (Questions 9, 10, 11)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	4.4 (1.27)	4.0 (1.48)	4.2 (1.35)
95% Confidence Interval of Mean	3.7, 5.0	3.1, 4.9	3.7, 4.7
Median	4.0	3.0	4.0
Q1, Q3	3.0, 5.0	3.0, 5.0	3.0, 5.0
Min, Max	3, 7	3, 7	3, 7
Change from Baseline			
n	17	12	29
Mean (SD)	-0.1 (1.60)	-0.4 (1.44)	-0.2 (1.52)
95% Confidence Interval of Mean	-0.9, 0.8	-1.3, 0.5	-0.8, 0.4
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-2, 4	-3, 3	-3, 4

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	43	37	80
Mean (SD)	5.3 (1.70)	5.0 (1.40)	5.2 (1.57)
95% Confidence Interval of Mean	4.8, 5.9	4.5, 5.4	4.8, 5.5
Median	5.0	4.0	4.0
Q1, Q3	4.0, 6.0	4.0, 6.0	4.0, 6.0
Min, Max	4, 10	4, 8	4, 10

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	39	37	76
Mean (SD)	4.8 (1.19)	4.9 (1.33)	4.9 (1.25)
95% Confidence Interval of Mean	4.4, 5.2	4.4, 5.3	4.6, 5.1
Median	4.0	4.0	4.0
Q1, Q3	4.0, 5.0	4.0, 6.0	4.0, 6.0
Min, Max	4, 8	4, 8	4, 8
Change from Baseline			
n	38	34	72
Mean (SD)	-0.6 (1.37)	-0.2 (1.34)	-0.4 (1.36)
95% Confidence Interval of Mean	-1.0, -0.1	-0.7, 0.3	-0.7, -0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	0.0, 0.0	-1.0, 0.0
Min, Max	-5, 2	-4, 2	-5, 2

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	37	38	75
Mean (SD)	4.9 (1.36)	4.8 (1.06)	4.8 (1.21)
95% Confidence Interval of Mean	4.4, 5.3	4.5, 5.2	4.6, 5.1
Median	4.0	4.0	4.0
Q1, Q3	4.0, 5.0	4.0, 6.0	4.0, 5.0
Min, Max	4, 9	4, 8	4, 9
Change from Baseline			
n	35	35	70
Mean (SD)	-0.7 (1.47)	-0.1 (1.09)	-0.4 (1.32)
95% Confidence Interval of Mean	-1.2, -0.2	-0.5, 0.3	-0.7, -0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	0.0, 0.0	-1.0, 0.0
Min, Max	-5, 4	-4, 2	-5, 4

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	31	64
Mean (SD)	5.1 (1.47)	4.8 (1.47)	5.0 (1.47)
95% Confidence Interval of Mean	4.6, 5.6	4.3, 5.3	4.6, 5.3
Median	5.0	4.0	4.0
Q1, Q3	4.0, 6.0	4.0, 5.0	4.0, 6.0
Min, Max	4, 10	4, 10	4, 10
Change from Baseline			
n	32	28	60
Mean (SD)	-0.3 (1.79)	-0.2 (1.62)	-0.3 (1.70)
95% Confidence Interval of Mean	-1.0, 0.3	-0.8, 0.4	-0.7, 0.2
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-0.5, 0.0	-1.0, 0.0
Min, Max	-5, 6	-4, 3	-5, 6

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	29	59
Mean (SD)	5.1 (1.68)	4.7 (1.03)	4.9 (1.40)
95% Confidence Interval of Mean	4.5, 5.8	4.3, 5.1	4.6, 5.3
Median	4.0	4.0	4.0
Q1, Q3	4.0, 6.0	4.0, 5.0	4.0, 6.0
Min, Max	4, 10	4, 8	4, 10
Change from Baseline			
n	29	26	55
Mean (SD)	-0.3 (1.37)	-0.3 (1.09)	-0.3 (1.23)
95% Confidence Interval of Mean	-0.9, 0.2	-0.7, 0.1	-0.7, 0.0
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-3, 2	-3, 1	-3, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	27	52
Mean (SD)	5.1 (1.47)	4.8 (1.00)	4.9 (1.24)
95% Confidence Interval of Mean	4.5, 5.7	4.4, 5.2	4.6, 5.3
Median	4.0	5.0	4.5
Q1, Q3	4.0, 6.0	4.0, 5.0	4.0, 6.0
Min, Max	4, 9	4, 8	4, 9
Change from Baseline			
n	24	24	48
Mean (SD)	-0.5 (1.25)	-0.3 (0.82)	-0.4 (1.05)
95% Confidence Interval of Mean	-1.0, 0.0	-0.7, 0.0	-0.7, -0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-4, 2	-2, 1	-4, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	26	49
Mean (SD)	4.7 (1.22)	4.7 (1.12)	4.7 (1.15)
95% Confidence Interval of Mean	4.2, 5.2	4.3, 5.2	4.4, 5.0
Median	4.0	4.0	4.0
Q1, Q3	4.0, 5.0	4.0, 5.0	4.0, 5.0
Min, Max	4, 8	4, 8	4, 8
Change from Baseline			
n	23	23	46
Mean (SD)	-0.9 (1.36)	-0.4 (1.20)	-0.7 (1.29)
95% Confidence Interval of Mean	-1.5, -0.3	-1.0, 0.1	-1.0, -0.3
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-5, 0	-3, 1	-5, 1

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	22	26	48
Mean (SD)	4.7 (1.17)	4.7 (0.89)	4.7 (1.02)
95% Confidence Interval of Mean	4.2, 5.2	4.3, 5.0	4.4, 5.0
Median	4.0	4.0	4.0
Q1, Q3	4.0, 5.0	4.0, 5.0	4.0, 5.0
Min, Max	4, 8	4, 7	4, 8
Change from Baseline			
n	21	23	44
Mean (SD)	-0.6 (1.16)	-0.6 (1.08)	-0.6 (1.11)
95% Confidence Interval of Mean	-1.1, -0.1	-1.0, -0.1	-0.9, -0.3
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-4, 1	-3, 1	-4, 1

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	23	45
Mean (SD)	4.6 (1.26)	4.7 (0.93)	4.6 (1.09)
95% Confidence Interval of Mean	4.0, 5.1	4.3, 5.1	4.3, 5.0
Median	4.0	4.0	4.0
Q1, Q3	4.0, 4.0	4.0, 5.0	4.0, 5.0
Min, Max	4, 8	4, 7	4, 8
Change from Baseline			
n	21	20	41
Mean (SD)	-0.9 (1.37)	-0.4 (1.23)	-0.7 (1.32)
95% Confidence Interval of Mean	-1.5, -0.3	-1.0, 0.2	-1.1, -0.2
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-5, 0	-3, 1	-5, 1

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	4.8 (1.47)	4.6 (0.72)	4.7 (1.13)
95% Confidence Interval of Mean	4.0, 5.6	4.2, 5.0	4.3, 5.1
Median	4.0	4.5	4.0
Q1, Q3	4.0, 6.0	4.0, 5.0	4.0, 5.0
Min, Max	4, 8	4, 6	4, 8
Change from Baseline			
n	15	14	29
Mean (SD)	-0.7 (1.87)	-0.4 (1.22)	-0.6 (1.57)
95% Confidence Interval of Mean	-1.8, 0.3	-1.1, 0.3	-1.2, 0.0
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-5, 3	-3, 1	-5, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	4.5 (1.29)	5.2 (0.83)	4.9 (1.14)
95% Confidence Interval of Mean	3.7, 5.4	4.6, 5.9	4.3, 5.4
Median	4.0	5.0	4.0
Q1, Q3	4.0, 4.0	5.0, 6.0	4.0, 6.0
Min, Max	4, 8	4, 6	4, 8
Change from Baseline			
n	11	7	18
Mean (SD)	-0.7 (1.27)	-0.4 (1.27)	-0.6 (1.24)
95% Confidence Interval of Mean	-1.6, 0.1	-1.6, 0.7	-1.2, 0.0
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-2.0, 1.0	-1.0, 0.0
Min, Max	-4, 0	-2, 1	-4, 1

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	5.5 (2.12)	6.0 (N/A)	5.7 (1.53)
95% Confidence Interval of Mean	-13.6, 24.6	N/A, N/A	1.9, 9.5
Median	5.5	6.0	6.0
Q1, Q3	4.0, 7.0	6.0, 6.0	4.0, 7.0
Min, Max	4, 7	6, 6	4, 7
Change from Baseline			
n	2	1	3
Mean (SD)	-2.5 (0.71)	0.0 (N/A)	-1.7 (1.53)
95% Confidence Interval of Mean	-8.9, 3.9	N/A, N/A	-5.5, 2.1
Median	-2.5	0.0	-2.0
Q1, Q3	-3.0, -2.0	0.0, 0.0	-3.0, 0.0
Min, Max	-3, -2	0, 0	-3, 0

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Anxiety (Questions 5, 7, 8, 21)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	4.6 (1.37)	5.0 (1.76)	4.8 (1.53)
95% Confidence Interval of Mean	3.9, 5.3	3.9, 6.1	4.2, 5.3
Median	4.0	4.0	4.0
Q1, Q3	4.0, 4.0	4.0, 5.5	4.0, 5.0
Min, Max	4, 8	4, 10	4, 10
Change from Baseline			
n	16	9	25
Mean (SD)	-1.0 (1.41)	-0.1 (2.20)	-0.7 (1.75)
95% Confidence Interval of Mean	-1.8, -0.2	-1.8, 1.6	-1.4, 0.0
Median	-1.0	0.0	0.0
Q1, Q3	-2.0, 0.0	-1.0, 0.0	-2.0, 0.0
Min, Max	-5, 1	-3, 5	-5, 5

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	41	38	79
Mean (SD)	5.1 (1.31)	4.9 (1.39)	5.0 (1.34)
95% Confidence Interval of Mean	4.7, 5.5	4.5, 5.4	4.7, 5.3
Median	5.0	5.0	5.0
Q1, Q3	4.0, 6.0	4.0, 6.0	4.0, 6.0
Min, Max	3, 8	3, 7	3, 8

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	40	38	78
Mean (SD)	4.7 (1.45)	4.7 (1.49)	4.7 (1.46)
95% Confidence Interval of Mean	4.3, 5.2	4.2, 5.2	4.4, 5.0
Median	5.0	5.0	5.0
Q1, Q3	3.0, 6.0	3.0, 6.0	3.0, 6.0
Min, Max	3, 7	3, 8	3, 8
Change from Baseline			
n	38	34	72
Mean (SD)	-0.4 (1.41)	-0.3 (1.64)	-0.4 (1.51)
95% Confidence Interval of Mean	-0.9, 0.0	-0.9, 0.3	-0.7, -0.0
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-4, 3	-3, 4	-4, 4

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	36	38	74
Mean (SD)	4.6 (1.42)	5.2 (1.59)	4.9 (1.53)
95% Confidence Interval of Mean	4.1, 5.0	4.7, 5.7	4.5, 5.2
Median	4.0	5.0	5.0
Q1, Q3	3.0, 6.0	4.0, 6.0	4.0, 6.0
Min, Max	3, 7	3, 9	3, 9
Change from Baseline			
n	33	34	67
Mean (SD)	-0.5 (1.42)	0.0 (1.67)	-0.3 (1.56)
95% Confidence Interval of Mean	-1.0, -0.0	-0.6, 0.6	-0.6, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 1.0	-1.0, 1.0
Min, Max	-4, 2	-3, 4	-4, 4

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	32	32	64
Mean (SD)	4.6 (1.41)	4.8 (1.42)	4.7 (1.41)
95% Confidence Interval of Mean	4.1, 5.1	4.3, 5.3	4.3, 5.0
Median	4.5	5.0	5.0
Q1, Q3	3.0, 5.0	4.0, 5.5	3.0, 5.0
Min, Max	3, 7	3, 8	3, 8
Change from Baseline			
n	30	29	59
Mean (SD)	-0.4 (1.65)	-0.2 (1.53)	-0.3 (1.58)
95% Confidence Interval of Mean	-1.1, 0.2	-0.8, 0.3	-0.8, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-4, 3	-3, 4	-4, 4

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	28	30	58
Mean (SD)	4.8 (1.27)	4.6 (1.61)	4.7 (1.44)
95% Confidence Interval of Mean	4.3, 5.2	4.0, 5.2	4.3, 5.1
Median	5.0	5.0	5.0
Q1, Q3	4.0, 5.5	3.0, 6.0	3.0, 6.0
Min, Max	3, 7	3, 9	3, 9
Change from Baseline			
n	26	27	53
Mean (SD)	-0.3 (1.19)	-0.3 (1.80)	-0.3 (1.52)
95% Confidence Interval of Mean	-0.8, 0.2	-1.0, 0.4	-0.7, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-3, 2	-4, 3	-4, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	24	28	52
Mean (SD)	4.7 (1.09)	4.6 (1.35)	4.6 (1.22)
95% Confidence Interval of Mean	4.2, 5.1	4.0, 5.1	4.3, 5.0
Median	5.0	4.5	5.0
Q1, Q3	4.0, 5.0	3.5, 5.0	4.0, 5.0
Min, Max	3, 7	3, 8	3, 8
Change from Baseline			
n	22	25	47
Mean (SD)	-0.5 (1.37)	-0.4 (1.47)	-0.4 (1.41)
95% Confidence Interval of Mean	-1.1, 0.2	-1.0, 0.2	-0.9, -0.0
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 1.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-3, 2	-4, 3	-4, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	25	48
Mean (SD)	4.7 (1.36)	4.7 (1.43)	4.7 (1.38)
95% Confidence Interval of Mean	4.1, 5.3	4.1, 5.3	4.3, 5.1
Median	5.0	5.0	5.0
Q1, Q3	3.0, 6.0	4.0, 5.0	3.5, 5.0
Min, Max	3, 7	3, 9	3, 9
Change from Baseline			
n	22	23	45
Mean (SD)	-0.5 (1.14)	-0.2 (1.76)	-0.4 (1.48)
95% Confidence Interval of Mean	-1.0, 0.0	-1.0, 0.5	-0.8, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-3, 2	-3, 4	-3, 4

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	21	26	47
Mean (SD)	4.6 (1.29)	4.6 (1.20)	4.6 (1.23)
95% Confidence Interval of Mean	4.0, 5.2	4.1, 5.1	4.2, 5.0
Median	4.0	5.0	4.0
Q1, Q3	4.0, 5.0	4.0, 5.0	4.0, 5.0
Min, Max	3, 7	3, 7	3, 7
Change from Baseline			
n	20	23	43
Mean (SD)	-0.6 (1.82)	-0.4 (1.70)	-0.5 (1.74)
95% Confidence Interval of Mean	-1.5, 0.3	-1.2, 0.3	-1.0, 0.0
Median	-0.5	-1.0	-1.0
Q1, Q3	-2.0, 1.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-4, 2	-3, 4	-4, 4

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	20	23	43
Mean (SD)	4.6 (1.23)	4.8 (1.48)	4.7 (1.36)
95% Confidence Interval of Mean	4.0, 5.1	4.1, 5.4	4.3, 5.1
Median	4.0	5.0	4.0
Q1, Q3	4.0, 5.0	4.0, 6.0	4.0, 5.0
Min, Max	3, 7	3, 8	3, 8
Change from Baseline			
n	19	20	39
Mean (SD)	-0.7 (1.67)	-0.5 (1.73)	-0.6 (1.68)
95% Confidence Interval of Mean	-1.5, 0.1	-1.3, 0.4	-1.1, -0.0
Median	0.0	-0.5	0.0
Q1, Q3	-2.0, 1.0	-2.0, 0.5	-2.0, 1.0
Min, Max	-4, 2	-3, 4	-4, 4

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	4.3 (1.23)	4.6 (1.31)	4.5 (1.26)
95% Confidence Interval of Mean	3.6, 5.0	3.9, 5.3	4.0, 4.9
Median	4.0	4.5	4.0
Q1, Q3	3.0, 5.0	3.5, 5.0	3.0, 5.0
Min, Max	3, 7	3, 7	3, 7
Change from Baseline			
n	15	14	29
Mean (SD)	-0.5 (1.36)	-0.6 (1.95)	-0.6 (1.64)
95% Confidence Interval of Mean	-1.2, 0.3	-1.8, 0.5	-1.2, 0.1
Median	0.0	-0.5	0.0
Q1, Q3	-2.0, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-3, 2	-3, 4	-3, 4

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	4.3 (1.01)	5.9 (2.03)	5.0 (1.72)
95% Confidence Interval of Mean	3.6, 5.0	4.3, 7.4	4.2, 5.8
Median	4.0	5.0	5.0
Q1, Q3	3.0, 5.0	5.0, 7.0	4.0, 5.0
Min, Max	3, 6	3, 9	3, 9
Change from Baseline			
n	11	7	18
Mean (SD)	-0.6 (0.92)	0.6 (3.26)	-0.2 (2.15)
95% Confidence Interval of Mean	-1.3, -0.0	-2.4, 3.6	-1.2, 0.9
Median	-1.0	0.0	-0.5
Q1, Q3	-1.0, 0.0	-2.0, 3.0	-2.0, 0.0
Min, Max	-2, 1	-3, 6	-3, 6

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	5.0 (0.00)	6.0 (N/A)	5.3 (0.58)
95% Confidence Interval of Mean	5.0, 5.0	N/A, N/A	3.9, 6.8
Median	5.0	6.0	5.0
Q1, Q3	5.0, 5.0	6.0, 6.0	5.0, 6.0
Min, Max	5, 5	6, 6	5, 6
Change from Baseline			
n	2	1	3
Mean (SD)	-1.0 (1.41)	-1.0 (N/A)	-1.0 (1.00)
95% Confidence Interval of Mean	-13.7, 11.7	N/A, N/A	-3.5, 1.5
Median	-1.0	-1.0	-1.0
Q1, Q3	-2.0, 0.0	-1.0, -1.0	-2.0, 0.0
Min, Max	-2, 0	-1, -1	-2, 0

Abbreviation: N/A=Not applicable.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Night Wakings (Questions 16, 24, 25)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	4.6 (1.32)	5.3 (1.37)	4.9 (1.36)
95% Confidence Interval of Mean	4.0, 5.3	4.5, 6.2	4.4, 5.4
Median	5.0	5.0	5.0
Q1, Q3	4.0, 5.0	4.5, 7.0	4.0, 6.0
Min, Max	3, 7	3, 7	3, 7
Change from Baseline			
n	15	9	24
Mean (SD)	-0.4 (1.40)	-0.2 (1.56)	-0.3 (1.43)
95% Confidence Interval of Mean	-1.2, 0.4	-1.4, 1.0	-0.9, 0.3
Median	0.0	0.0	0.0
Q1, Q3	-2.0, 1.0	-1.0, 1.0	-1.5, 1.0
Min, Max	-3, 2	-3, 2	-3, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	40	36	76
Mean (SD)	10.4 (2.38)	10.3 (2.05)	10.3 (2.22)
95% Confidence Interval of Mean	9.6, 11.1	9.6, 11.0	9.8, 10.8
Median	10.0	10.5	10.0
Q1, Q3	9.0, 11.5	9.0, 11.5	9.0, 11.5
Min, Max	7, 19	7, 15	7, 19

Abbreviation: N/A=Not applicable.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	40	37	77
Mean (SD)	9.5 (2.29)	9.2 (1.89)	9.4 (2.09)
95% Confidence Interval of Mean	8.7, 10.2	8.6, 9.8	8.9, 9.8
Median	9.0	9.0	9.0
Q1, Q3	8.0, 10.0	8.0, 10.0	8.0, 10.0
Min, Max	7, 15	7, 14	7, 15
Change from Baseline			
n	37	33	70
Mean (SD)	-1.0 (2.22)	-1.0 (1.87)	-1.0 (2.05)
95% Confidence Interval of Mean	-1.7, -0.3	-1.7, -0.3	-1.5, -0.5
Median	-1.0	-1.0	-1.0
Q1, Q3	-2.0, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-10, 2	-4, 3	-10, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	35	35	70
Mean (SD)	9.5 (1.72)	9.0 (1.77)	9.3 (1.75)
95% Confidence Interval of Mean	8.9, 10.1	8.4, 9.6	8.9, 9.7
Median	10.0	9.0	9.0
Q1, Q3	8.0, 11.0	8.0, 10.0	8.0, 10.0
Min, Max	7, 15	7, 13	7, 15
Change from Baseline			
n	33	31	64
Mean (SD)	-1.0 (2.39)	-1.2 (2.04)	-1.1 (2.21)
95% Confidence Interval of Mean	-1.9, -0.2	-1.9, -0.4	-1.7, -0.6
Median	0.0	-1.0	-1.0
Q1, Q3	-3.0, 1.0	-3.0, 0.0	-3.0, 1.0
Min, Max	-8, 3	-6, 3	-8, 3

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	31	64
Mean (SD)	9.4 (1.32)	9.6 (2.36)	9.5 (1.89)
95% Confidence Interval of Mean	8.9, 9.9	8.8, 10.5	9.0, 10.0
Median	9.0	9.0	9.0
Q1, Q3	9.0, 10.0	8.0, 11.0	8.0, 10.5
Min, Max	7, 12	7, 19	7, 19
Change from Baseline			
n	31	26	57
Mean (SD)	-1.3 (2.24)	-1.0 (2.10)	-1.2 (2.16)
95% Confidence Interval of Mean	-2.1, -0.5	-1.8, -0.2	-1.7, -0.6
Median	-1.0	-1.0	-1.0
Q1, Q3	-2.0, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-10, 2	-6, 4	-10, 4

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	29	28	57
Mean (SD)	9.8 (1.50)	9.1 (1.83)	9.4 (1.69)
95% Confidence Interval of Mean	9.2, 10.3	8.4, 9.8	9.0, 9.9
Median	10.0	9.0	9.0
Q1, Q3	9.0, 11.0	7.0, 11.0	8.0, 11.0
Min, Max	7, 12	7, 13	7, 13
Change from Baseline			
n	27	24	51
Mean (SD)	-0.9 (2.27)	-1.3 (1.98)	-1.1 (2.12)
95% Confidence Interval of Mean	-1.8, -0.0	-2.1, -0.4	-1.7, -0.5
Median	-1.0	-1.0	-1.0
Q1, Q3	-3.0, 1.0	-3.0, 0.0	-3.0, 1.0
Min, Max	-7, 3	-6, 2	-7, 3

Abbreviation: N/A=Not applicable.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	24	28	52
Mean (SD)	9.9 (1.91)	9.4 (2.06)	9.6 (1.99)
95% Confidence Interval of Mean	9.1, 10.7	8.6, 10.2	9.1, 10.2
Median	10.0	9.0	10.0
Q1, Q3	8.5, 11.0	8.0, 10.0	8.0, 10.0
Min, Max	7, 14	7, 17	7, 17
Change from Baseline			
n	22	23	45
Mean (SD)	-1.0 (2.45)	-1.0 (1.72)	-1.0 (2.08)
95% Confidence Interval of Mean	-2.1, 0.1	-1.8, -0.3	-1.6, -0.4
Median	-1.0	-1.0	-1.0
Q1, Q3	-2.0, 1.0	-2.0, 0.0	-2.0, 1.0
Min, Max	-9, 2	-4, 2	-9, 2

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	25	48
Mean (SD)	9.5 (1.75)	8.7 (1.52)	9.1 (1.67)
95% Confidence Interval of Mean	8.8, 10.3	8.1, 9.3	8.6, 9.6
Median	9.0	8.0	9.0
Q1, Q3	8.0, 11.0	8.0, 9.0	8.0, 10.0
Min, Max	7, 13	7, 13	7, 13
Change from Baseline			
n	22	22	44
Mean (SD)	-1.4 (2.46)	-1.4 (1.81)	-1.4 (2.14)
95% Confidence Interval of Mean	-2.5, -0.3	-2.2, -0.6	-2.0, -0.7
Median	-1.0	-2.0	-1.0
Q1, Q3	-3.0, 0.0	-2.0, 0.0	-2.5, 0.0
Min, Max	-10, 1	-5, 2	-10, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	21	24	45
Mean (SD)	9.4 (1.57)	8.9 (1.42)	9.1 (1.50)
95% Confidence Interval of Mean	8.7, 10.1	8.3, 9.5	8.7, 9.6
Median	10.0	9.0	9.0
Q1, Q3	8.0, 10.0	8.0, 9.0	8.0, 10.0
Min, Max	7, 13	7, 14	7, 14
Change from Baseline			
n	20	21	41
Mean (SD)	-1.4 (2.09)	-1.5 (2.02)	-1.5 (2.03)
95% Confidence Interval of Mean	-2.4, -0.4	-2.4, -0.6	-2.1, -0.8
Median	-0.5	-1.0	-1.0
Q1, Q3	-2.5, 0.0	-3.0, 0.0	-3.0, 0.0
Min, Max	-8, 1	-5, 3	-8, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	20	23	43
Mean (SD)	9.7 (1.84)	9.1 (2.63)	9.4 (2.29)
95% Confidence Interval of Mean	8.8, 10.6	8.0, 10.3	8.7, 10.1
Median	10.0	9.0	9.0
Q1, Q3	8.0, 11.5	7.0, 10.0	8.0, 10.0
Min, Max	7, 13	7, 19	7, 19
Change from Baseline			
n	19	19	38
Mean (SD)	-1.1 (2.40)	-1.8 (1.90)	-1.4 (2.16)
95% Confidence Interval of Mean	-2.3, 0.1	-2.7, -0.9	-2.2, -0.7
Median	0.0	-2.0	-1.0
Q1, Q3	-2.0, 0.0	-3.0, 0.0	-3.0, 0.0
Min, Max	-8, 3	-6, 1	-8, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	9.1 (1.68)	9.4 (2.19)	9.3 (1.94)
95% Confidence Interval of Mean	8.2, 10.1	8.3, 10.6	8.6, 10.0
Median	9.0	8.5	9.0
Q1, Q3	8.0, 11.0	7.5, 11.5	8.0, 11.0
Min, Max	7, 12	7, 13	7, 13
Change from Baseline			
n	15	13	28
Mean (SD)	-1.7 (2.69)	-0.8 (2.27)	-1.3 (2.50)
95% Confidence Interval of Mean	-3.2, -0.2	-2.2, 0.5	-2.3, -0.4
Median	-1.0	0.0	-1.0
Q1, Q3	-3.0, 0.0	-2.0, 0.0	-2.5, 0.0
Min, Max	-9, 1	-6, 3	-9, 3

