Omaveloxolon (Skyclarys™)

Biogen GmbH

Anhang 4-H zu Modul 4A und 4B

Behandlung der Friedreich-Ataxie (FA) bei Erwachsenen und Jugendlichen ab 16 Jahren

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MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis -ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	52	51
Mean score	37.81	40.67
SD	10.778	10.160
Week 4		
N	52	50
LS mean change from baseline	-1.10	-1.63
SE	0.509	0.521
95% CI	(-2.12, -0.09)	(-2.67, -0.60)
LS mean difference (Omaveloxolone-Placebo)		-0.53
SE		0.738
95% CI		(-2.00, 0.94)
p-value		0.4746
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.14
95% CI		(-0.53, 0.25)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm.sas **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis -ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 12		
N	52	50
LS mean change from baseline	-1.58	-1.06
SE	0.572	0.584
95% CI	(-2.71, -0.44)	(-2.22, 0.10)
LS mean difference (Omaveloxolone-Placebo)		0.52
SE		0.827
95% CI		(-1.13, 2.16)
p-value		0.5330
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.12
95% CI		(-0.27, 0.51)
Week 18		
N	51	47
LS mean change from baseline	-1.01	-1.59
SE	0.610	0.632
95% CI	(-2.23, 0.20)	(-2.85, -0.34)
LS mean difference (Omaveloxolone-Placebo)		-0.58
SE		0.887
95% CI		(-2.34, 1.18)
p-value		0.5144
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-0.53, 0.27)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm.sas **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis -ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 24		
N	51	45
LS mean change from baseline	-0.69	-1.45
SE	0.598	0.631
95% CI	(-1.88, 0.50)	(-2.71, -0.20)
LS mean difference (Omaveloxolone-Placebo)		-0.76
SE		0.879
95% CI		(-2.51, 0.98)
p-value		0.3874
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.17
95% CI		(-0.58, 0.23)
Week 36		
N	51	44
LS mean change from baseline	-0.13	-1.14
SE	0.667	0.708
95% CI	(-1.45, 1.20)	(-2.55, 0.26)
LS mean difference (Omaveloxolone-Placebo)		-1.02
SE		0.982
95% CI		(-2.97, 0.93)
p-value		0.3035
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.21
95% CI		(-0.61, 0.20)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm.sas **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis -ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 48		
N	50	42
LS mean change from baseline	0.82	-1.01
SE	0.600	0.642
95% CI	(-0.38, 2.01)	(-2.28, 0.27)
LS mean difference (Omaveloxolone-Placebo)		-1.82
SE		0.888
95% CI		(-3.59, -0.06)
p-value		0.0428
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.42
95% CI		(-0.84, -0.01)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm.sas **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	52	51
Mean score	0.63	0.73
SD	0.625	0.501
Week 4		
N	52	50
LS mean change from baseline	-0.02	-0.07
SE	0.045	0.046
95% CI	(-0.11, 0.07)	(-0.16, 0.02)
LS mean difference (Omaveloxolone-Placebo)		-0.05
SE		0.065
95% CI		(-0.18, 0.08)
p-value		0.4595
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.14
95% CI		(-0.53, 0.24)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
NOTE 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm.sas:t-mfars93-d-bul-mmrm-itt.rtf **Data Tag:** FINAL **Run Date:** 20SEP2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 12		
N	52	50
LS mean change from baseline	0.00	-0.06
SE	0.038	0.039
95% CI	(-0.08, 0.07)	(-0.14, 0.02)
LS mean difference (Omaveloxolone-Placebo)		-0.06
SE		0.055
95% CI		(-0.17, 0.05)
p-value		0.3048
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.20
95% CI		(-0.59, 0.19)
Week 18		
N	51	47
LS mean change from baseline	0.02	-0.01
SE	0.039	0.040
95% CI	(-0.06, 0.09)	(-0.09, 0.07)
LS mean difference (Omaveloxolone-Placebo)		-0.02
SE		0.056
95% CI		(-0.13, 0.09)
p-value		0.6968
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.08
95% CI		(-0.47, 0.32)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
NOTE 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm.sas:t-mfars93-d-bul-mmrm-itt.rtf **Data Tag:** FINAL **Run Date:** 20SEP2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 24		
N	51	45
LS mean change from baseline	0.10	-0.01
SE	0.041	0.044
95% CI	(0.01, 0.18)	(-0.09, 0.08)
LS mean difference (Omaveloxolone-Placebo)		-0.10
SE		0.060
95% CI		(-0.22, 0.02)
p-value		0.0946
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.34
95% CI		(-0.74, 0.07)
Week 36		
N	51	44
LS mean change from baseline	0.03	-0.04
SE	0.046	0.049
95% CI	(-0.06, 0.12)	(-0.14, 0.06)
LS mean difference (Omaveloxolone-Placebo)		-0.07
SE		0.068
95% CI		(-0.21, 0.06)
p-value		0.2737
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.22
95% CI		(-0.63, 0.18)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
NOTE 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm.sas:t-mfars93-d-bul-mmrm-itt.rtf **Data Tag:** FINAL **Run Date:** 20SEP2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 48		
N	50	42
LS mean change from baseline	-0.03	-0.08
SE	0.049	0.054
95% CI	(-0.13, 0.07)	(-0.18, 0.03)
LS mean difference (Omaveloxolone-Placebo)		-0.05
SE		0.073
95% CI		(-0.19, 0.10)
p-value		0.5079
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.14
95% CI		(-0.55, 0.27)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
NOTE 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm.sas:t-mfars93-d-bul-mmrm-itt.rtf **Data Tag:** FINAL **Run Date:** 20SEP2024

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Upper Limb Coordination Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	52	51
Mean score	9.90	10.75
SD	3.534	3.712
Week 4		
N	52	50
LS mean change from baseline	-0.62	-0.82
SE	0.298	0.305
95% CI	(-1.21, -0.03)	(-1.42, -0.21)
LS mean difference (Omaveloxolone-Placebo)		-0.20
SE		0.431
95% CI		(-1.06, 0.66)
p-value		0.6484
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.09
95% CI		(-0.48, 0.30)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-ul-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Upper Limb Coordination Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 12		
N	52	50
LS mean change from baseline	-0.58	-0.71
SE	0.302	0.308
95% CI	(-1.18, 0.02)	(-1.32, -0.09)
LS mean difference (Omaveloxolone-Placebo)		-0.13
SE		0.436
95% CI		(-0.99, 0.74)
p-value		0.7714
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.06
95% CI		(-0.44, 0.33)
Week 18		
N	51	47
LS mean change from baseline	-0.62	-0.76
SE	0.339	0.351
95% CI	(-1.30, 0.05)	(-1.46, -0.07)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.492
95% CI		(-1.12, 0.84)
p-value		0.7758
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.06
95% CI		(-0.45, 0.34)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-ul-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Upper Limb Coordination Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 24		
N	51	45
LS mean change from baseline	-0.52	-0.41
SE	0.299	0.316
95% CI	(-1.11, 0.08)	(-1.04, 0.22)
LS mean difference (Omaveloxolone-Placebo)		0.11
SE		0.438
95% CI		(-0.77, 0.98)
p-value		0.8101
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.05
95% CI		(-0.35, 0.45)
Week 36		
N	51	44
LS mean change from baseline	-0.29	-0.57
SE	0.341	0.361
95% CI	(-0.97, 0.39)	(-1.29, 0.15)
LS mean difference (Omaveloxolone-Placebo)		-0.28
SE		0.500
95% CI		(-1.28, 0.71)
p-value		0.5735
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.11
95% CI		(-0.52, 0.29)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-ul-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Upper Limb Coordination Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 48		
N	50	42
LS mean change from baseline	0.11	-0.72
SE	0.369	0.395
95% CI	(-0.62, 0.84)	(-1.51, 0.07)
LS mean difference (Omaveloxolone-Placebo)		-0.83
SE		0.543
95% CI		(-1.91, 0.25)
p-value		0.1306
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.31
95% CI		(-0.73, 0.10)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-ul-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Lower Limb Coordination Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	52	51
Mean score	6.25	6.29
SD	2.285	2.579
Week 4		
N	52	50
LS mean change from baseline	-0.71	-0.26
SE	0.234	0.239
95% CI	(-1.17, -0.24)	(-0.73, 0.22)
LS mean difference (Omaveloxolone-Placebo)		0.45
SE		0.337
95% CI		(-0.22, 1.12)
p-value		0.1826
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.26
95% CI		(-0.13, 0.65)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-ll-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Lower Limb Coordination Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 12		
N	52	50
LS mean change from baseline	-0.96	-0.27
SE	0.238	0.243
95% CI	(-1.44, -0.49)	(-0.75, 0.21)
LS mean difference (Omaveloxolone-Placebo)		0.70
SE		0.343
95% CI		(0.01, 1.38)
p-value		0.0455
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.39
95% CI		(0.00, 0.79)
Week 18		
N	51	47
LS mean change from baseline	-0.57	-0.21
SE	0.271	0.280
95% CI	(-1.11, -0.03)	(-0.77, 0.35)
LS mean difference (Omaveloxolone-Placebo)		0.36
SE		0.392
95% CI		(-0.42, 1.14)
p-value		0.3579
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.18
95% CI		(-0.21, 0.58)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-ll-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Lower Limb Coordination Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 24		
N	51	45
LS mean change from baseline	-0.47	-0.55
SE	0.251	0.265
95% CI	(-0.97, 0.03)	(-1.07, -0.02)
LS mean difference (Omaveloxolone-Placebo)		-0.08
SE		0.368
95% CI		(-0.81, 0.65)
p-value		0.8274
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.04
95% CI		(-0.44, 0.36)
Week 36		
N	51	44
LS mean change from baseline	-0.58	-0.22
SE	0.259	0.276
95% CI	(-1.09, -0.06)	(-0.77, 0.33)
LS mean difference (Omaveloxolone-Placebo)		0.36
SE		0.380
95% CI		(-0.40, 1.11)
p-value		0.3505
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.19
95% CI		(-0.22, 0.59)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-ll-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Lower Limb Coordination Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 48		
N	50	42
LS mean change from baseline	-0.30	-0.13
SE	0.283	0.303
95% CI	(-0.86, 0.26)	(-0.73, 0.48)
LS mean difference (Omaveloxolone-Placebo)		0.17
SE		0.417
95% CI		(-0.66, 1.00)
p-value		0.6822
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.08
95% CI		(-0.33, 0.49)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-ll-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Upright Stability Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	52	51
Mean score	21.02	22.89
SD	7.125	6.526
Week 4		
N	52	50
LS mean change from baseline	0.26	-0.51
SE	0.274	0.280
95% CI	(-0.28, 0.81)	(-1.07, 0.04)
LS mean difference (Omaveloxolone-Placebo)		-0.78
SE		0.396
95% CI		(-1.56, 0.01)
p-value		0.0531
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.38
95% CI		(-0.77, 0.01)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-us-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Upright Stability Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 12		
N	52	50
LS mean change from baseline	-0.07	0.02
SE	0.294	0.301
95% CI	(-0.65, 0.52)	(-0.58, 0.61)
LS mean difference (Omaveloxolone-Placebo)		0.08
SE		0.425
95% CI		(-0.76, 0.93)
p-value		0.8443
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.04
95% CI		(-0.35, 0.43)
Week 18		
N	51	47
LS mean change from baseline	0.10	-0.57
SE	0.292	0.304
95% CI	(-0.48, 0.68)	(-1.17, 0.04)
LS mean difference (Omaveloxolone-Placebo)		-0.67
SE		0.427
95% CI		(-1.52, 0.18)
p-value		0.1217
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.31
95% CI		(-0.71, 0.09)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-us-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Upright Stability Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 24		
N	51	45
LS mean change from baseline	0.10	-0.39
SE	0.336	0.354
95% CI	(-0.56, 0.77)	(-1.10, 0.31)
LS mean difference (Omaveloxolone-Placebo)		-0.50
SE		0.493
95% CI		(-1.48, 0.48)
p-value		0.3150
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.20
95% CI		(-0.60, 0.20)
Week 36		
N	51	44
LS mean change from baseline	0.61	-0.18
SE	0.373	0.396
95% CI	(-0.13, 1.35)	(-0.96, 0.61)
LS mean difference (Omaveloxolone-Placebo)		-0.79
SE		0.550
95% CI		(-1.88, 0.31)
p-value		0.1568
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.29
95% CI		(-0.69, 0.12)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-us-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Modified Friedreich's Ataxia Rating Scale (mFARS) Upright Stability Domain Score LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 48		
N	50	42
LS mean change from baseline	0.94	-0.11
SE	0.365	0.390
95% CI	(0.22, 1.67)	(-0.88, 0.67)
LS mean difference (Omaveloxolone-Placebo)		-1.05
SE		0.540
95% CI		(-2.12, 0.02)
p-value		0.0551
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.40
95% CI		(-0.81, 0.02)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.
Note 2: Baseline refers to the corresponding domain baseline score.
Source: biib141/valueaccess/amnog/t-mfars-d-us-mmrm.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score at Week 48 with imputation - ITT population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Number of subjects with week 48 assessment (%)	50 (96.2)	42 (82.4)
Number of subjects with event (%)	23 (44.2)	21 (41.2)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		0.93
95% CI		(0.59, 1.46)
p-value		0.7514
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		0.88
95% CI		(0.41, 1.92)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.03
95% CI		(-0.22, 0.16)

NOTE 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
NOTE 2: Worsening if change from baseline at Week 48 >= 1.9 in mFARS Total Score. Subject without week 48 assessment will be imputed as responder with a worsening event.

Source: biib141/valueaccess/amnog/t-mfars93-propw-imp.sas:t-mfars93-propw-imp-itt.rtf **Data Tag:** FINAL **Run Date:** 20SEP2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline at Week 48: Treatment by Subgroup Interaction from MMRM Analysis - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Age (<18, >=18)	0.7254
Gender (female, male)	0.5918
GAA1 repeat length >=675 (yes, no)	0.3786
Geographic location (US, other)	0.2811
Pes Cavus status (yes, no)	0.1322

Note 1: p-value is based on the MMRM model for the ITT population with three way interaction added (subgroup*treatment*time).
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm-int.sas **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change at Week 48 by Gender (Female, Male) from MMRM Analysis - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Baseline		
N	17	31
Mean score	32.36	41.50
SD	7.787	10.326
Week 48		
N	16	26
LS mean change from baseline	0.93	-0.55
SE	1.112	0.825
95% CI	(-1.28, 3.14)	(-2.19, 1.09)
LS mean difference (Omaveloxolone-Placebo)		-1.48
SE		1.409
95% CI		(-4.28, 1.32)
p-value		0.2957
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.32
95% CI		(-0.95, 0.30)

Note 1: LS Mean and Hedges'g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp-itt.sas:t-mfars93-mmrm-sgrp-itt-gen.rtf **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change at Week 48 by Gender (Female, Male) from MMRM Analysis - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Baseline		
N	35	20
Mean score	40.45	39.39
SD	11.123	10.022
Week 48		
N	34	16
LS mean change from baseline	0.75	-1.78
SE	0.742	1.068
95% CI	(-0.73, 2.22)	(-3.90, 0.34)
LS mean difference (Omaveloxolone-Placebo)		-2.53
SE		1.305
95% CI		(-5.12, 0.07)
p-value		0.0559
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.57
95% CI		(-1.17, 0.03)

Note 1: LS Mean and Hedges'g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp-itt.sas:t-mfars93-mmrm-sgrp-itt-gen.rtf **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93 points Rating Scale (mFARS) Total Score LS Mean Change from Baseline at Week 48 by Geographic Location (US, Other) from MMRM Analysis - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Baseline		
N	35	36
Mean score	37.49	42.66
SD	12.012	10.156
Week 48		
N	35	29
LS mean change from baseline	1.03	-1.34
SE	0.737	0.795
95% CI	(-0.43, 2.50)	(-2.92, 0.24)
LS mean difference (Omaveloxolone-Placebo)		-2.37
SE		1.094
95% CI		(-4.55, -0.20)
p-value		0.0327
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.53
95% CI		(-1.03, -0.03)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.
Note 3: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp-itt.sas:t-mfars93-mmrm-sgrp-itt-geo.rtf **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93 points Rating Scale (mFARS) Total Score LS Mean Change from Baseline at Week 48 by Geographic Location (US, Other) from MMRM Analysis - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Baseline		
N	17	15
Mean score	38.46	35.91
SD	7.937	8.744
Week 48		
N	15	13
LS mean change from baseline	0.19	-0.06
SE	1.093	1.187
95% CI	(-1.98, 2.36)	(-2.42, 2.30)
LS mean difference (Omaveloxolone-Placebo)		-0.25
SE		1.609
95% CI		(-3.44, 2.95)
p-value		0.8782
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.06
95% CI		(-0.80, 0.69)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.
Note 3: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp-itt.sas:t-mfars93-mmrm-sgrp-itt-geo.rtf **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline at Week 48 by GAA1 Repeat Length >=675 (Yes, No) from MMRM Analysis - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Baseline		
N	21	26
Mean score	42.06	44.32
SD	11.635	9.655
Week 48		
N	21	22
LS mean change from baseline	1.54	-1.03
SE	0.937	0.902
95% CI	(-0.33, 3.42)	(-2.83, 0.77)
LS mean difference (Omaveloxolone-Placebo)		-2.58
SE		1.276
95% CI		(-5.12, -0.03)
p-value		0.0477
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.59
95% CI		(-1.20, 0.02)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp.sas **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline at Week 48 by GAA1 Repeat Length >=675 (Yes, No) from MMRM Analysis - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Baseline		
N	22	15
Mean score	33.03	39.45
SD	9.763	9.474
Week 48		
N	21	13
LS mean change from baseline	0.67	-0.16
SE	0.970	1.170
95% CI	(-1.26, 2.61)	(-2.50, 2.17)
LS mean difference (Omaveloxolone-Placebo)		-0.84
SE		1.492
95% CI		(-3.82, 2.14)
p-value		0.5768
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.19
95% CI		(-0.88, 0.50)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp.sas **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline at Week 48: Treatment by Subgroup Interaction from MMRM Analysis - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Gender (female, male)	0.9615
GAA1 repeat length >=675 (yes, no)	0.3179
Geographic location (US, other)	0.8807