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	9.9 (1.70)	9.7 (2.40)	9.8 (1.99)
95% Confidence Interval of Mean	8.8, 11.1	7.8, 11.5	8.9, 10.7
Median	10.0	10.0	10.0
Q1, Q3	9.0, 11.0	8.0, 11.0	8.0, 11.0
Min, Max	8, 14	7, 14	7, 14
Change from Baseline			
n	11	7	18
Mean (SD)	-0.6 (2.01)	0.1 (1.07)	-0.3 (1.71)
95% Confidence Interval of Mean	-2.0, 0.7	-0.8, 1.1	-1.2, 0.5
Median	-1.0	0.0	0.0
Q1, Q3	-2.0, 1.0	0.0, 1.0	-2.0, 1.0
Min, Max	-3, 3	-2, 1	-3, 3

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	10.5 (0.71)	7.0 (N/A)	9.3 (2.08)
95% Confidence Interval of Mean	4.1, 16.9	N/A, N/A	4.2, 14.5
Median	10.5	7.0	10.0
Q1, Q3	10.0, 11.0	7.0, 7.0	7.0, 11.0
Min, Max	10, 11	7, 7	7, 11
Change from Baseline			
n	2	1	3
Mean (SD)	-2.5 (2.12)	-1.0 (N/A)	-2.0 (1.73)
95% Confidence Interval of Mean	-21.6, 16.6	N/A, N/A	-6.3, 2.3
Median	-2.5	-1.0	-1.0
Q1, Q3	-4.0, -1.0	-1.0, -1.0	-4.0, -1.0
Min, Max	-4, -1	-1, -1	-4, -1

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Parasomnias (Questions 12-15, 17, 22, 23)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	9.4 (1.80)	9.8 (4.00)	9.6 (2.86)
95% Confidence Interval of Mean	8.4, 10.3	7.3, 12.4	8.5, 10.6
Median	10.0	9.0	9.0
Q1, Q3	8.0, 10.0	7.5, 9.5	8.0, 10.0
Min, Max	7, 14	7, 21	7, 21
Change from Baseline			
n	15	9	24
Mean (SD)	-2.1 (2.80)	-0.8 (2.05)	-1.6 (2.58)
95% Confidence Interval of Mean	-3.7, -0.6	-2.4, 0.8	-2.7, -0.5
Median	-2.0	-1.0	-2.0
Q1, Q3	-4.0, 0.0	-2.0, 0.0	-3.0, 0.0
Min, Max	-9, 2	-4, 3	-9, 3

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	43	39	82
Mean (SD)	3.7 (1.30)	3.8 (1.15)	3.7 (1.23)
95% Confidence Interval of Mean	3.3, 4.1	3.4, 4.2	3.5, 4.0
Median	3.0	3.0	3.0
Q1, Q3	3.0, 4.0	3.0, 4.0	3.0, 4.0
Min, Max	3, 9	3, 7	3, 9

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	40	41	81
Mean (SD)	3.6 (1.03)	3.8 (1.01)	3.7 (1.02)
95% Confidence Interval of Mean	3.3, 3.9	3.5, 4.1	3.5, 3.9
Median	3.0	3.0	3.0
Q1, Q3	3.0, 4.0	3.0, 4.0	3.0, 4.0
Min, Max	3, 7	3, 7	3, 7
Change from Baseline			
n	39	38	77
Mean (SD)	-0.1 (1.42)	-0.0 (1.13)	-0.1 (1.27)
95% Confidence Interval of Mean	-0.6, 0.3	-0.4, 0.3	-0.4, 0.2
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	0.0, 0.0	0.0, 0.0
Min, Max	-6, 4	-3, 4	-6, 4

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	37	38	75
Mean (SD)	3.5 (0.93)	3.7 (1.19)	3.6 (1.07)
95% Confidence Interval of Mean	3.2, 3.9	3.3, 4.0	3.4, 3.8
Median	3.0	3.0	3.0
Q1, Q3	3.0, 4.0	3.0, 4.0	3.0, 4.0
Min, Max	3, 6	3, 9	3, 9
Change from Baseline			
n	35	35	70
Mean (SD)	-0.2 (1.42)	-0.2 (1.12)	-0.2 (1.27)
95% Confidence Interval of Mean	-0.7, 0.3	-0.6, 0.2	-0.5, 0.1
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	0.0, 0.0	0.0, 0.0
Min, Max	-6, 2	-4, 3	-6, 3

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	33	66
Mean (SD)	3.4 (0.56)	3.8 (1.17)	3.6 (0.93)
95% Confidence Interval of Mean	3.2, 3.6	3.3, 4.2	3.4, 3.8
Median	3.0	3.0	3.0
Q1, Q3	3.0, 4.0	3.0, 4.0	3.0, 4.0
Min, Max	3, 5	3, 7	3, 7
Change from Baseline			
n	32	29	61
Mean (SD)	-0.4 (1.52)	-0.1 (1.03)	-0.2 (1.31)
95% Confidence Interval of Mean	-1.0, 0.1	-0.5, 0.3	-0.6, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	0.0, 0.0	-1.0, 0.0
Min, Max	-6, 2	-3, 2	-6, 2

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	29	59
Mean (SD)	3.6 (0.82)	3.7 (0.86)	3.6 (0.83)
95% Confidence Interval of Mean	3.3, 3.9	3.3, 4.0	3.4, 3.8
Median	3.0	3.0	3.0
Q1, Q3	3.0, 4.0	3.0, 4.0	3.0, 4.0
Min, Max	3, 6	3, 6	3, 6
Change from Baseline			
n	29	26	55
Mean (SD)	-0.2 (1.61)	-0.2 (0.97)	-0.2 (1.33)
95% Confidence Interval of Mean	-0.8, 0.4	-0.5, 0.2	-0.5, 0.2
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 1.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-6, 3	-2, 2	-6, 3

Abbreviation: N/A=Not applicable.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	28	53
Mean (SD)	3.6 (0.71)	3.6 (1.03)	3.6 (0.88)
95% Confidence Interval of Mean	3.3, 3.9	3.2, 4.0	3.4, 3.9
Median	3.0	3.0	3.0
Q1, Q3	3.0, 4.0	3.0, 4.0	3.0, 4.0
Min, Max	3, 5	3, 7	3, 7
Change from Baseline			
n	24	24	48
Mean (SD)	-0.3 (1.49)	-0.4 (0.78)	-0.4 (1.18)
95% Confidence Interval of Mean	-1.0, 0.3	-0.7, -0.1	-0.7, -0.0
Median	0.0	0.0	0.0
Q1, Q3	-0.5, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-6, 1	-2, 1	-6, 1

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	26	49
Mean (SD)	3.7 (0.98)	3.8 (0.98)	3.7 (0.97)
95% Confidence Interval of Mean	3.2, 4.1	3.4, 4.2	3.5, 4.0
Median	3.0	4.0	3.0
Q1, Q3	3.0, 4.0	3.0, 4.0	3.0, 4.0
Min, Max	3, 7	3, 7	3, 7
Change from Baseline			
n	23	23	46
Mean (SD)	-0.3 (1.43)	-0.1 (0.87)	-0.2 (1.17)
95% Confidence Interval of Mean	-0.9, 0.3	-0.5, 0.2	-0.6, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-6, 2	-2, 2	-6, 2

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	22	26	48
Mean (SD)	3.7 (1.09)	4.0 (1.26)	3.9 (1.18)
95% Confidence Interval of Mean	3.2, 4.2	3.5, 4.5	3.5, 4.2
Median	3.0	4.0	3.0
Q1, Q3	3.0, 4.0	3.0, 4.0	3.0, 4.0
Min, Max	3, 7	3, 8	3, 8
Change from Baseline			
n	21	22	43
Mean (SD)	-0.1 (1.20)	0.1 (0.97)	-0.0 (1.08)
95% Confidence Interval of Mean	-0.7, 0.4	-0.3, 0.5	-0.4, 0.3
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	0.0, 1.0	0.0, 0.0
Min, Max	-4, 2	-3, 2	-4, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	21	23	44
Mean (SD)	3.6 (0.81)	3.7 (0.93)	3.6 (0.87)
95% Confidence Interval of Mean	3.2, 3.9	3.2, 4.1	3.3, 3.9
Median	3.0	3.0	3.0
Q1, Q3	3.0, 4.0	3.0, 4.0	3.0, 4.0
Min, Max	3, 5	3, 6	3, 6
Change from Baseline			
n	20	19	39
Mean (SD)	-0.3 (1.68)	-0.4 (0.96)	-0.3 (1.36)
95% Confidence Interval of Mean	-1.0, 0.5	-0.8, 0.1	-0.7, 0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.5	-1.0, 0.0	-1.0, 0.0
Min, Max	-6, 2	-3, 1	-6, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	3.5 (0.74)	3.7 (1.08)	3.6 (0.92)
95% Confidence Interval of Mean	3.1, 3.9	3.1, 4.3	3.2, 3.9
Median	3.0	3.0	3.0
Q1, Q3	3.0, 4.0	3.0, 4.0	3.0, 4.0
Min, Max	3, 5	3, 6	3, 6
Change from Baseline			
n	15	13	28
Mean (SD)	-0.3 (1.76)	0.0 (0.41)	-0.2 (1.31)
95% Confidence Interval of Mean	-1.3, 0.6	-0.2, 0.2	-0.7, 0.3
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	0.0, 0.0	0.0, 0.0
Min, Max	-6, 2	-1, 1	-6, 2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	3.5 (0.93)	4.3 (1.41)	3.9 (1.23)
95% Confidence Interval of Mean	2.8, 4.1	3.2, 5.4	3.3, 4.4
Median	3.0	5.0	3.0
Q1, Q3	3.0, 4.0	3.0, 5.0	3.0, 5.0
Min, Max	3, 6	3, 7	3, 7
Change from Baseline			
n	11	7	18
Mean (SD)	0.0 (0.89)	0.1 (0.38)	0.1 (0.73)
95% Confidence Interval of Mean	-0.6, 0.6	-0.2, 0.5	-0.3, 0.4
Median	0.0	0.0	0.0
Q1, Q3	0.0, 0.0	0.0, 0.0	0.0, 0.0
Min, Max	-2, 2	0, 1	-2, 2

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	3.5 (0.71)	6.0 (N/A)	4.3 (1.53)
95% Confidence Interval of Mean	-2.9, 9.9	N/A, N/A	0.5, 8.1
Median	3.5	6.0	4.0
Q1, Q3	3.0, 4.0	6.0, 6.0	3.0, 6.0
Min, Max	3, 4	6, 6	3, 6
Change from Baseline			
n	2	1	3
Mean (SD)	-0.5 (0.71)	0.0 (N/A)	-0.3 (0.58)
95% Confidence Interval of Mean	-6.9, 5.9	N/A, N/A	-1.8, 1.1
Median	-0.5	0.0	0.0
Q1, Q3	-1.0, 0.0	0.0, 0.0	-1.0, 0.0
Min, Max	-1, 0	0, 0	-1, 0

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Sleep Disordered Breathing (Questions 18, 19, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	3.6 (1.00)	4.3 (1.15)	3.9 (1.10)
95% Confidence Interval of Mean	3.1, 4.2	3.6, 5.1	3.5, 4.3
Median	3.0	4.5	3.0
Q1, Q3	3.0, 4.0	3.0, 5.0	3.0, 5.0
Min, Max	3, 6	3, 6	3, 6
Change from Baseline			
n	16	9	25
Mean (SD)	-0.3 (1.82)	0.6 (0.73)	0.0 (1.55)
95% Confidence Interval of Mean	-1.3, 0.7	-0.0, 1.1	-0.6, 0.6
Median	0.0	0.0	0.0
Q1, Q3	-0.5, 0.0	0.0, 1.0	0.0, 1.0
Min, Max	-6, 2	0, 2	-6, 2

Abbreviation: N/A=Not applicable.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	36	32	68
Mean (SD)	12.3 (3.03)	12.2 (2.62)	12.3 (2.83)
95% Confidence Interval of Mean	11.3, 13.3	11.3, 13.2	11.6, 12.9
Median	12.0	12.0	12.0
Q1, Q3	10.5, 13.0	10.5, 14.0	10.5, 14.0
Min, Max	8, 19	8, 18	8, 19

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	38	35	73
Mean (SD)	12.2 (3.17)	12.1 (2.93)	12.2 (3.03)
95% Confidence Interval of Mean	11.2, 13.3	11.1, 13.1	11.5, 12.9
Median	12.0	12.0	12.0
Q1, Q3	10.0, 14.0	10.0, 13.0	10.0, 13.0
Min, Max	8, 20	9, 20	8, 20
Change from Baseline			
n	33	28	61
Mean (SD)	0.2 (2.35)	0.3 (2.17)	0.2 (2.25)
95% Confidence Interval of Mean	-0.6, 1.1	-0.6, 1.1	-0.3, 0.8
Median	0.0	1.0	0.0
Q1, Q3	-1.0, 2.0	-2.0, 2.0	-1.0, 2.0
Min, Max	-8, 4	-4, 4	-8, 4

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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	34	36	70
Mean (SD)	11.5 (2.81)	12.1 (3.02)	11.8 (2.91)
95% Confidence Interval of Mean	10.5, 12.5	11.0, 13.1	11.1, 12.5
Median	11.0	12.0	11.5
Q1, Q3	9.0, 13.0	10.0, 13.5	10.0, 13.0
Min, Max	8, 18	8, 19	8, 19
Change from Baseline			
n	28	29	57
Mean (SD)	-0.5 (2.65)	0.0 (2.90)	-0.3 (2.77)
95% Confidence Interval of Mean	-1.6, 0.5	-1.1, 1.1	-1.0, 0.5
Median	0.0	-1.0	0.0
Q1, Q3	-1.5, 1.0	-2.0, 2.0	-2.0, 1.0
Min, Max	-7, 5	-6, 7	-7, 7

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	32	30	62
Mean (SD)	11.8 (2.81)	12.3 (3.06)	12.0 (2.92)
95% Confidence Interval of Mean	10.7, 12.8	11.2, 13.5	11.3, 12.8
Median	12.0	12.0	12.0
Q1, Q3	10.0, 13.0	10.0, 13.0	10.0, 13.0
Min, Max	8, 19	9, 20	8, 20
Change from Baseline			
n	28	23	51
Mean (SD)	-0.5 (2.60)	0.2 (2.35)	-0.2 (2.49)
95% Confidence Interval of Mean	-1.5, 0.5	-0.8, 1.2	-0.9, 0.5
Median	-0.5	0.0	0.0
Q1, Q3	-1.5, 1.0	-1.0, 1.0	-1.0, 1.0
Min, Max	-7, 6	-4, 4	-7, 6

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	29	28	57
Mean (SD)	11.7 (2.62)	11.8 (2.72)	11.8 (2.65)
95% Confidence Interval of Mean	10.7, 12.7	10.8, 12.9	11.1, 12.5
Median	12.0	12.0	12.0
Q1, Q3	9.0, 14.0	10.0, 13.0	10.0, 13.0
Min, Max	8, 18	8, 20	8, 20
Change from Baseline			
n	25	21	46
Mean (SD)	-0.9 (3.38)	0.0 (2.24)	-0.5 (2.92)
95% Confidence Interval of Mean	-2.3, 0.5	-1.0, 1.0	-1.3, 0.4
Median	0.0	0.0	0.0
Q1, Q3	-2.0, 1.0	-1.0, 2.0	-2.0, 1.0
Min, Max	-10, 5	-4, 4	-10, 5

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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	24	28	52
Mean (SD)	11.9 (2.69)	12.8 (2.30)	12.4 (2.50)
95% Confidence Interval of Mean	10.8, 13.1	11.9, 13.7	11.7, 13.1
Median	12.0	13.0	12.0
Q1, Q3	10.0, 14.0	11.0, 14.0	10.0, 14.0
Min, Max	8, 19	10, 18	8, 19
Change from Baseline			
n	20	21	41
Mean (SD)	-0.4 (2.80)	0.8 (2.47)	0.2 (2.67)
95% Confidence Interval of Mean	-1.7, 0.9	-0.4, 1.9	-0.6, 1.0
Median	0.0	0.0	0.0
Q1, Q3	-2.0, 1.5	0.0, 1.0	-1.0, 1.0
Min, Max	-6, 4	-3, 7	-6, 7

Abbreviation: N/A=Not applicable.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	26	49
Mean (SD)	11.3 (2.85)	11.8 (2.63)	11.6 (2.72)
95% Confidence Interval of Mean	10.1, 12.5	10.7, 12.8	10.8, 12.3
Median	11.0	11.0	11.0
Q1, Q3	8.0, 13.0	10.0, 14.0	10.0, 13.0
Min, Max	8, 18	8, 17	8, 18
Change from Baseline			
n	19	21	40
Mean (SD)	-0.8 (3.25)	-0.5 (2.93)	-0.7 (3.05)
95% Confidence Interval of Mean	-2.4, 0.7	-1.9, 0.8	-1.7, 0.3
Median	0.0	-1.0	0.0
Q1, Q3	-2.0, 1.0	-3.0, 1.0	-3.0, 1.0
Min, Max	-9, 5	-4, 7	-9, 7

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	20	25	45
Mean (SD)	11.3 (2.41)	12.9 (3.35)	12.2 (3.05)
95% Confidence Interval of Mean	10.2, 12.4	11.5, 14.3	11.3, 13.1
Median	11.0	13.0	12.0
Q1, Q3	9.5, 12.5	10.0, 15.0	10.0, 14.0
Min, Max	8, 16	8, 20	8, 20
Change from Baseline			
n	16	19	35
Mean (SD)	-0.2 (2.14)	0.6 (2.89)	0.2 (2.57)
95% Confidence Interval of Mean	-1.3, 1.0	-0.8, 2.0	-0.7, 1.1
Median	0.0	0.0	0.0
Q1, Q3	-1.5, 1.0	-1.0, 3.0	-1.0, 2.0
Min, Max	-5, 4	-4, 7	-5, 7

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	19	22	41
Mean (SD)	11.1 (2.34)	12.2 (2.60)	11.7 (2.52)
95% Confidence Interval of Mean	9.9, 12.2	11.1, 13.4	10.9, 12.5
Median	11.0	12.0	12.0
Q1, Q3	9.0, 12.0	10.0, 14.0	10.0, 13.0
Min, Max	8, 17	8, 17	8, 17
Change from Baseline			
n	16	17	33
Mean (SD)	-0.1 (2.38)	0.8 (2.68)	0.4 (2.53)
95% Confidence Interval of Mean	-1.3, 1.2	-0.6, 2.1	-0.5, 1.3
Median	0.0	1.0	0.0
Q1, Q3	-1.5, 1.0	-1.0, 3.0	-1.0, 2.0
Min, Max	-5, 4	-4, 5	-5, 5

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	10.9 (2.10)	11.8 (2.40)	11.4 (2.27)
95% Confidence Interval of Mean	9.7, 12.0	10.5, 13.1	10.5, 12.2
Median	11.0	11.5	11.0
Q1, Q3	9.0, 12.0	10.0, 14.0	10.0, 13.0
Min, Max	8, 16	9, 16	8, 16
Change from Baseline			
n	12	11	23
Mean (SD)	-0.6 (2.91)	0.5 (2.54)	-0.0 (2.74)
95% Confidence Interval of Mean	-2.4, 1.3	-1.2, 2.3	-1.2, 1.1
Median	-0.5	0.0	0.0
Q1, Q3	-2.5, 1.5	-1.0, 3.0	-2.0, 2.0
Min, Max	-6, 4	-3, 5	-6, 5

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	11.8 (2.52)	10.9 (1.62)	11.4 (2.16)
95% Confidence Interval of Mean	10.1, 13.5	9.6, 12.1	10.4, 12.4
Median	12.0	11.0	11.0
Q1, Q3	10.0, 14.0	10.0, 12.0	10.0, 13.0
Min, Max	8, 16	8, 13	8, 16
Change from Baseline			
n	8	7	15
Mean (SD)	0.4 (1.60)	-0.4 (2.64)	0.0 (2.10)
95% Confidence Interval of Mean	-1.0, 1.7	-2.9, 2.0	-1.2, 1.2
Median	1.0	-1.0	1.0
Q1, Q3	-1.0, 1.0	-2.0, 1.0	-2.0, 1.0
Min, Max	-2, 3	-4, 4	-4, 4

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	11.5 (3.54)	9.0 (N/A)	10.7 (2.89)
95% Confidence Interval of Mean	-20.3, 43.3	N/A, N/A	3.5, 17.8
Median	11.5	9.0	9.0
Q1, Q3	9.0, 14.0	9.0, 9.0	9.0, 14.0
Min, Max	9, 14	9, 9	9, 14
Change from Baseline			
n	2	1	3
Mean (SD)	-1.0 (2.83)	-1.0 (N/A)	-1.0 (2.00)
95% Confidence Interval of Mean	-26.4, 24.4	N/A, N/A	-6.0, 4.0
Median	-1.0	-1.0	-1.0
Q1, Q3	-3.0, 1.0	-1.0, -1.0	-3.0, 1.0
Min, Max	-3, 1	-1, -1	-3, 1

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Daytime Sleepiness (Questions 26-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	16	12	28
Mean (SD)	11.3 (2.44)	11.2 (2.98)	11.2 (2.63)
95% Confidence Interval of Mean	10.0, 12.5	9.3, 13.1	10.2, 12.2
Median	11.0	10.0	10.5
Q1, Q3	10.0, 12.0	9.0, 12.5	9.0, 12.0
Min, Max	8, 17	8, 18	8, 18
Change from Baseline			
n	12	9	21
Mean (SD)	-1.8 (2.76)	-0.3 (1.80)	-1.2 (2.46)
95% Confidence Interval of Mean	-3.6, -0.1	-1.7, 1.1	-2.3, -0.1
Median	-1.0	0.0	0.0
Q1, Q3	-4.0, 0.0	-1.0, 1.0	-3.0, 0.0
Min, Max	-7, 2	-4, 2	-7, 2

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	34	30	64
Mean (SD)	49.9 (9.53)	47.9 (6.00)	49.0 (8.07)
95% Confidence Interval of Mean	46.6, 53.3	45.7, 50.1	47.0, 51.0
Median	49.0	47.0	48.0
Q1, Q3	43.0, 54.0	43.0, 52.0	43.0, 53.5
Min, Max	35, 76	37, 60	35, 76

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	37	32	69
Mean (SD)	46.8 (7.74)	45.7 (5.26)	46.3 (6.68)
95% Confidence Interval of Mean	44.2, 49.4	43.8, 47.6	44.7, 47.9
Median	46.0	45.0	46.0
Q1, Q3	41.0, 50.0	42.0, 49.0	42.0, 49.0
Min, Max	35, 68	38, 58	35, 68
Change from Baseline			
n	30	25	55
Mean (SD)	-2.3 (8.50)	-2.2 (5.01)	-2.3 (7.07)
95% Confidence Interval of Mean	-5.5, 0.8	-4.3, -0.2	-4.2, -0.4
Median	-1.5	-2.0	-2.0
Q1, Q3	-5.0, 2.0	-5.0, 0.0	-5.0, 1.0
Min, Max	-38, 8	-12, 10	-38, 10

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	33	33	66
Mean (SD)	45.4 (6.92)	46.3 (5.96)	45.9 (6.42)
95% Confidence Interval of Mean	43.0, 47.9	44.2, 48.4	44.3, 47.4
Median	44.0	45.0	45.0
Q1, Q3	41.0, 48.0	42.0, 50.0	42.0, 49.0
Min, Max	36, 68	35, 58	35, 68
Change from Baseline			
n	28	26	54
Mean (SD)	-4.1 (8.58)	-1.1 (5.58)	-2.6 (7.38)
95% Confidence Interval of Mean	-7.4, -0.7	-3.3, 1.2	-4.6, -0.6
Median	-2.0	-0.5	-1.5
Q1, Q3	-6.0, 1.0	-4.0, 3.0	-5.0, 2.0
Min, Max	-33, 8	-12, 7	-33, 8

Abbreviation: N/A=Not applicable.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	31	29	60
Mean (SD)	45.6 (6.92)	45.9 (5.50)	45.8 (6.22)
95% Confidence Interval of Mean	43.1, 48.2	43.8, 48.0	44.2, 47.4
Median	46.0	45.0	45.0
Q1, Q3	40.0, 49.0	42.0, 49.0	41.0, 49.0
Min, Max	35, 65	37, 58	35, 65
Change from Baseline			
n	26	21	47
Mean (SD)	-3.8 (9.23)	-2.0 (5.40)	-3.0 (7.73)
95% Confidence Interval of Mean	-7.5, -0.1	-4.5, 0.5	-5.3, -0.7
Median	-1.5	-1.0	-1.0
Q1, Q3	-7.0, 1.0	-5.0, 2.0	-6.0, 1.0
Min, Max	-37, 9	-13, 9	-37, 9

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	27	27	54
Mean (SD)	46.3 (6.69)	45.1 (5.63)	45.7 (6.16)
95% Confidence Interval of Mean	43.7, 49.0	42.9, 47.3	44.0, 47.4
Median	47.0	44.0	45.0
Q1, Q3	40.0, 52.0	41.0, 50.0	41.0, 51.0
Min, Max	36, 61	33, 56	33, 61
Change from Baseline			
n	22	20	42
Mean (SD)	-2.2 (7.73)	-2.2 (4.19)	-2.2 (6.22)
95% Confidence Interval of Mean	-5.7, 1.2	-4.2, -0.2	-4.2, -0.3
Median	0.5	-2.0	-1.0
Q1, Q3	-4.0, 2.0	-5.0, 0.5	-5.0, 2.0
Min, Max	-21, 9	-12, 8	-21, 9

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	23	27	50
Mean (SD)	47.3 (7.13)	46.6 (5.31)	46.9 (6.16)
95% Confidence Interval of Mean	44.2, 50.4	44.5, 48.7	45.1, 48.7
Median	46.0	46.0	46.0
Q1, Q3	44.0, 51.0	42.0, 49.0	43.0, 51.0
Min, Max	35, 64	37, 56	35, 64
Change from Baseline			
n	18	20	38
Mean (SD)	-2.8 (8.28)	-1.7 (5.31)	-2.2 (6.81)
95% Confidence Interval of Mean	-6.9, 1.3	-4.1, 0.8	-4.4, 0.0
Median	0.0	-1.0	-0.5
Q1, Q3	-3.0, 0.0	-5.0, 2.5	-4.0, 2.0
Min, Max	-32, 7	-13, 7	-32, 7

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[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	25	48
Mean (SD)	45.0 (6.28)	44.2 (4.94)	44.6 (5.58)
95% Confidence Interval of Mean	42.3, 47.8	42.2, 46.2	43.0, 46.2
Median	44.0	43.0	43.5
Q1, Q3	40.0, 48.0	42.0, 48.0	40.5, 48.0
Min, Max	37, 60	34, 55	34, 60
Change from Baseline			
n	18	20	38
Mean (SD)	-5.9 (9.54)	-3.9 (5.36)	-4.8 (7.59)
95% Confidence Interval of Mean	-10.6, -1.1	-6.4, -1.4	-7.3, -2.3
Median	-4.5	-3.0	-3.0
Q1, Q3	-10.0, 0.0	-7.5, -1.5	-9.0, 0.0
Min, Max	-34, 8	-14, 8	-34, 8

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	19	23	42
Mean (SD)	45.0 (6.28)	45.8 (5.80)	45.4 (5.96)
95% Confidence Interval of Mean	42.0, 48.0	43.3, 48.3	43.6, 47.3
Median	46.0	47.0	46.5
Q1, Q3	40.0, 50.0	41.0, 50.0	40.0, 50.0
Min, Max	35, 56	35, 57	35, 57
Change from Baseline			
n	15	17	32
Mean (SD)	-4.7 (8.48)	-1.8 (6.55)	-3.2 (7.54)
95% Confidence Interval of Mean	-9.4, -0.0	-5.2, 1.5	-5.9, -0.5
Median	-3.0	-2.0	-2.5
Q1, Q3	-7.0, 0.0	-5.0, 4.0	-6.0, 2.0
Min, Max	-26, 8	-20, 6	-26, 8

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	19	22	41
Mean (SD)	44.6 (5.86)	45.1 (4.55)	44.9 (5.14)
95% Confidence Interval of Mean	41.8, 47.4	43.1, 47.1	43.2, 46.5
Median	44.0	44.5	44.0
Q1, Q3	41.0, 49.0	42.0, 49.0	42.0, 49.0
Min, Max	35, 58	38, 55	35, 58
Change from Baseline			
n	15	16	31
Mean (SD)	-4.4 (10.18)	-2.7 (5.96)	-3.5 (8.18)
95% Confidence Interval of Mean	-10.0, 1.2	-5.9, 0.5	-6.5, -0.5
Median	-2.0	-2.5	-2.0
Q1, Q3	-11.0, 1.0	-7.0, 2.0	-8.0, 1.0
Min, Max	-32, 7	-15, 7	-32, 7

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-cshq-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	43.5 (7.02)	45.6 (4.88)	44.5 (6.00)
95% Confidence Interval of Mean	39.6, 47.4	43.0, 48.2	42.3, 46.8
Median	42.0	46.5	45.0
Q1, Q3	38.0, 47.0	41.0, 47.5	40.0, 47.0
Min, Max	36, 59	38, 54	36, 59
Change from Baseline			
n	12	10	22
Mean (SD)	-5.0 (12.61)	-1.4 (5.46)	-3.4 (9.97)
95% Confidence Interval of Mean	-13.0, 3.0	-5.3, 2.5	-7.8, 1.1
Median	-2.0	-1.0	-1.0
Q1, Q3	-9.0, 1.0	-6.0, 1.0	-6.0, 1.0
Min, Max	-36, 14	-8, 8	-36, 14

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	44.6 (5.46)	47.7 (5.05)	46.0 (5.37)
95% Confidence Interval of Mean	41.0, 48.3	43.8, 51.5	43.5, 48.5
Median	44.0	48.0	45.0
Q1, Q3	41.0, 47.0	45.0, 50.0	42.0, 49.5
Min, Max	38, 57	41, 57	38, 57
Change from Baseline			
n	8	6	14
Mean (SD)	-3.4 (5.48)	-2.2 (7.70)	-2.9 (6.27)
95% Confidence Interval of Mean	-8.0, 1.2	-10.3, 5.9	-6.5, 0.8
Median	-3.0	-5.5	-3.5
Q1, Q3	-7.5, 1.5	-7.0, 3.0	-7.0, 2.0
Min, Max	-12, 3	-9, 11	-12, 11

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	51.5 (4.95)	43.0 (N/A)	48.7 (6.03)
95% Confidence Interval of Mean	7.0, 96.0	N/A, N/A	33.7, 63.6
Median	51.5	43.0	48.0
Q1, Q3	48.0, 55.0	43.0, 43.0	43.0, 55.0
Min, Max	48, 55	43, 43	43, 55
Change from Baseline			
n	2	1	3
Mean (SD)	-8.0 (8.49)	-9.0 (N/A)	-8.3 (6.03)
95% Confidence Interval of Mean	-84.2, 68.2	N/A, N/A	-23.3, 6.6
Median	-8.0	-9.0	-9.0
Q1, Q3	-14.0, -2.0	-9.0, -9.0	-14.0, -2.0
Min, Max	-14, -2	-9, -9	-14, -2

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-01a-cshq-ole.rtf

Table 14.2.6.1A
Summary of Children's Sleep Habit Questionnaire
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Total (Questions 1-33)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	16	12	28
Mean (SD)	44.3 (6.61)	46.7 (8.16)	45.3 (7.27)
95% Confidence Interval of Mean	40.7, 47.8	41.5, 51.9	42.5, 48.1
Median	42.5	44.5	43.0
Q1, Q3	40.5, 46.5	42.0, 50.0	41.0, 47.5
Min, Max	37, 58	36, 63	36, 63
Change from Baseline			
n	11	8	19
Mean (SD)	-8.4 (12.07)	-2.3 (7.85)	-5.8 (10.70)
95% Confidence Interval of Mean	-16.5, -0.3	-8.8, 4.3	-10.9, -0.6
Median	-8.0	-3.5	-5.0
Q1, Q3	-13.0, -2.0	-7.0, 0.5	-12.0, 0.0
Min, Max	-35, 13	-12, 14	-35, 14

Abbreviation: N/A=Not applicable.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.1A

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4.8.6 Ergebnisse zum Endpunkt Morbidität - Veränderung der ADAMS gegenüber Baseline während der OLE

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	44	41	85
Mean (SD)	5.8 (3.79)	7.4 (4.22)	6.6 (4.06)
95% Confidence Interval of Mean	4.6, 6.9	6.1, 8.7	5.7, 7.4
Median	6.0	8.0	7.0
Q1, Q3	2.0, 8.5	4.0, 11.0	3.0, 10.0
Min, Max	0, 12	0, 15	0, 15

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-adams-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 5 (Week 17)			
n	0	1	1
Mean (SD)		10.0 (N/A)	10.0 (N/A)
95% Confidence Interval of Mean		N/A, N/A	N/A, N/A
Median		10.0	10.0
Q1, Q3		10.0, 10.0	10.0, 10.0
Min, Max		10, 10	10, 10
Change from Baseline			
n	0	1	1
Mean (SD)		-1.0 (N/A)	-1.0 (N/A)
95% Confidence Interval of Mean		N/A, N/A	N/A, N/A
Median		-1.0	-1.0
Q1, Q3		-1.0, -1.0	-1.0, -1.0
Min, Max		-1, -1	-1, -1

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-adams-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	42	39	81
Mean (SD)	3.9 (3.47)	5.9 (4.03)	4.9 (3.87)
95% Confidence Interval of Mean	2.8, 5.0	4.6, 7.3	4.0, 5.7
Median	3.0	6.0	4.0
Q1, Q3	1.0, 6.0	2.0, 10.0	2.0, 8.0
Min, Max	0, 14	0, 14	0, 14
Change from Baseline			
n	41	39	80
Mean (SD)	-2.1 (2.76)	-1.6 (3.90)	-1.9 (3.36)
95% Confidence Interval of Mean	-3.0, -1.3	-2.9, -0.3	-2.6, -1.1
Median	-2.0	-1.0	-2.0
Q1, Q3	-3.0, 0.0	-4.0, 0.0	-4.0, 0.0
Min, Max	-8, 3	-10, 6	-10, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-adams-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	36	38	74
Mean (SD)	4.6 (3.76)	6.3 (3.71)	5.5 (3.81)
95% Confidence Interval of Mean	3.3, 5.9	5.1, 7.5	4.6, 6.4
Median	4.5	6.0	5.0
Q1, Q3	1.5, 7.0	4.0, 9.0	2.0, 9.0
Min, Max	0, 12	0, 13	0, 13
Change from Baseline			
n	36	38	74
Mean (SD)	-0.9 (3.22)	-1.0 (3.51)	-1.0 (3.35)
95% Confidence Interval of Mean	-2.0, 0.1	-2.1, 0.2	-1.7, -0.2
Median	-1.0	-1.0	-1.0
Q1, Q3	-2.5, 1.0	-3.0, 2.0	-3.0, 1.0
Min, Max	-8, 5	-10, 5	-10, 5

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-adams-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	32	65
Mean (SD)	4.8 (3.50)	5.7 (3.87)	5.2 (3.68)
95% Confidence Interval of Mean	3.6, 6.1	4.3, 7.0	4.3, 6.1
Median	5.0	6.0	6.0
Q1, Q3	2.0, 7.0	2.5, 9.0	2.0, 8.0
Min, Max	0, 12	0, 13	0, 13
Change from Baseline			
n	32	32	64
Mean (SD)	-0.9 (2.98)	-1.4 (2.87)	-1.1 (2.92)
95% Confidence Interval of Mean	-1.9, 0.2	-2.4, -0.4	-1.9, -0.4
Median	0.0	-1.0	-1.0
Q1, Q3	-2.0, 0.5	-3.0, 0.0	-3.0, 0.0
Min, Max	-9, 6	-7, 5	-9, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	28	58
Mean (SD)	5.2 (3.89)	5.4 (4.21)	5.3 (4.01)
95% Confidence Interval of Mean	3.7, 6.6	3.8, 7.1	4.2, 6.3
Median	6.0	5.5	6.0
Q1, Q3	2.0, 8.0	2.0, 9.0	2.0, 9.0
Min, Max	0, 12	0, 13	0, 13
Change from Baseline			
n	29	28	57
Mean (SD)	-0.2 (3.44)	-2.0 (3.42)	-1.1 (3.52)
95% Confidence Interval of Mean	-1.5, 1.1	-3.4, -0.7	-2.0, -0.2
Median	0.0	-1.0	-1.0
Q1, Q3	-2.0, 2.0	-4.0, 0.0	-4.0, 1.0
Min, Max	-9, 6	-12, 4	-12, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	24	27	51
Mean (SD)	4.3 (3.54)	5.0 (3.92)	4.7 (3.73)
95% Confidence Interval of Mean	2.8, 5.7	3.5, 6.6	3.6, 5.7
Median	4.0	5.0	4.0
Q1, Q3	1.0, 8.0	2.0, 7.0	1.0, 8.0
Min, Max	0, 10	0, 14	0, 14
Change from Baseline			
n	23	27	50
Mean (SD)	-0.8 (3.26)	-2.3 (3.22)	-1.6 (3.30)
95% Confidence Interval of Mean	-2.2, 0.6	-3.6, -1.0	-2.5, -0.7
Median	0.0	-2.0	-1.0
Q1, Q3	-3.0, 1.0	-4.0, 0.0	-4.0, 0.0
Min, Max	-8, 7	-9, 4	-9, 7

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-adams-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	26	49
Mean (SD)	3.6 (3.29)	4.8 (4.19)	4.2 (3.80)
95% Confidence Interval of Mean	2.2, 5.0	3.1, 6.5	3.2, 5.3
Median	4.0	4.0	4.0
Q1, Q3	0.0, 6.0	1.0, 8.0	1.0, 7.0
Min, Max	0, 10	0, 13	0, 13
Change from Baseline			
n	23	26	49
Mean (SD)	-1.5 (3.31)	-2.7 (3.47)	-2.1 (3.42)
95% Confidence Interval of Mean	-2.9, -0.0	-4.1, -1.3	-3.1, -1.1
Median	-2.0	-2.5	-2.0
Q1, Q3	-4.0, 0.0	-4.0, 0.0	-4.0, 0.0
Min, Max	-8, 6	-11, 4	-11, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	22	25	47
Mean (SD)	4.1 (4.30)	5.3 (4.50)	4.7 (4.40)
95% Confidence Interval of Mean	2.2, 6.0	3.4, 7.1	3.4, 6.0
Median	3.0	5.0	4.0
Q1, Q3	0.0, 8.0	1.0, 9.0	0.0, 8.0
Min, Max	0, 13	0, 14	0, 14
Change from Baseline			
n	21	25	46
Mean (SD)	-0.9 (2.62)	-2.0 (3.59)	-1.5 (3.20)
95% Confidence Interval of Mean	-2.1, 0.3	-3.5, -0.5	-2.5, -0.5
Median	0.0	-1.0	-1.0
Q1, Q3	-2.0, 0.0	-4.0, 0.0	-3.0, 0.0
Min, Max	-8, 4	-12, 3	-12, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	21	43
Mean (SD)	4.3 (4.51)	4.3 (4.37)	4.3 (4.39)
95% Confidence Interval of Mean	2.3, 6.3	2.3, 6.3	3.0, 5.7
Median	3.0	3.0	3.0
Q1, Q3	0.0, 9.0	1.0, 7.0	0.0, 8.0
Min, Max	0, 13	0, 14	0, 14
Change from Baseline			
n	21	21	42
Mean (SD)	-1.0 (3.11)	-2.8 (3.86)	-1.9 (3.58)
95% Confidence Interval of Mean	-2.4, 0.4	-4.5, -1.0	-3.0, -0.8
Median	-1.0	-2.0	-1.0
Q1, Q3	-2.0, 1.0	-4.0, 0.0	-4.0, 0.0
Min, Max	-9, 5	-10, 4	-10, 5

Abbreviation: N/A=Not applicable.
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[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	14	15	29
Mean (SD)	3.1 (3.78)	4.5 (4.34)	3.9 (4.07)
95% Confidence Interval of Mean	1.0, 5.3	2.1, 6.9	2.3, 5.4
Median	0.5	4.0	3.0
Q1, Q3	0.0, 6.0	1.0, 7.0	0.0, 6.0
Min, Max	0, 9	0, 14	0, 14
Change from Baseline			
n	14	15	29
Mean (SD)	-1.4 (2.74)	-1.9 (3.25)	-1.7 (2.97)
95% Confidence Interval of Mean	-3.0, 0.2	-3.7, -0.1	-2.8, -0.5
Median	-1.0	-2.0	-1.0
Q1, Q3	-2.0, 0.0	-5.0, 0.0	-3.0, 0.0
Min, Max	-8, 3	-6, 5	-8, 5

Abbreviation: N/A=Not applicable.
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[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	8	19
Mean (SD)	3.2 (3.97)	5.4 (3.74)	4.1 (3.93)
95% Confidence Interval of Mean	0.5, 5.8	2.2, 8.5	2.2, 6.0
Median	0.0	6.0	5.0
Q1, Q3	0.0, 6.0	2.5, 8.0	0.0, 7.0
Min, Max	0, 10	0, 10	0, 10
Change from Baseline			
n	11	8	19
Mean (SD)	-1.4 (3.07)	-1.4 (3.50)	-1.4 (3.17)
95% Confidence Interval of Mean	-3.4, 0.7	-4.3, 1.6	-2.9, 0.2
Median	0.0	-2.5	-1.0
Q1, Q3	-2.0, 0.0	-3.5, 1.0	-3.0, 0.0
Min, Max	-8, 3	-6, 5	-8, 5

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	6.5 (3.54)	3.0 (N/A)	5.3 (3.21)
95% Confidence Interval of Mean	-25.3, 38.3	N/A, N/A	-2.7, 13.3
Median	6.5	3.0	4.0
Q1, Q3	4.0, 9.0	3.0, 3.0	3.0, 9.0
Min, Max	4, 9	3, 3	3, 9
Change from Baseline			
n	2	1	3
Mean (SD)	-2.5 (0.71)	-5.0 (N/A)	-3.3 (1.53)
95% Confidence Interval of Mean	-8.9, 3.9	N/A, N/A	-7.1, 0.5
Median	-2.5	-5.0	-3.0
Q1, Q3	-3.0, -2.0	-5.0, -5.0	-5.0, -2.0
Min, Max	-3, -2	-5, -5	-5, -2

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Manic/Hyperactive Behavior (Questions 3, 4, 12, 17, 22)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	11	28
Mean (SD)	3.1 (3.13)	4.0 (4.05)	3.4 (3.48)
95% Confidence Interval of Mean	1.4, 4.7	1.3, 6.7	2.1, 4.8
Median	3.0	3.0	3.0
Q1, Q3	0.0, 6.0	0.0, 7.0	0.0, 6.0
Min, Max	0, 10	0, 12	0, 12
Change from Baseline			
n	16	11	27
Mean (SD)	-1.7 (3.07)	-1.5 (1.81)	-1.6 (2.59)
95% Confidence Interval of Mean	-3.3, -0.1	-2.8, -0.3	-2.7, -0.6
Median	-1.0	-2.0	-1.0
Q1, Q3	-2.5, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-8, 4	-6, 1	-8, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	44	41	85
Mean (SD)	4.4 (3.55)	6.0 (3.72)	5.2 (3.70)
95% Confidence Interval of Mean	3.4, 5.5	4.8, 7.2	4.4, 6.0
Median	4.0	5.0	5.0
Q1, Q3	1.0, 8.0	3.0, 7.0	2.0, 8.0
Min, Max	0, 12	1, 16	0, 16

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 5 (Week 17)			
n	0	1	1
Mean (SD)		3.0 (N/A)	3.0 (N/A)
95% Confidence Interval of Mean		N/A, N/A	N/A, N/A
Median		3.0	3.0
Q1, Q3		3.0, 3.0	3.0, 3.0
Min, Max		3, 3	3, 3
Change from Baseline			
n	0	1	1
Mean (SD)		1.0 (N/A)	1.0 (N/A)
95% Confidence Interval of Mean		N/A, N/A	N/A, N/A
Median		1.0	1.0
Q1, Q3		1.0, 1.0	1.0, 1.0
Min, Max		1, 1	1, 1

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	41	39	80
Mean (SD)	3.8 (3.90)	4.9 (3.07)	4.3 (3.55)
95% Confidence Interval of Mean	2.5, 5.0	3.9, 5.9	3.5, 5.1
Median	3.0	5.0	4.0
Q1, Q3	1.0, 5.0	2.0, 8.0	1.0, 7.0
Min, Max	0, 14	0, 10	0, 14
Change from Baseline			
n	40	39	79
Mean (SD)	-0.6 (2.91)	-1.1 (4.14)	-0.8 (3.55)
95% Confidence Interval of Mean	-1.5, 0.4	-2.4, 0.3	-1.6, -0.0
Median	-0.5	0.0	0.0
Q1, Q3	-3.0, 1.0	-4.0, 2.0	-3.0, 1.0
Min, Max	-7, 6	-12, 7	-12, 7

Abbreviation: N/A=Not applicable.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	36	38	74
Mean (SD)	3.8 (3.72)	4.6 (2.78)	4.2 (3.27)
95% Confidence Interval of Mean	2.5, 5.0	3.7, 5.5	3.4, 4.9
Median	3.0	5.0	4.0
Q1, Q3	0.0, 6.0	3.0, 7.0	1.0, 6.0
Min, Max	0, 14	0, 9	0, 14
Change from Baseline			
n	36	38	74
Mean (SD)	-0.4 (2.99)	-1.1 (4.05)	-0.8 (3.57)
95% Confidence Interval of Mean	-1.4, 0.6	-2.5, 0.2	-1.6, 0.0
Median	0.0	0.0	0.0
Q1, Q3	-2.5, 1.0	-5.0, 1.0	-4.0, 1.0
Min, Max	-7, 7	-11, 8	-11, 8

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	32	65
Mean (SD)	3.6 (3.56)	5.0 (3.48)	4.3 (3.57)
95% Confidence Interval of Mean	2.3, 4.8	3.7, 6.3	3.4, 5.2
Median	3.0	4.5	3.0
Q1, Q3	1.0, 6.0	2.0, 9.0	1.0, 7.0
Min, Max	0, 14	0, 11	0, 14
Change from Baseline			
n	32	32	64
Mean (SD)	-0.8 (2.81)	-0.8 (3.97)	-0.8 (3.41)
95% Confidence Interval of Mean	-1.8, 0.2	-2.2, 0.7	-1.6, 0.1
Median	-1.0	-1.0	-1.0
Q1, Q3	-2.0, 1.0	-3.5, 3.0	-3.0, 1.5
Min, Max	-10, 3	-10, 6	-10, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Source Data: Listing 16.2.9.2A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	29	59
Mean (SD)	3.8 (3.94)	4.3 (3.81)	4.0 (3.85)
95% Confidence Interval of Mean	2.3, 5.2	2.8, 5.7	3.0, 5.0
Median	3.0	3.0	3.0
Q1, Q3	0.0, 6.0	1.0, 7.0	0.0, 6.0
Min, Max	0, 13	0, 11	0, 13
Change from Baseline			
n	29	29	58
Mean (SD)	-0.5 (2.61)	-1.8 (4.87)	-1.2 (3.93)
95% Confidence Interval of Mean	-1.5, 0.5	-3.7, 0.0	-2.2, -0.1
Median	-1.0	-2.0	-2.0
Q1, Q3	-2.0, 2.0	-4.0, 0.0	-4.0, 1.0
Min, Max	-5, 6	-13, 9	-13, 9

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	27	52
Mean (SD)	3.1 (3.19)	4.4 (4.13)	3.8 (3.73)
95% Confidence Interval of Mean	1.8, 4.4	2.7, 6.0	2.7, 4.8
Median	2.0	4.0	3.0
Q1, Q3	0.0, 5.0	1.0, 6.0	1.0, 6.0
Min, Max	0, 10	0, 17	0, 17
Change from Baseline			
n	24	27	51
Mean (SD)	-0.9 (3.06)	-1.7 (3.83)	-1.3 (3.48)
95% Confidence Interval of Mean	-2.2, 0.4	-3.2, -0.2	-2.3, -0.3
Median	-1.0	-2.0	-1.0
Q1, Q3	-2.0, 0.0	-3.0, 0.0	-3.0, 0.0
Min, Max	-9, 5	-13, 5	-13, 5

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	26	49
Mean (SD)	3.1 (2.82)	4.3 (4.51)	3.8 (3.82)
95% Confidence Interval of Mean	1.9, 4.3	2.5, 6.1	2.7, 4.9
Median	3.0	2.5	3.0
Q1, Q3	1.0, 5.0	1.0, 6.0	1.0, 6.0
Min, Max	0, 11	0, 17	0, 17
Change from Baseline			
n	23	26	49
Mean (SD)	-1.0 (3.27)	-1.8 (4.21)	-1.4 (3.77)
95% Confidence Interval of Mean	-2.5, 0.4	-3.5, -0.1	-2.5, -0.3
Median	-1.0	-2.0	-1.0
Q1, Q3	-2.0, 0.0	-4.0, -1.0	-4.0, 0.0
Min, Max	-9, 7	-8, 9	-9, 9

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	25	48
Mean (SD)	3.0 (3.35)	4.0 (3.66)	3.5 (3.51)
95% Confidence Interval of Mean	1.6, 4.5	2.5, 5.5	2.5, 4.6
Median	2.0	4.0	2.5
Q1, Q3	0.0, 5.0	0.0, 6.0	0.0, 6.0
Min, Max	0, 11	0, 12	0, 12
Change from Baseline			
n	22	25	47
Mean (SD)	-0.8 (1.84)	-2.0 (3.71)	-1.5 (3.02)
95% Confidence Interval of Mean	-1.6, -0.0	-3.6, -0.5	-2.4, -0.6
Median	-1.0	-1.0	-1.0
Q1, Q3	-2.0, 0.0	-5.0, 0.0	-3.0, 0.0
Min, Max	-5, 2	-8, 5	-8, 5

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	22	44
Mean (SD)	3.0 (3.48)	3.4 (3.40)	3.2 (3.41)
95% Confidence Interval of Mean	1.5, 4.5	1.9, 4.9	2.1, 4.2
Median	2.0	3.0	2.0
Q1, Q3	0.0, 6.0	0.0, 4.0	0.0, 4.5
Min, Max	0, 11	0, 12	0, 12
Change from Baseline			
n	21	22	43
Mean (SD)	-1.0 (2.44)	-2.4 (4.17)	-1.7 (3.46)
95% Confidence Interval of Mean	-2.2, 0.1	-4.3, -0.6	-2.8, -0.7
Median	-1.0	-3.0	-2.0
Q1, Q3	-2.0, 0.0	-5.0, 0.0	-3.0, 0.0
Min, Max	-9, 4	-12, 7	-12, 7

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	15	30
Mean (SD)	2.5 (2.53)	3.1 (3.31)	2.8 (2.91)
95% Confidence Interval of Mean	1.1, 3.9	1.3, 5.0	1.7, 3.9
Median	3.0	2.0	2.5
Q1, Q3	0.0, 4.0	0.0, 6.0	0.0, 5.0
Min, Max	0, 9	0, 11	0, 11
Change from Baseline			
n	15	15	30
Mean (SD)	-0.8 (1.42)	-2.0 (3.96)	-1.4 (2.99)
95% Confidence Interval of Mean	-1.6, -0.0	-4.2, 0.2	-2.5, -0.3
Median	-1.0	-2.0	-1.0
Q1, Q3	-2.0, 0.0	-5.0, 0.0	-2.0, 0.0
Min, Max	-3, 2	-11, 6	-11, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	8	19
Mean (SD)	2.5 (3.05)	4.1 (3.80)	3.2 (3.39)
95% Confidence Interval of Mean	0.4, 4.5	1.0, 7.3	1.5, 4.8
Median	1.0	4.0	3.0
Q1, Q3	0.0, 5.0	1.5, 5.0	0.0, 5.0
Min, Max	0, 9	0, 12	0, 12
Change from Baseline			
n	11	8	19
Mean (SD)	-0.2 (1.78)	-1.9 (5.74)	-0.9 (3.91)
95% Confidence Interval of Mean	-1.4, 1.0	-6.7, 2.9	-2.8, 1.0
Median	0.0	-1.5	0.0
Q1, Q3	-2.0, 1.0	-4.0, 1.0	-2.0, 1.0
Min, Max	-3, 3	-13, 7	-13, 7