NOTE 1: p-value is based on the MMRM model for the ITT population with three way interaction added (subgroup*treatment*time).
Source: biib141/valueaccess/amnog/t-mfars93-bul-mmrm-int.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Baseline		
N	17	31
Mean score	0.46	0.82
SD	0.607	0.529
Week 4		
N	17	31
LS mean change from baseline	-0.10	-0.13
SE	0.080	0.059
95% CI	(-0.26, 0.06)	(-0.25, -0.01)
LS mean difference (Omaveloxolone-Placebo)		-0.02
SE		0.101
95% CI		(-0.23, 0.18)
p-value		0.8139
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.07
95% CI		(-0.66, 0.52)
Week 12		
N	17	31
LS mean change from baseline	0.05	-0.11
SE	0.068	0.050
95% CI	(-0.09, 0.18)	(-0.21, -0.01)
LS mean difference (Omaveloxolone-Placebo)		-0.16
SE		0.086
95% CI		(-0.33, 0.01)
p-value		0.0702
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.54
95% CI		(-1.14, 0.06)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 18		
N	17	31
LS mean change from baseline	0.01	0.04
SE	0.069	0.050
95% CI	(-0.13, 0.15)	(-0.06, 0.14)
LS mean difference (Omaveloxolone-Placebo)		0.03
SE		0.086
95% CI		(-0.14, 0.20)
p-value		0.7494
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.09
95% CI		(-0.50, 0.69)
Week 24		
N	17	29
LS mean change from baseline	-0.01	0.00
SE	0.073	0.055
95% CI	(-0.16, 0.13)	(-0.11, 0.11)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.092
95% CI		(-0.17, 0.20)
p-value		0.8830
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.04
95% CI		(-0.56, 0.64)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 36		
N	17	28
LS mean change from baseline	0.21	-0.02
SE	0.079	0.060
95% CI	(0.05, 0.36)	(-0.14, 0.10)
LS mean difference (Omaveloxolone-Placebo)		-0.23
SE		0.100
95% CI		(-0.43, -0.03)
p-value		0.0255
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.68
95% CI		(-1.30, -0.06)
Week 48		
N	16	26
LS mean change from baseline	-0.04	-0.09
SE	0.090	0.069
95% CI	(-0.22, 0.14)	(-0.23, 0.05)
LS mean difference (Omaveloxolone-Placebo)		-0.05
SE		0.115
95% CI		(-0.28, 0.18)
p-value		0.6753
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-0.75, 0.49)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Baseline		
N	35	20
Mean score	0.71	0.58
SD	0.625	0.422
Week 4		
N	35	19
LS mean change from baseline	0.02	0.02
SE	0.055	0.075
95% CI	(-0.09, 0.13)	(-0.13, 0.17)
LS mean difference (Omaveloxolone-Placebo)		0.00
SE		0.094
95% CI		(-0.18, 0.19)
p-value		0.9706
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.01
95% CI		(-0.55, 0.57)
Week 12		
N	35	19
LS mean change from baseline	-0.03	0.02
SE	0.047	0.064
95% CI	(-0.12, 0.07)	(-0.10, 0.15)
LS mean difference (Omaveloxolone-Placebo)		0.05
SE		0.080
95% CI		(-0.11, 0.21)
p-value		0.5504
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.17
95% CI		(-0.39, 0.73)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 18		
N	34	16
LS mean change from baseline	0.02	-0.09
SE	0.048	0.070
95% CI	(-0.08, 0.11)	(-0.23, 0.05)
LS mean difference (Omaveloxolone-Placebo)		-0.11
SE		0.085
95% CI		(-0.28, 0.06)
p-value		0.2028
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.38
95% CI		(-0.98, 0.22)
Week 24		
N	34	16
LS mean change from baseline	0.15	-0.02
SE	0.050	0.074
95% CI	(0.05, 0.25)	(-0.17, 0.13)
LS mean difference (Omaveloxolone-Placebo)		-0.17
SE		0.090
95% CI		(-0.35, 0.01)
p-value		0.0618
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.56
95% CI		(-1.16, 0.05)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 36		
N	34	16
LS mean change from baseline	-0.05	-0.09
SE	0.055	0.080
95% CI	(-0.16, 0.06)	(-0.25, 0.07)
LS mean difference (Omaveloxolone-Placebo)		-0.04
SE		0.098
95% CI		(-0.23, 0.16)
p-value		0.7098
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.11
95% CI		(-0.70, 0.48)
Week 48		
N	34	16
LS mean change from baseline	-0.02	-0.06
SE	0.061	0.090
95% CI	(-0.14, 0.10)	(-0.24, 0.12)
LS mean difference (Omaveloxolone-Placebo)		-0.04
SE		0.109
95% CI		(-0.26, 0.18)
p-value		0.7114
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.11
95% CI		(-0.70, 0.49)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Baseline		
N	35	36
Mean score	0.57	0.68
SD	0.688	0.559
Week 4		
N	35	35
LS mean change from baseline	0.01	-0.13
SE	0.056	0.056
95% CI	(-0.10, 0.13)	(-0.24, -0.02)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.080
95% CI		(-0.30, 0.02)
p-value		0.0781
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.42
95% CI		(-0.89, 0.06)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 12		
N	35	35
LS mean change from baseline	0.03	-0.08
SE	0.051	0.051
95% CI	(-0.07, 0.13)	(-0.19, 0.02)
LS mean difference (Omaveloxolone-Placebo)		-0.11
SE		0.072
95% CI		(-0.26, 0.03)
p-value		0.1208
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.37
95% CI		(-0.84, 0.11)
Week 18		
N	35	32
LS mean change from baseline	0.00	-0.04
SE	0.048	0.050
95% CI	(-0.10, 0.10)	(-0.14, 0.06)
LS mean difference (Omaveloxolone-Placebo)		-0.04
SE		0.070
95% CI		(-0.18, 0.10)
p-value		0.6003
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-0.61, 0.35)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 24		
N	35	31
LS mean change from baseline	0.13	-0.03
SE	0.054	0.057
95% CI	(0.02, 0.24)	(-0.15, 0.08)
LS mean difference (Omaveloxolone-Placebo)		-0.17
SE		0.079
95% CI		(-0.32, -0.01)
p-value		0.0374
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.51
95% CI		(-1.00, -0.02)
Week 36		
N	35	31
LS mean change from baseline	0.02	-0.08
SE	0.060	0.063
95% CI	(-0.10, 0.14)	(-0.20, 0.05)
LS mean difference (Omaveloxolone-Placebo)		-0.10
SE		0.088
95% CI		(-0.27, 0.08)
p-value		0.2619
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.27
95% CI		(-0.76, 0.21)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 48		
N	35	29
LS mean change from baseline	-0.05	-0.11
SE	0.066	0.071
95% CI	(-0.18, 0.08)	(-0.25, 0.03)
LS mean difference (Omaveloxolone-Placebo)		-0.06
SE		0.097
95% CI		(-0.25, 0.13)
p-value		0.5271
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.16
95% CI		(-0.65, 0.34)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Baseline		
N	17	15
Mean score	0.75	0.83
SD	0.468	0.309
Week 4		
N	17	15
LS mean change from baseline	-0.09	0.06
SE	0.081	0.087
95% CI	(-0.25, 0.07)	(-0.11, 0.23)
LS mean difference (Omaveloxolone-Placebo)		0.15
SE		0.118
95% CI		(-0.09, 0.38)
p-value		0.2119
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.43
95% CI		(-0.27, 1.13)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 12		
N	17	15
LS mean change from baseline	-0.06	-0.01
SE	0.073	0.079
95% CI	(-0.21, 0.08)	(-0.16, 0.15)
LS mean difference (Omaveloxolone-Placebo)		0.05
SE		0.107
95% CI		(-0.16, 0.27)
p-value		0.6248
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.17
95% CI		(-0.53, 0.86)
Week 18		
N	16	15
LS mean change from baseline	0.06	0.06
SE	0.071	0.074
95% CI	(-0.08, 0.20)	(-0.09, 0.21)
LS mean difference (Omaveloxolone-Placebo)		0.00
SE		0.103
95% CI		(-0.20, 0.20)
p-value		0.9992
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.00
95% CI		(-0.70, 0.70)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 24		
N	16	14
LS mean change from baseline	0.03	0.05
SE	0.080	0.085
95% CI	(-0.13, 0.19)	(-0.12, 0.22)
LS mean difference (Omaveloxolone-Placebo)		0.03
SE		0.116
95% CI		(-0.20, 0.26)
p-value		0.8207
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.08
95% CI		(-0.64, 0.80)
Week 36		
N	16	13
LS mean change from baseline	0.07	0.04
SE	0.089	0.098
95% CI	(-0.11, 0.24)	(-0.15, 0.24)
LS mean difference (Omaveloxolone-Placebo)		-0.03
SE		0.132
95% CI		(-0.29, 0.24)
p-value		0.8491
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.07
95% CI		(-0.80, 0.66)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 48		
N	15	13
LS mean change from baseline	0.04	0.00
SE	0.099	0.108
95% CI	(-0.16, 0.23)	(-0.21, 0.21)
LS mean difference (Omaveloxolone-Placebo)		-0.04
SE		0.146
95% CI		(-0.33, 0.26)
p-value		0.8099
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.09
95% CI		(-0.83, 0.66)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Baseline		
N	21	26
Mean score	0.77	0.72
SD	0.766	0.443
Week 4		
N	21	26
LS mean change from baseline	0.08	-0.03
SE	0.068	0.061
95% CI	(-0.06, 0.21)	(-0.15, 0.10)
LS mean difference (Omaveloxolone-Placebo)		-0.10
SE		0.090
95% CI		(-0.28, 0.08)
p-value		0.2578
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.32
95% CI		(-0.90, 0.26)
Week 12		
N	21	26
LS mean change from baseline	0.07	-0.07
SE	0.058	0.052
95% CI	(-0.04, 0.19)	(-0.17, 0.04)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.077
95% CI		(-0.29, 0.01)
p-value		0.0738
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.51
95% CI		(-1.10, 0.07)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 18		
N	21	24
LS mean change from baseline	0.03	0.03
SE	0.060	0.056
95% CI	(-0.09, 0.15)	(-0.08, 0.14)
LS mean difference (Omaveloxolone-Placebo)		0.00
SE		0.081
95% CI		(-0.16, 0.16)
p-value		0.9852
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.01
95% CI		(-0.59, 0.58)
Week 24		
N	21	23
LS mean change from baseline	0.21	0.01
SE	0.063	0.060
95% CI	(0.09, 0.34)	(-0.11, 0.12)
LS mean difference (Omaveloxolone-Placebo)		-0.21
SE		0.085
95% CI		(-0.38, -0.04)
p-value		0.0171
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.71
95% CI		(-1.32, -0.10)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 36		
N	21	23
LS mean change from baseline	0.13	0.00
SE	0.071	0.067
95% CI	(-0.01, 0.27)	(-0.14, 0.13)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.096
95% CI		(-0.33, 0.06)
p-value		0.1600
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.41
95% CI		(-1.01, 0.18)
Week 48		
N	21	22
LS mean change from baseline	0.05	-0.09
SE	0.062	0.059
95% CI	(-0.08, 0.17)	(-0.21, 0.02)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.085
95% CI		(-0.31, 0.03)
p-value		0.1021
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.49
95% CI		(-1.09, 0.12)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Baseline		
N	22	15
Mean score	0.42	0.60
SD	0.446	0.596
Week 4		
N	22	14
LS mean change from baseline	-0.05	-0.18
SE	0.067	0.083
95% CI	(-0.18, 0.09)	(-0.34, -0.01)
LS mean difference (Omaveloxolone-Placebo)		-0.13
SE		0.105
95% CI		(-0.34, 0.08)
p-value		0.2216
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.41
95% CI		(-1.08, 0.27)
Week 12		
N	22	14
LS mean change from baseline	-0.01	0.01
SE	0.057	0.070
95% CI	(-0.13, 0.10)	(-0.14, 0.15)
LS mean difference (Omaveloxolone-Placebo)		0.02
SE		0.089
95% CI		(-0.16, 0.20)
p-value		0.8232
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.07
95% CI		(-0.60, 0.74)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 18		
N	22	14
LS mean change from baseline	-0.03	-0.07
SE	0.059	0.073
95% CI	(-0.15, 0.09)	(-0.21, 0.08)
LS mean difference (Omaveloxolone-Placebo)		-0.04
SE		0.092
95% CI		(-0.23, 0.14)
p-value		0.6536
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.15
95% CI		(-0.82, 0.52)
Week 24		
N	22	14
LS mean change from baseline	0.01	-0.07
SE	0.062	0.076
95% CI	(-0.12, 0.13)	(-0.22, 0.09)
LS mean difference (Omaveloxolone-Placebo)		-0.07
SE		0.097
95% CI		(-0.27, 0.12)
p-value		0.4496
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.25
95% CI		(-0.92, 0.42)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 36		
N	22	13
LS mean change from baseline	-0.10	-0.11
SE	0.070	0.089
95% CI	(-0.24, 0.04)	(-0.29, 0.07)
LS mean difference (Omaveloxolone-Placebo)		-0.01
SE		0.111
95% CI		(-0.23, 0.22)
p-value		0.9559
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.02
95% CI		(-0.70, 0.67)
Week 48		
N	21	13
LS mean change from baseline	-0.01	-0.02
SE	0.062	0.077
95% CI	(-0.13, 0.11)	(-0.17, 0.13)
LS mean difference (Omaveloxolone-Placebo)		-0.01
SE		0.098
95% CI		(-0.20, 0.19)
p-value		0.9219
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.03
95% CI		(-0.73, 0.66)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline at Week 48: Treatment by Subgroup Interaction from MMRM Analysis - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Gender (female, male)	0.2337
GAA1 repeat length >=675 (yes, no)	0.6658
Geographic location (US, other)	0.2996