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	6.5 (4.95)	5.0 (N/A)	6.0 (3.61)
95% Confidence Interval of Mean	-38.0, 51.0	N/A, N/A	-3.0, 15.0
Median	6.5	5.0	5.0
Q1, Q3	3.0, 10.0	5.0, 5.0	3.0, 10.0
Min, Max	3, 10	5, 5	3, 10
Change from Baseline			
n	2	1	3
Mean (SD)	2.5 (3.54)	-2.0 (N/A)	1.0 (3.61)
95% Confidence Interval of Mean	-29.3, 34.3	N/A, N/A	-8.0, 10.0
Median	2.5	-2.0	0.0
Q1, Q3	0.0, 5.0	-2.0, -2.0	-2.0, 5.0
Min, Max	0, 5	-2, -2	-2, 5

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Depressed Mood (Questions 5, 9, 10, 14, 18, 23, 28)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	16	11	27
Mean (SD)	2.4 (2.68)	3.5 (2.98)	2.9 (2.81)
95% Confidence Interval of Mean	0.9, 3.8	1.5, 5.5	1.7, 4.0
Median	1.5	4.0	2.0
Q1, Q3	0.0, 4.0	1.0, 5.0	0.0, 5.0
Min, Max	0, 8	0, 10	0, 10
Change from Baseline			
n	15	11	26
Mean (SD)	-1.1 (2.34)	-1.6 (3.75)	-1.3 (2.96)
95% Confidence Interval of Mean	-2.4, 0.2	-4.2, 0.9	-2.5, -0.1
Median	0.0	-2.0	-1.0
Q1, Q3	-2.0, 1.0	-5.0, 0.0	-3.0, 0.0
Min, Max	-7, 2	-7, 5	-7, 5

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	44	40	84
Mean (SD)	6.2 (4.32)	7.9 (4.58)	7.0 (4.51)
95% Confidence Interval of Mean	4.8, 7.5	6.4, 9.4	6.0, 8.0
Median	5.0	6.5	6.0
Q1, Q3	3.0, 9.0	4.5, 10.5	4.0, 10.0
Min, Max	0, 18	0, 18	0, 18

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 5 (Week 17)			
n	0	1	1
Mean (SD)		7.0 (N/A)	7.0 (N/A)
95% Confidence Interval of Mean		N/A, N/A	N/A, N/A
Median		7.0	7.0
Q1, Q3		7.0, 7.0	7.0, 7.0
Min, Max		7, 7	7, 7
Change from Baseline			
n	0	1	1
Mean (SD)		4.0 (N/A)	4.0 (N/A)
95% Confidence Interval of Mean		N/A, N/A	N/A, N/A
Median		4.0	4.0
Q1, Q3		4.0, 4.0	4.0, 4.0
Min, Max		4, 4	4, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	41	39	80
Mean (SD)	4.5 (3.98)	6.6 (4.24)	5.5 (4.22)
95% Confidence Interval of Mean	3.2, 5.7	5.3, 8.0	4.6, 6.5
Median	3.0	6.0	5.0
Q1, Q3	2.0, 7.0	3.0, 9.0	2.0, 9.0
Min, Max	0, 15	0, 17	0, 17
Change from Baseline			
n	40	38	78
Mean (SD)	-1.8 (2.75)	-1.5 (4.64)	-1.6 (3.77)
95% Confidence Interval of Mean	-2.7, -0.9	-3.0, 0.1	-2.5, -0.8
Median	-1.0	-1.0	-1.0
Q1, Q3	-4.0, 0.0	-3.0, 2.0	-3.0, 0.0
Min, Max	-9, 4	-13, 6	-13, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	35	37	72
Mean (SD)	5.5 (4.18)	5.6 (3.99)	5.6 (4.06)
95% Confidence Interval of Mean	4.1, 7.0	4.3, 7.0	4.6, 6.5
Median	6.0	6.0	6.0
Q1, Q3	2.0, 8.0	3.0, 7.0	2.0, 8.0
Min, Max	0, 16	0, 17	0, 17
Change from Baseline			
n	35	37	72
Mean (SD)	-0.7 (3.13)	-2.1 (4.44)	-1.4 (3.89)
95% Confidence Interval of Mean	-1.8, 0.3	-3.5, -0.6	-2.3, -0.5
Median	0.0	-1.0	-0.5
Q1, Q3	-3.0, 1.0	-4.0, 1.0	-3.0, 1.0
Min, Max	-9, 6	-16, 4	-16, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	31	64
Mean (SD)	5.1 (3.40)	6.0 (4.27)	5.5 (3.84)
95% Confidence Interval of Mean	3.9, 6.3	4.4, 7.6	4.6, 6.5
Median	4.0	5.0	5.0
Q1, Q3	3.0, 7.0	3.0, 9.0	3.0, 8.0
Min, Max	0, 13	0, 17	0, 17
Change from Baseline			
n	32	30	62
Mean (SD)	-1.6 (2.83)	-1.9 (5.46)	-1.7 (4.27)
95% Confidence Interval of Mean	-2.6, -0.6	-3.9, 0.2	-2.8, -0.7
Median	-2.0	-1.5	-2.0
Q1, Q3	-3.0, 0.0	-4.0, 1.0	-3.0, 1.0
Min, Max	-9, 5	-13, 11	-13, 11

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	29	27	56
Mean (SD)	5.3 (4.17)	5.0 (3.50)	5.2 (3.83)
95% Confidence Interval of Mean	3.8, 6.9	3.7, 6.4	4.2, 6.2
Median	5.0	5.0	5.0
Q1, Q3	2.0, 8.0	2.0, 8.0	2.0, 8.0
Min, Max	0, 16	0, 11	0, 16
Change from Baseline			
n	28	26	54
Mean (SD)	-1.5 (3.25)	-3.4 (4.39)	-2.4 (3.93)
95% Confidence Interval of Mean	-2.8, -0.2	-5.2, -1.6	-3.5, -1.4
Median	-1.0	-2.0	-2.0
Q1, Q3	-3.0, 0.5	-4.0, 0.0	-4.0, 0.0
Min, Max	-9, 6	-15, 3	-15, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	26	51
Mean (SD)	5.0 (4.30)	5.4 (3.66)	5.2 (3.95)
95% Confidence Interval of Mean	3.3, 6.8	3.9, 6.9	4.1, 6.3
Median	4.0	6.5	5.0
Q1, Q3	2.0, 9.0	2.0, 8.0	2.0, 9.0
Min, Max	0, 12	0, 11	0, 12
Change from Baseline			
n	24	25	49
Mean (SD)	-1.2 (2.81)	-2.8 (3.30)	-2.0 (3.15)
95% Confidence Interval of Mean	-2.4, 0.0	-4.2, -1.5	-2.9, -1.1
Median	-1.0	-2.0	-2.0
Q1, Q3	-3.0, 0.5	-4.0, -1.0	-3.0, 0.0
Min, Max	-9, 4	-11, 2	-11, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	25	48
Mean (SD)	4.3 (4.22)	5.6 (4.59)	5.0 (4.42)
95% Confidence Interval of Mean	2.5, 6.2	3.7, 7.5	3.7, 6.3
Median	3.0	6.0	4.0
Q1, Q3	0.0, 9.0	2.0, 8.0	2.0, 8.0
Min, Max	0, 14	0, 21	0, 21
Change from Baseline			
n	22	24	46
Mean (SD)	-2.0 (2.67)	-3.0 (5.09)	-2.5 (4.10)
95% Confidence Interval of Mean	-3.2, -0.8	-5.1, -0.8	-3.7, -1.3
Median	-1.5	-2.5	-2.0
Q1, Q3	-3.0, 0.0	-5.0, -0.5	-4.0, 0.0
Min, Max	-9, 2	-14, 10	-14, 10

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	22	44
Mean (SD)	4.4 (3.69)	5.6 (3.91)	5.0 (3.81)
95% Confidence Interval of Mean	2.8, 6.0	3.9, 7.4	3.9, 6.2
Median	3.5	5.5	5.0
Q1, Q3	2.0, 8.0	4.0, 7.0	2.0, 7.0
Min, Max	0, 13	0, 15	0, 15
Change from Baseline			
n	21	21	42
Mean (SD)	-2.0 (3.25)	-2.7 (5.22)	-2.4 (4.30)
95% Confidence Interval of Mean	-3.5, -0.6	-5.0, -0.3	-3.7, -1.0
Median	-2.0	-2.0	-2.0
Q1, Q3	-4.0, 0.0	-5.0, 0.0	-4.0, 0.0
Min, Max	-9, 3	-14, 6	-14, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	15	30
Mean (SD)	4.1 (3.77)	4.3 (3.28)	4.2 (3.47)
95% Confidence Interval of Mean	2.0, 6.2	2.4, 6.1	2.9, 5.5
Median	3.0	5.0	3.5
Q1, Q3	0.0, 9.0	1.0, 6.0	1.0, 6.0
Min, Max	0, 10	0, 9	0, 10
Change from Baseline			
n	15	15	30
Mean (SD)	-1.5 (2.33)	-3.3 (4.27)	-2.4 (3.51)
95% Confidence Interval of Mean	-2.8, -0.2	-5.7, -1.0	-3.7, -1.1
Median	-1.0	-3.0	-2.0
Q1, Q3	-4.0, 0.0	-5.0, 0.0	-4.0, 0.0
Min, Max	-5, 3	-15, 1	-15, 3

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	8	19
Mean (SD)	3.9 (5.05)	5.0 (4.34)	4.4 (4.67)
95% Confidence Interval of Mean	0.5, 7.3	1.4, 8.6	2.1, 6.6
Median	2.0	4.0	3.0
Q1, Q3	0.0, 7.0	1.5, 8.5	0.0, 7.0
Min, Max	0, 15	0, 12	0, 15
Change from Baseline			
n	11	8	19
Mean (SD)	-0.4 (2.58)	-2.3 (5.50)	-1.2 (4.05)
95% Confidence Interval of Mean	-2.1, 1.4	-6.8, 2.3	-3.1, 0.8
Median	0.0	-2.0	-1.0
Q1, Q3	-3.0, 1.0	-3.5, -0.5	-3.0, 0.0
Min, Max	-4, 5	-13, 7	-13, 7

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	7.0 (4.24)	5.0 (N/A)	6.3 (3.21)
95% Confidence Interval of Mean	-31.1, 45.1	N/A, N/A	-1.7, 14.3
Median	7.0	5.0	5.0
Q1, Q3	4.0, 10.0	5.0, 5.0	4.0, 10.0
Min, Max	4, 10	5, 5	4, 10
Change from Baseline			
n	2	1	3
Mean (SD)	1.0 (0.00)	-4.0 (N/A)	-0.7 (2.89)
95% Confidence Interval of Mean	1.0, 1.0	N/A, N/A	-7.8, 6.5
Median	1.0	-4.0	1.0
Q1, Q3	1.0, 1.0	-4.0, -4.0	-4.0, 1.0
Min, Max	1, 1	-4, -4	-4, 1

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Social Avoidance (Questions 2, 6, 13, 19, 21, 25, 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	11	28
Mean (SD)	4.4 (3.74)	4.5 (3.96)	4.4 (3.76)
95% Confidence Interval of Mean	2.4, 6.3	1.9, 7.2	3.0, 5.9
Median	3.0	3.0	3.0
Q1, Q3	2.0, 8.0	1.0, 9.0	1.5, 8.5
Min, Max	0, 10	0, 10	0, 10
Change from Baseline			
n	16	10	26
Mean (SD)	-1.3 (2.32)	-2.7 (4.24)	-1.8 (3.20)
95% Confidence Interval of Mean	-2.5, -0.0	-5.7, 0.3	-3.1, -0.5
Median	-1.5	-2.5	-2.0
Q1, Q3	-3.0, 0.0	-5.0, 0.0	-3.0, 0.0
Min, Max	-6, 3	-10, 4	-10, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	44	41	85
Mean (SD)	4.0 (3.57)	4.2 (3.66)	4.1 (3.59)
95% Confidence Interval of Mean	2.9, 5.1	3.0, 5.3	3.3, 4.9
Median	3.0	3.0	3.0
Q1, Q3	2.0, 5.0	2.0, 6.0	2.0, 6.0
Min, Max	0, 16	0, 14	0, 16

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 5 (Week 17)			
n	0	1	1
Mean (SD)		3.0 (N/A)	3.0 (N/A)
95% Confidence Interval of Mean		N/A, N/A	N/A, N/A
Median		3.0	3.0
Q1, Q3		3.0, 3.0	3.0, 3.0
Min, Max		3, 3	3, 3
Change from Baseline			
n	0	1	1
Mean (SD)		-2.0 (N/A)	-2.0 (N/A)
95% Confidence Interval of Mean		N/A, N/A	N/A, N/A
Median		-2.0	-2.0
Q1, Q3		-2.0, -2.0	-2.0, -2.0
Min, Max		-2, -2	-2, -2

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	41	39	80
Mean (SD)	2.2 (3.36)	3.2 (3.33)	2.7 (3.36)
95% Confidence Interval of Mean	1.1, 3.3	2.1, 4.3	1.9, 3.4
Median	1.0	2.0	1.0
Q1, Q3	0.0, 3.0	1.0, 5.0	0.0, 4.0
Min, Max	0, 16	0, 11	0, 16
Change from Baseline			
n	40	39	79
Mean (SD)	-1.9 (2.78)	-1.1 (3.37)	-1.5 (3.09)
95% Confidence Interval of Mean	-2.8, -1.0	-2.2, -0.0	-2.2, -0.8
Median	-2.0	-1.0	-1.0
Q1, Q3	-3.0, 0.0	-3.0, 0.0	-3.0, 0.0
Min, Max	-14, 5	-10, 8	-14, 8

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-adams-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	36	38	74
Mean (SD)	2.6 (3.70)	2.7 (2.42)	2.7 (3.08)
95% Confidence Interval of Mean	1.4, 3.9	1.9, 3.5	1.9, 3.4
Median	1.0	2.0	2.0
Q1, Q3	0.0, 3.5	1.0, 4.0	0.0, 4.0
Min, Max	0, 15	0, 9	0, 15
Change from Baseline			
n	36	38	74
Mean (SD)	-1.4 (3.17)	-1.4 (3.59)	-1.4 (3.37)
95% Confidence Interval of Mean	-2.5, -0.3	-2.6, -0.3	-2.2, -0.6
Median	-1.0	-1.0	-1.0
Q1, Q3	-3.5, 0.0	-3.0, 0.0	-3.0, 0.0
Min, Max	-13, 8	-10, 6	-13, 8

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	32	65
Mean (SD)	2.5 (3.64)	2.8 (3.11)	2.7 (3.36)
95% Confidence Interval of Mean	1.3, 3.8	1.7, 3.9	1.8, 3.5
Median	2.0	2.0	2.0
Q1, Q3	0.0, 3.0	0.5, 4.0	0.0, 3.0
Min, Max	0, 15	0, 12	0, 15
Change from Baseline			
n	32	32	64
Mean (SD)	-1.5 (2.77)	-1.5 (3.47)	-1.5 (3.12)
95% Confidence Interval of Mean	-2.5, -0.5	-2.7, -0.2	-2.3, -0.7
Median	-1.5	-1.0	-1.0
Q1, Q3	-2.5, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-12, 3	-10, 7	-12, 7

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	29	59
Mean (SD)	2.6 (3.49)	2.4 (2.63)	2.5 (3.07)
95% Confidence Interval of Mean	1.3, 3.9	1.4, 3.4	1.7, 3.3
Median	2.0	2.0	2.0
Q1, Q3	0.0, 3.0	1.0, 3.0	0.0, 3.0
Min, Max	0, 13	0, 9	0, 13
Change from Baseline			
n	29	29	58
Mean (SD)	-1.3 (2.37)	-2.1 (3.55)	-1.7 (3.02)
95% Confidence Interval of Mean	-2.2, -0.4	-3.4, -0.7	-2.5, -0.9
Median	-1.0	-1.0	-1.0
Q1, Q3	-3.0, 0.0	-3.0, 0.0	-3.0, 0.0
Min, Max	-7, 5	-12, 5	-12, 5

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	27	52
Mean (SD)	2.6 (3.16)	3.3 (3.58)	2.9 (3.37)
95% Confidence Interval of Mean	1.3, 3.9	1.8, 4.7	2.0, 3.9
Median	2.0	2.0	2.0
Q1, Q3	1.0, 3.0	0.0, 6.0	0.0, 4.0
Min, Max	0, 11	0, 13	0, 13
Change from Baseline			
n	24	27	51
Mean (SD)	-1.0 (2.14)	-1.5 (3.38)	-1.3 (2.84)
95% Confidence Interval of Mean	-1.9, -0.1	-2.9, -0.2	-2.1, -0.5
Median	-1.5	-1.0	-1.0
Q1, Q3	-2.0, 0.5	-3.0, 0.0	-3.0, 0.0
Min, Max	-5, 4	-11, 5	-11, 5

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	26	49
Mean (SD)	2.0 (3.59)	3.3 (4.24)	2.7 (3.96)
95% Confidence Interval of Mean	0.4, 3.6	1.6, 5.0	1.5, 3.8
Median	1.0	1.0	1.0
Q1, Q3	0.0, 2.0	0.0, 4.0	0.0, 3.0
Min, Max	0, 16	0, 13	0, 16
Change from Baseline			
n	23	26	49
Mean (SD)	-1.7 (1.86)	-1.4 (2.89)	-1.6 (2.44)
95% Confidence Interval of Mean	-2.5, -0.9	-2.6, -0.3	-2.3, -0.9
Median	-2.0	-1.0	-1.0
Q1, Q3	-3.0, 0.0	-3.0, 1.0	-3.0, 0.0
Min, Max	-6, 0	-10, 4	-10, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	25	48
Mean (SD)	2.5 (3.98)	3.6 (4.16)	3.1 (4.07)
95% Confidence Interval of Mean	0.8, 4.2	1.9, 5.3	1.9, 4.3
Median	1.0	2.0	1.0
Q1, Q3	0.0, 3.0	1.0, 4.0	0.0, 4.0
Min, Max	0, 15	0, 14	0, 15
Change from Baseline			
n	22	25	47
Mean (SD)	-1.2 (2.11)	-1.4 (3.16)	-1.3 (2.69)
95% Confidence Interval of Mean	-2.2, -0.3	-2.7, -0.1	-2.1, -0.5
Median	-1.0	-1.0	-1.0
Q1, Q3	-3.0, 0.0	-3.0, 1.0	-3.0, 0.0
Min, Max	-7, 2	-10, 4	-10, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	22	44
Mean (SD)	2.6 (4.10)	2.8 (3.87)	2.7 (3.94)
95% Confidence Interval of Mean	0.8, 4.4	1.1, 4.5	1.5, 3.9
Median	1.0	1.0	1.0
Q1, Q3	0.0, 4.0	0.0, 4.0	0.0, 4.0
Min, Max	0, 14	0, 14	0, 14
Change from Baseline			
n	21	22	43
Mean (SD)	-1.3 (2.71)	-2.1 (3.65)	-1.7 (3.21)
95% Confidence Interval of Mean	-2.6, -0.1	-3.7, -0.5	-2.7, -0.7
Median	-2.0	-2.0	-2.0
Q1, Q3	-3.0, 0.0	-4.0, 0.0	-3.0, 0.0
Min, Max	-7, 6	-10, 5	-10, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	15	30
Mean (SD)	1.7 (3.24)	2.5 (3.18)	2.1 (3.18)
95% Confidence Interval of Mean	-0.1, 3.5	0.7, 4.2	0.9, 3.3
Median	0.0	1.0	0.5
Q1, Q3	0.0, 2.0	0.0, 4.0	0.0, 4.0
Min, Max	0, 12	0, 9	0, 12
Change from Baseline			
n	15	15	30
Mean (SD)	-2.0 (2.17)	-1.3 (3.37)	-1.7 (2.81)
95% Confidence Interval of Mean	-3.2, -0.8	-3.2, 0.5	-2.7, -0.6
Median	-2.0	-1.0	-1.5
Q1, Q3	-4.0, 0.0	-3.0, 1.0	-3.0, 0.0
Min, Max	-7, 1	-8, 5	-8, 5

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	8	19
Mean (SD)	1.5 (3.01)	3.3 (5.15)	2.2 (4.02)
95% Confidence Interval of Mean	-0.6, 3.5	-1.1, 7.6	0.3, 4.1
Median	0.0	1.5	0.0
Q1, Q3	0.0, 2.0	0.0, 4.0	0.0, 2.0
Min, Max	0, 10	0, 15	0, 15
Change from Baseline			
n	11	8	19
Mean (SD)	-1.8 (1.47)	-0.4 (4.81)	-1.2 (3.28)
95% Confidence Interval of Mean	-2.8, -0.8	-4.4, 3.6	-2.8, 0.4
Median	-2.0	-1.0	-2.0
Q1, Q3	-3.0, 0.0	-2.5, 3.5	-3.0, 0.0
Min, Max	-4, 0	-9, 6	-9, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	3.5 (2.12)	0.0 (N/A)	2.3 (2.52)
95% Confidence Interval of Mean	-15.6, 22.6	N/A, N/A	-3.9, 8.6
Median	3.5	0.0	2.0
Q1, Q3	2.0, 5.0	0.0, 0.0	0.0, 5.0
Min, Max	2, 5	0, 0	0, 5
Change from Baseline			
n	2	1	3
Mean (SD)	-1.0 (1.41)	-3.0 (N/A)	-1.7 (1.53)
95% Confidence Interval of Mean	-13.7, 11.7	N/A, N/A	-5.5, 2.1
Median	-1.0	-3.0	-2.0
Q1, Q3	-2.0, 0.0	-3.0, -3.0	-3.0, 0.0
Min, Max	-2, 0	-3, -3	-3, 0

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: General Anxiety (Questions 1, 3, 7, 11, 15, 24, 26)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	11	28
Mean (SD)	1.8 (2.74)	3.2 (3.87)	2.4 (3.23)
95% Confidence Interval of Mean	0.4, 3.2	0.6, 5.8	1.1, 3.6
Median	0.0	2.0	1.0
Q1, Q3	0.0, 3.0	0.0, 6.0	0.0, 4.0
Min, Max	0, 9	0, 12	0, 12
Change from Baseline			
n	16	11	27
Mean (SD)	-1.3 (1.66)	-0.3 (2.80)	-0.9 (2.21)
95% Confidence Interval of Mean	-2.2, -0.4	-2.2, 1.6	-1.8, -0.0
Median	-1.0	-1.0	-1.0
Q1, Q3	-2.5, 0.0	-2.0, 2.0	-2.0, 0.0
Min, Max	-4, 2	-5, 5	-5, 5

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	44	41	85
Mean (SD)	2.1 (2.49)	2.3 (2.26)	2.2 (2.37)
95% Confidence Interval of Mean	1.3, 2.8	1.6, 3.0	1.7, 2.7
Median	1.5	2.0	2.0
Q1, Q3	0.0, 3.5	0.0, 4.0	0.0, 4.0
Min, Max	0, 9	0, 6	0, 9

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-adams-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 5 (Week 17)			
n	0	1	1
Mean (SD)		3.0 (N/A)	3.0 (N/A)
95% Confidence Interval of Mean		N/A, N/A	N/A, N/A
Median		3.0	3.0
Q1, Q3		3.0, 3.0	3.0, 3.0
Min, Max		3, 3	3, 3
Change from Baseline			
n	0	1	1
Mean (SD)		1.0 (N/A)	1.0 (N/A)
95% Confidence Interval of Mean		N/A, N/A	N/A, N/A
Median		1.0	1.0
Q1, Q3		1.0, 1.0	1.0, 1.0
Min, Max		1, 1	1, 1

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	41	39	80
Mean (SD)	1.6 (2.38)	1.7 (2.03)	1.6 (2.20)
95% Confidence Interval of Mean	0.8, 2.3	1.1, 2.4	1.1, 2.1
Median	0.0	1.0	1.0
Q1, Q3	0.0, 3.0	0.0, 4.0	0.0, 3.0
Min, Max	0, 9	0, 7	0, 9
Change from Baseline			
n	40	39	79
Mean (SD)	-0.7 (1.93)	-0.7 (1.66)	-0.7 (1.79)
95% Confidence Interval of Mean	-1.3, -0.0	-1.2, -0.2	-1.1, -0.3
Median	0.0	-1.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-7, 3	-6, 2	-7, 3

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	37	38	75
Mean (SD)	1.7 (2.38)	1.2 (1.77)	1.5 (2.09)
95% Confidence Interval of Mean	0.9, 2.5	0.6, 1.8	1.0, 1.9
Median	1.0	0.0	1.0
Q1, Q3	0.0, 3.0	0.0, 2.0	0.0, 2.0
Min, Max	0, 9	0, 6	0, 9
Change from Baseline			
n	36	38	74
Mean (SD)	-0.4 (2.02)	-1.1 (1.77)	-0.8 (1.92)
95% Confidence Interval of Mean	-1.1, 0.3	-1.7, -0.5	-1.2, -0.3
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-2.0, 0.0	-1.0, 0.0
Min, Max	-5, 6	-6, 1	-6, 6

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	31	64
Mean (SD)	1.8 (2.20)	1.4 (1.96)	1.6 (2.08)
95% Confidence Interval of Mean	1.0, 2.6	0.7, 2.1	1.1, 2.1
Median	1.0	0.0	1.0
Q1, Q3	0.0, 2.0	0.0, 2.0	0.0, 2.0
Min, Max	0, 9	0, 6	0, 9
Change from Baseline			
n	32	31	63
Mean (SD)	-0.5 (2.27)	-1.0 (1.92)	-0.8 (2.11)
95% Confidence Interval of Mean	-1.4, 0.3	-1.7, -0.3	-1.3, -0.2
Median	0.0	-1.0	0.0
Q1, Q3	-1.5, 1.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-6, 3	-6, 2	-6, 3

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	29	28	57
Mean (SD)	1.7 (2.25)	1.1 (1.94)	1.4 (2.10)
95% Confidence Interval of Mean	0.8, 2.5	0.4, 1.9	0.9, 2.0
Median	1.0	0.0	0.0
Q1, Q3	0.0, 3.0	0.0, 2.0	0.0, 2.0
Min, Max	0, 9	0, 9	0, 9
Change from Baseline			
n	28	28	56
Mean (SD)	-0.4 (1.20)	-1.4 (2.30)	-0.9 (1.88)
95% Confidence Interval of Mean	-0.9, 0.1	-2.2, -0.5	-1.4, -0.4
Median	0.0	-0.5	0.0
Q1, Q3	-1.0, 0.0	-3.0, 0.0	-1.5, 0.0
Min, Max	-3, 3	-6, 3	-6, 3

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	24	27	51
Mean (SD)	1.4 (1.74)	1.4 (1.85)	1.4 (1.78)
95% Confidence Interval of Mean	0.6, 2.1	0.7, 2.2	0.9, 1.9
Median	0.0	0.0	0.0
Q1, Q3	0.0, 2.5	0.0, 3.0	0.0, 3.0
Min, Max	0, 6	0, 5	0, 6
Change from Baseline			
n	23	27	50
Mean (SD)	-0.5 (1.78)	-1.3 (2.01)	-0.9 (1.93)
95% Confidence Interval of Mean	-1.3, 0.2	-2.1, -0.5	-1.5, -0.4
Median	0.0	-1.0	0.0
Q1, Q3	-2.0, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-4, 4	-6, 3	-6, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	26	49
Mean (SD)	1.4 (1.78)	1.6 (1.75)	1.5 (1.75)
95% Confidence Interval of Mean	0.6, 2.2	0.9, 2.3	1.0, 2.0
Median	0.0	1.0	1.0
Q1, Q3	0.0, 2.0	0.0, 3.0	0.0, 3.0
Min, Max	0, 6	0, 5	0, 6
Change from Baseline			
n	23	26	49
Mean (SD)	-0.6 (1.83)	-1.2 (1.90)	-0.9 (1.87)
95% Confidence Interval of Mean	-1.4, 0.2	-2.0, -0.4	-1.4, -0.4
Median	0.0	-1.0	0.0
Q1, Q3	-2.0, 0.0	-2.0, 0.0	-2.0, 0.0
Min, Max	-5, 4	-6, 2	-6, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	25	48
Mean (SD)	1.7 (2.63)	1.5 (1.64)	1.6 (2.15)
95% Confidence Interval of Mean	0.6, 2.9	0.8, 2.2	1.0, 2.2
Median	0.0	1.0	1.0
Q1, Q3	0.0, 2.0	0.0, 3.0	0.0, 2.5
Min, Max	0, 9	0, 5	0, 9
Change from Baseline			
n	22	25	47
Mean (SD)	-0.3 (1.83)	-1.4 (2.10)	-0.9 (2.03)
95% Confidence Interval of Mean	-1.1, 0.5	-2.2, -0.5	-1.4, -0.3
Median	0.0	-1.0	0.0
Q1, Q3	-1.0, 0.0	-3.0, 0.0	-2.0, 0.0
Min, Max	-4, 4	-6, 2	-6, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	22	44
Mean (SD)	1.7 (2.10)	1.6 (2.15)	1.7 (2.10)
95% Confidence Interval of Mean	0.8, 2.7	0.6, 2.5	1.0, 2.3
Median	1.0	0.0	0.5
Q1, Q3	0.0, 3.0	0.0, 3.0	0.0, 3.0
Min, Max	0, 7	0, 6	0, 7
Change from Baseline			
n	21	22	43
Mean (SD)	-0.3 (1.56)	-1.0 (2.14)	-0.7 (1.89)
95% Confidence Interval of Mean	-1.0, 0.4	-1.9, -0.1	-1.3, -0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-4, 4	-6, 2	-6, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	14	15	29
Mean (SD)	1.7 (2.05)	1.3 (2.13)	1.5 (2.06)
95% Confidence Interval of Mean	0.5, 2.9	0.2, 2.5	0.7, 2.3
Median	1.0	0.0	0.0
Q1, Q3	0.0, 3.0	0.0, 2.0	0.0, 2.0
Min, Max	0, 6	0, 6	0, 6
Change from Baseline			
n	14	15	29
Mean (SD)	-0.4 (1.09)	-0.7 (1.22)	-0.6 (1.15)
95% Confidence Interval of Mean	-1.1, 0.2	-1.4, -0.1	-1.0, -0.1
Median	0.0	0.0	0.0
Q1, Q3	-1.0, 0.0	-1.0, 0.0	-1.0, 0.0
Min, Max	-3, 1	-4, 1	-4, 1

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	8	19
Mean (SD)	1.3 (2.05)	1.6 (1.92)	1.4 (1.95)
95% Confidence Interval of Mean	-0.1, 2.7	0.0, 3.2	0.5, 2.4
Median	0.0	1.0	0.0
Q1, Q3	0.0, 2.0	0.0, 3.0	0.0, 2.0
Min, Max	0, 6	0, 5	0, 6
Change from Baseline			
n	11	8	19
Mean (SD)	-0.5 (1.04)	-0.8 (1.28)	-0.6 (1.12)
95% Confidence Interval of Mean	-1.2, 0.2	-1.8, 0.3	-1.2, -0.1
Median	0.0	-0.5	0.0
Q1, Q3	-1.0, 0.0	-1.5, 0.0	-1.0, 0.0
Min, Max	-3, 0	-3, 1	-3, 1

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
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Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	1.5 (2.12)	0.0 (N/A)	1.0 (1.73)
95% Confidence Interval of Mean	-17.6, 20.6	N/A, N/A	-3.3, 5.3
Median	1.5	0.0	0.0
Q1, Q3	0.0, 3.0	0.0, 0.0	0.0, 3.0
Min, Max	0, 3	0, 0	0, 3
Change from Baseline			
n	2	1	3
Mean (SD)	0.5 (0.71)	-1.0 (N/A)	0.0 (1.00)
95% Confidence Interval of Mean	-5.9, 6.9	N/A, N/A	-2.5, 2.5
Median	0.5	-1.0	0.0
Q1, Q3	0.0, 1.0	-1.0, -1.0	-1.0, 1.0
Min, Max	0, 1	-1, -1	-1, 1

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-adams-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

Table 14.2.6.2A
Summary of Anxiety Depression and Mood Scale (ADAMS)
(Open-Label Extension Phase, Intent-to-Treat Population)

Subscale: Obsessive/Compulsive Behavior (Questions 8, 16, 20)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	11	28
Mean (SD)	1.5 (1.94)	1.4 (2.73)	1.4 (2.23)
95% Confidence Interval of Mean	0.5, 2.5	-0.5, 3.2	0.6, 2.3
Median	1.0	0.0	0.0
Q1, Q3	0.0, 2.0	0.0, 2.0	0.0, 2.0
Min, Max	0, 7	0, 9	0, 9
Change from Baseline			
n	16	11	27
Mean (SD)	-0.3 (1.61)	-0.9 (2.17)	-0.5 (1.85)
95% Confidence Interval of Mean	-1.1, 0.6	-2.4, 0.5	-1.2, 0.2
Median	0.0	0.0	0.0
Q1, Q3	-0.5, 0.0	-2.0, 0.0	-1.0, 0.0
Min, Max	-3, 4	-5, 3	-5, 4

Abbreviation: N/A=Not applicable.
Note: The questions are based on subject's behavior during the past 6 months at Baseline and since the last visit at subsequent visits.
[1]Last non-missing value prior to the first dose of the DB phase.
[2]Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.2A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-adams-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-02a-adams-ole.rtf

4.8.7 Ergebnisse zum Endpunkt Lebensqualität – gemäß dem Quality of Life Inventory-Disability (QI-Disability) während der OLE

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	44	43	87
Mean (SD)	66.34 (17.791)	62.21 (16.250)	64.30 (17.074)
95% Confidence Interval of Mean	60.93, 71.74	57.21, 67.21	60.66, 67.93
Median	68.75	62.50	68.75
Q1, Q3	56.25, 75.00	50.00, 68.75	56.25, 75.00
Min, Max	18.8, 100.0	25.0, 93.8	18.8, 100.0

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	42	42	84
Mean (SD)	66.22 (17.189)	66.82 (16.068)	66.52 (16.540)
95% Confidence Interval of Mean	60.86, 71.58	61.81, 71.82	62.93, 70.11
Median	68.75	68.75	68.75
Q1, Q3	56.25, 75.00	56.25, 75.00	56.25, 75.00
Min, Max	18.8, 100.0	31.3, 100.0	18.8, 100.0
Change from Baseline			
n	41	42	83
Mean (SD)	0.61 (19.355)	4.61 (17.078)	2.64 (18.238)
95% Confidence Interval of Mean	-5.50, 6.72	-0.71, 9.93	-1.35, 6.62
Median	0.00	3.13	0.00
Q1, Q3	-12.50, 12.50	-6.25, 12.50	-6.25, 12.50
Min, Max	-50.0, 50.0	-31.3, 56.3	-50.0, 56.3

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	38	39	77
Mean (SD)	68.91 (15.884)	65.38 (17.316)	67.13 (16.610)
95% Confidence Interval of Mean	63.69, 74.14	59.77, 71.00	63.36, 70.90
Median	71.88	62.50	68.75
Q1, Q3	56.25, 81.25	56.25, 81.25	56.25, 81.25
Min, Max	31.3, 100.0	25.0, 100.0	25.0, 100.0
Change from Baseline			
n	37	39	76
Mean (SD)	3.72 (16.302)	2.88 (18.241)	3.29 (17.214)
95% Confidence Interval of Mean	-1.72, 9.15	-3.03, 8.80	-0.64, 7.22
Median	0.00	0.00	0.00
Q1, Q3	-6.25, 18.75	-6.25, 12.50	-6.25, 12.50
Min, Max	-31.3, 37.5	-56.3, 62.5	-56.3, 62.5

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	33	66
Mean (SD)	71.02 (15.053)	65.34 (16.835)	68.18 (16.102)
95% Confidence Interval of Mean	65.68, 76.36	59.37, 71.31	64.22, 72.14
Median	75.00	68.75	68.75
Q1, Q3	62.50, 81.25	50.00, 75.00	50.00, 81.25
Min, Max	37.5, 100.0	37.5, 100.0	37.5, 100.0
Change from Baseline			
n	32	33	65
Mean (SD)	5.27 (20.583)	2.08 (16.877)	3.65 (18.714)
95% Confidence Interval of Mean	-2.15, 12.69	-3.90, 8.07	-0.98, 8.29
Median	6.25	0.00	0.00
Q1, Q3	-12.50, 18.75	-6.25, 12.50	-12.50, 12.50
Min, Max	-31.3, 43.8	-25.0, 56.3	-31.3, 56.3

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	30	60
Mean (SD)	68.96 (16.038)	67.50 (17.555)	68.23 (16.687)
95% Confidence Interval of Mean	62.97, 74.95	60.94, 74.06	63.92, 72.54
Median	68.75	68.75	68.75
Q1, Q3	62.50, 81.25	56.25, 81.25	56.25, 81.25
Min, Max	37.5, 100.0	31.3, 100.0	31.3, 100.0
Change from Baseline			
n	29	30	59
Mean (SD)	1.51 (19.166)	5.21 (18.209)	3.39 (18.617)
95% Confidence Interval of Mean	-5.78, 8.80	-1.59, 12.01	-1.46, 8.24
Median	0.00	3.13	0.00
Q1, Q3	-12.50, 12.50	-6.25, 12.50	-6.25, 12.50
Min, Max	-31.3, 37.5	-25.0, 68.8	-31.3, 68.8

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	28	53
Mean (SD)	68.00 (17.052)	70.54 (17.748)	69.34 (17.302)
95% Confidence Interval of Mean	60.96, 75.04	63.65, 77.42	64.57, 74.11
Median	68.75	68.75	68.75
Q1, Q3	56.25, 75.00	62.50, 84.38	62.50, 81.25
Min, Max	31.3, 100.0	37.5, 100.0	31.3, 100.0
Change from Baseline			
n	25	28	53
Mean (SD)	3.75 (15.309)	8.26 (19.695)	6.13 (17.741)
95% Confidence Interval of Mean	-2.57, 10.07	0.62, 15.90	1.24, 11.02
Median	0.00	6.25	6.25
Q1, Q3	-6.25, 12.50	-6.25, 21.88	-6.25, 18.75
Min, Max	-18.8, 31.3	-25.0, 68.8	-25.0, 68.8

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	27	50
Mean (SD)	70.38 (15.678)	70.60 (15.870)	70.50 (15.621)
95% Confidence Interval of Mean	63.60, 77.16	64.32, 76.88	66.06, 74.94
Median	68.75	68.75	68.75
Q1, Q3	62.50, 81.25	56.25, 81.25	62.50, 81.25
Min, Max	43.8, 100.0	37.5, 100.0	37.5, 100.0
Change from Baseline			
n	23	27	50
Mean (SD)	5.43 (15.228)	8.56 (17.434)	7.13 (16.367)
95% Confidence Interval of Mean	-1.15, 12.02	1.67, 15.46	2.47, 11.78
Median	0.00	6.25	6.25
Q1, Q3	-6.25, 12.50	0.00, 25.00	0.00, 12.50
Min, Max	-12.5, 43.8	-25.0, 62.5	-25.0, 62.5

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	26	49
Mean (SD)	71.74 (14.942)	64.66 (16.292)	67.98 (15.916)
95% Confidence Interval of Mean	65.28, 78.20	58.08, 71.24	63.41, 72.56
Median	75.00	62.50	68.75
Q1, Q3	62.50, 81.25	56.25, 75.00	56.25, 75.00
Min, Max	43.8, 100.0	37.5, 100.0	37.5, 100.0
Change from Baseline			
n	23	26	49
Mean (SD)	4.89 (14.833)	3.13 (18.729)	3.95 (16.862)
95% Confidence Interval of Mean	-1.52, 11.31	-4.44, 10.69	-0.89, 8.80
Median	6.25	3.13	6.25
Q1, Q3	0.00, 12.50	-6.25, 12.50	-6.25, 12.50
Min, Max	-37.5, 37.5	-25.0, 62.5	-37.5, 62.5

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	23	45
Mean (SD)	74.15 (16.286)	66.58 (15.380)	70.28 (16.109)
95% Confidence Interval of Mean	66.93, 81.37	59.93, 73.23	65.44, 75.12
Median	78.13	68.75	68.75
Q1, Q3	62.50, 87.50	56.25, 81.25	56.25, 81.25
Min, Max	43.8, 100.0	37.5, 93.8	37.5, 100.0
Change from Baseline			
n	22	23	45
Mean (SD)	7.39 (17.849)	4.35 (16.149)	5.83 (16.876)
95% Confidence Interval of Mean	-0.53, 15.30	-2.64, 11.33	0.76, 10.90
Median	6.25	6.25	6.25
Q1, Q3	0.00, 12.50	-6.25, 12.50	-6.25, 12.50
Min, Max	-25.0, 43.8	-18.8, 56.3	-25.0, 56.3

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	74.17 (14.536)	67.97 (19.348)	70.97 (17.196)
95% Confidence Interval of Mean	66.12, 82.22	57.66, 78.28	64.66, 77.28
Median	75.00	68.75	75.00
Q1, Q3	62.50, 87.50	56.25, 81.25	62.50, 81.25
Min, Max	43.8, 100.0	37.5, 100.0	37.5, 100.0
Change from Baseline			
n	15	16	31
Mean (SD)	4.17 (16.480)	4.69 (17.751)	4.44 (16.863)
95% Confidence Interval of Mean	-4.96, 13.29	-4.77, 14.15	-1.75, 10.62
Median	0.00	6.25	0.00
Q1, Q3	-6.25, 18.75	-6.25, 12.50	-6.25, 12.50
Min, Max	-18.8, 37.5	-18.8, 56.3	-18.8, 56.3

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	73.30 (16.557)	65.97 (18.517)	70.00 (17.396)
95% Confidence Interval of Mean	62.17, 84.42	51.74, 80.21	61.86, 78.14
Median	75.00	62.50	71.88
Q1, Q3	62.50, 87.50	50.00, 75.00	53.13, 84.38
Min, Max	43.8, 100.0	43.8, 93.8	43.8, 100.0
Change from Baseline			
n	11	9	20
Mean (SD)	0.00 (18.114)	3.47 (12.148)	1.56 (15.426)
95% Confidence Interval of Mean	-12.17, 12.17	-5.87, 12.81	-5.66, 8.78
Median	0.00	0.00	0.00
Q1, Q3	-12.50, 12.50	0.00, 6.25	-6.25, 6.25
Min, Max	-37.5, 31.3	-12.5, 31.3	-37.5, 31.3

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	65.63 (4.419)	87.50 (N/A)	72.92 (13.010)
95% Confidence Interval of Mean	25.92, 105.33	N/A, N/A	40.60, 105.24
Median	65.63	87.50	68.75
Q1, Q3	62.50, 68.75	87.50, 87.50	62.50, 87.50
Min, Max	62.5, 68.8	87.5, 87.5	62.5, 87.5
Change from Baseline			
n	2	1	3
Mean (SD)	-6.25 (17.678)	18.75 (N/A)	2.08 (19.094)
95% Confidence Interval of Mean	-165.08, 152.58	N/A, N/A	-45.35, 49.52
Median	-6.25	18.75	6.25
Q1, Q3	-18.75, 6.25	18.75, 18.75	-18.75, 18.75
Min, Max	-18.8, 6.3	18.8, 18.8	-18.8, 18.8

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Physical Health (Items 1-4)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	73.53 (21.484)	69.79 (16.174)	71.98 (19.236)
95% Confidence Interval of Mean	62.48, 84.58	59.52, 80.07	64.67, 79.30
Median	81.25	62.50	75.00
Q1, Q3	56.25, 87.50	59.38, 84.38	56.25, 87.50
Min, Max	25.0, 100.0	43.8, 93.8	25.0, 100.0
Change from Baseline			
n	17	12	29
Mean (SD)	3.68 (19.271)	4.17 (14.678)	3.88 (17.231)
95% Confidence Interval of Mean	-6.23, 13.58	-5.16, 13.49	-2.68, 10.43
Median	0.00	6.25	6.25
Q1, Q3	0.00, 12.50	-9.38, 12.50	-6.25, 12.50
Min, Max	-50.0, 37.5	-18.8, 31.3	-50.0, 37.5

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	44	42	86
Mean (SD)	60.23 (25.003)	54.61 (24.502)	57.49 (24.775)
95% Confidence Interval of Mean	52.63, 67.83	46.98, 62.25	52.17, 62.80
Median	62.50	50.00	56.25
Q1, Q3	50.00, 75.00	43.75, 68.75	43.75, 75.00
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	42	41	83
Mean (SD)	61.01 (22.758)	58.99 (23.009)	60.02 (22.765)
95% Confidence Interval of Mean	53.92, 68.10	51.73, 66.26	55.04, 64.99
Median	62.50	56.25	56.25
Q1, Q3	50.00, 75.00	43.75, 75.00	43.75, 75.00
Min, Max	0.0, 100.0	12.5, 100.0	0.0, 100.0
Change from Baseline			
n	41	41	82
Mean (SD)	0.30 (19.057)	3.96 (19.251)	2.13 (19.125)
95% Confidence Interval of Mean	-5.71, 6.32	-2.11, 10.04	-2.07, 6.34
Median	0.00	6.25	0.00
Q1, Q3	-12.50, 12.50	-6.25, 12.50	-12.50, 12.50
Min, Max	-43.8, 56.3	-37.5, 43.8	-43.8, 56.3

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	38	39	77
Mean (SD)	59.87 (23.816)	60.10 (21.680)	59.98 (22.609)
95% Confidence Interval of Mean	52.04, 67.70	53.07, 67.12	54.85, 65.12
Median	62.50	62.50	62.50
Q1, Q3	50.00, 75.00	43.75, 75.00	50.00, 75.00
Min, Max	0.0, 100.0	25.0, 100.0	0.0, 100.0
Change from Baseline			
n	37	39	76
Mean (SD)	1.01 (23.823)	5.29 (21.438)	3.21 (22.581)
95% Confidence Interval of Mean	-6.93, 8.96	-1.66, 12.24	-1.95, 8.37
Median	0.00	6.25	6.25
Q1, Q3	-12.50, 12.50	-6.25, 18.75	-12.50, 18.75
Min, Max	-50.0, 56.3	-43.8, 50.0	-50.0, 56.3

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	33	66
Mean (SD)	59.28 (22.433)	58.90 (22.644)	59.09 (22.366)
95% Confidence Interval of Mean	51.33, 67.23	50.87, 66.93	53.59, 64.59
Median	56.25	56.25	56.25
Q1, Q3	43.75, 75.00	43.75, 68.75	43.75, 75.00
Min, Max	18.8, 93.8	18.8, 100.0	18.8, 100.0
Change from Baseline			
n	32	33	65
Mean (SD)	0.00 (22.563)	4.17 (18.923)	2.12 (20.737)
95% Confidence Interval of Mean	-8.13, 8.13	-2.54, 10.88	-3.02, 7.25
Median	3.13	6.25	6.25
Q1, Q3	-15.63, 18.75	-6.25, 18.75	-12.50, 18.75
Min, Max	-43.8, 37.5	-37.5, 50.0	-43.8, 50.0

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	30	60
Mean (SD)	59.17 (21.509)	61.04 (23.482)	60.10 (22.346)
95% Confidence Interval of Mean	51.14, 67.20	52.27, 69.81	54.33, 65.88
Median	62.50	59.38	62.50
Q1, Q3	50.00, 75.00	43.75, 75.00	50.00, 75.00
Min, Max	12.5, 93.8	18.8, 100.0	12.5, 100.0
Change from Baseline			
n	29	30	59
Mean (SD)	0.00 (22.720)	7.92 (16.408)	4.03 (19.993)
95% Confidence Interval of Mean	-8.64, 8.64	1.79, 14.04	-1.18, 9.24
Median	0.00	3.13	0.00
Q1, Q3	-12.50, 12.50	-6.25, 18.75	-6.25, 18.75
Min, Max	-50.0, 56.3	-12.5, 50.0	-50.0, 56.3

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	28	53
Mean (SD)	55.50 (20.910)	61.38 (21.044)	58.61 (20.989)
95% Confidence Interval of Mean	46.87, 64.13	53.22, 69.54	52.82, 64.39
Median	56.25	56.25	56.25
Q1, Q3	43.75, 68.75	50.00, 75.00	50.00, 75.00
Min, Max	12.5, 100.0	12.5, 100.0	12.5, 100.0
Change from Baseline			
n	25	28	53
Mean (SD)	-4.00 (23.377)	7.37 (23.635)	2.00 (23.981)
95% Confidence Interval of Mean	-13.65, 5.65	-1.80, 16.53	-4.61, 8.61
Median	0.00	9.38	0.00
Q1, Q3	-18.75, 12.50	-6.25, 21.88	-12.50, 18.75
Min, Max	-43.8, 37.5	-68.8, 50.0	-68.8, 50.0

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	27	50
Mean (SD)	57.61 (23.076)	62.50 (18.750)	60.25 (20.777)
95% Confidence Interval of Mean	47.63, 67.59	55.08, 69.92	54.35, 66.15
Median	56.25	62.50	59.38
Q1, Q3	43.75, 75.00	50.00, 75.00	50.00, 75.00
Min, Max	12.5, 100.0	31.3, 100.0	12.5, 100.0
Change from Baseline			
n	23	27	50
Mean (SD)	0.27 (30.061)	8.10 (21.845)	4.50 (25.971)
95% Confidence Interval of Mean	-12.73, 13.27	-0.54, 16.74	-2.88, 11.88
Median	0.00	0.00	0.00
Q1, Q3	-37.50, 25.00	-6.25, 25.00	-12.50, 25.00
Min, Max	-50.0, 43.8	-50.0, 43.8	-50.0, 43.8

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	26	49
Mean (SD)	58.15 (19.982)	58.17 (21.122)	58.16 (20.381)
95% Confidence Interval of Mean	49.51, 66.79	49.64, 66.70	52.31, 64.02
Median	56.25	59.38	56.25
Q1, Q3	43.75, 75.00	37.50, 68.75	43.75, 68.75
Min, Max	25.0, 100.0	25.0, 100.0	25.0, 100.0
Change from Baseline			
n	23	26	49
Mean (SD)	-1.90 (28.482)	4.57 (24.467)	1.53 (26.348)
95% Confidence Interval of Mean	-14.22, 10.41	-5.31, 14.45	-6.04, 9.10
Median	0.00	0.00	0.00
Q1, Q3	-25.00, 25.00	-12.50, 25.00	-18.75, 25.00
Min, Max	-56.3, 50.0	-37.5, 50.0	-56.3, 50.0

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	23	45
Mean (SD)	58.52 (20.279)	61.41 (21.786)	60.00 (20.874)
95% Confidence Interval of Mean	49.53, 67.51	51.99, 70.83	53.73, 66.27
Median	59.38	56.25	56.25
Q1, Q3	43.75, 68.75	43.75, 75.00	43.75, 75.00
Min, Max	25.0, 100.0	25.0, 100.0	25.0, 100.0
Change from Baseline			
n	22	23	45
Mean (SD)	-2.27 (29.539)	8.70 (21.793)	3.33 (26.166)
95% Confidence Interval of Mean	-15.37, 10.82	-0.73, 18.12	-4.53, 11.19
Median	12.50	6.25	12.50
Q1, Q3	-31.25, 18.75	-12.50, 25.00	-12.50, 25.00
Min, Max	-50.0, 43.8	-31.3, 50.0	-50.0, 50.0

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	64.17 (18.819)	62.11 (26.267)	63.10 (22.613)
95% Confidence Interval of Mean	53.74, 74.59	48.11, 76.11	54.81, 71.40
Median	62.50	50.00	62.50
Q1, Q3	50.00, 81.25	40.63, 90.63	43.75, 81.25
Min, Max	31.3, 100.0	31.3, 100.0	31.3, 100.0
Change from Baseline			
n	15	16	31
Mean (SD)	-1.67 (30.387)	7.42 (21.797)	3.02 (26.263)
95% Confidence Interval of Mean	-18.49, 15.16	-4.19, 19.04	-6.61, 12.66
Median	6.25	0.00	6.25
Q1, Q3	-37.50, 18.75	-9.38, 21.88	-12.50, 18.75
Min, Max	-56.3, 37.5	-18.8, 50.0	-56.3, 50.0