NOTE 1: p-value is based on the MMRM model for the ITT population with three way interaction added (subgroup*treatment*time).
Source: biib141/valueaccess/amnog/t-mfars93-ul-mmrm-int.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Baseline		
N	17	31
Mean score	8.71	10.85
SD	2.941	3.793
Week 4		
N	17	31
LS mean change from baseline	-0.26	-0.62
SE	0.549	0.396
95% CI	(-1.35, 0.83)	(-1.41, 0.17)
LS mean difference (Omaveloxolone-Placebo)		-0.35
SE		0.686
95% CI		(-1.72, 1.01)
p-value		0.6071
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.15
95% CI		(-0.74, 0.44)
Week 12		
N	17	31
LS mean change from baseline	-0.21	-0.43
SE	0.547	0.395
95% CI	(-1.29, 0.88)	(-1.22, 0.35)
LS mean difference (Omaveloxolone-Placebo)		-0.23
SE		0.684
95% CI		(-1.58, 1.13)
p-value		0.7425
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.10
95% CI		(-0.69, 0.50)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 18		
N	17	31
LS mean change from baseline	0.09	-0.45
SE	0.605	0.438
95% CI	(-1.11, 1.29)	(-1.32, 0.42)
LS mean difference (Omaveloxolone-Placebo)		-0.54
SE		0.756
95% CI		(-2.05, 0.96)
p-value		0.4734
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.21
95% CI		(-0.80, 0.38)
Week 24		
N	17	29
LS mean change from baseline	0.07	0.02
SE	0.526	0.388
95% CI	(-0.98, 1.11)	(-0.75, 0.80)
LS mean difference (Omaveloxolone-Placebo)		-0.04
SE		0.661
95% CI		(-1.36, 1.28)
p-value		0.9507
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.02
95% CI		(-0.62, 0.58)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 36		
N	17	28
LS mean change from baseline	0.19	-0.36
SE	0.613	0.458
95% CI	(-1.03, 1.41)	(-1.27, 0.55)
LS mean difference (Omaveloxolone-Placebo)		-0.56
SE		0.773
95% CI		(-2.09, 0.98)
p-value		0.4728
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.21
95% CI		(-0.82, 0.39)
Week 48		
N	16	26
LS mean change from baseline	-0.05	-0.29
SE	0.671	0.506
95% CI	(-1.38, 1.29)	(-1.30, 0.71)
LS mean difference (Omaveloxolone-Placebo)		-0.25
SE		0.846
95% CI		(-1.93, 1.44)
p-value		0.7716
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.09
95% CI		(-0.71, 0.53)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Baseline		
N	35	20
Mean score	10.48	10.61
SD	3.688	3.674
Week 4		
N	35	19
LS mean change from baseline	-0.78	-1.17
SE	0.372	0.506
95% CI	(-1.52, -0.04)	(-2.17, -0.16)
LS mean difference (Omaveloxolone-Placebo)		-0.39
SE		0.630
95% CI		(-1.64, 0.87)
p-value		0.5418
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.17
95% CI		(-0.73, 0.39)
Week 12		
N	35	19
LS mean change from baseline	-0.75	-1.17
SE	0.370	0.504
95% CI	(-1.48, -0.01)	(-2.17, -0.17)
LS mean difference (Omaveloxolone-Placebo)		-0.43
SE		0.627
95% CI		(-1.67, 0.82)
p-value		0.4975
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.19
95% CI		(-0.75, 0.37)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 18		
N	34	16
LS mean change from baseline	-0.96	-1.33
SE	0.414	0.588
95% CI	(-1.78, -0.14)	(-2.50, -0.16)
LS mean difference (Omaveloxolone-Placebo)		-0.37
SE		0.721
95% CI		(-1.80, 1.06)
p-value		0.6109
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.15
95% CI		(-0.75, 0.44)
Week 24		
N	34	16
LS mean change from baseline	-0.79	-1.21
SE	0.360	0.524
95% CI	(-1.51, -0.08)	(-2.25, -0.17)
LS mean difference (Omaveloxolone-Placebo)		-0.42
SE		0.638
95% CI		(-1.69, 0.85)
p-value		0.5140
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.19
95% CI		(-0.79, 0.40)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 36		
N	34	16
LS mean change from baseline	-0.51	-0.94
SE	0.420	0.602
95% CI	(-1.35, 0.32)	(-2.14, 0.25)
LS mean difference (Omaveloxolone-Placebo)		-0.43
SE		0.736
95% CI		(-1.89, 1.03)
p-value		0.5596
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.17
95% CI		(-0.77, 0.42)
Week 48		
N	34	16
LS mean change from baseline	0.18	-1.47
SE	0.452	0.650
95% CI	(-0.72, 1.08)	(-2.76, -0.18)
LS mean difference (Omaveloxolone-Placebo)		-1.65
SE		0.794
95% CI		(-3.23, -0.07)
p-value		0.0407
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.61
95% CI		(-1.22, -0.01)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Baseline		
N	35	36
Mean score	9.82	11.09
SD	3.988	3.804
Week 4		
N	35	35
LS mean change from baseline	-1.07	-0.97
SE	0.377	0.381
95% CI	(-1.82, -0.33)	(-1.73, -0.22)
LS mean difference (Omaveloxolone-Placebo)		0.10
SE		0.538
95% CI		(-0.97, 1.17)
p-value		0.8538
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.04
95% CI		(-0.43, 0.51)
Week 12		
N	35	35
LS mean change from baseline	-0.93	-0.99
SE	0.366	0.370
95% CI	(-1.66, -0.21)	(-1.72, -0.25)
LS mean difference (Omaveloxolone-Placebo)		-0.05
SE		0.523
95% CI		(-1.09, 0.98)
p-value		0.9176
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.02
95% CI		(-0.49, 0.44)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 18		
N	35	32
LS mean change from baseline	-0.88	-0.97
SE	0.417	0.431
95% CI	(-1.71, -0.06)	(-1.83, -0.12)
LS mean difference (Omaveloxolone-Placebo)		-0.09
SE		0.602
95% CI		(-1.28, 1.11)
p-value		0.8860
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.03
95% CI		(-0.51, 0.45)
Week 24		
N	35	31
LS mean change from baseline	-0.60	-0.24
SE	0.352	0.373
95% CI	(-1.30, 0.10)	(-0.98, 0.50)
LS mean difference (Omaveloxolone-Placebo)		0.36
SE		0.514
95% CI		(-0.66, 1.38)
p-value		0.4825
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.17
95% CI		(-0.31, 0.65)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 36		
N	35	31
LS mean change from baseline	-0.39	-0.71
SE	0.422	0.442
95% CI	(-1.23, 0.45)	(-1.59, 0.16)
LS mean difference (Omaveloxolone-Placebo)		-0.33
SE		0.613
95% CI		(-1.54, 0.89)
p-value		0.5966
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-0.61, 0.36)
Week 48		
N	35	29
LS mean change from baseline	-0.15	-1.30
SE	0.439	0.473
95% CI	(-1.02, 0.72)	(-2.24, -0.36)
LS mean difference (Omaveloxolone-Placebo)		-1.15
SE		0.647
95% CI		(-2.44, 0.13)
p-value		0.0786
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.44
95% CI		(-0.93, 0.06)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Baseline		
N	17	15
Mean score	10.06	9.95
SD	2.442	3.470
Week 4		
N	17	15
LS mean change from baseline	0.26	-0.38
SE	0.539	0.577
95% CI	(-0.81, 1.33)	(-1.52, 0.77)
LS mean difference (Omaveloxolone-Placebo)		-0.64
SE		0.789
95% CI		(-2.20, 0.93)
p-value		0.4222
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.27
95% CI		(-0.97, 0.42)
Week 12		
N	17	15
LS mean change from baseline	0.10	0.02
SE	0.523	0.560
95% CI	(-0.94, 1.14)	(-1.09, 1.13)
LS mean difference (Omaveloxolone-Placebo)		-0.08
SE		0.766
95% CI		(-1.60, 1.44)
p-value		0.9150
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.04
95% CI		(-0.73, 0.66)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 18		
N	16	15
LS mean change from baseline	-0.15	-0.22
SE	0.607	0.638
95% CI	(-1.36, 1.05)	(-1.49, 1.04)
LS mean difference (Omaveloxolone-Placebo)		-0.07
SE		0.880
95% CI		(-1.82, 1.68)
p-value		0.9377
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.03
95% CI		(-0.73, 0.68)
Week 24		
N	16	14
LS mean change from baseline	-0.44	-0.69
SE	0.516	0.555
95% CI	(-1.46, 0.59)	(-1.79, 0.41)
LS mean difference (Omaveloxolone-Placebo)		-0.25
SE		0.757
95% CI		(-1.76, 1.25)
p-value		0.7375
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.12
95% CI		(-0.84, 0.60)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 36		
N	16	13
LS mean change from baseline	-0.17	-0.15
SE	0.616	0.676
95% CI	(-1.40, 1.05)	(-1.49, 1.20)
LS mean difference (Omaveloxolone-Placebo)		0.03
SE		0.914
95% CI		(-1.79, 1.84)
p-value		0.9774
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.01
95% CI		(-0.72, 0.74)
Week 48		
N	15	13
LS mean change from baseline	0.59	0.66
SE	0.658	0.708
95% CI	(-0.72, 1.90)	(-0.75, 2.06)
LS mean difference (Omaveloxolone-Placebo)		0.07
SE		0.967
95% CI		(-1.85, 1.99)
p-value		0.9457
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.02
95% CI		(-0.72, 0.77)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Baseline		
N	21	26
Mean score	10.77	11.76
SD	3.950	3.821
Week 4		
N	21	26
LS mean change from baseline	-1.63	-1.02
SE	0.482	0.444
95% CI	(-2.59, -0.67)	(-1.91, -0.13)
LS mean difference (Omaveloxolone-Placebo)		0.61
SE		0.646
95% CI		(-0.68, 1.90)
p-value		0.3487
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.27
95% CI		(-0.31, 0.84)
Week 12		
N	21	26
LS mean change from baseline	-0.93	-1.22
SE	0.510	0.470
95% CI	(-1.94, 0.09)	(-2.16, -0.28)
LS mean difference (Omaveloxolone-Placebo)		-0.29
SE		0.685
95% CI		(-1.66, 1.07)
p-value		0.6708
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.12
95% CI		(-0.70, 0.45)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 18		
N	21	24
LS mean change from baseline	-0.63	-1.06
SE	0.554	0.520
95% CI	(-1.73, 0.47)	(-2.09, -0.02)
LS mean difference (Omaveloxolone-Placebo)		-0.43
SE		0.751
95% CI		(-1.92, 1.07)
p-value		0.5712
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.16
95% CI		(-0.75, 0.42)
Week 24		
N	21	23
LS mean change from baseline	-1.17	-0.34
SE	0.467	0.447
95% CI	(-2.10, -0.23)	(-1.23, 0.55)
LS mean difference (Omaveloxolone-Placebo)		0.83
SE		0.637
95% CI		(-0.45, 2.10)
p-value		0.1995
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.38
95% CI		(-0.22, 0.97)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 36		
N	21	23
LS mean change from baseline	-0.61	-1.01
SE	0.549	0.522
95% CI	(-1.71, 0.48)	(-2.05, 0.03)
LS mean difference (Omaveloxolone-Placebo)		-0.40
SE		0.749
95% CI		(-1.89, 1.10)
p-value		0.5969
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.15
95% CI		(-0.75, 0.44)
Week 48		
N	21	22
LS mean change from baseline	-0.02	-1.27
SE	0.614	0.594
95% CI	(-1.24, 1.21)	(-2.45, -0.08)
LS mean difference (Omaveloxolone-Placebo)		-1.25
SE		0.846
95% CI		(-2.93, 0.44)
p-value		0.1449
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.43
95% CI		(-1.04, 0.17)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Baseline		
N	22	15
Mean score	9.35	9.93
SD	3.518	3.600
Week 4		
N	22	14
LS mean change from baseline	-0.26	-0.59
SE	0.478	0.592
95% CI	(-1.21, 0.70)	(-1.77, 0.59)
LS mean difference (Omaveloxolone-Placebo)		-0.33
SE		0.737
95% CI		(-1.80, 1.14)
p-value		0.6554
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.15
95% CI		(-0.82, 0.52)
Week 12		
N	22	14
LS mean change from baseline	-0.34	0.33
SE	0.506	0.626
95% CI	(-1.35, 0.66)	(-0.91, 1.58)
LS mean difference (Omaveloxolone-Placebo)		0.68
SE		0.782
95% CI		(-0.88, 2.24)
p-value		0.3895
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.28
95% CI		(-0.39, 0.96)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 18		
N	22	14
LS mean change from baseline	-0.45	-0.55
SE	0.549	0.680
95% CI	(-1.55, 0.64)	(-1.90, 0.81)
LS mean difference (Omaveloxolone-Placebo)		-0.09
SE		0.852
95% CI		(-1.79, 1.61)
p-value		0.9134
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.04
95% CI		(-0.71, 0.63)
Week 24		
N	22	14
LS mean change from baseline	0.06	-0.24
SE	0.463	0.573
95% CI	(-0.87, 0.98)	(-1.39, 0.90)
LS mean difference (Omaveloxolone-Placebo)		-0.30
SE		0.713
95% CI		(-1.72, 1.12)
p-value		0.6748
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.14
95% CI		(-0.81, 0.53)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 36		
N	22	13
LS mean change from baseline	-0.26	0.13
SE	0.544	0.690
95% CI	(-1.34, 0.83)	(-1.24, 1.51)
LS mean difference (Omaveloxolone-Placebo)		0.39
SE		0.856
95% CI		(-1.32, 2.10)
p-value		0.6485
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.15
95% CI		(-0.53, 0.84)
Week 48		
N	21	13
LS mean change from baseline	0.03	-0.66
SE	0.617	0.773
95% CI	(-1.20, 1.26)	(-2.20, 0.88)
LS mean difference (Omaveloxolone-Placebo)		-0.69
SE		0.967
95% CI		(-2.62, 1.24)
p-value		0.4781
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.24
95% CI		(-0.94, 0.45)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline at Week 48: Treatment by Subgroup Interaction from MMRM Analysis - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Gender (female, male)	0.6647
GAA1 repeat length >=675 (yes, no)	0.7323
Geographic location (US, other)	0.8836