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	63.64 (20.883)	61.11 (29.610)	62.50 (24.502)
95% Confidence Interval of Mean	49.61, 77.67	38.35, 83.87	51.03, 73.97
Median	62.50	50.00	59.38
Q1, Q3	50.00, 81.25	31.25, 93.75	43.75, 84.38
Min, Max	31.3, 100.0	31.3, 100.0	31.3, 100.0
Change from Baseline			
n	11	9	20
Mean (SD)	-1.70 (28.517)	4.17 (22.097)	0.94 (25.349)
95% Confidence Interval of Mean	-20.86, 17.45	-12.82, 21.15	-10.93, 12.80
Median	0.00	0.00	0.00
Q1, Q3	-25.00, 18.75	-6.25, 12.50	-18.75, 18.75
Min, Max	-50.0, 37.5	-25.0, 50.0	-50.0, 50.0

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	43.75 (26.517)	43.75 (N/A)	43.75 (18.750)
95% Confidence Interval of Mean	-194.49, 281.99	N/A, N/A	-2.83, 90.33
Median	43.75	43.75	43.75
Q1, Q3	25.00, 62.50	43.75, 43.75	25.00, 62.50
Min, Max	25.0, 62.5	43.8, 43.8	25.0, 62.5
Change from Baseline			
n	2	1	3
Mean (SD)	-50.00 (17.678)	-12.50 (N/A)	-37.50 (25.000)
95% Confidence Interval of Mean	-208.83, 108.83	N/A, N/A	-99.60, 24.60
Median	-50.00	-12.50	-37.50
Q1, Q3	-62.50, -37.50	-12.50, -12.50	-62.50, -12.50
Min, Max	-62.5, -37.5	-12.5, -12.5	-62.5, -12.5

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Positive Emotions (Items 5-8)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	59.93 (20.853)	66.15 (26.309)	62.50 (23.025)
95% Confidence Interval of Mean	49.20, 70.65	49.43, 82.86	53.74, 71.26
Median	62.50	62.50	62.50
Q1, Q3	43.75, 68.75	43.75, 96.88	43.75, 75.00
Min, Max	25.0, 100.0	31.3, 100.0	25.0, 100.0
Change from Baseline			
n	17	12	29
Mean (SD)	3.68 (33.150)	5.21 (22.110)	4.31 (28.646)
95% Confidence Interval of Mean	-13.37, 20.72	-8.84, 19.26	-6.59, 15.21
Median	12.50	0.00	12.50
Q1, Q3	-12.50, 25.00	-12.50, 15.63	-12.50, 25.00
Min, Max	-50.0, 43.8	-25.0, 50.0	-50.0, 50.0

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	44	42	86
Mean (SD)	77.68 (16.323)	79.08 (15.469)	78.36 (15.834)
95% Confidence Interval of Mean	72.72, 82.64	74.26, 83.90	74.97, 81.76
Median	82.14	78.57	82.14
Q1, Q3	67.86, 89.29	75.00, 89.29	71.43, 89.29
Min, Max	28.6, 100.0	17.9, 100.0	17.9, 100.0

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	41	41	82
Mean (SD)	84.41 (13.068)	84.67 (13.104)	84.54 (13.005)
95% Confidence Interval of Mean	80.28, 88.53	80.53, 88.81	81.68, 87.40
Median	89.29	89.29	89.29
Q1, Q3	75.00, 92.86	78.57, 92.86	75.00, 92.86
Min, Max	46.4, 100.0	42.9, 100.0	42.9, 100.0
Change from Baseline			
n	40	41	81
Mean (SD)	7.50 (13.957)	5.49 (12.553)	6.48 (13.220)
95% Confidence Interval of Mean	3.04, 11.96	1.53, 9.45	3.56, 9.40
Median	7.14	3.57	7.14
Q1, Q3	-1.79, 14.29	-3.57, 10.71	-3.57, 14.29
Min, Max	-21.4, 42.9	-25.0, 42.9	-25.0, 42.9

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	37	39	76
Mean (SD)	85.81 (13.135)	84.07 (11.496)	84.92 (12.270)
95% Confidence Interval of Mean	81.43, 90.19	80.34, 87.79	82.11, 87.72
Median	89.29	85.71	85.71
Q1, Q3	78.57, 96.43	75.00, 92.86	75.00, 92.86
Min, Max	42.9, 100.0	50.0, 100.0	42.9, 100.0
Change from Baseline			
n	36	39	75
Mean (SD)	7.94 (11.864)	4.58 (11.985)	6.19 (11.966)
95% Confidence Interval of Mean	3.92, 11.95	0.69, 8.46	3.44, 8.94
Median	7.14	3.57	3.57
Q1, Q3	0.00, 14.29	-3.57, 14.29	-3.57, 14.29
Min, Max	-10.7, 35.7	-21.4, 32.1	-21.4, 35.7

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	33	66
Mean (SD)	82.90 (13.883)	80.41 (15.647)	81.66 (14.731)
95% Confidence Interval of Mean	77.98, 87.82	74.86, 85.96	78.03, 85.28
Median	82.14	82.14	82.14
Q1, Q3	75.00, 92.86	71.43, 92.86	75.00, 92.86
Min, Max	35.7, 100.0	35.7, 100.0	35.7, 100.0
Change from Baseline			
n	32	33	65
Mean (SD)	6.03 (12.286)	0.54 (13.367)	3.24 (13.042)
95% Confidence Interval of Mean	1.60, 10.46	-4.20, 5.28	0.01, 6.47
Median	3.57	0.00	3.57
Q1, Q3	0.00, 10.71	-7.14, 7.14	-3.57, 10.71
Min, Max	-17.9, 42.9	-28.6, 25.0	-28.6, 42.9

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	30	60
Mean (SD)	80.83 (14.635)	83.69 (14.399)	82.26 (14.466)
95% Confidence Interval of Mean	75.37, 86.30	78.31, 89.07	78.53, 86.00
Median	82.14	83.93	82.14
Q1, Q3	71.43, 92.86	78.57, 92.86	75.00, 92.86
Min, Max	46.4, 100.0	32.1, 100.0	32.1, 100.0
Change from Baseline			
n	29	30	59
Mean (SD)	3.08 (12.617)	4.29 (11.729)	3.69 (12.083)
95% Confidence Interval of Mean	-1.72, 7.88	-0.09, 8.67	0.54, 6.84
Median	0.00	1.79	0.00
Q1, Q3	-3.57, 10.71	-3.57, 14.29	-3.57, 14.29
Min, Max	-28.6, 35.7	-17.9, 28.6	-28.6, 35.7

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	24	28	52
Mean (SD)	81.85 (16.313)	81.76 (15.410)	81.80 (15.676)
95% Confidence Interval of Mean	74.96, 88.73	75.78, 87.74	77.44, 86.16
Median	85.71	83.93	85.71
Q1, Q3	71.43, 94.64	76.79, 92.86	75.00, 92.86
Min, Max	39.3, 100.0	42.9, 100.0	39.3, 100.0
Change from Baseline			
n	24	28	52
Mean (SD)	0.74 (11.194)	3.06 (14.955)	1.99 (13.277)
95% Confidence Interval of Mean	-3.98, 5.47	-2.74, 8.86	-1.70, 5.69
Median	0.00	1.79	0.00
Q1, Q3	-5.36, 8.93	-7.14, 10.71	-7.14, 8.93
Min, Max	-28.6, 21.4	-21.4, 39.3	-28.6, 39.3

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	27	50
Mean (SD)	83.07 (15.724)	82.01 (15.232)	82.50 (15.310)
95% Confidence Interval of Mean	76.28, 89.87	75.98, 88.04	78.15, 86.85
Median	85.71	85.71	85.71
Q1, Q3	71.43, 96.43	75.00, 92.86	75.00, 92.86
Min, Max	35.7, 100.0	32.1, 100.0	32.1, 100.0
Change from Baseline			
n	23	27	50
Mean (SD)	3.42 (11.622)	3.84 (12.682)	3.64 (12.084)
95% Confidence Interval of Mean	-1.61, 8.44	-1.18, 8.85	0.21, 7.08
Median	3.57	3.57	3.57
Q1, Q3	-7.14, 14.29	-3.57, 10.71	-3.57, 10.71
Min, Max	-17.9, 28.6	-28.6, 39.3	-28.6, 39.3

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	26	49
Mean (SD)	83.39 (17.317)	81.04 (16.060)	82.14 (16.528)
95% Confidence Interval of Mean	75.90, 90.87	74.56, 87.53	77.40, 86.89
Median	92.86	85.71	85.71
Q1, Q3	67.86, 96.43	78.57, 89.29	71.43, 92.86
Min, Max	32.1, 100.0	35.7, 100.0	32.1, 100.0
Change from Baseline			
n	23	26	49
Mean (SD)	3.57 (12.278)	3.16 (14.994)	3.35 (13.647)
95% Confidence Interval of Mean	-1.74, 8.88	-2.90, 9.22	-0.57, 7.27
Median	3.57	3.57	3.57
Q1, Q3	-7.14, 14.29	-3.57, 17.86	-3.57, 14.29
Min, Max	-21.4, 25.0	-32.1, 28.6	-32.1, 28.6

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	23	45
Mean (SD)	81.98 (16.550)	79.66 (17.109)	80.79 (16.687)
95% Confidence Interval of Mean	74.64, 89.32	72.26, 87.06	75.78, 85.81
Median	85.71	85.71	85.71
Q1, Q3	71.43, 92.86	67.86, 89.29	71.43, 92.86
Min, Max	39.3, 100.0	32.1, 100.0	32.1, 100.0
Change from Baseline			
n	22	23	45
Mean (SD)	3.08 (10.759)	0.31 (17.023)	1.67 (14.217)
95% Confidence Interval of Mean	-1.69, 7.85	-7.05, 7.67	-2.60, 5.94
Median	3.57	3.57	3.57
Q1, Q3	-7.14, 14.29	-14.29, 10.71	-7.14, 10.71
Min, Max	-17.9, 17.9	-32.1, 35.7	-32.1, 35.7

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	84.05 (14.020)	83.48 (10.901)	83.76 (12.297)
95% Confidence Interval of Mean	76.28, 91.81	77.67, 89.29	79.25, 88.27
Median	85.71	85.71	85.71
Q1, Q3	78.57, 92.86	82.14, 89.29	82.14, 89.29
Min, Max	39.3, 100.0	50.0, 96.4	39.3, 100.0
Change from Baseline			
n	15	16	31
Mean (SD)	5.48 (13.823)	1.79 (12.234)	3.57 (12.943)
95% Confidence Interval of Mean	-2.18, 13.13	-4.73, 8.30	-1.18, 8.32
Median	7.14	5.36	7.14
Q1, Q3	-10.71, 14.29	-5.36, 10.71	-7.14, 10.71
Min, Max	-17.9, 32.1	-21.4, 17.9	-21.4, 32.1

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Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	88.64 (9.150)	83.33 (12.372)	86.25 (10.763)
95% Confidence Interval of Mean	82.49, 94.78	73.82, 92.84	81.21, 91.29
Median	89.29	78.57	87.50
Q1, Q3	78.57, 96.43	78.57, 92.86	78.57, 96.43
Min, Max	75.0, 100.0	60.7, 100.0	60.7, 100.0
Change from Baseline			
n	11	9	20
Mean (SD)	8.12 (11.304)	1.59 (12.891)	5.18 (12.179)
95% Confidence Interval of Mean	0.52, 15.71	-8.32, 11.50	-0.52, 10.88
Median	10.71	3.57	8.93
Q1, Q3	3.57, 14.29	-10.71, 10.71	0.00, 14.29
Min, Max	-21.4, 17.9	-17.9, 21.4	-21.4, 21.4

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	82.14 (15.152)	78.57 (N/A)	80.95 (10.911)
95% Confidence Interval of Mean	-54.00, 218.28	N/A, N/A	53.85, 108.06
Median	82.14	78.57	78.57
Q1, Q3	71.43, 92.86	78.57, 78.57	71.43, 92.86
Min, Max	71.4, 92.9	78.6, 78.6	71.4, 92.9
Change from Baseline			
n	2	1	3
Mean (SD)	1.79 (2.525)	-7.14 (N/A)	-1.19 (5.455)
95% Confidence Interval of Mean	-20.90, 24.48	N/A, N/A	-14.74, 12.36
Median	1.79	-7.14	0.00
Q1, Q3	0.00, 3.57	-7.14, -7.14	-7.14, 3.57
Min, Max	0.0, 3.6	-7.1, -7.1	-7.1, 3.6

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Negative Emotions (Items 9-15)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	84.45 (17.630)	83.63 (11.939)	84.11 (15.290)
95% Confidence Interval of Mean	75.39, 93.52	76.05, 91.22	78.30, 89.93
Median	82.14	85.71	85.71
Q1, Q3	78.57, 100.00	78.57, 91.07	78.57, 96.43
Min, Max	32.1, 100.0	53.6, 100.0	32.1, 100.0
Change from Baseline			
n	17	12	29
Mean (SD)	6.30 (11.609)	0.30 (13.829)	3.82 (12.696)
95% Confidence Interval of Mean	0.33, 12.27	-8.49, 9.08	-1.01, 8.65
Median	7.14	3.57	7.14
Q1, Q3	3.57, 14.29	-7.14, 12.50	0.00, 14.29
Min, Max	-21.4, 21.4	-25.0, 17.9	-25.0, 21.4

[1] Last non-missing value prior to the first dose.
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Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	43	42	85
Mean (SD)	42.19 (27.491)	42.26 (25.957)	42.23 (26.585)
95% Confidence Interval of Mean	33.73, 50.65	34.17, 50.35	36.49, 47.96
Median	42.86	42.86	42.86
Q1, Q3	25.00, 64.29	17.86, 64.29	17.86, 64.29
Min, Max	0.0, 96.4	0.0, 100.0	0.0, 100.0

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	42	41	83
Mean (SD)	44.22 (24.579)	50.26 (24.188)	47.20 (24.428)
95% Confidence Interval of Mean	36.56, 51.88	42.63, 57.90	41.87, 52.54
Median	44.64	46.43	46.43
Q1, Q3	28.57, 57.14	35.71, 67.86	35.71, 60.71
Min, Max	0.0, 100.0	3.6, 100.0	0.0, 100.0
Change from Baseline			
n	40	41	81
Mean (SD)	1.16 (20.290)	7.14 (19.233)	4.19 (19.867)
95% Confidence Interval of Mean	-5.33, 7.65	1.07, 13.21	-0.20, 8.58
Median	0.00	3.57	3.57
Q1, Q3	-10.71, 12.50	-3.57, 14.29	-7.14, 14.29
Min, Max	-42.9, 67.9	-28.6, 78.6	-42.9, 78.6

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	36	39	75
Mean (SD)	44.94 (25.971)	52.29 (25.353)	48.76 (25.744)
95% Confidence Interval of Mean	36.15, 53.73	44.07, 60.51	42.84, 54.69
Median	46.43	46.43	46.43
Q1, Q3	28.57, 60.71	32.14, 71.43	32.14, 67.86
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	34	39	73
Mean (SD)	4.73 (23.751)	9.16 (20.200)	7.09 (21.883)
95% Confidence Interval of Mean	-3.56, 13.01	2.61, 15.71	1.99, 12.20
Median	3.57	3.57	3.57
Q1, Q3	-7.14, 14.29	0.00, 14.29	-3.57, 14.29
Min, Max	-53.6, 67.9	-35.7, 78.6	-53.6, 78.6

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	33	66
Mean (SD)	45.56 (25.474)	54.55 (26.596)	50.05 (26.233)
95% Confidence Interval of Mean	36.53, 54.60	45.12, 63.98	43.61, 56.50
Median	42.86	57.14	53.57
Q1, Q3	32.14, 60.71	35.71, 75.00	32.14, 64.29
Min, Max	0.0, 100.0	7.1, 100.0	0.0, 100.0
Change from Baseline			
n	31	33	64
Mean (SD)	4.95 (17.921)	11.26 (19.645)	8.20 (18.948)
95% Confidence Interval of Mean	-1.62, 11.53	4.29, 18.22	3.47, 12.94
Median	3.57	7.14	3.57
Q1, Q3	-7.14, 14.29	0.00, 17.86	-3.57, 17.86
Min, Max	-42.9, 50.0	-14.3, 78.6	-42.9, 78.6

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	30	30	60
Mean (SD)	41.90 (23.874)	50.48 (29.759)	46.19 (27.095)
95% Confidence Interval of Mean	32.99, 50.82	39.36, 61.59	39.19, 53.19
Median	42.86	51.79	46.43
Q1, Q3	25.00, 57.14	21.43, 64.29	23.21, 64.29
Min, Max	3.6, 100.0	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	28	30	58
Mean (SD)	2.55 (18.259)	7.50 (23.789)	5.11 (21.262)
95% Confidence Interval of Mean	-4.53, 9.63	-1.38, 16.38	-0.48, 10.70
Median	0.00	3.57	3.57
Q1, Q3	-8.93, 14.29	0.00, 10.71	-3.57, 14.29
Min, Max	-35.7, 46.4	-42.9, 78.6	-42.9, 78.6

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	28	53
Mean (SD)	45.29 (25.872)	54.97 (27.789)	50.40 (27.088)
95% Confidence Interval of Mean	34.61, 55.97	44.20, 65.75	42.94, 57.87
Median	46.43	64.29	50.00
Q1, Q3	32.14, 64.29	33.93, 75.00	32.14, 75.00
Min, Max	0.0, 78.6	3.6, 100.0	0.0, 100.0
Change from Baseline			
n	24	28	52
Mean (SD)	6.99 (21.363)	10.97 (27.006)	9.13 (24.412)
95% Confidence Interval of Mean	-2.03, 16.01	0.50, 21.44	2.34, 15.93
Median	1.79	5.36	3.57
Q1, Q3	-7.14, 16.07	-1.79, 19.64	-5.36, 19.64
Min, Max	-17.9, 75.0	-64.3, 78.6	-64.3, 78.6

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	22	27	49
Mean (SD)	44.81 (26.284)	53.31 (30.673)	49.49 (28.812)
95% Confidence Interval of Mean	33.15, 56.46	41.17, 65.44	41.21, 57.77
Median	50.00	53.57	53.57
Q1, Q3	32.14, 60.71	28.57, 78.57	28.57, 71.43
Min, Max	0.0, 96.4	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	22	27	49
Mean (SD)	9.90 (23.091)	8.20 (27.142)	8.97 (25.160)
95% Confidence Interval of Mean	-0.34, 20.14	-2.54, 18.94	1.74, 16.19
Median	5.36	3.57	3.57
Q1, Q3	-7.14, 25.00	-3.57, 14.29	-3.57, 14.29
Min, Max	-28.6, 71.4	-53.6, 78.6	-53.6, 78.6

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	26	49
Mean (SD)	44.10 (26.280)	50.27 (27.216)	47.38 (26.684)
95% Confidence Interval of Mean	32.74, 55.46	39.28, 61.27	39.71, 55.04
Median	42.86	50.00	46.43
Q1, Q3	28.57, 60.71	35.71, 71.43	32.14, 64.29
Min, Max	0.0, 96.4	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	22	26	48
Mean (SD)	8.44 (23.781)	5.08 (23.193)	6.62 (23.274)
95% Confidence Interval of Mean	-2.10, 18.99	-4.29, 14.45	-0.14, 13.38
Median	7.14	0.00	1.79
Q1, Q3	-10.71, 17.86	-7.14, 14.29	-7.14, 17.86
Min, Max	-28.6, 75.0	-39.3, 67.9	-39.3, 75.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	23	45
Mean (SD)	46.59 (24.484)	54.97 (26.829)	50.87 (25.767)
95% Confidence Interval of Mean	35.74, 57.45	43.37, 66.57	43.13, 58.61
Median	50.00	46.43	50.00
Q1, Q3	32.14, 57.14	35.71, 75.00	35.71, 71.43
Min, Max	0.0, 92.9	3.6, 100.0	0.0, 100.0
Change from Baseline			
n	21	23	44
Mean (SD)	8.84 (24.579)	9.47 (21.794)	9.17 (22.893)
95% Confidence Interval of Mean	-2.34, 20.03	0.05, 18.90	2.21, 16.13
Median	3.57	3.57	3.57
Q1, Q3	-10.71, 17.86	-3.57, 21.43	-5.36, 19.64
Min, Max	-28.6, 67.9	-28.6, 64.3	-28.6, 67.9

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	56.19 (22.404)	58.26 (32.699)	57.26 (27.748)
95% Confidence Interval of Mean	43.78, 68.60	40.83, 75.68	47.08, 67.44
Median	57.14	55.36	57.14
Q1, Q3	32.14, 75.00	39.29, 91.07	35.71, 78.57
Min, Max	25.0, 100.0	3.6, 100.0	3.6, 100.0
Change from Baseline			
n	15	16	31
Mean (SD)	14.76 (24.849)	12.50 (24.293)	13.59 (24.177)
95% Confidence Interval of Mean	1.00, 28.52	-0.44, 25.44	4.73, 22.46
Median	10.71	3.57	10.71
Q1, Q3	3.57, 28.57	-1.79, 17.86	0.00, 25.00
Min, Max	-25.0, 75.0	-10.7, 71.4	-25.0, 75.0

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	56.17 (24.800)	54.76 (37.330)	55.54 (30.182)
95% Confidence Interval of Mean	39.51, 72.83	26.07, 83.46	41.41, 69.66
Median	53.57	50.00	53.57
Q1, Q3	39.29, 75.00	32.14, 96.43	33.93, 76.79
Min, Max	10.7, 100.0	3.6, 100.0	3.6, 100.0
Change from Baseline			
n	11	9	20
Mean (SD)	10.39 (16.862)	3.97 (11.356)	7.50 (14.652)
95% Confidence Interval of Mean	-0.94, 21.72	-4.76, 12.70	0.64, 14.36
Median	3.57	3.57	3.57
Q1, Q3	-3.57, 25.00	0.00, 10.71	-1.79, 17.86
Min, Max	-10.7, 39.3	-14.3, 21.4	-14.3, 39.3

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	37.50 (2.525)	50.00 (N/A)	41.67 (7.435)
95% Confidence Interval of Mean	14.81, 60.19	N/A, N/A	23.20, 60.14
Median	37.50	50.00	39.29
Q1, Q3	35.71, 39.29	50.00, 50.00	35.71, 50.00
Min, Max	35.7, 39.3	50.0, 50.0	35.7, 50.0
Change from Baseline			
n	2	1	3
Mean (SD)	-23.21 (7.576)	7.14 (N/A)	-13.10 (18.327)
95% Confidence Interval of Mean	-91.28, 44.85	N/A, N/A	-58.62, 32.43
Median	-23.21	7.14	-17.86
Q1, Q3	-28.57, -17.86	7.14, 7.14	-28.57, 7.14
Min, Max	-28.6, -17.9	7.1, 7.1	-28.6, 7.1

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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Social Interaction (Items 16-22)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	49.37 (29.430)	63.69 (29.640)	55.30 (29.860)
95% Confidence Interval of Mean	34.24, 64.50	44.86, 82.52	43.94, 66.65
Median	42.86	57.14	50.00
Q1, Q3	28.57, 67.86	48.21, 94.64	32.14, 75.00
Min, Max	7.1, 100.0	7.1, 100.0	7.1, 100.0
Change from Baseline			
n	16	12	28
Mean (SD)	10.94 (24.063)	8.93 (17.196)	10.08 (21.052)
95% Confidence Interval of Mean	-1.88, 23.76	-2.00, 19.85	1.91, 18.24
Median	12.50	3.57	7.14
Q1, Q3	-8.93, 23.21	-1.79, 16.07	-3.57, 17.86
Min, Max	-25.0, 53.6	-14.3, 50.0	-25.0, 53.6

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	43	42	85
Mean (SD)	49.53 (31.049)	47.26 (25.500)	48.41 (28.297)
95% Confidence Interval of Mean	39.98, 59.09	39.32, 55.21	42.31, 54.52
Median	50.00	50.00	50.00
Q1, Q3	25.00, 75.00	30.00, 70.00	25.00, 70.00
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	42	41	83
Mean (SD)	53.93 (31.245)	53.41 (24.378)	53.67 (27.894)
95% Confidence Interval of Mean	44.19, 63.67	45.72, 61.11	47.58, 59.77
Median	60.00	50.00	55.00
Q1, Q3	30.00, 75.00	40.00, 70.00	35.00, 75.00
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	40	41	81
Mean (SD)	3.63 (25.894)	5.49 (19.000)	4.57 (22.544)
95% Confidence Interval of Mean	-4.66, 11.91	-0.51, 11.48	-0.42, 9.55
Median	2.50	0.00	0.00
Q1, Q3	-12.50, 20.00	-5.00, 15.00	-10.00, 15.00
Min, Max	-50.0, 75.0	-30.0, 55.0	-50.0, 75.0