NOTE 1: p-value is based on the MMRM model for the ITT population with three way interaction added (subgroup*treatment*time).
Source: biib141/valueaccess/amnog/t-mfars93-ll-mmrm-int.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Baseline		
N	17	31
Mean score	5.56	6.94
SD	2.392	2.765
Week 4		
N	17	31
LS mean change from baseline	-0.57	-0.25
SE	0.423	0.312
95% CI	(-1.41, 0.27)	(-0.87, 0.37)
LS mean difference (Omaveloxolone-Placebo)		0.32
SE		0.533
95% CI		(-0.74, 1.38)
p-value		0.5466
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.18
95% CI		(-0.42, 0.77)
Week 12		
N	17	31
LS mean change from baseline	-0.84	-0.34
SE	0.430	0.317
95% CI	(-1.70, 0.01)	(-0.97, 0.29)
LS mean difference (Omaveloxolone-Placebo)		0.51
SE		0.541
95% CI		(-0.57, 1.58)
p-value		0.3513
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.27
95% CI		(-0.32, 0.87)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 18		
N	17	31
LS mean change from baseline	-0.27	-0.09
SE	0.482	0.356
95% CI	(-1.23, 0.69)	(-0.80, 0.61)
LS mean difference (Omaveloxolone-Placebo)		0.18
SE		0.607
95% CI		(-1.03, 1.39)
p-value		0.7704
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.09
95% CI		(-0.51, 0.68)
Week 24		
N	17	29
LS mean change from baseline	-0.68	-0.57
SE	0.450	0.340
95% CI	(-1.57, 0.22)	(-1.24, 0.11)
LS mean difference (Omaveloxolone-Placebo)		0.11
SE		0.571
95% CI		(-1.02, 1.25)
p-value		0.8464
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.06
95% CI		(-0.54, 0.66)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 36		
N	17	28
LS mean change from baseline	-1.00	-0.21
SE	0.460	0.353
95% CI	(-1.92, -0.09)	(-0.91, 0.49)
LS mean difference (Omaveloxolone-Placebo)		0.79
SE		0.588
95% CI		(-0.38, 1.96)
p-value		0.1829
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.40
95% CI		(-0.21, 1.01)
Week 48		
N	16	26
LS mean change from baseline	-0.62	-0.16
SE	0.510	0.394
95% CI	(-1.63, 0.40)	(-0.94, 0.62)
LS mean difference (Omaveloxolone-Placebo)		0.46
SE		0.653
95% CI		(-0.84, 1.75)
p-value		0.4863
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.21
95% CI		(-0.41, 0.84)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Baseline		
N	35	20
Mean score	6.59	5.29
SD	2.188	1.918
Week 4		
N	35	19
LS mean change from baseline	-0.77	-0.26
SE	0.291	0.399
95% CI	(-1.35, -0.19)	(-1.06, 0.53)
LS mean difference (Omaveloxolone-Placebo)		0.51
SE		0.499
95% CI		(-0.48, 1.50)
p-value		0.3105
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.28
95% CI		(-0.28, 0.84)
Week 12		
N	35	19
LS mean change from baseline	-1.02	-0.16
SE	0.296	0.405
95% CI	(-1.61, -0.43)	(-0.96, 0.65)
LS mean difference (Omaveloxolone-Placebo)		0.86
SE		0.507
95% CI		(-0.14, 1.87)
p-value		0.0917
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.47
95% CI		(-0.09, 1.04)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 18		
N	34	16
LS mean change from baseline	-0.72	-0.44
SE	0.335	0.485
95% CI	(-1.39, -0.05)	(-1.40, 0.52)
LS mean difference (Omaveloxolone-Placebo)		0.28
SE		0.594
95% CI		(-0.90, 1.46)
p-value		0.6367
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.14
95% CI		(-0.46, 0.73)
Week 24		
N	34	16
LS mean change from baseline	-0.36	-0.52
SE	0.313	0.459
95% CI	(-0.98, 0.26)	(-1.43, 0.39)
LS mean difference (Omaveloxolone-Placebo)		-0.16
SE		0.560
95% CI		(-1.27, 0.95)
p-value		0.7782
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.08
95% CI		(-0.68, 0.51)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 36		
N	34	16
LS mean change from baseline	-0.37	-0.24
SE	0.320	0.468
95% CI	(-1.00, 0.27)	(-1.17, 0.69)
LS mean difference (Omaveloxolone-Placebo)		0.13
SE		0.572
95% CI		(-1.01, 1.26)
p-value		0.8222
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.07
95% CI		(-0.53, 0.66)
Week 48		
N	34	16
LS mean change from baseline	-0.15	-0.09
SE	0.349	0.510
95% CI	(-0.84, 0.55)	(-1.10, 0.92)
LS mean difference (Omaveloxolone-Placebo)		0.06
SE		0.622
95% CI		(-1.18, 1.29)
p-value		0.9283
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.03
95% CI		(-0.57, 0.62)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Baseline		
N	35	36
Mean score	6.14	6.31
SD	2.105	2.758
Week 4		
N	35	35
LS mean change from baseline	-0.72	-0.19
SE	0.273	0.274
95% CI	(-1.27, -0.18)	(-0.73, 0.36)
LS mean difference (Omaveloxolone-Placebo)		0.54
SE		0.387
95% CI		(-0.23, 1.31)
p-value		0.1670
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.33
95% CI		(-0.15, 0.80)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-llmmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 12		
N	35	35
LS mean change from baseline	-0.67	-0.05
SE	0.306	0.306
95% CI	(-1.27, -0.06)	(-0.65, 0.56)
LS mean difference (Omaveloxolone-Placebo)		0.62
SE		0.433
95% CI		(-0.24, 1.48)
p-value		0.1540
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.34
95% CI		(-0.14, 0.81)
Week 18		
N	35	32
LS mean change from baseline	-0.49	0.02
SE	0.328	0.340
95% CI	(-1.15, 0.16)	(-0.66, 0.69)
LS mean difference (Omaveloxolone-Placebo)		0.51
SE		0.473
95% CI		(-0.43, 1.45)
p-value		0.2832
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.26
95% CI		(-0.22, 0.74)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-llmmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 24		
N	35	31
LS mean change from baseline	-0.20	-0.19
SE	0.309	0.325
95% CI	(-0.81, 0.42)	(-0.84, 0.45)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.449
95% CI		(-0.89, 0.90)
p-value		0.9901
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.00
95% CI		(-0.48, 0.49)
Week 36		
N	35	31
LS mean change from baseline	-0.10	0.02
SE	0.309	0.326
95% CI	(-0.72, 0.51)	(-0.63, 0.66)
LS mean difference (Omaveloxolone-Placebo)		0.12
SE		0.449
95% CI		(-0.77, 1.01)
p-value		0.7890
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.06
95% CI		(-0.42, 0.55)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-llmmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 48		
N	35	29
LS mean change from baseline	-0.12	0.02
SE	0.341	0.366
95% CI	(-0.80, 0.56)	(-0.70, 0.75)
LS mean difference (Omaveloxolone-Placebo)		0.14
SE		0.501
95% CI		(-0.85, 1.14)
p-value		0.7741
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.07
95% CI		(-0.42, 0.56)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-llmmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Baseline		
N	17	15
Mean score	6.47	6.27
SD	2.675	2.176
Week 4		
N	17	15
LS mean change from baseline	-0.73	-0.41
SE	0.392	0.419
95% CI	(-1.51, 0.05)	(-1.24, 0.42)
LS mean difference (Omaveloxolone-Placebo)		0.32
SE		0.574
95% CI		(-0.82, 1.46)
p-value		0.5743
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.19
95% CI		(-0.50, 0.89)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-llmmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 12		
N	17	15
LS mean change from baseline	-1.63	-0.78
SE	0.438	0.469
95% CI	(-2.50, -0.76)	(-1.71, 0.15)
LS mean difference (Omaveloxolone-Placebo)		0.85
SE		0.641
95% CI		(-0.42, 2.12)
p-value		0.1881
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.45
95% CI		(-0.25, 1.16)
Week 18		
N	16	15
LS mean change from baseline	-0.78	-0.71
SE	0.481	0.503
95% CI	(-1.73, 0.18)	(-1.71, 0.29)
LS mean difference (Omaveloxolone-Placebo)		0.07
SE		0.696
95% CI		(-1.31, 1.45)
p-value		0.9178
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.04
95% CI		(-0.67, 0.74)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-llmmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 24		
N	16	14
LS mean change from baseline	-1.08	-1.30
SE	0.454	0.486
95% CI	(-1.98, -0.18)	(-2.26, -0.33)
LS mean difference (Omaveloxolone-Placebo)		-0.22
SE		0.665
95% CI		(-1.54, 1.10)
p-value		0.7417
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.12
95% CI		(-0.83, 0.60)
Week 36		
N	16	13
LS mean change from baseline	-1.65	-0.72
SE	0.455	0.500
95% CI	(-2.55, -0.74)	(-1.71, 0.27)
LS mean difference (Omaveloxolone-Placebo)		0.93
SE		0.675
95% CI		(-0.41, 2.27)
p-value		0.1731
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.49
95% CI		(-0.25, 1.23)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-llmmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 48		
N	15	13
LS mean change from baseline	-0.71	-0.43
SE	0.512	0.550
95% CI	(-1.73, 0.31)	(-1.52, 0.66)
LS mean difference (Omaveloxolone-Placebo)		0.28
SE		0.751
95% CI		(-1.21, 1.77)
p-value		0.7132
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.13
95% CI		(-0.61, 0.88)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-llmmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Baseline		
N	21	26
Mean score	6.82	7.23
SD	2.111	2.797
Week 4		
N	21	26
LS mean change from baseline	-0.72	0.17
SE	0.368	0.337
95% CI	(-1.46, 0.01)	(-0.50, 0.85)
LS mean difference (Omaveloxolone-Placebo)		0.90
SE		0.490
95% CI		(-0.08, 1.87)
p-value		0.0709
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.52
95% CI		(-0.06, 1.10)
Week 12		
N	21	26
LS mean change from baseline	-0.52	0.06
SE	0.403	0.370
95% CI	(-1.33, 0.28)	(-0.68, 0.80)
LS mean difference (Omaveloxolone-Placebo)		0.58
SE		0.537
95% CI		(-0.49, 1.65)
p-value		0.2814
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.31
95% CI		(-0.27, 0.89)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 18		
N	21	24
LS mean change from baseline	-0.34	0.09
SE	0.440	0.414
95% CI	(-1.22, 0.54)	(-0.74, 0.91)
LS mean difference (Omaveloxolone-Placebo)		0.43
SE		0.593
95% CI		(-0.75, 1.62)
p-value		0.4696
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.21
95% CI		(-0.38, 0.80)
Week 24		
N	21	23
LS mean change from baseline	-0.27	-0.21
SE	0.422	0.405
95% CI	(-1.11, 0.57)	(-1.02, 0.59)
LS mean difference (Omaveloxolone-Placebo)		0.06
SE		0.574
95% CI		(-1.09, 1.20)
p-value		0.9190
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.03
95% CI		(-0.56, 0.62)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 36		
N	21	23
LS mean change from baseline	-0.34	0.14
SE	0.435	0.416
95% CI	(-1.21, 0.53)	(-0.69, 0.97)
LS mean difference (Omaveloxolone-Placebo)		0.49
SE		0.592
95% CI		(-0.69, 1.67)
p-value		0.4142
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.24
95% CI		(-0.35, 0.83)
Week 48		
N	21	22
LS mean change from baseline	0.19	-0.11
SE	0.461	0.449
95% CI	(-0.73, 1.11)	(-1.01, 0.78)
LS mean difference (Omaveloxolone-Placebo)		-0.31
SE		0.632
95% CI		(-1.57, 0.96)
p-value		0.6310
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.14
95% CI		(-0.74, 0.46)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Baseline		
N	22	15
Mean score	5.15	5.25
SD	2.183	1.902
Week 4		
N	22	14
LS mean change from baseline	-0.85	-0.71
SE	0.368	0.452
95% CI	(-1.58, -0.12)	(-1.61, 0.19)
LS mean difference (Omaveloxolone-Placebo)		0.14
SE		0.559
95% CI		(-0.98, 1.25)
p-value		0.8040
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.08
95% CI		(-0.59, 0.75)
Week 12		
N	22	14
LS mean change from baseline	-1.03	-0.19
SE	0.403	0.495
95% CI	(-1.84, -0.23)	(-1.17, 0.80)
LS mean difference (Omaveloxolone-Placebo)		0.84
SE		0.615
95% CI		(-0.38, 2.07)
p-value		0.1741
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.45
95% CI		(-0.23, 1.13)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 18		
N	22	14
LS mean change from baseline	-0.39	-0.68
SE	0.440	0.540
95% CI	(-1.27, 0.48)	(-1.75, 0.40)
LS mean difference (Omaveloxolone-Placebo)		-0.28
SE		0.673
95% CI		(-1.63, 1.06)
p-value		0.6745
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.14
95% CI		(-0.81, 0.53)
Week 24		
N	22	14
LS mean change from baseline	-0.37	-0.71
SE	0.422	0.518
95% CI	(-1.21, 0.47)	(-1.74, 0.32)
LS mean difference (Omaveloxolone-Placebo)		-0.34
SE		0.645
95% CI		(-1.63, 0.95)
p-value		0.6009
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.17
95% CI		(-0.84, 0.50)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 36		
N	22	13
LS mean change from baseline	-0.28	-0.34
SE	0.435	0.546
95% CI	(-1.15, 0.59)	(-1.43, 0.75)
LS mean difference (Omaveloxolone-Placebo)		-0.06
SE		0.676
95% CI		(-1.41, 1.29)
p-value		0.9263
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.03
95% CI		(-0.72, 0.65)
Week 48		
N	21	13
LS mean change from baseline	-0.34	-0.32
SE	0.467	0.580
95% CI	(-1.27, 0.59)	(-1.47, 0.84)
LS mean difference (Omaveloxolone-Placebo)		0.02
SE		0.722
95% CI		(-1.42, 1.47)
p-value		0.9730
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.01
95% CI		(-0.68, 0.70)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich\'s Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline at Week 48: Treatment by Subgroup Interaction from MMRM Analysis - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Gender (female, male)	0.8589
GAA1 repeat length >=675 (yes, no)	0.2170
Geographic location (US, other)	0.4868

NOTE 1: p-value is based on the MMRM model for the ITT population with three way interaction added (subgroup*treatment*time).
Source: biib141/valueaccess/amnog/t-mfars93-us-mmrm-int.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Baseline		
N	17	31
Mean score	17.65	22.89
SD	5.736	6.756
Week 4		
N	17	31
LS mean change from baseline	0.50	-0.53
SE	0.507	0.362
95% CI	(-0.51, 1.51)	(-1.25, 0.19)
LS mean difference (Omaveloxolone-Placebo)		-1.04
SE		0.634
95% CI		(-2.30, 0.22)
p-value		0.1056
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.48
95% CI		(-1.08, 0.12)
Week 12		
N	17	31
LS mean change from baseline	-0.12	0.13
SE	0.543	0.388
95% CI	(-1.20, 0.96)	(-0.64, 0.90)
LS mean difference (Omaveloxolone-Placebo)		0.25
SE		0.678
95% CI		(-1.09, 1.60)
p-value		0.7105
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.11
95% CI		(-0.48, 0.70)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 18		
N	17	31
LS mean change from baseline	0.41	-0.58
SE	0.535	0.381
95% CI	(-0.65, 1.47)	(-1.34, 0.18)
LS mean difference (Omaveloxolone-Placebo)		-0.99
SE		0.667
95% CI		(-2.31, 0.34)
p-value		0.1428
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.43
95% CI		(-1.03, 0.17)
Week 24		
N	17	29
LS mean change from baseline	0.46	-0.47
SE	0.612	0.447
95% CI	(-0.76, 1.68)	(-1.36, 0.42)
LS mean difference (Omaveloxolone-Placebo)		-0.93
SE		0.769
95% CI		(-2.46, 0.60)
p-value		0.2284
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.36
95% CI		(-0.96, 0.24)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 36		
N	17	28
LS mean change from baseline	0.41	-0.06
SE	0.682	0.504
95% CI	(-0.94, 1.77)	(-1.06, 0.94)
LS mean difference (Omaveloxolone-Placebo)		-0.47
SE		0.860
95% CI		(-2.18, 1.24)
p-value		0.5838
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.16
95% CI		(-0.77, 0.44)
Week 48		
N	16	26
LS mean change from baseline	1.27	-0.01
SE	0.670	0.497
95% CI	(-0.06, 2.60)	(-0.99, 0.98)
LS mean difference (Omaveloxolone-Placebo)		-1.28
SE		0.846
95% CI		(-2.96, 0.40)
p-value		0.1345
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.46
95% CI		(-1.10, 0.17)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Baseline		
N	35	20
Mean score	22.66	22.90
SD	7.227	6.326
Week 4		
N	35	19
LS mean change from baseline	0.15	-0.50
SE	0.340	0.463
95% CI	(-0.52, 0.83)	(-1.42, 0.42)
LS mean difference (Omaveloxolone-Placebo)		-0.65
SE		0.577
95% CI		(-1.79, 0.50)
p-value		0.2644
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.31
95% CI		(-0.87, 0.25)
Week 12		
N	35	19
LS mean change from baseline	-0.04	-0.17
SE	0.364	0.496
95% CI	(-0.77, 0.68)	(-1.16, 0.81)
LS mean difference (Omaveloxolone-Placebo)		-0.13
SE		0.617
95% CI		(-1.35, 1.10)
p-value		0.8341
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.06
95% CI		(-0.62, 0.50)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 18		
N	34	16
LS mean change from baseline	-0.04	-0.55
SE	0.362	0.524
95% CI	(-0.76, 0.68)	(-1.59, 0.49)
LS mean difference (Omaveloxolone-Placebo)		-0.51
SE		0.639
95% CI		(-1.78, 0.76)
p-value		0.4309
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.23
95% CI		(-0.83, 0.36)
Week 24		
N	34	16
LS mean change from baseline	-0.06	-0.27
SE	0.415	0.599
95% CI	(-0.89, 0.76)	(-1.46, 0.92)
LS mean difference (Omaveloxolone-Placebo)		-0.20
SE		0.730
95% CI		(-1.65, 1.25)
p-value		0.7803
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.08
95% CI		(-0.68, 0.51)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 36		
N	34	16
LS mean change from baseline	0.70	-0.35
SE	0.462	0.667
95% CI	(-0.22, 1.62)	(-1.68, 0.97)
LS mean difference (Omaveloxolone-Placebo)		-1.05
SE		0.813
95% CI		(-2.67, 0.56)
p-value		0.1986
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.38
95% CI		(-0.98, 0.22)
Week 48		
N	34	16
LS mean change from baseline	0.79	-0.28
SE	0.450	0.654
95% CI	(-0.10, 1.69)	(-1.58, 1.02)
LS mean difference (Omaveloxolone-Placebo)		-1.07
SE		0.795
95% CI		(-2.65, 0.51)
p-value		0.1816
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.40
95% CI		(-1.00, 0.20)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Baseline		
N	35	36
Mean score	20.95	24.57
SD	8.131	6.394
Week 4		
N	35	35
LS mean change from baseline	0.45	-0.18
SE	0.332	0.343
95% CI	(-0.21, 1.11)	(-0.86, 0.50)
LS mean difference (Omaveloxolone-Placebo)		-0.63
SE		0.482
95% CI		(-1.58, 0.33)
p-value		0.1966
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.30
95% CI		(-0.77, 0.17)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 12		
N	35	35
LS mean change from baseline	0.36	0.41
SE	0.361	0.372
95% CI	(-0.35, 1.08)	(-0.33, 1.15)
LS mean difference (Omaveloxolone-Placebo)		0.04
SE		0.524
95% CI		(-1.00, 1.08)
p-value		0.9336
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.02
95% CI		(-0.45, 0.49)
Week 18		
N	35	32
LS mean change from baseline	0.13	-0.49
SE	0.352	0.376
95% CI	(-0.56, 0.83)	(-1.24, 0.25)
LS mean difference (Omaveloxolone-Placebo)		-0.63
SE		0.520
95% CI		(-1.66, 0.41)
p-value		0.2321
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.29
95% CI		(-0.77, 0.19)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 24		
N	35	31
LS mean change from baseline	0.38	-0.24
SE	0.404	0.435
95% CI	(-0.42, 1.18)	(-1.10, 0.63)
LS mean difference (Omaveloxolone-Placebo)		-0.62
SE		0.600
95% CI		(-1.81, 0.57)
p-value		0.3057
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.25
95% CI		(-0.73, 0.24)
Week 36		
N	35	31
LS mean change from baseline	0.97	-0.12
SE	0.448	0.483
95% CI	(0.08, 1.86)	(-1.08, 0.84)
LS mean difference (Omaveloxolone-Placebo)		-1.10
SE		0.666
95% CI		(-2.42, 0.23)
p-value		0.1028
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.40
95% CI		(-0.88, 0.09)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 48		
N	35	29
LS mean change from baseline	1.28	0.01
SE	0.440	0.482
95% CI	(0.40, 2.15)	(-0.94, 0.97)
LS mean difference (Omaveloxolone-Placebo)		-1.26
SE		0.659
95% CI		(-2.57, 0.05)
p-value		0.0584
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.47
95% CI		(-0.97, 0.03)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Baseline		
N	17	15
Mean score	21.17	18.87
SD	4.615	5.031
Week 4		
N	17	15
LS mean change from baseline	-0.22	-1.20
SE	0.475	0.516
95% CI	(-1.16, 0.73)	(-2.22, -0.17)
LS mean difference (Omaveloxolone-Placebo)		-0.98
SE		0.698
95% CI		(-2.37, 0.41)
p-value		0.1643
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.48
95% CI		(-1.18, 0.23)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 12		
N	17	15
LS mean change from baseline	-1.06	-0.79
SE	0.517	0.561
95% CI	(-2.08, -0.03)	(-1.90, 0.33)
LS mean difference (Omaveloxolone-Placebo)		0.27
SE		0.760
95% CI		(-1.24, 1.78)
p-value		0.7245
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.12
95% CI		(-0.57, 0.82)
Week 18		
N	16	15
LS mean change from baseline	-0.01	-0.75
SE	0.516	0.547
95% CI	(-1.04, 1.01)	(-1.84, 0.34)
LS mean difference (Omaveloxolone-Placebo)		-0.74
SE		0.747
95% CI		(-2.22, 0.75)
p-value		0.3253
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.34
95% CI		(-1.05, 0.37)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 24		
N	16	14
LS mean change from baseline	-0.55	-0.72
SE	0.593	0.640
95% CI	(-1.73, 0.62)	(-1.99, 0.55)
LS mean difference (Omaveloxolone-Placebo)		-0.16
SE		0.867
95% CI		(-1.88, 1.56)
p-value		0.8522
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.07
95% CI		(-0.78, 0.65)
Week 36		
N	16	13
LS mean change from baseline	-0.23	-0.26
SE	0.658	0.725
95% CI	(-1.53, 1.08)	(-1.70, 1.18)
LS mean difference (Omaveloxolone-Placebo)		-0.04
SE		0.974
95% CI		(-1.97, 1.90)
p-value		0.9714
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.01
95% CI		(-0.74, 0.72)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 48		
N	15	13
LS mean change from baseline	0.13	-0.31
SE	0.655	0.713
95% CI	(-1.17, 1.43)	(-1.73, 1.10)
LS mean difference (Omaveloxolone-Placebo)		-0.44
SE		0.963
95% CI		(-2.36, 1.47)
p-value		0.6474
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.17
95% CI		(-0.91, 0.58)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time).
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 29APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Baseline		
N	21	26
Mean score	23.68	24.60
SD	8.519	6.177
Week 4		
N	21	26
LS mean change from baseline	0.60	-0.28
SE	0.413	0.376
95% CI	(-0.22, 1.42)	(-1.03, 0.47)
LS mean difference (Omaveloxolone-Placebo)		-0.88
SE		0.547
95% CI		(-1.97, 0.21)
p-value		0.1121
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.46
95% CI		(-1.04, 0.13)
Week 12		
N	21	26
LS mean change from baseline	0.84	0.13
SE	0.434	0.395
95% CI	(-0.03, 1.70)	(-0.66, 0.92)
LS mean difference (Omaveloxolone-Placebo)		-0.71
SE		0.576
95% CI		(-1.85, 0.44)
p-value		0.2237
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.35
95% CI		(-0.93, 0.23)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 18		
N	21	24
LS mean change from baseline	0.32	-0.52
SE	0.435	0.408
95% CI	(-0.55, 1.18)	(-1.33, 0.29)
LS mean difference (Omaveloxolone-Placebo)		-0.84
SE		0.587
95% CI		(-2.00, 0.33)
p-value		0.1584
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.41
95% CI		(-1.00, 0.18)
Week 24		
N	21	23
LS mean change from baseline	0.81	0.17
SE	0.441	0.418
95% CI	(-0.07, 1.69)	(-0.66, 1.00)
LS mean difference (Omaveloxolone-Placebo)		-0.64
SE		0.598
95% CI		(-1.83, 0.55)
p-value		0.2888
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.31
95% CI		(-0.91, 0.28)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 36		
N	21	23
LS mean change from baseline	1.35	0.32
SE	0.470	0.447
95% CI	(0.41, 2.29)	(-0.57, 1.21)
LS mean difference (Omaveloxolone-Placebo)		-1.03
SE		0.639
95% CI		(-2.30, 0.25)
p-value		0.1125
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.47
95% CI		(-1.07, 0.13)
Week 48		
N	21	22
LS mean change from baseline	1.49	0.53
SE	0.447	0.430
95% CI	(0.60, 2.38)	(-0.33, 1.38)
LS mean difference (Omaveloxolone-Placebo)		-0.96
SE		0.611
95% CI		(-2.18, 0.26)
p-value		0.1209
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.46
95% CI		(-1.07, 0.14)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Baseline		
N	22	15
Mean score	18.11	23.67
SD	5.455	6.268
Week 4		
N	22	14
LS mean change from baseline	-0.17	0.23
SE	0.422	0.505
95% CI	(-1.01, 0.67)	(-0.77, 1.24)
LS mean difference (Omaveloxolone-Placebo)		0.40
SE		0.650
95% CI		(-0.89, 1.70)
p-value		0.5379
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.20
95% CI		(-0.47, 0.88)
Week 12		
N	22	14
LS mean change from baseline	-0.45	0.27
SE	0.444	0.531
95% CI	(-1.33, 0.44)	(-0.79, 1.33)
LS mean difference (Omaveloxolone-Placebo)		0.72
SE		0.685
95% CI		(-0.65, 2.09)
p-value		0.2969
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.35
95% CI		(-0.33, 1.02)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 18		
N	22	14
LS mean change from baseline	-0.12	-0.05
SE	0.446	0.533
95% CI	(-1.01, 0.77)	(-1.11, 1.02)
LS mean difference (Omaveloxolone-Placebo)		0.08
SE		0.688
95% CI		(-1.30, 1.45)
p-value		0.9116
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.04
95% CI		(-0.63, 0.71)
Week 24		
N	22	14
LS mean change from baseline	-0.38	0.15
SE	0.453	0.541
95% CI	(-1.28, 0.52)	(-0.92, 1.23)
LS mean difference (Omaveloxolone-Placebo)		0.54
SE		0.699
95% CI		(-0.86, 1.93)
p-value		0.4468
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.25
95% CI		(-0.42, 0.92)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 36		
N	22	13
LS mean change from baseline	0.11	0.33
SE	0.482	0.593
95% CI	(-0.85, 1.07)	(-0.85, 1.51)
LS mean difference (Omaveloxolone-Placebo)		0.22
SE		0.763
95% CI		(-1.30, 1.74)
p-value		0.7787
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.09
95% CI		(-0.59, 0.78)
Week 48		
N	21	13
LS mean change from baseline	0.61	0.83
SE	0.463	0.563
95% CI	(-0.32, 1.53)	(-0.29, 1.96)
LS mean difference (Omaveloxolone-Placebo)		0.23
SE		0.727
95% CI		(-1.22, 1.68)
p-value		0.7560
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.11
95% CI		(-0.59, 0.80)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 17APR2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	52	51
Mean score	9.85	11.03
SD	4.716	4.486
Week 24		
N	51	45
LS mean change from baseline	0.42	0.02
SE	0.388	0.414
95% CI	(-0.35, 1.20)	(-0.81, 0.84)
LS mean difference (Omaveloxolone-Placebo)		-0.41
SE		0.573
95% CI		(-1.55, 0.73)
p-value		0.4768
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.14
95% CI		(-0.54, 0.26)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-adl-mmrm.sas **Data Tag:** FINAL **Run Date:** 08DEC2023

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 36		
N	51	45
LS mean change from baseline	0.53	0.78
SE	0.396	0.422
95% CI	(-0.25, 1.32)	(-0.06, 1.62)
LS mean difference (Omaveloxolone-Placebo)		0.24
SE		0.584
95% CI		(-0.92, 1.41)
p-value		0.6769
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.08
95% CI		(-0.32, 0.48)
Week 48		
N	51	44
LS mean change from baseline	1.05	0.28
SE	0.393	0.421
95% CI	(0.27, 1.84)	(-0.56, 1.12)
LS mean difference (Omaveloxolone-Placebo)		-0.78
SE		0.582
95% CI		(-1.93, 0.38)
p-value		0.1865
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.27
95% CI		(-0.67, 0.14)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-adl-mmrm.sas **Data Tag:** FINAL **Run Date:** 08DEC2023

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline at Week 48: Treatment by Subgroup Interaction from MMRM Analysis - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Gender (female, male)	0.1921
GAA1 repeat length >=675 (yes, no)	0.3033
Geographic location (US, other)	0.6744
Pes Cavus status (yes, no)	0.0993

NOTE 1: p-value is based on the MMRM model for the ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-adl-mmrm-int.sas **Data Tag:** FINAL **Run Date:** 19MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Baseline		
N	17	31
Mean score	7.85	11.56
SD	4.475	4.635
Week 24		
N	17	29
LS mean change from baseline	0.37	-0.14
SE	0.714	0.534
95% CI	(-1.05, 1.79)	(-1.20, 0.92)
LS mean difference (Omaveloxolone-Placebo)		-0.51
SE		0.910
95% CI		(-2.32, 1.30)
p-value		0.5773
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.17
95% CI		(-0.77, 0.43)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 36		
N	17	29
LS mean change from baseline	0.81	0.67
SE	0.726	0.543
95% CI	(-0.63, 2.25)	(-0.41, 1.75)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.924
95% CI		(-1.97, 1.70)
p-value		0.8838
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.04
95% CI		(-0.64, 0.56)
Week 48		
N	17	28
LS mean change from baseline	1.90	0.10
SE	0.713	0.536
95% CI	(0.48, 3.32)	(-0.97, 1.17)
LS mean difference (Omaveloxolone-Placebo)		-1.80
SE		0.909
95% CI		(-3.61, 0.01)
p-value		0.0514
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.59
95% CI		(-1.20, 0.03)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Baseline		
N	35	20
Mean score	10.81	10.20
SD	4.581	4.225
Week 24		
N	34	16
LS mean change from baseline	0.45	0.31
SE	0.488	0.729
95% CI	(-0.52, 1.42)	(-1.14, 1.76)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.889
95% CI		(-1.91, 1.63)
p-value		0.8729
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.05
95% CI		(-0.64, 0.55)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 36		
N	34	16
LS mean change from baseline	0.40	0.97
SE	0.496	0.740
95% CI	(-0.59, 1.39)	(-0.50, 2.44)
LS mean difference (Omaveloxolone-Placebo)		0.57
SE		0.902
95% CI		(-1.23, 2.36)
p-value		0.5303
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.19
95% CI		(-0.41, 0.78)
Week 48		
N	34	16
LS mean change from baseline	0.64	0.57
SE	0.487	0.728
95% CI	(-0.33, 1.61)	(-0.88, 2.02)
LS mean difference (Omaveloxolone-Placebo)		-0.08
SE		0.887
95% CI		(-1.84, 1.69)
p-value		0.9328
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.02
95% CI		(-0.62, 0.57)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by Geographic Location (US, Other) - ITT Population

Geographical location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Baseline		
N	35	36
Mean score	10.26	12.15
SD	4.678	4.441
Week 24		
N	35	31
LS mean change from baseline	0.44	0.10
SE	0.457	0.500
95% CI	(-0.47, 1.35)	(-0.89, 1.10)
LS mean difference (Omaveloxolone-Placebo)		-0.34
SE		0.678
95% CI		(-1.68, 1.01)
p-value		0.6225
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.12
95% CI		(-0.60, 0.37)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by Geographic Location (US, Other) - ITT Population

Geographical location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 36		
N	35	31
LS mean change from baseline	0.60	1.03
SE	0.470	0.515
95% CI	(-0.34, 1.53)	(0.01, 2.06)
LS mean difference (Omaveloxolone-Placebo)		0.44
SE		0.698
95% CI		(-0.95, 1.83)
p-value		0.5309
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.15
95% CI		(-0.33, 0.64)
Week 48		
N	35	30
LS mean change from baseline	1.21	0.59
SE	0.471	0.519
95% CI	(0.27, 2.14)	(-0.44, 1.62)
LS mean difference (Omaveloxolone-Placebo)		-0.62
SE		0.702
95% CI		(-2.01, 0.77)
p-value		0.3793
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.21
95% CI		(-0.70, 0.27)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by Geographic Location (US, Other) - ITT Population

Geographical location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Baseline		
N	17	15
Mean score	9.00	8.33
SD	4.822	3.395
Week 24		
N	16	14
LS mean change from baseline	0.44	-0.21
SE	0.681	0.738
95% CI	(-0.91, 1.79)	(-1.67, 1.26)
LS mean difference (Omaveloxolone-Placebo)		-0.64
SE		0.993
95% CI		(-2.62, 1.33)
p-value		0.5178
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.23
95% CI		(-0.95, 0.49)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by by Geographic Location (US, Other) - ITT Population

Geographical location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 36		
N	16	14
LS mean change from baseline	0.43	0.21
SE	0.701	0.760
95% CI	(-0.96, 1.82)	(-1.30, 1.72)
LS mean difference (Omaveloxolone-Placebo)		-0.22
SE		1.022
95% CI		(-2.25, 1.81)
p-value		0.8297
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.08
95% CI		(-0.79, 0.64)
Week 48		
N	16	14
LS mean change from baseline	0.73	-0.42
SE	0.703	0.762
95% CI	(-0.67, 2.13)	(-1.93, 1.10)
LS mean difference (Omaveloxolone-Placebo)		-1.15
SE		1.024
95% CI		(-3.18, 0.89)
p-value		0.2661
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.39
95% CI		(-1.12, 0.33)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Y

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Baseline		
N	21	26
Mean score	11.10	11.92
SD	4.543	4.465
Week 24		
N	21	23
LS mean change from baseline	1.04	-0.32
SE	0.662	0.637
95% CI	(-0.28, 2.36)	(-1.59, 0.95)
LS mean difference (Omaveloxolone-Placebo)		-1.36
SE		0.903
95% CI		(-3.16, 0.44)
p-value		0.1366
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.44
95% CI		(-1.04, 0.16)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Y

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 36		
N	21	23
LS mean change from baseline	1.23	0.49
SE	0.665	0.639
95% CI	(-0.09, 2.56)	(-0.78, 1.77)
LS mean difference (Omaveloxolone-Placebo)		-0.74
SE		0.907
95% CI		(-2.55, 1.07)
p-value		0.4156
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.24
95% CI		(-0.83, 0.36)
Week 48		
N	21	22
LS mean change from baseline	1.69	0.40
SE	0.654	0.634
95% CI	(0.39, 3.00)	(-0.87, 1.66)
LS mean difference (Omaveloxolone-Placebo)		-1.30
SE		0.895
95% CI		(-3.08, 0.49)
p-value		0.1523
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.43
95% CI		(-1.03, 0.18)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: N

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Baseline		
N	22	15
Mean score	8.68	10.77
SD	4.458	4.795
Week 24		
N	22	14
LS mean change from baseline	-0.15	0.60
SE	0.649	0.811
95% CI	(-1.44, 1.15)	(-1.02, 2.22)
LS mean difference (Omaveloxolone-Placebo)		0.75
SE		1.004
95% CI		(-1.26, 2.75)
p-value		0.4596
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.24
95% CI		(-0.43, 0.92)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: N

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 36		
N	22	14
LS mean change from baseline	0.01	1.55
SE	0.652	0.815
95% CI	(-1.29, 1.31)	(-0.07, 3.18)
LS mean difference (Omaveloxolone-Placebo)		1.55
SE		1.009
95% CI		(-0.47, 3.56)
p-value		0.1298
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.50
95% CI		(-0.18, 1.18)
Week 48		
N	22	14
LS mean change from baseline	0.45	0.55
SE	0.641	0.801
95% CI	(-0.83, 1.73)	(-1.05, 2.14)
LS mean difference (Omaveloxolone-Placebo)		0.09
SE		0.990
95% CI		(-1.88, 2.07)
p-value		0.9248
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.03
95% CI		(-0.64, 0.70)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean Change from Baseline up to Week 48 by Visit from ANCOVA+MI Analysis - ITT Population

Visit Statistic	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 12		
Number of observations per imputation	52 (100)	50 (98.0)
Number of imputed values per imputation	0	0
LS mean change	3.66	3.72
SE	0.164	0.161
95% CI	(3.34, 3.99)	(3.40, 4.04)
LS mean difference (Omaveloxolone-Placebo)		0.06
SE		0.177
95% CI		(-0.29, 0.41)
p-value		0.7338
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.07
95% CI		(-0.32, 0.45)
Week 24		
Number of observations per imputation	51 (98.1)	45 (88.2)
Number of imputed values per imputation	1 (1.9)	5 (9.8)
LS mean change	3.91	3.69
SE	0.200	0.205
95% CI	(3.51, 4.30)	(3.29, 4.09)
LS mean difference (Omaveloxolone-Placebo)		-0.21
SE		0.219
95% CI		(-0.64, 0.21)
p-value		0.3265
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.20
95% CI		(-0.60, 0.20)