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	37	39	76
Mean (SD)	48.78 (29.496)	53.85 (28.916)	51.38 (29.116)
95% Confidence Interval of Mean	38.95, 58.62	44.47, 63.22	44.73, 58.03
Median	55.00	50.00	55.00
Q1, Q3	30.00, 75.00	30.00, 75.00	30.00, 75.00
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	35	39	74
Mean (SD)	2.43 (27.875)	5.77 (22.406)	4.19 (25.021)
95% Confidence Interval of Mean	-7.15, 12.00	-1.49, 13.03	-1.61, 9.99
Median	0.00	5.00	5.00
Q1, Q3	-15.00, 20.00	-5.00, 20.00	-10.00, 20.00
Min, Max	-60.0, 60.0	-55.0, 55.0	-60.0, 60.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	33	66
Mean (SD)	47.27 (29.211)	57.88 (27.840)	52.58 (28.813)
95% Confidence Interval of Mean	36.92, 57.63	48.01, 67.75	45.49, 59.66
Median	50.00	55.00	55.00
Q1, Q3	25.00, 65.00	30.00, 80.00	30.00, 75.00
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	31	33	64
Mean (SD)	0.16 (17.344)	8.79 (20.880)	4.61 (19.585)
95% Confidence Interval of Mean	-6.20, 6.52	1.38, 16.19	-0.28, 9.50
Median	0.00	10.00	0.00
Q1, Q3	-10.00, 10.00	0.00, 20.00	-5.00, 12.50
Min, Max	-55.0, 40.0	-30.0, 65.0	-55.0, 65.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	29	30	59
Mean (SD)	53.79 (24.189)	55.17 (30.891)	54.49 (27.570)
95% Confidence Interval of Mean	44.59, 62.99	43.63, 66.70	47.31, 61.68
Median	50.00	55.00	55.00
Q1, Q3	40.00, 70.00	30.00, 80.00	35.00, 75.00
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	27	30	57
Mean (SD)	9.26 (23.192)	4.83 (18.592)	6.93 (20.826)
95% Confidence Interval of Mean	0.08, 18.43	-2.11, 11.78	1.40, 12.46
Median	10.00	5.00	5.00
Q1, Q3	-5.00, 20.00	-5.00, 15.00	-5.00, 20.00
Min, Max	-45.0, 50.0	-30.0, 50.0	-45.0, 50.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	28	53
Mean (SD)	48.40 (24.140)	56.43 (28.767)	52.64 (26.740)
95% Confidence Interval of Mean	38.44, 58.36	45.27, 67.58	45.27, 60.01
Median	50.00	55.00	50.00
Q1, Q3	40.00, 70.00	37.50, 80.00	40.00, 75.00
Min, Max	0.0, 85.0	10.0, 100.0	0.0, 100.0
Change from Baseline			
n	24	28	52
Mean (SD)	4.17 (21.349)	5.89 (18.711)	5.10 (19.790)
95% Confidence Interval of Mean	-4.85, 13.18	-1.36, 13.15	-0.41, 10.61
Median	5.00	5.00	5.00
Q1, Q3	-5.00, 12.50	-5.00, 15.00	-5.00, 15.00
Min, Max	-40.0, 50.0	-35.0, 50.0	-40.0, 50.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	27	50
Mean (SD)	47.61 (23.735)	56.85 (23.824)	52.60 (23.995)
95% Confidence Interval of Mean	37.35, 57.87	47.43, 66.28	45.78, 59.42
Median	50.00	60.00	50.00
Q1, Q3	35.00, 60.00	40.00, 65.00	35.00, 65.00
Min, Max	0.0, 100.0	10.0, 100.0	0.0, 100.0
Change from Baseline			
n	23	27	50
Mean (SD)	4.57 (20.277)	6.30 (19.345)	5.50 (19.594)
95% Confidence Interval of Mean	-4.20, 13.33	-1.36, 13.95	-0.07, 11.07
Median	0.00	5.00	0.00
Q1, Q3	-10.00, 15.00	-5.00, 15.00	-5.00, 15.00
Min, Max	-20.0, 50.0	-25.0, 60.0	-25.0, 60.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	26	49
Mean (SD)	48.26 (30.955)	55.00 (28.671)	51.84 (29.645)
95% Confidence Interval of Mean	34.87, 61.65	43.42, 66.58	43.32, 60.35
Median	45.00	50.00	50.00
Q1, Q3	25.00, 80.00	40.00, 85.00	30.00, 80.00
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	22	26	48
Mean (SD)	5.45 (27.469)	4.42 (18.018)	4.90 (22.585)
95% Confidence Interval of Mean	-6.72, 17.63	-2.85, 11.70	-1.66, 11.45
Median	5.00	2.50	5.00
Q1, Q3	-10.00, 25.00	-5.00, 15.00	-5.00, 17.50
Min, Max	-65.0, 70.0	-40.0, 35.0	-65.0, 70.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	23	45
Mean (SD)	49.77 (26.479)	56.52 (29.712)	53.22 (28.066)
95% Confidence Interval of Mean	38.03, 61.51	43.67, 69.37	44.79, 61.65
Median	50.00	50.00	50.00
Q1, Q3	30.00, 70.00	30.00, 80.00	30.00, 75.00
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	21	23	44
Mean (SD)	5.95 (26.675)	5.22 (16.547)	5.57 (21.707)
95% Confidence Interval of Mean	-6.19, 18.09	-1.94, 12.37	-1.03, 12.17
Median	5.00	0.00	2.50
Q1, Q3	-10.00, 10.00	-5.00, 15.00	-5.00, 15.00
Min, Max	-50.0, 65.0	-25.0, 50.0	-50.0, 65.0

[1] Last non-missing value prior to the first dose.
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Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	61.67 (21.685)	63.13 (29.658)	62.42 (25.686)
95% Confidence Interval of Mean	49.66, 73.68	47.32, 78.93	53.00, 71.84
Median	65.00	67.50	65.00
Q1, Q3	40.00, 75.00	35.00, 85.00	40.00, 85.00
Min, Max	30.0, 95.0	10.0, 100.0	10.0, 100.0
Change from Baseline			
n	15	16	31
Mean (SD)	10.33 (24.529)	6.56 (19.211)	8.39 (21.656)
95% Confidence Interval of Mean	-3.25, 23.92	-3.67, 16.80	0.44, 16.33
Median	5.00	2.50	5.00
Q1, Q3	-5.00, 30.00	-2.50, 17.50	-5.00, 25.00
Min, Max	-25.0, 55.0	-30.0, 50.0	-30.0, 55.0

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	58.64 (22.813)	53.89 (33.706)	56.50 (27.535)
95% Confidence Interval of Mean	43.31, 73.96	27.98, 79.80	43.61, 69.39
Median	60.00	55.00	60.00
Q1, Q3	45.00, 75.00	25.00, 75.00	42.50, 75.00
Min, Max	10.0, 100.0	10.0, 100.0	10.0, 100.0
Change from Baseline			
n	11	9	20
Mean (SD)	2.27 (20.780)	6.67 (15.000)	4.25 (18.084)
95% Confidence Interval of Mean	-11.69, 16.23	-4.86, 18.20	-4.21, 12.71
Median	5.00	10.00	7.50
Q1, Q3	-10.00, 15.00	-5.00, 20.00	-5.00, 17.50
Min, Max	-30.0, 40.0	-20.0, 25.0	-30.0, 40.0

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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	50.00 (7.071)	55.00 (N/A)	51.67 (5.774)
95% Confidence Interval of Mean	-13.53, 113.53	N/A, N/A	37.32, 66.01
Median	50.00	55.00	55.00
Q1, Q3	45.00, 55.00	55.00, 55.00	45.00, 55.00
Min, Max	45.0, 55.0	55.0, 55.0	45.0, 55.0
Change from Baseline			
n	2	1	3
Mean (SD)	-30.00 (28.284)	-5.00 (N/A)	-21.67 (24.664)
95% Confidence Interval of Mean	-284.12, 224.12	N/A, N/A	-82.94, 39.60
Median	-30.00	-5.00	-10.00
Q1, Q3	-50.00, -10.00	-5.00, -5.00	-50.00, -5.00
Min, Max	-50.0, -10.0	-5.0, -5.0	-50.0, -5.0

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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Leisure and the Outdoors (Items 23- 27)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	50.00 (28.777)	57.08 (31.727)	52.93 (29.686)
95% Confidence Interval of Mean	35.20, 64.80	36.92, 77.24	41.64, 64.22
Median	50.00	52.50	50.00
Q1, Q3	30.00, 70.00	35.00, 85.00	30.00, 70.00
Min, Max	0.0, 100.0	5.0, 100.0	0.0, 100.0
Change from Baseline			
n	16	12	28
Mean (SD)	1.25 (28.431)	7.92 (11.572)	4.11 (22.692)
95% Confidence Interval of Mean	-13.90, 16.40	0.56, 15.27	-4.69, 12.91
Median	2.50	7.50	5.00
Q1, Q3	-15.00, 17.50	0.00, 17.50	-7.50, 17.50
Min, Max	-70.0, 55.0	-10.0, 25.0	-70.0, 55.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	44	43	87
Mean (SD)	28.41 (22.146)	21.98 (19.885)	25.23 (21.185)
95% Confidence Interval of Mean	21.68, 35.14	15.86, 28.10	20.71, 29.74
Median	27.50	15.00	20.00
Q1, Q3	10.00, 45.00	5.00, 30.00	5.00, 40.00
Min, Max	0.0, 75.0	0.0, 90.0	0.0, 90.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	42	41	83
Mean (SD)	30.71 (22.212)	29.15 (20.824)	29.94 (21.420)
95% Confidence Interval of Mean	23.79, 37.64	22.57, 35.72	25.26, 34.62
Median	32.50	30.00	30.00
Q1, Q3	10.00, 45.00	15.00, 45.00	10.00, 45.00
Min, Max	0.0, 80.0	0.0, 75.0	0.0, 80.0
Change from Baseline			
n	41	41	82
Mean (SD)	0.85 (14.954)	6.10 (20.172)	3.48 (17.842)
95% Confidence Interval of Mean	-3.87, 5.57	-0.27, 12.46	-0.44, 7.40
Median	0.00	5.00	0.00
Q1, Q3	-10.00, 5.00	-5.00, 20.00	-10.00, 15.00
Min, Max	-30.0, 45.0	-35.0, 50.0	-35.0, 50.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	37	38	75
Mean (SD)	29.05 (23.887)	33.29 (23.546)	31.20 (23.651)
95% Confidence Interval of Mean	21.09, 37.02	25.55, 41.03	25.76, 36.64
Median	25.00	32.50	25.00
Q1, Q3	10.00, 40.00	10.00, 45.00	10.00, 45.00
Min, Max	0.0, 90.0	0.0, 90.0	0.0, 90.0
Change from Baseline			
n	36	38	74
Mean (SD)	1.25 (10.848)	10.13 (23.380)	5.81 (18.800)
95% Confidence Interval of Mean	-2.42, 4.92	2.45, 17.82	1.46, 10.17
Median	0.00	10.00	5.00
Q1, Q3	-7.50, 7.50	-10.00, 30.00	-10.00, 20.00
Min, Max	-25.0, 25.0	-30.0, 65.0	-30.0, 65.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	33	66
Mean (SD)	26.82 (21.243)	35.91 (24.983)	31.36 (23.461)
95% Confidence Interval of Mean	19.29, 34.35	27.05, 44.77	25.60, 37.13
Median	25.00	35.00	30.00
Q1, Q3	10.00, 40.00	15.00, 60.00	15.00, 50.00
Min, Max	0.0, 80.0	0.0, 90.0	0.0, 90.0
Change from Baseline			
n	32	33	65
Mean (SD)	-4.38 (14.960)	10.91 (27.055)	3.38 (23.101)
95% Confidence Interval of Mean	-9.77, 1.02	1.32, 20.50	-2.34, 9.11
Median	-7.50	5.00	0.00
Q1, Q3	-15.00, 5.00	-10.00, 30.00	-15.00, 15.00
Min, Max	-35.0, 25.0	-30.0, 75.0	-35.0, 75.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	29	30	59
Mean (SD)	29.66 (23.258)	33.17 (25.274)	31.44 (24.159)
95% Confidence Interval of Mean	20.81, 38.50	23.73, 42.60	25.14, 37.74
Median	25.00	35.00	30.00
Q1, Q3	10.00, 45.00	10.00, 45.00	10.00, 45.00
Min, Max	0.0, 90.0	0.0, 90.0	0.0, 90.0
Change from Baseline			
n	28	30	58
Mean (SD)	-1.25 (16.022)	7.33 (24.059)	3.19 (20.852)
95% Confidence Interval of Mean	-7.46, 4.96	-1.65, 16.32	-2.29, 8.67
Median	0.00	2.50	0.00
Q1, Q3	-10.00, 12.50	-10.00, 25.00	-10.00, 20.00
Min, Max	-40.0, 20.0	-35.0, 65.0	-40.0, 65.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	25	28	53
Mean (SD)	28.00 (23.979)	36.43 (23.993)	32.45 (24.132)
95% Confidence Interval of Mean	18.10, 37.90	27.13, 45.73	25.80, 39.10
Median	20.00	40.00	30.00
Q1, Q3	10.00, 45.00	17.50, 50.00	10.00, 50.00
Min, Max	0.0, 75.0	0.0, 95.0	0.0, 95.0
Change from Baseline			
n	25	28	53
Mean (SD)	-1.40 (19.068)	10.36 (22.066)	4.81 (21.348)
95% Confidence Interval of Mean	-9.27, 6.47	1.80, 18.91	-1.07, 10.70
Median	0.00	7.50	5.00
Q1, Q3	-10.00, 10.00	-5.00, 25.00	-10.00, 15.00
Min, Max	-65.0, 35.0	-30.0, 65.0	-65.0, 65.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	23	27	50
Mean (SD)	33.48 (27.112)	38.70 (24.282)	36.30 (25.491)
95% Confidence Interval of Mean	21.75, 45.20	29.10, 48.31	29.06, 43.54
Median	25.00	40.00	35.00
Q1, Q3	10.00, 55.00	20.00, 50.00	20.00, 50.00
Min, Max	0.0, 90.0	0.0, 85.0	0.0, 90.0
Change from Baseline			
n	23	27	50
Mean (SD)	4.35 (16.397)	12.22 (22.589)	8.60 (20.179)
95% Confidence Interval of Mean	-2.74, 11.44	3.29, 21.16	2.87, 14.33
Median	0.00	15.00	10.00
Q1, Q3	-5.00, 10.00	-5.00, 30.00	-5.00, 20.00
Min, Max	-25.0, 50.0	-30.0, 60.0	-30.0, 60.0

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	26	49
Mean (SD)	35.65 (27.729)	35.77 (27.302)	35.71 (27.214)
95% Confidence Interval of Mean	23.66, 47.64	24.74, 46.80	27.90, 43.53
Median	30.00	37.50	35.00
Q1, Q3	15.00, 50.00	10.00, 50.00	15.00, 50.00
Min, Max	0.0, 100.0	0.0, 100.0	0.0, 100.0
Change from Baseline			
n	23	26	49
Mean (SD)	5.65 (19.028)	8.85 (23.677)	7.35 (21.460)
95% Confidence Interval of Mean	-2.58, 13.88	-0.72, 18.41	1.18, 13.51
Median	5.00	5.00	5.00
Q1, Q3	-5.00, 15.00	-10.00, 30.00	-5.00, 20.00
Min, Max	-30.0, 60.0	-40.0, 50.0	-40.0, 60.0

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	23	45
Mean (SD)	36.59 (22.381)	37.39 (25.400)	37.00 (23.703)
95% Confidence Interval of Mean	26.67, 46.51	26.41, 48.38	29.88, 44.12
Median	32.50	35.00	35.00
Q1, Q3	20.00, 55.00	15.00, 65.00	15.00, 55.00
Min, Max	0.0, 80.0	5.0, 85.0	0.0, 85.0
Change from Baseline			
n	22	23	45
Mean (SD)	5.23 (20.147)	9.13 (23.387)	7.22 (21.705)
95% Confidence Interval of Mean	-3.71, 14.16	-0.98, 19.24	0.70, 13.74
Median	5.00	10.00	10.00
Q1, Q3	-5.00, 15.00	-10.00, 25.00	-5.00, 20.00
Min, Max	-30.0, 50.0	-40.0, 45.0	-40.0, 50.0

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	48.00 (24.553)	39.69 (26.862)	43.71 (25.689)
95% Confidence Interval of Mean	34.40, 61.60	25.37, 54.00	34.29, 53.13
Median	40.00	42.50	40.00
Q1, Q3	30.00, 70.00	17.50, 55.00	25.00, 70.00
Min, Max	10.0, 100.0	5.0, 100.0	5.0, 100.0
Change from Baseline			
n	15	16	31
Mean (SD)	10.67 (19.167)	12.81 (20.974)	11.77 (19.814)
95% Confidence Interval of Mean	0.05, 21.28	1.64, 23.99	4.51, 19.04
Median	10.00	10.00	10.00
Q1, Q3	0.00, 25.00	0.00, 30.00	0.00, 30.00
Min, Max	-25.0, 40.0	-30.0, 50.0	-30.0, 50.0

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	37.27 (23.597)	32.78 (30.116)	35.25 (26.081)
95% Confidence Interval of Mean	21.42, 53.13	9.63, 55.93	23.04, 47.46
Median	30.00	25.00	30.00
Q1, Q3	15.00, 65.00	10.00, 35.00	15.00, 50.00
Min, Max	10.0, 75.0	5.0, 85.0	5.0, 85.0
Change from Baseline			
n	11	9	20
Mean (SD)	5.00 (17.464)	8.89 (22.048)	6.75 (19.213)
95% Confidence Interval of Mean	-6.73, 16.73	-8.06, 25.84	-2.24, 15.74
Median	0.00	5.00	2.50
Q1, Q3	-10.00, 15.00	-5.00, 10.00	-10.00, 12.50
Min, Max	-15.0, 35.0	-10.0, 60.0	-15.0, 60.0

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	22.50 (3.536)	40.00 (N/A)	28.33 (10.408)
95% Confidence Interval of Mean	-9.27, 54.27	N/A, N/A	2.48, 54.19
Median	22.50	40.00	25.00
Q1, Q3	20.00, 25.00	40.00, 40.00	20.00, 40.00
Min, Max	20.0, 25.0	40.0, 40.0	20.0, 40.0
Change from Baseline			
n	2	1	3
Mean (SD)	-17.50 (3.536)	-5.00 (N/A)	-13.33 (7.638)
95% Confidence Interval of Mean	-49.27, 14.27	N/A, N/A	-32.31, 5.64
Median	-17.50	-5.00	-15.00
Q1, Q3	-20.00, -15.00	-5.00, -5.00	-20.00, -5.00
Min, Max	-20.0, -15.0	-5.0, -5.0	-20.0, -5.0

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

Domain: Independence (Items 28-32)

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	32.65 (25.988)	42.50 (30.711)	36.72 (27.944)
95% Confidence Interval of Mean	19.29, 46.01	22.99, 62.01	26.09, 47.35
Median	25.00	40.00	35.00
Q1, Q3	15.00, 40.00	22.50, 62.50	15.00, 45.00
Min, Max	0.0, 90.0	0.0, 95.0	0.0, 95.0
Change from Baseline			
n	17	12	29
Mean (SD)	1.76 (19.360)	15.83 (19.867)	7.59 (20.469)
95% Confidence Interval of Mean	-8.19, 11.72	3.21, 28.46	-0.20, 15.37
Median	0.00	7.50	5.00
Q1, Q3	-10.00, 15.00	2.50, 27.50	-5.00, 20.00
Min, Max	-35.0, 40.0	-5.0, 60.0	-35.0, 60.0

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Baseline [1]			
n	43	42	85
Mean (SD)	54.14 (15.914)	52.05 (14.113)	53.11 (14.999)
95% Confidence Interval of Mean	49.24, 59.04	47.65, 56.44	49.87, 56.34
Median	54.69	51.17	53.91
Q1, Q3	43.75, 63.28	40.63, 60.16	41.41, 60.94
Min, Max	15.6, 89.1	29.7, 91.4	15.6, 91.4

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 7 (Week 21)			
n	41	41	82
Mean (SD)	57.13 (15.293)	58.17 (14.093)	57.65 (14.623)
95% Confidence Interval of Mean	52.30, 61.95	53.73, 62.62	54.44, 60.86
Median	60.16	57.03	57.42
Q1, Q3	45.31, 67.19	50.00, 64.06	49.22, 66.41
Min, Max	18.8, 88.3	32.0, 92.2	18.8, 92.2
Change from Baseline			
n	39	41	80
Mean (SD)	2.84 (12.646)	5.68 (9.443)	4.30 (11.140)
95% Confidence Interval of Mean	-1.25, 6.94	2.70, 8.66	1.82, 6.78
Median	3.13	3.13	3.13
Q1, Q3	-6.25, 7.81	-1.56, 11.72	-3.13, 10.94
Min, Max	-20.3, 43.8	-8.6, 36.7	-20.3, 43.8

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 8 (Week 34)			
n	36	38	74
Mean (SD)	57.25 (14.854)	59.11 (15.082)	58.20 (14.898)
95% Confidence Interval of Mean	52.22, 62.27	54.15, 64.06	54.75, 61.65
Median	60.16	58.20	59.38
Q1, Q3	47.66, 67.19	46.88, 67.97	47.66, 67.97
Min, Max	22.7, 89.1	33.6, 91.4	22.7, 91.4
Change from Baseline			
n	34	38	72
Mean (SD)	4.11 (13.007)	6.58 (11.360)	5.41 (12.142)
95% Confidence Interval of Mean	-0.43, 8.65	2.84, 10.31	2.56, 8.27
Median	3.52	6.64	4.69
Q1, Q3	-3.13, 10.94	0.00, 12.50	-0.78, 11.33
Min, Max	-24.2, 38.3	-30.5, 35.9	-30.5, 38.3

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 9 (Week 52)			
n	33	33	66
Mean (SD)	55.97 (14.768)	59.71 (15.952)	57.84 (15.369)
95% Confidence Interval of Mean	50.73, 61.20	54.05, 65.36	54.06, 61.61
Median	59.38	57.81	58.59
Q1, Q3	42.97, 64.84	50.00, 67.97	46.09, 67.97
Min, Max	27.3, 86.7	32.0, 94.5	27.3, 94.5
Change from Baseline			
n	31	33	64
Mean (SD)	2.49 (9.821)	6.44 (11.021)	4.53 (10.563)
95% Confidence Interval of Mean	-1.11, 6.10	2.53, 10.35	1.89, 7.17
Median	3.13	6.25	5.47
Q1, Q3	-5.47, 10.16	-1.56, 14.06	-1.95, 10.94
Min, Max	-18.8, 27.3	-15.6, 39.1	-18.8, 39.1

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 10 (Week 68)			
n	29	30	59
Mean (SD)	56.09 (14.433)	59.22 (17.791)	57.68 (16.165)
95% Confidence Interval of Mean	50.60, 61.58	52.58, 65.86	53.47, 61.89
Median	57.81	60.55	59.38
Q1, Q3	40.63, 64.84	50.78, 66.41	41.41, 65.63
Min, Max	33.6, 86.7	26.6, 96.1	26.6, 96.1
Change from Baseline			
n	27	30	57
Mean (SD)	3.39 (11.371)	6.12 (11.583)	4.82 (11.463)
95% Confidence Interval of Mean	-1.11, 7.88	1.79, 10.44	1.78, 7.87
Median	4.69	7.03	4.69
Q1, Q3	-3.91, 10.16	-1.56, 13.28	-2.34, 12.50
Min, Max	-24.2, 27.3	-14.1, 40.6	-24.2, 40.6

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 11 (Week 84)			
n	24	28	52
Mean (SD)	54.88 (14.613)	60.91 (17.028)	58.13 (16.094)
95% Confidence Interval of Mean	48.71, 61.05	54.31, 67.51	53.65, 62.61
Median	55.47	64.45	59.77
Q1, Q3	47.27, 66.02	48.05, 71.09	47.66, 69.92
Min, Max	22.7, 79.7	30.5, 93.0	22.7, 93.0
Change from Baseline			
n	23	28	51
Mean (SD)	2.48 (11.498)	7.56 (13.342)	5.27 (12.681)
95% Confidence Interval of Mean	-2.49, 7.45	2.39, 12.73	1.70, 8.84
Median	3.13	5.86	5.47
Q1, Q3	-6.25, 8.59	0.00, 16.80	-3.13, 12.50
Min, Max	-13.3, 35.2	-36.7, 37.5	-36.7, 37.5

[1] Last non-missing value prior to the first dose.
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Subjects are grouped by the treatment received in DB phase.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 12 (Week 100)			
n	22	27	49
Mean (SD)	55.89 (15.531)	61.17 (15.206)	58.80 (15.421)
95% Confidence Interval of Mean	49.01, 62.78	55.15, 67.18	54.37, 63.23
Median	56.64	58.59	57.03
Q1, Q3	46.88, 65.63	50.78, 69.53	50.00, 67.97
Min, Max	27.3, 86.7	34.4, 93.8	27.3, 93.8
Change from Baseline			
n	22	27	49
Mean (SD)	4.65 (13.887)	7.61 (11.573)	6.28 (12.614)
95% Confidence Interval of Mean	-1.51, 10.81	3.03, 12.19	2.66, 9.91
Median	1.95	7.03	5.47
Q1, Q3	-5.47, 10.94	0.00, 13.28	-1.56, 11.72
Min, Max	-14.8, 38.3	-25.0, 36.7	-25.0, 38.3

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 13 (Week 116)			
n	23	26	49
Mean (SD)	57.24 (16.228)	58.26 (16.334)	57.78 (16.122)
95% Confidence Interval of Mean	50.22, 64.25	51.67, 64.86	53.15, 62.41
Median	56.25	55.86	56.25
Q1, Q3	43.75, 64.06	45.31, 70.31	45.31, 67.19
Min, Max	28.9, 96.1	33.6, 97.7	28.9, 97.7
Change from Baseline			
n	22	26	48
Mean (SD)	5.08 (14.637)	4.84 (12.996)	4.95 (13.623)
95% Confidence Interval of Mean	-1.41, 11.57	-0.41, 10.09	0.99, 8.90
Median	5.86	6.64	6.25
Q1, Q3	-1.56, 11.72	-3.91, 11.72	-2.73, 11.72
Min, Max	-26.6, 41.4	-25.0, 23.4	-26.6, 41.4

[1] Last non-missing value prior to the first dose.
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 14 (Week 132)			
n	22	23	45
Mean (SD)	58.20 (14.064)	60.12 (16.339)	59.18 (15.127)
95% Confidence Interval of Mean	51.97, 64.44	53.06, 67.19	54.64, 63.73
Median	58.59	55.47	56.25
Q1, Q3	49.22, 69.53	45.31, 72.66	46.88, 70.31
Min, Max	35.2, 81.3	32.8, 91.4	32.8, 91.4
Change from Baseline			
n	21	23	44
Mean (SD)	5.17 (15.561)	6.01 (11.330)	5.61 (13.360)
95% Confidence Interval of Mean	-1.91, 12.25	1.11, 10.91	1.55, 9.67
Median	4.69	7.81	5.47
Q1, Q3	-5.47, 15.63	-0.78, 13.28	-3.52, 14.45
Min, Max	-22.7, 41.4	-18.8, 25.0	-22.7, 41.4