Note 1: LS Mean and Hedges' g are based on the ANCOVA model that includes site, pes cavus status, baseline, treatment group.
Note 2: Multiple imputation including treatment, site, pes cavus status, postbaseline for the endpoint
Note 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean Change from Baseline up to Week 48 by Visit from ANCOVA+MI Analysis - ITT Population

Visit Statistic	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 36		
Number of observations per imputation	51 (98.1)	45 (88.2)
Number of imputed values per imputation	1 (1.9)	5 (9.8)
LS mean change	4.14	3.75
SE	0.209	0.211
95% CI	(3.73, 4.55)	(3.33, 4.16)
LS mean difference (Omaveloxolone-Placebo)		-0.39
SE		0.228
95% CI		(-0.84, 0.06)
p-value		0.0870
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.35
95% CI		(-0.75, 0.06)
Week 48		
Number of observations per imputation	51 (98.1)	44 (86.3)
Number of imputed values per imputation	1 (1.9)	6 (11.8)
LS mean change	4.47	3.91
SE	0.229	0.241
95% CI	(4.02, 4.92)	(3.44, 4.38)
LS mean difference (Omaveloxolone-Placebo)		-0.56
SE		0.254
95% CI		(-1.06, -0.06)
p-value		0.0282
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.45
95% CI		(-0.86, -0.04)

Note 1: LS Mean and Hedges' g are based on the ANCOVA model that includes site, pes cavus status, baseline, treatment group.
Note 2: Multiple imputation including treatment, site, pes cavus status, postbaseline for the endpoint
Note 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Summary of Proportion of Improvement in Patient Global Impression of Change (PGI-C) at Week 48 - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Number of subjects with week 48 assessment (%)	51 (100)	44 (100)
Number of subjects with event (%)	13 (25.5)	19 (43.2)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.69
95% CI		(0.95, 3.01)
p-value		0.0754
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		2.22
95% CI		(0.93, 5.29)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.18
95% CI		(-0.01, 0.37)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement if PGI-C value at Week 48 in (1,2,3).
Source: biib141/valueaccess/amnog/t-pgi-propi.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Summary of Proportion of Improvement and Stable in Patient Global Impression of Change (PGI-C) at Week 48 - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Number of subjects with week 48 assessment (%)	51 (100)	44 (100)
Number of subjects with event (%)	28 (54.9)	31 (70.5)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.28
95% CI		(0.94, 1.75)
p-value		0.1223
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		1.94
95% CI		(0.83, 4.53)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.16
95% CI		(-0.04, 0.35)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement and stable if PGI-C value at Week 48 in (1,2,3,4).
Source: biib141/valueaccess/amnog/t-pgi-propis.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Patient Global Impression of Change (PGI-C) at Week 48 - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Number of subjects with week 48 assessment (%)	51 (100)	44 (100)
Number of subjects with event (%)	23 (45.1)	13 (29.5)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		0.66
95% CI		(0.38, 1.14)
p-value		0.1345
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		0.52
95% CI		(0.22, 1.20)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.16
95% CI		(-0.35, 0.04)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Worsening if PGI-C value at Week 48 in (5,6,7).
Source: biib141/valueaccess/amnog/t-pgi-propw.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean Change from Baseline at Week 48: Treatment by Subgroup Interaction from ANCOVA+MI Analysis - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Gender (female, male)	0.7657
GAA1 repeat length >=675 (yes, no)	0.0760
Geographic location (US, other)	0.9613
Pes Cavus status (yes, no)	0.5835

NOTE 1: p-value is based on the ANOVA + MI analysis for the ITT population with interaction added (subgroup*treatment).

Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-int.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by Gender (Female, Male) - ITT Population

Gender: Female

Visit Statistic	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 12		
Number of observations per imputation	17 (100)	31 (100)
Number of imputed values per imputation	0	0
LS mean	3.86	3.60
SE	0.240	0.182
95% CI	(3.38, 4.33)	(3.24, 3.96)
LS mean difference (Omaveloxolone-Placebo)		-0.26
SE		0.273
95% CI		(-0.80, 0.29)
p-value		0.3505
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.27
95% CI		(-0.87, 0.32)
Week 24		
Number of observations per imputation	17 (100)	29 (93.5)
Number of imputed values per imputation	0	2 (6.5)
LS mean	3.96	3.63
SE	0.294	0.228
95% CI	(3.39, 4.54)	(3.19, 4.08)
LS mean difference (Omaveloxolone-Placebo)		-0.33
SE		0.335
95% CI		(-0.99, 0.33)
p-value		0.3254
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.30
95% CI		(-0.90, 0.31)

NOTE 1: LS Mean and Hedges' g are from the ANCOVA model for ITT population with two way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, site, gender, pes cavus status, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by Gender (Female, Male) - ITT Population

Gender: Female

Visit Statistic	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 36		
Number of observations per imputation	17 (100)	29 (93.5)
Number of imputed values per imputation	0	2 (6.5)
LS mean	4.50	3.70
SE	0.304	0.234
95% CI	(3.91, 5.10)	(3.24, 4.16)
LS mean difference (Omaveloxolone-Placebo)		-0.80
SE		0.346
95% CI		(-1.48, -0.12)
p-value		0.0206
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.70
95% CI		(-1.31, -0.08)
Week 48		
Number of observations per imputation	17 (100)	28 (90.3)
Number of imputed values per imputation	0	3 (9.7)
LS mean	4.57	3.90
SE	0.337	0.270
95% CI	(3.90, 5.23)	(3.37, 4.43)
LS mean difference (Omaveloxolone-Placebo)		-0.66
SE		0.387
95% CI		(-1.42, 0.09)
p-value		0.0859
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.52
95% CI		(-1.13, 0.09)

NOTE 1: LS Mean and Hedges' g are from the ANCOVA model for ITT population with two way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, site, gender, pes cavus status, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by Gender (Female, Male) - ITT Population

Gender: Male

Visit Statistic	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 12		
Number of observations per imputation	35 (100)	19 (95.0)
Number of imputed values per imputation	0	0
LS mean	3.59	3.96
SE	0.189	0.240
95% CI	(3.21, 3.97)	(3.48, 4.43)
LS mean difference (Omaveloxolone-Placebo)		0.37
SE		0.256
95% CI		(-0.14, 0.88)
p-value		0.1542
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.40
95% CI		(-0.17, 0.96)
Week 24		
Number of observations per imputation	34 (97.1)	16 (80.0)
Number of imputed values per imputation	1 (2.9)	3 (15.0)
LS mean	3.93	3.88
SE	0.237	0.333
95% CI	(3.46, 4.39)	(3.23, 4.53)
LS mean difference (Omaveloxolone-Placebo)		-0.05
SE		0.340
95% CI		(-0.71, 0.62)
p-value		0.8939
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.04
95% CI		(-0.63, 0.55)

NOTE 1: LS Mean and Hedges' g are from the ANCOVA model for ITT population with two way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, site, gender, pes cavus status, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by Gender (Female, Male) - ITT Population

Gender: Male

Visit Statistic	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 36		
Number of observations per imputation	34 (97.1)	16 (80.0)
Number of imputed values per imputation	1 (2.9)	3 (15.0)
LS mean	3.96	3.82
SE	0.244	0.343
95% CI	(3.48, 4.44)	(3.15, 4.49)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.350
95% CI		(-0.83, 0.54)
p-value		0.6864
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.12
95% CI		(-0.72, 0.47)
Week 48		
Number of observations per imputation	34 (97.1)	16 (80.0)
Number of imputed values per imputation	1 (2.9)	3 (15.0)
LS mean	4.42	3.92
SE	0.270	0.382
95% CI	(3.89, 4.95)	(3.17, 4.67)
LS mean difference (Omaveloxolone-Placebo)		-0.50
SE		0.389
95% CI		(-1.26, 0.26)
p-value		0.1996
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.38
95% CI		(-0.98, 0.22)

NOTE 1: LS Mean and Hedges' g are from the ANCOVA model for ITT population with two way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, site, gender, pes cavus status, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by Geographic Location (US, Other) - ITT Population

Geographical Region: US

Visit Statistic	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 12		
Number of observations per imputation	35 (100)	35 (97.2)
Number of imputed values per imputation	0	0
LS mean	3.53	3.68
SE	0.165	0.160
95% CI	(3.21, 3.86)	(3.37, 4.00)
LS mean difference (Omaveloxolone-Placebo)		0.15
SE		0.213
95% CI		(-0.27, 0.57)
p-value		0.4820
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.16
95% CI		(-0.30, 0.63)
Week 24		
Number of observations per imputation	35 (100)	31 (86.1)
Number of imputed values per imputation	0	4 (11.1)
LS mean	3.80	3.47
SE	0.173	0.199
95% CI	(3.46, 4.14)	(3.08, 3.86)
LS mean difference (Omaveloxolone-Placebo)		-0.31
SE		0.257
95% CI		(-0.81, 0.20)
p-value		0.2345
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.29
95% CI		(-0.78, 0.20)

NOTE 1: LS Mean and Hedges'g are from the ANCOVA model for ITT population with two-way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, region, pes cavus status, postbaseline for the endpoint.
NOTE 3: Site was not included as a covariate in ANCOVA subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.
NOTE 4: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by Geographic Location (US, Other) - ITT Population

Geographical Region: US

Visit Statistic	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 36		
Number of observations per imputation	35 (100)	31 (86.1)
Number of imputed values per imputation	0	4 (11.1)
LS mean	3.86	3.54
SE	0.185	0.209
95% CI	(3.49, 4.23)	(3.13, 3.94)
LS mean difference (Omaveloxolone-Placebo)		-0.38
SE		0.272
95% CI		(-0.91, 0.15)
p-value		0.1599
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.34
95% CI		(-0.83, 0.14)
Week 48		
Number of observations per imputation	35 (100)	30 (83.3)
Number of imputed values per imputation	0	5 (13.9)
LS mean	4.11	3.63
SE	0.201	0.242
95% CI	(3.71, 4.51)	(3.16, 4.11)
LS mean difference (Omaveloxolone-Placebo)		-0.51
SE		0.302
95% CI		(-1.10, 0.08)
p-value		0.0895
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.42
95% CI		(-0.91, 0.08)

NOTE 1: LS Mean and Hedges'g are from the ANCOVA model for ITT population with two-way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, region, pes cavus status, postbaseline for the endpoint.
NOTE 3: Site was not included as a covariate in ANCOVA subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.
NOTE 4: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by Geographic Location (US, Other) - ITT Population

Geographical Region: Other

Visit Statistic	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 12		
Number of observations per imputation	17 (100)	15 (100)
Number of imputed values per imputation	0	0
LS mean	3.61	3.61
SE	0.228	0.249
95% CI	(3.15, 4.06)	(3.12, 4.11)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.316
95% CI		(-0.62, 0.63)
p-value		0.9865
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.01
95% CI		(-0.69, 0.70)
Week 24		
Number of observations per imputation	16 (94.1)	14 (93.3)
Number of imputed values per imputation	1 (5.9)	1 (6.7)
LS mean	3.94	3.96
SE	0.256	0.301
95% CI	(3.43, 4.45)	(3.37, 4.56)
LS mean difference (Omaveloxolone-Placebo)		0.05
SE		0.381
95% CI		(-0.70, 0.79)
p-value		0.9026
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.04
95% CI		(-0.67, 0.76)

NOTE 1: LS Mean and Hedges'g are from the ANCOVA model for ITT population with two-way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, region, pes cavus status, postbaseline for the endpoint.
NOTE 3: Site was not included as a covariate in ANCOVA subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.
NOTE 4: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by Geographic Location (US, Other) - ITT Population

Geographical Region: Other

Visit Statistic	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 36		
Number of observations per imputation	16 (94.1)	14 (93.3)
Number of imputed values per imputation	1 (5.9)	1 (6.7)
LS mean	4.13	3.79
SE	0.274	0.316
95% CI	(3.58, 4.67)	(3.18, 4.41)
LS mean difference (Omaveloxolone-Placebo)		-0.40
SE		0.402
95% CI		(-1.19, 0.39)
p-value		0.3196
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.35
95% CI		(-1.08, 0.37)
Week 48		
Number of observations per imputation	16 (94.1)	14 (93.3)
Number of imputed values per imputation	1 (5.9)	1 (6.7)
LS mean	4.50	4.04
SE	0.297	0.349
95% CI	(3.91, 5.09)	(3.36, 4.73)
LS mean difference (Omaveloxolone-Placebo)		-0.49
SE		0.440
95% CI		(-1.35, 0.38)
p-value		0.2688
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.39
95% CI		(-1.12, 0.33)

NOTE 1: LS Mean and Hedges'g are from the ANCOVA model for ITT population with two-way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, region, pes cavus status, postbaseline for the endpoint.
NOTE 3: Site was not included as a covariate in ANCOVA subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.
NOTE 4: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Y

Visit Statistic	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 12		
Number of observations per imputation	21 (100)	26 (100)
Number of imputed values per imputation	0	0
LS mean	3.92	3.67
SE	0.239	0.196
95% CI	(3.44, 4.40)	(3.28, 4.06)
LS mean difference (Omaveloxolone-Placebo)		-0.25
SE		0.258
95% CI		(-0.76, 0.27)
p-value		0.3376
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.27
95% CI		(-0.85, 0.30)
Week 24		
Number of observations per imputation	21 (100)	23 (88.5)
Number of imputed values per imputation	0	3 (11.5)
LS mean	4.38	3.64
SE	0.304	0.263
95% CI	(3.78, 4.97)	(3.13, 4.16)
LS mean difference (Omaveloxolone-Placebo)		-0.73
SE		0.335
95% CI		(-1.39, -0.08)
p-value		0.0284
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.65
95% CI		(-1.26, -0.04)

NOTE 1: LS Mean and Hedges' g are from the ANCOVA model for ITT population with two way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, site, pes cavus status, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Y

Visit Statistic	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 36		
Number of observations per imputation	21 (100)	23 (88.5)
Number of imputed values per imputation	0	3 (11.5)
LS mean	4.52	3.72
SE	0.305	0.262
95% CI	(3.92, 5.12)	(3.21, 4.23)
LS mean difference (Omaveloxolone-Placebo)		-0.80
SE		0.335
95% CI		(-1.45, -0.14)
p-value		0.0175
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.70
95% CI		(-1.31, -0.09)
Week 48		
Number of observations per imputation	21 (100)	22 (84.6)
Number of imputed values per imputation	0	4 (15.4)
LS mean	4.76	3.68
SE	0.335	0.298
95% CI	(4.11, 5.42)	(3.10, 4.27)
LS mean difference (Omaveloxolone-Placebo)		-1.08
SE		0.374
95% CI		(-1.81, -0.35)
p-value		0.0038
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.87
95% CI		(-1.49, -0.24)

NOTE 1: LS Mean and Hedges' g are from the ANCOVA model for ITT population with two way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, site, pes cavus status, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: N

Visit Statistic	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 12		
Number of observations per imputation	22 (100)	14 (93.3)
Number of imputed values per imputation	0	0
LS mean	3.29	3.53
SE	0.228	0.272
95% CI	(2.84, 3.75)	(2.98, 4.07)
LS mean difference (Omaveloxolone-Placebo)		0.23
SE		0.291
95% CI		(-0.35, 0.81)
p-value		0.4249
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.26
95% CI		(-0.41, 0.94)
Week 24		
Number of observations per imputation	22 (100)	14 (93.3)
Number of imputed values per imputation	0	0
LS mean	3.52	3.72
SE	0.292	0.348
95% CI	(2.95, 4.09)	(3.03, 4.40)
LS mean difference (Omaveloxolone-Placebo)		0.20
SE		0.368
95% CI		(-0.52, 0.92)
p-value		0.5937
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.18
95% CI		(-0.49, 0.85)

NOTE 1: LS Mean and Hedges' g are from the ANCOVA model for ITT population with two way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, site, pes cavus status, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: N

Visit Statistic	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 36		
Number of observations per imputation	22 (100)	14 (93.3)
Number of imputed values per imputation	0	0
LS mean	3.83	3.78
SE	0.293	0.350
95% CI	(3.25, 4.40)	(3.10, 4.47)
LS mean difference (Omaveloxolone-Placebo)		-0.04
SE		0.369
95% CI		(-0.77, 0.68)
p-value		0.9062
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.04
95% CI		(-0.71, 0.63)
Week 48		
Number of observations per imputation	22 (100)	14 (93.3)
Number of imputed values per imputation	0	0
LS mean	4.39	4.29
SE	0.322	0.384
95% CI	(3.76, 5.02)	(3.54, 5.05)
LS mean difference (Omaveloxolone-Placebo)		-0.10
SE		0.405
95% CI		(-0.89, 0.70)
p-value		0.8111
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.08
95% CI		(-0.75, 0.59)

NOTE 1: LS Mean and Hedges' g are from the ANCOVA model for ITT population with two way interaction added (subgroup*treatment) at each timepoint.
NOTE 2: Multiple imputation including treatment, site, pes cavus status, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Summary of Proportion of Improvement in Patient Global Impression of Change (PGI-C) at Week 48: Treatment by Subgroup Interaction - ITT Population

Subgroup	p-value based on adjusted RR for Treatment by Subgroup Interaction
Age (<18, >=18)	0.3244
Gender (female, male)	0.3372
GAA1 repeat length >=675 (yes, no)	0.1137
Geographic location (US, other)	0.9789
Pes Cavus status (yes, no)	0.8831

Note 1: Cochran-Mantel-Haenszel adjusted RR is calculated adjusting for pes cavus status.
Note 2: Improvement if PGI-C value at Week 48 in (1,2,3)
Note 3: P-value for pes cavus status is based on RR
Source: biib141/valueaccess/amnog/t-pgi-propi-int.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Summary of Proportion of Improvement in Patient Global Impression of Change (PGI-C) at Week 48 by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Number of subjects with week 48 assessment (%)	17 (100)	28 (100)
Number of subjects with event (%)	4 (23.5)	14 (50.0)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		2.11
95% CI		(0.83, 5.35)
p-value		0.1150
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		3.22
95% CI		(0.84, 12.34)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.26
95% CI		(-0.01, 0.54)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement if PGI-C value at Week 48 in (1,2,3).

Source: biib141/valueaccess/amnog/t-pgi-propi-sgrp.sas:t-pgi-propi-sgrp-gen.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement in Patient Global Impression of Change (PGI-C) at Week 48 by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Number of subjects with week 48 assessment (%)	34 (100)	16 (100)
Number of subjects with event (%)	9 (26.5)	5 (31.3)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.11
95% CI		(0.45, 2.78)
p-value		0.8161
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		1.17
95% CI		(0.31, 4.44)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.05
95% CI		(-0.22, 0.32)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement if PGI-C value at Week 48 in (1,2,3).

Source: biib141/valueaccess/amnog/t-pgi-propi-sgrp.sas:t-pgi-propi-sgrp-gen.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement in Patient Global Impression of Change (PGI-C) at Week 48 by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Number of subjects with week 48 assessment (%)	35 (100)	30 (100)
Number of subjects with event (%)	10 (28.6)	14 (46.7)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.64
95% CI		(0.86, 3.13)
p-value		0.1366
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		2.19
95% CI		(0.79, 6.13)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.18
95% CI		(-0.05, 0.41)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement if PGI-C value at Week 48 in (1,2,3).

Source: biib141/valueaccess/amnog/t-pgi-propi-sgrp.sas:t-pgi-propi-sgrp-geo.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement in Patient Global Impression of Change (PGI-C) at Week 48 by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Number of subjects with week 48 assessment (%)	16 (100)	14 (100)
Number of subjects with event (%)	3 (18.8)	5 (35.7)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.67
95% CI		(0.50, 5.57)
p-value		0.4070
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		2.08
95% CI		(0.38, 11.48)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.17
95% CI		(-0.15, 0.49)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement if PGI-C value at Week 48 in (1,2,3).

Source: biib141/valueaccess/amnog/t-pgi-propi-sgrp.sas:t-pgi-propi-sgrp-geo.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement in Patient Global Impression of Change (PGI-C) at Week 48 by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Number of subjects with week 48 assessment (%)	21 (100)	22 (100)
Number of subjects with event (%)	4 (19.0)	12 (54.5)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		2.86
95% CI		(1.09, 7.49)
p-value		0.0330
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		4.92
95% CI		(1.26, 19.14)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.35
95% CI		(0.09, 0.62)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement if PGI-C value at Week 48 in (1,2,3).

Source: biib141/valueaccess/amnog/t-pgi-propi-sgrp.sas:t-pgi-propi-sgrp-gaa1.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement in Patient Global Impression of Change (PGI-C) at Week 48 by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Number of subjects with week 48 assessment (%)	22 (100)	14 (100)
Number of subjects with event (%)	6 (27.3)	3 (21.4)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		0.84
95% CI		(0.26, 2.70)
p-value		0.7745
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		0.80
95% CI		(0.16, 3.87)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.06
95% CI		(-0.34, 0.23)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement if PGI-C value at Week 48 in (1,2,3).

Source: biib141/valueaccess/amnog/t-pgi-propi-sgrp.sas:t-pgi-propi-sgrp-gaa1.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement and Stable in Patient Global Impression of Change (PGI-C) at Week 48: Treatment by Subgroup Interaction - ITT Population

Subgroup	p-value based on adjusted RR for Treatment by Subgroup Interaction
Age (<18, >=18)	0.2624
Gender (female, male)	0.7323
GAA1 repeat length >=675 (yes, no)	0.3598
Geographic location (US, other)	0.5726
Pes Cavus status (yes, no)	0.3304

Note 1: Cochran-Mantel-Haenszel adjusted RR is calculated adjusting for pes cavus status.
Note 2: Improvement and stable if PGI-C value at Week 48 in (1,2,3,4).
Note 3: P-value for pes cavus status is based on RR
Source: biib141/valueaccess/amnog/t-pgi-propis-int.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Summary of Proportion of Improvement and Stable in Patient Global Impression of Change (PGI-C) at Week 48 by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Number of subjects with week 48 assessment (%)	17 (100)	28 (100)
Number of subjects with event (%)	8 (47.1)	17 (60.7)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.28
95% CI		(0.72, 2.28)
p-value		0.4071
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		1.71
95% CI		(0.50, 5.83)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.14
95% CI		(-0.16, 0.43)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement and stable if PGI-C value at Week 48 in (1,2,3,4).

Source: biib141/valueaccess/amnog/t-pgi-propis-sgrp.sas:t-pgi-propis-sgrp-gen.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement and Stable in Patient Global Impression of Change (PGI-C) at Week 48 by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Number of subjects with week 48 assessment (%)	34 (100)	16 (100)
Number of subjects with event (%)	20 (58.8)	14 (87.5)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.44
95% CI		(1.04, 1.99)
p-value		0.0299
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		4.27
95% CI		(0.87, 20.91)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.29
95% CI		(0.06, 0.52)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement and stable if PGI-C value at Week 48 in (1,2,3,4).

Source: biib141/valueaccess/amnog/t-pgi-propis-sgrp.sas:t-pgi-propis-sgrp-gen.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement and Stable in Patient Global Impression of Change (PGI-C) at Week 48 by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Number of subjects with week 48 assessment (%)	35 (100)	30 (100)
Number of subjects with event (%)	19 (54.3)	22 (73.3)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.36
95% CI		(0.93, 1.97)
p-value		0.1095
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		2.35
95% CI		(0.82, 6.72)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.19
95% CI		(-0.04, 0.42)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement and stable if PGI-C value at Week 48 in (1,2,3,4).

Source: biib141/valueaccess/amnog/t-pgi-propis-sgrp.sas:t-pgi-propis-sgrp-geo.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement and Stable in Patient Global Impression of Change (PGI-C) at Week 48 by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Number of subjects with week 48 assessment (%)	16 (100)	14 (100)
Number of subjects with event (%)	9 (56.3)	9 (64.3)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.12
95% CI		(0.64, 1.96)
p-value		0.6984
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		1.33
95% CI		(0.30, 5.78)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.08
95% CI		(-0.27, 0.43)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement and stable if PGI-C value at Week 48 in (1,2,3,4).

Source: biib141/valueaccess/amnog/t-pgi-propis-sgrp.sas:t-pgi-propis-sgrp-geo.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement and Stable in Patient Global Impression of Change (PGI-C) at Week 48 by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Number of subjects with week 48 assessment (%)	21 (100)	22 (100)
Number of subjects with event (%)	10 (47.6)	16 (72.7)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.52
95% CI		(0.91, 2.53)
p-value		0.1067
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		2.98
95% CI		(0.82, 10.82)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.25
95% CI		(-0.03, 0.53)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement and stable if PGI-C value at Week 48 in (1,2,3,4).

Source: biib141/valueaccess/amnog/t-pgi-propis-sgrp.sas:t-pgi-propis-sgrp-gaa1.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement and Stable in Patient Global Impression of Change (PGI-C) at Week 48 by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Number of subjects with week 48 assessment (%)	22 (100)	14 (100)
Number of subjects with event (%)	14 (63.6)	10 (71.4)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.11
95% CI		(0.70, 1.74)
p-value		0.6666
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		1.37
95% CI		(0.32, 5.91)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.08
95% CI		(-0.23, 0.39)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Improvement and stable if PGI-C value at Week 48 in (1,2,3,4).

Source: biib141/valueaccess/amnog/t-pgi-propis-sgrp.sas:t-pgi-propis-sgrp-gaa1.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Patient Global Impression of Change (PGI-C) at Week 48: Treatment by Subgroup Interaction - ITT Population

Subgroup	p-value based on adjusted RR for Treatment by Subgroup Interaction
Age (<18, >=18)	0.1531
Gender (female, male)	0.2753
GAA1 repeat length >=675 (yes, no)	0.5030
Geographic location (US, other)	0.5554
Pes Cavus status (yes, no)	0.4374

Note 1: Cochran-Mantel-Haenszel adjusted RR is calculated adjusting for pes cavus status.
Note 2: Worsening if PGI-C value at Week 48 in (5,6,7).
Note 3: P-value for pes cavus status is based on RR
Source: biib141/valueaccess/amnog/t-pgi-propw-int.sas **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Patient Global Impression of Change (PGI-C) at Week 48 by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Number of subjects with week 48 assessment (%)	17 (100)	28 (100)
Number of subjects with event (%)	9 (52.9)	11 (39.3)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		0.75
95% CI		(0.39, 1.43)
p-value		0.3799
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		0.58
95% CI		(0.17, 1.98)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.14
95% CI		(-0.43, 0.16)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Worsening if PGI-C value at Week 48 in (5,6,7).

Source: biib141/valueaccess/amnog/t-pgi-propw-sgrp.sas:t-pgi-propw-sgrp-gen.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Patient Global Impression of Change (PGI-C) at Week 48 by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Number of subjects with week 48 assessment (%)	34 (100)	16 (100)
Number of subjects with event (%)	14 (41.2)	2 (12.5)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		0.31
95% CI		(0.08, 1.30)
p-value		0.1110
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		0.23
95% CI		(0.05, 1.15)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.29
95% CI		(-0.52, -0.06)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Worsening if PGI-C value at Week 48 in (5,6,7).

Source: biib141/valueaccess/amnog/t-pgi-propw-sgrp.sas:t-pgi-propw-sgrp-gen.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Patient Global Impression of Change (PGI-C) at Week 48 by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Number of subjects with week 48 assessment (%)	35 (100)	30 (100)
Number of subjects with event (%)	16 (45.7)	8 (26.7)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		0.58
95% CI		(0.29, 1.16)
p-value		0.1224
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		0.43
95% CI		(0.15, 1.22)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.19
95% CI		(-0.42, 0.04)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Worsening if PGI-C value at Week 48 in (5,6,7).

Source: biib141/valueaccess/amnog/t-pgi-propw-sgrp.sas:t-pgi-propw-sgrp-geo.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Patient Global Impression of Change (PGI-C) at Week 48 by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Number of subjects with week 48 assessment (%)	16 (100)	14 (100)
Number of subjects with event (%)	7 (43.8)	5 (35.7)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		0.83
95% CI		(0.31, 2.24)
p-value		0.7181
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		0.75
95% CI		(0.17, 3.29)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.08
95% CI		(-0.43, 0.27)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Worsening if PGI-C value at Week 48 in (5,6,7).

Source: biib141/valueaccess/amnog/t-pgi-propw-sgrp.sas:t-pgi-propw-sgrp-geo.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Patient Global Impression of Change (PGI-C) at Week 48 by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Number of subjects with week 48 assessment (%)	21 (100)	22 (100)
Number of subjects with event (%)	11 (52.4)	6 (27.3)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		0.52
95% CI		(0.24, 1.15)
p-value		0.1073
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		0.34
95% CI		(0.09, 1.22)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.25
95% CI		(-0.53, 0.03)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Worsening if PGI-C value at Week 48 in (5,6,7).

Source: biib141/valueaccess/amnog/t-pgi-propw-sgrp.sas:t-pgi-propw-sgrp-gaa1.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Patient Global Impression of Change (PGI-C) at Week 48 by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Number of subjects with week 48 assessment (%)	22 (100)	14 (100)
Number of subjects with event (%)	8 (36.4)	4 (28.6)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		0.81
95% CI		(0.30, 2.21)
p-value		0.6807
Adjusted OR - Odds Ratio (Omaveloxolone/Placebo)		0.73
95% CI		(0.17, 3.16)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.08
95% CI		(-0.39, 0.23)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Worsening if PGI-C value at Week 48 in (5,6,7).

Source: biib141/valueaccess/amnog/t-pgi-propw-sgrp.sas:t-pgi-propw-sgrp-gaa1.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	52	51
Mean score	0.0227	0.0229
SD	0.00758	0.00770
Week 24		
N	50	45
LS mean change from baseline	-0.0003	-0.0003
SE	0.00040	0.00042
95% CI	(-0.0011, 0.0005)	(-0.0011, 0.0005)
LS mean difference (Omaveloxolone-Placebo)		0.0000
SE		0.00058
95% CI		(-0.0011, 0.0012)
p-value		0.9837
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.0041
95% CI		(-0.3986, 0.4069)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9pht-mmrm.sas:t-9pht-mmrm-dh.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 48		
N	51	44
LS mean change from baseline	-0.0007	-0.0002
SE	0.00045	0.00048
95% CI	(-0.0016, 0.0002)	(-0.0011, 0.0008)
LS mean difference (Omaveloxolone-Placebo)		0.0005
SE		0.00066
95% CI		(-0.0008, 0.0018)
p-value		0.4223
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.1623
95% CI		(-0.2417, 0.5662)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9pht-mmrm.sas:t-9pht-mmrm-dh.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	52	51
Mean score	0.0204	0.0205
SD	0.00699	0.00797
Week 24		
N	51	45
LS mean change from baseline	-0.0007	-0.0014
SE	0.00048	0.00051
95% CI	(-0.0016, 0.0003)	(-0.0024, -0.0004)
LS mean difference (Omaveloxolone-Placebo)		-0.0007
SE		0.00071
95% CI		(-0.0021, 0.0007)
p-value		0.2974
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.2096
95% CI		(-0.6116, 0.1923)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9pht-mmrm.sas:t-9pht-mmrm-ndh.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 48		
N	51	44
LS mean change from baseline	-0.0002	-0.0010
SE	0.00053	0.00056
95% CI	(-0.0013, 0.0008)	(-0.0022, 0.0001)
LS mean difference (Omaveloxolone-Placebo)		-0.0008
SE		0.00078
95% CI		(-0.0024, 0.0007)
p-value		0.2976
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.2108
95% CI		(-0.6152, 0.1936)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9pht-mmrm.sas:t-9pht-mmrm-ndh.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline at Week 48: Treatment by Subgroup Interaction from MMRM Analysis - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Gender (female, male)	0.3053
GAA1 repeat length >=675 (yes, no)	0.2671
Geographic location (US, other)	0.3249
Pes Cavus status (yes, no)	0.5689

NOTE 1: p-value is based on the MMRM model for the ITT population with three way interaction added (subgroup*treatment*time).