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 15 (Week 148)			
n	15	16	31
Mean (SD)	65.10 (14.071)	63.33 (18.708)	64.19 (16.377)
95% Confidence Interval of Mean	57.31, 72.90	53.36, 73.30	58.18, 70.20
Median	64.84	64.06	64.84
Q1, Q3	53.13, 78.13	48.83, 77.73	50.78, 78.13
Min, Max	43.8, 90.6	33.6, 96.9	33.6, 96.9
Change from Baseline			
n	15	16	31
Mean (SD)	8.02 (14.910)	7.67 (11.104)	7.84 (12.862)
95% Confidence Interval of Mean	-0.24, 16.28	1.75, 13.58	3.12, 12.56
Median	12.50	8.59	9.38
Q1, Q3	-3.91, 15.63	-2.34, 16.80	-3.13, 15.63
Min, Max	-11.7, 38.3	-10.2, 27.3	-11.7, 38.3

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 16 (Week 164)			
n	11	9	20
Mean (SD)	63.78 (14.779)	59.64 (21.601)	61.91 (17.773)
95% Confidence Interval of Mean	53.85, 73.71	43.03, 76.24	53.60, 70.23
Median	63.28	59.38	61.72
Q1, Q3	55.47, 78.91	37.50, 80.47	50.39, 79.69
Min, Max	32.8, 84.4	35.9, 91.4	32.8, 91.4
Change from Baseline			
n	11	9	20
Mean (SD)	4.97 (12.957)	4.60 (9.452)	4.80 (11.225)
95% Confidence Interval of Mean	-3.73, 13.68	-2.66, 11.87	-0.45, 10.06
Median	1.56	0.00	0.78
Q1, Q3	-3.13, 19.53	-1.56, 11.72	-2.73, 14.06
Min, Max	-17.2, 25.8	-7.0, 19.5	-17.2, 25.8

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

Visit	Treatment Group during Double-Blind Phase: Placebo (N=45)	Treatment Group during Double-Blind Phase: Ganaxolone (N=43)	Total (N=88)
Visit 17 (Week 180)			
n	2	1	3
Mean (SD)	51.17 (0.552)	59.38 (N/A)	53.91 (4.752)
95% Confidence Interval of Mean	46.21, 56.14	N/A, N/A	42.10, 65.71
Median	51.17	59.38	51.56
Q1, Q3	50.78, 51.56	59.38, 59.38	50.78, 59.38
Min, Max	50.8, 51.6	59.4, 59.4	50.8, 59.4
Change from Baseline			
n	2	1	3
Mean (SD)	-19.14 (10.496)	-0.78 (N/A)	-13.02 (12.940)
95% Confidence Interval of Mean	-113.44, 75.16	N/A, N/A	-45.17, 19.12
Median	-19.14	-0.78	-11.72
Q1, Q3	-26.56, -11.72	-0.78, -0.78	-26.56, -0.78
Min, Max	-26.6, -11.7	-0.8, -0.8	-26.6, -0.8

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-qidis-ole.sas
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Table 14.2.6.5A
Summary of Quality of Life Inventory - Disability (QI-Disability)
(Open-Label Extension Phase, Intent-to-Treat Population)

All Items

	Treatment Group during Double-Blind Phase		
Visit	Placebo (N=45)	Ganaxolone (N=43)	Total (N=88)
Taper Visit [2]			
n	17	12	29
Mean (SD)	58.87 (19.172)	64.78 (18.104)	61.31 (18.643)
95% Confidence Interval of Mean	49.01, 68.73	53.28, 76.28	54.22, 68.41
Median	57.03	62.89	60.16
Q1, Q3	46.09, 70.31	50.00, 80.08	49.22, 74.22
Min, Max	28.1, 91.4	33.6, 94.5	28.1, 94.5
Change from Baseline			
n	16	12	28
Mean (SD)	5.42 (17.598)	6.90 (9.338)	6.05 (14.427)
95% Confidence Interval of Mean	-3.96, 14.80	0.97, 12.83	0.46, 11.65
Median	5.08	5.86	5.86
Q1, Q3	-1.56, 16.41	2.73, 13.28	1.17, 16.41
Min, Max	-29.7, 31.3	-7.8, 21.1	-29.7, 31.3

[1] Last non-missing value prior to the first dose.
[2] Only subjects not continuing into the open label phase of the study would have this visit during the DB phase. If the subject prematurely discontinued, the Taper Visit was reassigned to the first visit at which the assessment was scheduled subsequent to the discontinuation as long as the scheduled day of that visit was within 32 days of the Taper Visit.
Subjects are grouped by the treatment received in DB phase.
Source Data: Listing 16.2.9.5A

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Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-02-06-05a-qidis-ole.rtf

4.8.8 Ergebnisse zum Endpunkt Sicherheit während der OLE

4.8.8.1 Unerwünschte Ereignisse auf Ebene der SOC und PT nach Schweregrad während der OLE

Marinus, Inc.
Study No. 1042-CDD-3001

Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Any TEAE [1]	Mild	9 (20.0)	204	11 (25.6)	140	20 (22.7)	344
	Moderate	16 (35.6)	133	16 (37.2)	84	32 (36.4)	217
	Severe	16 (35.6)	37	12 (27.9)	21	28 (31.8)	58
Infections And Infestations	Mild	10 (22.2)	51	9 (20.9)	44	19 (21.6)	95
	Moderate	9 (20.0)	25	10 (23.3)	19	19 (21.6)	44
	Severe	6 (13.3)	13	5 (11.6)	6	11 (12.5)	19
Upper Respiratory Tract Infection	Mild	8 (17.8)	9	4 (9.3)	4	12 (13.6)	13
	Moderate	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Covid-19	Mild	5 (11.1)	5	6 (14.0)	6	11 (12.5)	11
	Moderate	2 (4.4)	2	1 (2.3)	1	3 (3.4)	3
	Severe	0	0	0	0	0	0
Nasopharyngitis	Mild	6 (13.3)	10	3 (7.0)	7	9 (10.2)	17
	Moderate	1 (2.2)	1	3 (7.0)	3	4 (4.5)	4
	Severe	0	0	0	0	0	0
Urinary Tract Infection	Mild	0	1	3 (7.0)	3	3 (3.4)	4
	Moderate	3 (6.7)	3	1 (2.3)	1	4 (4.5)	4
	Severe	1 (2.2)	1	0	0	1 (1.1)	1

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-teae-soc-pt-sev-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-03-02-04-01a-ae-spt-sev-ole.rtf

Marinus, Inc.
Study No. 1042-CDD-3001

Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Bronchitis	Mild	1 (2.2)	1	2 (4.7)	2	3 (3.4)	3
	Moderate	2 (4.4)	2	2 (4.7)	2	4 (4.5)	4
	Severe	0	0	0	0	0	0
Ear Infection	Mild	4 (8.9)	5	0	0	4 (4.5)	5
	Moderate	2 (4.4)	2	1 (2.3)	1	3 (3.4)	3
	Severe	0	0	0	0	0	0
Rhinitis	Mild	2 (4.4)	2	5 (11.6)	6	7 (8.0)	8
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Pneumonia	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	3 (6.7)	3	2 (4.7)	2	5 (5.7)	5
Gastroenteritis	Mild	3 (6.7)	3	1 (2.3)	1	4 (4.5)	4
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Viral Infection	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	1 (2.2)	1	2 (4.7)	2	3 (3.4)	3
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Influenza	Mild	0	1	0	0	0	1
	Moderate	3 (6.7)	3	0	0	3 (3.4)	3
	Severe	0	0	1 (2.3)	1	1 (1.1)	1

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-teae-soc-pt-sev-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-03-02-04-01a-ae-spt-sev-ole.rtf

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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Sepsis	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
Gastrointestinal Viral Infection	Mild	2 (4.4)	2	0	0	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Lower Respiratory Tract Infection	Mild	0	0	0	1	0	1
	Moderate	1 (2.2)	2	1 (2.3)	1	2 (2.3)	3
	Severe	0	0	0	0	0	0
Lower Respiratory Tract Infection Viral	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Metapneumovirus Infection	Mild	0	0	1 (2.3)	2	1 (1.1)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Pharyngitis Streptococcal	Mild	0	0	1 (2.3)	2	1 (1.1)	2
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Pneumonia Viral	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	1 (2.3)	1	1 (1.1)	1

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-teae-soc-pt-sev-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-03-02-04-01a-ae-spt-sev-ole.rtf

Marinus, Inc.
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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase Placebo (N=45) Subjects n (%)	 Events n	 Ganaxolone (N=43) Subjects n (%)	 Events n	 Total (N=88) Subjects n (%)	 Events n
Respiratory Tract Infection	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Respiratory Tract Infection Viral	Mild	1 (2.2)	2	1 (2.3)	1	2 (2.3)	3
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Sinusitis	Mild	2 (4.4)	2	0	0	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Acute Sinusitis	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Adenovirus Infection	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Asymptomatic Covid-19	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Bacterial Disease Carrier	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-teae-soc-pt-sev-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-03-02-04-01a-ae-spt-sev-ole.rtf

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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone Events n	Total (N=88) Subjects n (%)	Total Events n
Bacterial Vaginosis	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Body Tinea	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Bronchiolitis	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Eczema Infected	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Enterovirus Infection	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Escherichia Urinary Tract Infection	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Gingivitis	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-teae-soc-pt-sev-ole.sas
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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Impetigo	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Localised Infection	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Oral Candidiasis	Mild	0	0	1 (2.3)	2	1 (1.1)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Otitis Media	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Otitis Media Acute	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Pharyngitis	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Postoperative Wound Infection	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-teae-soc-pt-sev-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-03-02-04-01a-ae-spt-sev-ole.rtf

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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Pyelonephritis	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Respiratory Syncytial Virus Infection	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Rhinovirus Infection	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Roseola	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Septic Shock	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Staphylococcal Scalded Skin Syndrome	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Tinea Capitis	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase Placebo (N=45) Subjects n (%)	 Events n	 Ganaxolone (N=43) Subjects n (%)	 Events n	 Total (N=88) Subjects n (%)	 Events n
Upper Respiratory Tract Infection Bacterial	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Viral Upper Respiratory Tract Infection	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Nervous System Disorders	Mild	11 (24.4)	25	4 (9.3)	13	15 (17.0)	38
	Moderate	9 (20.0)	16	11 (25.6)	18	20 (22.7)	34
	Severe	6 (13.3)	9	4 (9.3)	5	10 (11.4)	14
Seizure	Mild	4 (8.9)	8	2 (4.7)	4	6 (6.8)	12
	Moderate	7 (15.6)	10	6 (14.0)	9	13 (14.8)	19
	Severe	2 (4.4)	4	1 (2.3)	1	3 (3.4)	5
Somnolence	Mild	7 (15.6)	10	2 (4.7)	3	9 (10.2)	13
	Moderate	4 (8.9)	4	4 (9.3)	5	8 (9.1)	9
	Severe	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
Lethargy	Mild	2 (4.4)	2	1 (2.3)	1	3 (3.4)	3
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Drooling	Mild	2 (4.4)	2	0	0	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Headache	Mild	0	0	2 (4.7)	2	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Hypersomnia	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Hypotonia	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Status Epilepticus	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
Ataxia	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Chorea	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Circadian Rhythm Sleep Disorder	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

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Marinus, Inc.
Study No. 1042-CDD-3001

Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Coma	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Dyskinesia	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Epilepsy	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Nystagmus	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Poor Quality Sleep	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Sedation	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Slow Response To Stimuli	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
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Source Data: Listing 16.2.10A

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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Tonic Convulsion	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Unresponsive To Stimuli	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Gastrointestinal Disorders	Mild	10 (22.2)	33	11 (25.6)	15	21 (23.9)	48
	Moderate	9 (20.0)	22	2 (4.7)	4	11 (12.5)	26
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Vomiting	Mild	8 (17.8)	12	7 (16.3)	9	15 (17.0)	21
	Moderate	3 (6.7)	6	1 (2.3)	1	4 (4.5)	7
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Diarrhoea	Mild	4 (8.9)	7	2 (4.7)	2	6 (6.8)	9
	Moderate	2 (4.4)	2	0	0	2 (2.3)	2
	Severe	0	0	0	0	0	0
Constipation	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	2 (4.4)	2	0	0	2 (2.3)	2
	Severe	0	0	0	0	0	0
Toothache	Mild	1 (2.2)	4	0	0	1 (1.1)	4
	Moderate	2 (4.4)	12	0	0	2 (2.3)	12
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-teae-soc-pt-sev-ole.sas
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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Nausea	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Salivary Hypersecretion	Mild	2 (4.4)	2	0	0	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Abdominal Pain	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Dysphagia	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	2	1 (1.1)	2
	Severe	0	0	0	0	0	0
Eructation	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Faeces Discoloured	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Gastrointestinal Disorder	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

		Treatment Group during Double-Blind Phase					
		Placebo (N=45)		Ganaxolone (N=43)		Total (N=88)	
System Organ Class Preferred Term	Severity [2]	Subjects n (%)	Events n	Subjects n (%)	Events n	Subjects n (%)	Events n
Gastrooesophageal Reflux Disease	Mild	0	0	1 (2.3)	2	1 (1.1)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Gingival Swelling	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Regurgitation	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Tooth Development Disorder	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Tooth Discolouration	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
General Disorders And Administration Site Conditions	Mild	9 (20.0)	26	7 (16.3)	17	16 (18.2)	43
	Moderate	2 (4.4)	18	7 (16.3)	14	9 (10.2)	32
	Severe	0	0	2 (4.7)	2	2 (2.3)	2
Pyrexia	Mild	6 (13.3)	9	6 (14.0)	10	12 (13.6)	19
	Moderate	0	0	4 (9.3)	7	4 (4.5)	7
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Gait Disturbance	Mild	2 (4.4)	2	1 (2.3)	1	3 (3.4)	3
	Moderate	0	0	2 (4.7)	2	2 (2.3)	2
	Severe	0	0	0	0	0	0
Fatigue	Mild	1 (2.2)	1	2 (4.7)	3	3 (3.4)	4
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Asthenia	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Severe	0	0	0	0	0	0
Pain	Mild	0	13	0	1	0	14
	Moderate	1 (2.2)	17	0	0	1 (1.1)	17
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Peripheral Swelling	Mild	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Crying	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	3	1 (1.1)	3
	Severe	0	0	0	0	0	0
Influenza Like Illness	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

		Treatment Group during Double-Blind Phase					
		Placebo (N=45)		Ganaxolone (N=43)		Total (N=88)	
System Organ Class Preferred Term	Severity [2]	Subjects n (%)	Events n	Subjects n (%)	Events n	Subjects n (%)	Events n
Sudden Unexplained Death In Epilepsy	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Swelling Face	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Respiratory, Thoracic And Mediastinal Disorders	Mild	1 (2.2)	8	6 (14.0)	9	7 (8.0)	17
	Moderate	7 (15.6)	9	5 (11.6)	8	12 (13.6)	17
	Severe	4 (8.9)	6	2 (4.7)	2	6 (6.8)	8
Cough	Mild	2 (4.4)	3	4 (9.3)	4	6 (6.8)	7
	Moderate	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Severe	0	0	0	0	0	0
Pneumonia Aspiration	Mild	0	0	0	0	0	0
	Moderate	2 (4.4)	2	2 (4.7)	2	4 (4.5)	4
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Acute Respiratory Failure	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	1 (2.2)	1	2 (4.7)	2	3 (3.4)	3
Respiratory Disorder	Mild	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-teae-soc-pt-sev-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-03-02-04-01a-ae-spt-sev-ole.rtf

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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Aspiration	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Dysphonia	Mild	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Lower Respiratory Tract Congestion	Mild	1 (2.2)	1	1 (2.3)	2	2 (2.3)	3
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Productive Cough	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Respiratory Distress	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	2 (4.4)	2	0	0	2 (2.3)	2
Respiratory Tract Congestion	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Sleep Apnoea Syndrome	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Decreased Bronchial Secretion	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Hypoxia	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Nasal Congestion	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Pneumonitis	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Upper Airway Obstruction	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Upper Respiratory Tract Inflammation	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Metabolism And Nutrition Disorders	Mild	3 (6.7)	6	8 (18.6)	8	11 (12.5)	14
	Moderate	3 (6.7)	6	3 (7.0)	6	6 (6.8)	12
	Severe	3 (6.7)	4	1 (2.3)	1	4 (4.5)	5

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Decreased Appetite	Mild	1 (2.2)	2	5 (11.6)	5	6 (6.8)	7
	Moderate	0	0	2 (4.7)	4	2 (2.3)	4
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Dehydration	Mild	0	1	1 (2.3)	1	1 (1.1)	2
	Moderate	3 (6.7)	4	0	0	3 (3.4)	4
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Hyperkalaemia	Mild	0	1	1 (2.3)	1	1 (1.1)	2
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Feeding Intolerance	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Hyponatraemia	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Hypercalcaemia	Mild	0	0	0	0	0	0
	Moderate	0	1	0	0	0	1
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Hypokalaemia	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
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Table 14.3.2.4.1A

Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

		Treatment Group during Double-Blind Phase					
		Placebo (N=45)		Ganaxolone (N=43)		Total (N=88)	
System Organ Class Preferred Term	Severity [2]	Subjects n (%)	Events n	Subjects n (%)	Events n	Subjects n (%)	Events n
Vitamin D Deficiency	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Investigations	Mild	2 (4.4)	6	6 (14.0)	8	8 (9.1)	14
	Moderate	4 (8.9)	5	4 (9.3)	5	8 (9.1)	10
	Severe	0	0	0	0	0	0
Weight Decreased	Mild	0	0	2 (4.7)	2	2 (2.3)	2
	Moderate	1 (2.2)	1	3 (7.0)	4	4 (4.5)	5
	Severe	0	0	0	0	0	0
Electrocardiogram Qt Prolonged	Mild	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Sars-Cov-2 Test Positive	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Alanine Aminotransferase Increased	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Blood Pressure Increased	Mild	0	1	0	0	0	1
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Body Temperature Increased	Mild	0	0	1 (2.3)	2	1 (1.1)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Bone Density Decreased	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Electrocardiogram Change	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Oxygen Saturation Decreased	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Pco2 Increased	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Platelet Count Increased	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Respiratory Syncytial Virus Test Positive	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

		Treatment Group during Double-Blind Phase					
		Placebo (N=45)		Ganaxolone (N=43)		Total (N=88)	
System Organ Class Preferred Term	Severity [2]	Subjects n (%)	Events n	Subjects n (%)	Events n	Subjects n (%)	Events n
Urine Analysis Abnormal	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Weight Increased	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Skin And Subcutaneous Tissue Disorders	Mild	7 (15.6)	8	7 (16.3)	7	14 (15.9)	15
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Rash	Mild	3 (6.7)	4	0	0	3 (3.4)	4
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Alopecia	Mild	2 (4.4)	2	0	0	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Dry Skin	Mild	0	0	2 (4.7)	2	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Erythema	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
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Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Hypohidrosis	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Keratosis Pilaris	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Pityriasis Rosea	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Rash Maculo-Papular	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Rash Maculovesicular	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Urticaria	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Injury, Poisoning And Procedural Complications	Mild	5 (11.1)	11	3 (7.0)	3	8 (9.1)	14
	Moderate	3 (6.7)	4	1 (2.3)	1	4 (4.5)	5
	Severe	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
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Table 14.3.2.4.1A

Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase Placebo (N=45) Subjects n (%)	 Events n	 Ganaxolone (N=43) Subjects n (%)	 Events n	 Total (N=88) Subjects n (%)	 Events n
Contusion	Mild	2 (4.4)	2	0	0	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Postoperative Ileus	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Product Dose Omission	Mild	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Skin Laceration	Mild	1 (2.2)	2	1 (2.3)	1	2 (2.3)	3
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Endotracheal Intubation Complication	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Femur Fracture	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Head Injury	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Hip Fracture	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Incorrect Dose Administered	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Joint Dislocation	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Lip Injury	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Procedural Pain	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Stoma Site Haemorrhage	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Tibia Fracture	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
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[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
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Table 14.3.2.4.1A
Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

		Treatment Group during Double-Blind Phase					
		Placebo (N=45)		Ganaxolone (N=43)		Total (N=88)	
System Organ Class Preferred Term	Severity [2]	Subjects n (%)	Events n	Subjects n (%)	Events n	Subjects n (%)	Events n
Torus Fracture	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Wrong Dose	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Psychiatric Disorders	Mild	5 (11.1)	10	2 (4.7)	5	7 (8.0)	15
	Moderate	0	17	4 (9.3)	6	4 (4.5)	23
	Severe	2 (4.4)	2	1 (2.3)	1	3 (3.4)	3
Agitation	Mild	0	0	1 (2.3)	2	1 (1.1)	2
	Moderate	0	0	2 (4.7)	3	2 (2.3)	3
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Attention-Seeking Behaviour	Mild	2 (4.4)	2	1 (2.3)	1	3 (3.4)	3
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Insomnia	Mild	1 (2.2)	5	1 (2.3)	1	2 (2.3)	6
	Moderate	1 (2.2)	16	0	0	1 (1.1)	16
	Severe	0	0	0	0	0	0
Sleep Disorder	Mild	1 (2.2)	1	0	1	1 (1.1)	2
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
[1]TEAE=Treatment emergent adverse event, defined as an AE that occurred or worsened during the open-label extension phase.
[2]Highest severity for subjects.
Source Data: Listing 16.2.10A

Program Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\t-teae-soc-pt-sev-ole.sas
Output Location: B:\Marinus_Pharma_Inc\1042-CDD-3001\bs\csr-DBL\prog\tlfs\output\t-14-03-02-04-01a-ae-spt-sev-ole.rtf

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System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Abnormal Behaviour	Mild	0	1	0	0	0	1
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Irritability	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Mood Altered	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Restlessness	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Social Avoidant Behaviour	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Sopor	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Blood And Lymphatic System Disorders	Mild	3 (6.7)	4	2 (4.7)	2	5 (5.7)	6
	Moderate	2 (4.4)	2	1 (2.3)	1	3 (3.4)	3
	Severe	0	0	0	0	0	0

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Anaemia	Mild	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Thrombocytosis	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Anaemia Macrocytic	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Hypochromic Anaemia	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Leukocytosis	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Thrombocytopenia	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Renal And Urinary Disorders	Mild	2 (4.4)	2	3 (7.0)	3	5 (5.7)	5
	Moderate	2 (4.4)	2	0	0	2 (2.3)	2
	Severe	1 (2.2)	1	0	0	1 (1.1)	1

Note: If a subject experiences more than one adverse event in a category, the subject is counted only once according to the highest severity in that category.
Subjects are grouped by the treatment received in DB phase.
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System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Urinary Retention	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Acute Kidney Injury	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Crystalluria	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Glycosuria	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Proteinuria	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Renal Failure	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	1 (2.2)	1	0	0	1 (1.1)	1
Urine Odour Abnormal	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

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Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
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System Organ Class Preferred Term	Severity [2]	Treatment Group during Double-Blind Phase: Placebo (N=45) Subjects n (%)	Placebo (N=45) Events n	Ganaxolone (N=43) Subjects n (%)	Ganaxolone (N=43) Events n	Total (N=88) Subjects n (%)	Total (N=88) Events n
Reproductive System And Breast Disorders	Mild	4 (8.9)	6	2 (4.7)	2	6 (6.8)	8
	Moderate	2 (4.4)	3	0	0	2 (2.3)	3
	Severe	0	0	0	0	0	0
Menstruation Irregular	Mild	2 (4.4)	2	1 (2.3)	1	3 (3.4)	3
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Menorrhagia	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Polymenorrhoea	Mild	2 (4.4)	2	0	0	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Breast Mass	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Dysmenorrhoea	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	2	0	0	1 (1.1)	2
	Severe	0	0	0	0	0	0
Oligomenorrhoea	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

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Summary of Treatment Emergent Adverse Events by System Organ Class, Preferred Term and Severity
(Open-Label Extension Phase, Safety Population)

		Treatment Group during Double-Blind Phase					
		Placebo (N=45)		Ganaxolone (N=43)		Total (N=88)	
System Organ Class Preferred Term	Severity [2]	Subjects n (%)	Events n	Subjects n (%)	Events n	Subjects n (%)	Events n
Musculoskeletal And Connective Tissue Disorders	Mild	3 (6.7)	4	0	0	3 (3.4)	4
	Moderate	1 (2.2)	1	1 (2.3)	1	2 (2.3)	2
	Severe	0	0	0	0	0	0
Arthralgia	Mild	2 (4.4)	2	0	0	2 (2.3)	2
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Bone Pain	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Hypermobility Syndrome	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Pain In Extremity	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Scoliosis	Mild	0	0	0	0	0	0
	Moderate	0	0	1 (2.3)	1	1 (1.1)	1
	Severe	0	0	0	0	0	0
Cardiac Disorders	Mild	1 (2.2)	2	1 (2.3)	1	2 (2.3)	3
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	1 (2.3)	1	1 (1.1)	1

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Cardiac Arrest	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Defect Conduction Intraventricular	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Sinus Tachycardia	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Tachycardia	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Tachycardia Paroxysmal	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Immune System Disorders	Mild	1 (2.2)	1	0	1	1 (1.1)	2
	Moderate	0	0	0	1	0	1
	Severe	0	0	2 (4.7)	2	2 (2.3)	2
Hypersensitivity	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

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System Organ Class Preferred Term	Severity [2]	Subjects n (%)	Events n	Subjects n (%)	Events n	Subjects n (%)	Events n
Immune System Disorder	Mild	0	0	0	0	0	0
	Moderate	0	0	0	0	0	0
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Seasonal Allergy	Mild	0	0	0	1	0	1
	Moderate	0	0	0	1	0	1
	Severe	0	0	1 (2.3)	1	1 (1.1)	1
Endocrine Disorders	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Precocious Puberty	Mild	1 (2.2)	1	0	0	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Eye Disorders	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Ocular Hyperaemia	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Social Circumstances	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0

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System Organ Class Preferred Term	Severity [2]	Subjects n (%)	Events n	Subjects n (%)	Events n	Subjects n (%)	Events n
Social Problem	Mild	0	0	1 (2.3)	1	1 (1.1)	1
	Moderate	0	0	0	0	0	0
	Severe	0	0	0	0	0	0
Vascular Disorders	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0
Hypovolaemic Shock	Mild	0	0	0	0	0	0
	Moderate	1 (2.2)	1	0	0	1 (1.1)	1
	Severe	0	0	0	0	0	0

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