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-dh-int.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Baseline		
N	17	31
Mean score	0.0272	0.0240
SD	0.00759	0.00780
Week 24		
N	17	29
LS mean change from baseline	0.0011	-0.0002
SE	0.00070	0.00052
95% CI	(-0.0003, 0.0025)	(-0.0013, 0.0008)
LS mean difference (Omaveloxolone-Placebo)		-0.0014
SE		0.00087
95% CI		(-0.0031, 0.0003)
p-value		0.1123
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.4748
95% CI		(-1.0813, 0.1317)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 24APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 48		
N	17	28
LS mean change from baseline	0.0002	-0.0002
SE	0.00081	0.00061
95% CI	(-0.0014, 0.0018)	(-0.0014, 0.0010)
LS mean difference (Omaveloxolone-Placebo)		-0.0004
SE		0.00100
95% CI		(-0.0024, 0.0016)
p-value		0.6801
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.1232
95% CI		(-0.7264, 0.4799)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 24APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Baseline		
N	35	20
Mean score	0.0206	0.0213
SD	0.00666	0.00744
Week 24		
N	33	16
LS mean change from baseline	-0.0011	-0.0004
SE	0.00049	0.00072
95% CI	(-0.0020, -0.0001)	(-0.0018, 0.0011)
LS mean difference (Omaveloxolone-Placebo)		0.0007
SE		0.00087
95% CI		(-0.0010, 0.0024)
p-value		0.4326
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.2329
95% CI		(-0.3659, 0.8318)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 24APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 48		
N	34	16
LS mean change from baseline	-0.0012	-0.0001
SE	0.00056	0.00083
95% CI	(-0.0023, 0.0000)	(-0.0017, 0.0015)
LS mean difference (Omaveloxolone-Placebo)		0.0011
SE		0.00100
95% CI		(-0.0009, 0.0030)
p-value		0.2892
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.3138
95% CI		(-0.2836, 0.9112)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 24APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic Location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Baseline		
N	35	36
Mean score	0.0237	0.0218
SD	0.00861	0.00788
Week 24		
N	35	31
LS mean change from baseline	-0.0002	-0.0007
SE	0.00046	0.00050
95% CI	(-0.0012, 0.0007)	(-0.0017, 0.0003)
LS mean difference (Omaveloxolone-Placebo)		-0.0005
SE		0.00068
95% CI		(-0.0018, 0.0009)
p-value		0.4727
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.1734
95% CI		(-0.6577, 0.3109)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 24APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic Location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 48		
N	35	30
LS mean change from baseline	-0.0006	-0.0005
SE	0.00053	0.00057
95% CI	(-0.0016, 0.0005)	(-0.0016, 0.0006)
LS mean difference (Omaveloxolone-Placebo)		0.0001
SE		0.00078
95% CI		(-0.0015, 0.0016)
p-value		0.9059
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.0287
95% CI		(-0.4589, 0.5164)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 24APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic Location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Baseline		
N	17	15
Mean score	0.0208	0.0257
SD	0.00445	0.00671
Week 24		
N	15	14
LS mean change from baseline	-0.0005	0.0006
SE	0.00071	0.00075
95% CI	(-0.0019, 0.0009)	(-0.0008, 0.0021)
LS mean difference (Omaveloxolone-Placebo)		0.0011
SE		0.00104
95% CI		(-0.0009, 0.0032)
p-value		0.2869
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.3818
95% CI		(-0.3531, 1.1167)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 24APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic Location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 48		
N	16	14
LS mean change from baseline	-0.0010	0.0005
SE	0.00078	0.00085
95% CI	(-0.0026, 0.0006)	(-0.0012, 0.0022)
LS mean difference (Omaveloxolone-Placebo)		0.0015
SE		0.00116
95% CI		(-0.0008, 0.0038)
p-value		0.2031
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.4504
95% CI		(-0.2759, 1.1767)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 24APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Y

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Baseline		
N	21	26
Mean score	0.0224	0.0219
SD	0.00911	0.00702
Week 24		
N	21	23
LS mean change from baseline	-0.0003	-0.0001
SE	0.00062	0.00059
95% CI	(-0.0015, 0.0010)	(-0.0013, 0.0010)
LS mean difference (Omaveloxolone-Placebo)		0.0001
SE		0.00084
95% CI		(-0.0015, 0.0018)
p-value		0.8657
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.0494
95% CI		(-0.5422, 0.6411)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 30APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Y

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 48		
N	21	22
LS mean change from baseline	-0.0001	-0.0001
SE	0.00065	0.00064
95% CI	(-0.0014, 0.0012)	(-0.0014, 0.0012)
LS mean difference (Omaveloxolone-Placebo)		0.0000
SE		0.00090
95% CI		(-0.0018, 0.0018)
p-value		0.9981
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.0007
95% CI		(-0.5986, 0.5973)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 30APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: N

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Baseline		
N	22	15
Mean score	0.0239	0.0221
SD	0.00621	0.00834
Week 24		
N	21	14
LS mean change from baseline	-0.0010	-0.0006
SE	0.00061	0.00076
95% CI	(-0.0022, 0.0002)	(-0.0021, 0.0009)
LS mean difference (Omaveloxolone-Placebo)		0.0004
SE		0.00094
95% CI		(-0.0015, 0.0022)
p-value		0.7005
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.1279
95% CI		(-0.5490, 0.8048)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 30APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: N

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 48		
N	22	14
LS mean change from baseline	-0.0019	-0.0004
SE	0.00064	0.00080
95% CI	(-0.0032, -0.0006)	(-0.0020, 0.0012)
LS mean difference (Omaveloxolone-Placebo)		0.0015
SE		0.00100
95% CI		(-0.0005, 0.0035)
p-value		0.1346
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.4972
95% CI		(-0.1826, 1.1771)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-dh-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 30APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline at Week 48: Treatment by Subgroup Interaction from MMRM Analysis - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Gender (female, male)	0.0741
GAA1 repeat length >=675 (yes, no)	0.0266
Geographic location (US, other)	0.1302
Pes Cavus status (yes, no)	0.3354

NOTE 1: p-value is based on the MMRM model for the ITT population with three way interaction added (subgroup*treatment*time).
Source: biib141/valueaccess/amnog/t-9hpt-mmrm-ndh-int.sas **Data Tag:** FINAL **Run Date:** 16MAY2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Baseline		
N	17	31
Mean score	0.0239	0.0216
SD	0.00689	0.00868
Week 24		
N	17	29
LS mean change from baseline	0.0000	-0.0015
SE	0.00087	0.00066
95% CI	(-0.0018, 0.0017)	(-0.0028, -0.0002)
LS mean difference (Omaveloxolone-Placebo)		-0.0015
SE		0.00109
95% CI		(-0.0036, 0.0007)
p-value		0.1830
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.3972
95% CI		(-1.0013, 0.2070)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 28APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Week 48		
N	17	28
LS mean change from baseline	0.0008	-0.0016
SE	0.00094	0.00071
95% CI	(-0.0011, 0.0026)	(-0.0030, -0.0002)
LS mean difference (Omaveloxolone-Placebo)		-0.0024
SE		0.00117
95% CI		(-0.0047, 0.0000)
p-value		0.0464
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.6018
95% CI		(-1.2172, 0.0135)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 28APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Baseline		
N	35	20
Mean score	0.0188	0.0189
SD	0.00650	0.00659
Week 24		
N	34	16
LS mean change from baseline	-0.0010	-0.0013
SE	0.00061	0.00091
95% CI	(-0.0022, 0.0002)	(-0.0031, 0.0005)
LS mean difference (Omaveloxolone-Placebo)		-0.0003
SE		0.00109
95% CI		(-0.0024, 0.0019)
p-value		0.8054
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.0727
95% CI		(-0.6670, 0.5217)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 28APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Week 48		
N	34	16
LS mean change from baseline	-0.0007	0.0000
SE	0.00065	0.00097
95% CI	(-0.0020, 0.0006)	(-0.0020, 0.0019)
LS mean difference (Omaveloxolone-Placebo)		0.0007
SE		0.00117
95% CI		(-0.0017, 0.0030)
p-value		0.5661
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.1694
95% CI		(-0.4257, 0.7645)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-gen.sas **Data Tag:** FINAL **Run Date:** 28APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic Location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Baseline		
N	35	36
Mean score	0.0208	0.0198
SD	0.00801	0.00861
Week 24		
N	35	31
LS mean change from baseline	-0.0005	-0.0017
SE	0.00059	0.00063
95% CI	(-0.0017, 0.0006)	(-0.0029, -0.0004)
LS mean difference (Omaveloxolone-Placebo)		-0.0011
SE		0.00086
95% CI		(-0.0029, 0.0006)
p-value		0.1848
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.3214
95% CI		(-0.8079, 0.1651)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 28APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic Location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Week 48		
N	35	30
LS mean change from baseline	0.0002	-0.0012
SE	0.00062	0.00067
95% CI	(-0.0010, 0.0014)	(-0.0026, 0.0001)
LS mean difference (Omaveloxolone-Placebo)		-0.0014
SE		0.00091
95% CI		(-0.0032, 0.0004)
p-value		0.1186
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.3822
95% CI		(-0.8742, 0.1099)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 28APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic Location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Baseline		
N	17	15
Mean score	0.0198	0.0223
SD	0.00432	0.00606
Week 24		
N	16	14
LS mean change from baseline	-0.0013	-0.0006
SE	0.00087	0.00094
95% CI	(-0.0030, 0.0005)	(-0.0024, 0.0013)
LS mean difference (Omaveloxolone-Placebo)		0.0007
SE		0.00128
95% CI		(-0.0019, 0.0032)
p-value		0.5948
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.1875
95% CI		(-0.5313, 0.9063)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 28APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by Geographic Location (US, Other) - ITT Population

Geographic Location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Week 48		
N	16	14
LS mean change from baseline	-0.0014	-0.0003
SE	0.00092	0.00099
95% CI	(-0.0032, 0.0005)	(-0.0023, 0.0017)
LS mean difference (Omaveloxolone-Placebo)		0.0011
SE		0.00135
95% CI		(-0.0016, 0.0037)
p-value		0.4330
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.2767
95% CI		(-0.4440, 0.9974)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
NOTE 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-geo.sas **Data Tag:** FINAL **Run Date:** 28APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Y

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Baseline		
N	21	26
Mean score	0.0194	0.0201
SD	0.00864	0.00849
Week 24		
N	21	23
LS mean change from baseline	-0.0004	-0.0019
SE	0.00084	0.00080
95% CI	(-0.0021, 0.0013)	(-0.0035, -0.0002)
LS mean difference (Omaveloxolone-Placebo)		-0.0014
SE		0.00114
95% CI		(-0.0037, 0.0008)
p-value		0.2113
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.3673
95% CI		(-0.9638, 0.2293)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 30APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Y

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Week 48		
N	21	22
LS mean change from baseline	0.0007	-0.0022
SE	0.00088	0.00085
95% CI	(-0.0011, 0.0024)	(-0.0039, -0.0005)
LS mean difference (Omaveloxolone-Placebo)		-0.0029
SE		0.00120
95% CI		(-0.0053, -0.0005)
p-value		0.0180
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.7130
95% CI		(-1.3297, -0.0964)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 30APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: N

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Baseline		
N	22	15
Mean score	0.0218	0.0193
SD	0.00531	0.00778
Week 24		
N	22	14
LS mean change from baseline	-0.0007	-0.0014
SE	0.00082	0.00103
95% CI	(-0.0024, 0.0009)	(-0.0035, 0.0006)
LS mean difference (Omaveloxolone-Placebo)		-0.0007
SE		0.00127
95% CI		(-0.0032, 0.0018)
p-value		0.5791
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.1829
95% CI		(-0.8543, 0.4885)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 30APR2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: N

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Week 48		
N	22	14
LS mean change from baseline	-0.0008	0.0004
SE	0.00085	0.00107
95% CI	(-0.0025, 0.0009)	(-0.0018, 0.0025)
LS mean difference (Omaveloxolone-Placebo)		0.0012
SE		0.00133
95% CI		(-0.0015, 0.0038)
p-value		0.3727
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.2945
95% CI		(-0.3790, 0.9680)

NOTE 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-ndh-sgrp-gaa1.sas **Data Tag:** FINAL **Run Date:** 30APR2025

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	47	45
Mean score	0.14	0.13
SD	0.063	0.067
Week 24		
N	44	39
LS mean change from baseline	-0.01	0.00
SE	0.004	0.004
95% CI	(-0.02, 0.00)	(-0.01, 0.00)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.006
95% CI		(0.00, 0.02)
p-value		0.2028
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.28
95% CI		(-0.16, 0.71)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-ftwk-mmrm.sas **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 48		
N	43	37
LS mean change from baseline	-0.02	-0.02
SE	0.005	0.005
95% CI	(-0.03, -0.01)	(-0.03, -0.01)
LS mean difference (Omaveloxolone-Placebo)		0.00
SE		0.007
95% CI		(-0.01, 0.02)
p-value		0.5042
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.15
95% CI		(-0.29, 0.59)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-ftwk-mmrm.sas **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) at Week 48 : Treatment by Subgroup Interaction from MMRM Analysis - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Age (<18, >=18)	0.0431
Gender (female, male)	0.8665
GAA1 repeat length >=675 (yes, no)	0.2551
Geographic location (US, other)	0.2757
Pes Cavus status (yes, no)	0.9089

Note 1: P-value is based on the MMRM model for the ITT population with three way interaction added (subgroup*treatment*time).
Note 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-int.sas **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) at Week 48 by Gender (Female, Male) from MMRM Analysis - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Baseline		
N	16	28
Mean score	0.15	0.13
SD	0.064	0.067
Week 48		
N	15	25
LS mean change from baseline	-0.03	-0.02
SE	0.008	0.006
95% CI	(-0.05, -0.01)	(-0.03, -0.01)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.011
95% CI		(-0.01, 0.03)
p-value		0.3867
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.27
95% CI		(-0.37, 0.92)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-sgrp.sas:t-ftwk-mmrm-sgrp-gen.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) at Week 48 by Gender (Female, Male) from MMRM Analysis - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Baseline		
N	31	17
Mean score	0.13	0.14
SD	0.063	0.069
Week 48		
N	28	12
LS mean change from baseline	-0.02	-0.01
SE	0.006	0.009
95% CI	(-0.03, -0.01)	(-0.03, 0.01)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.011
95% CI		(-0.02, 0.03)
p-value		0.5563
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.20
95% CI		(-0.48, 0.87)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-sgrp.sas:t-ftwk-mmrm-sgrp-gen.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) at Week 48 by Geographic Location (US , Other) from MMRM Analysis - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Baseline		
N	30	31
Mean score	0.14	0.12
SD	0.066	0.070
Week 48		
N	28	24
LS mean change from baseline	-0.02	-0.02
SE	0.006	0.006
95% CI	(-0.03, -0.01)	(-0.03, -0.01)
LS mean difference (Omaveloxolone-Placebo)		0.00
SE		0.009
95% CI		(-0.02, 0.02)
p-value		0.8726
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.04
95% CI		(-0.59, 0.50)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-sgrp.sas:t-ftwk-mmrm-sgrp-geo.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) at Week 48 by Geographic Location (US , Other) from MMRM Analysis - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Baseline		
N	17	14
Mean score	0.13	0.16
SD	0.061	0.050
Week 48		
N	15	13
LS mean change from baseline	-0.02	-0.01
SE	0.008	0.009
95% CI	(-0.04, -0.01)	(-0.03, 0.01)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.012
95% CI		(-0.01, 0.04)
p-value		0.2112
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.46
95% CI		(-0.30, 1.21)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: Site was not included as a covariate in MMRM subgroup analysis of Geographic Location (US, Other) due to multicollinearity which leads to nonestimable LSMs.

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-sgrp.sas:t-ftwk-mmrm-sgrp-geo.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) at Week 48 by GAA1 Repeat Length >=675 (Yes, No) from MMRM Analysis - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Baseline		
N	16	24
Mean score	0.13	0.11
SD	0.067	0.070
Week 48		
N	14	19
LS mean change from baseline	-0.03	-0.02
SE	0.009	0.008
95% CI	(-0.04, -0.01)	(-0.03, 0.00)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.012
95% CI		(-0.01, 0.03)
p-value		0.4252
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.27
95% CI		(-0.42, 0.96)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-sgrp.sas:t-ftwk-mmrm-sgrp-gaa1.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) at Week 48 by GAA1 Repeat Length >=675 (Yes, No) from MMRM Analysis - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Baseline		
N	22	11
Mean score	0.15	0.14
SD	0.060	0.063
Week 48		
N	21	10
LS mean change from baseline	-0.02	-0.03
SE	0.007	0.011
95% CI	(-0.03, 0.00)	(-0.05, -0.01)
LS mean difference (Omaveloxolone-Placebo)		-0.01
SE		0.012
95% CI		(-0.03, 0.01)
p-value		0.4270
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.29
95% CI		(-1.05, 0.46)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-sgrp.sas:t-ftwk-mmrm-sgrp-gaa1.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Number of Falls from Baseline to Week 48 from Poisson Regression Model Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Total number of falls from baseline to week 48		
Mean	15.00	11.24
SD	23.666	18.975
Adjusted incidence rate of falls	0.05	0.04
95% CI	(0.03, 0.06)	(0.03, 0.05)
Rate ratio (Omaveloxolone/Placebo)		0.82
95% CI		(0.50, 1.34)
p-value		0.4249

Note 1: Incidence rate of falls and rate ratio are estimated from the Poisson model with Pes Cavus status as a covariate and the natural logarithm of time on study (days) as an offset term.

Source: biib141/valueaccess/amnog/t-fall-prm.sas **Data Tag:** FINAL **Run Date:** 14DEC2023

MOXie Part 2: Number of Falls from Baseline to Week 48 from Poisson Regression Model Analysis: Treatment by Subgroup Interaction - ITT Population

Subgroup	p-value for Treatment by Subgroup Interaction
Age (<18, >=18)	0.9447
Gender (female, male)	0.2778
GAA1 repeat length >=675 (yes, no)	0.1890
Geographic location (US, other)	0.9535
Pes Cavus status (yes, no)	0.2927

Note 1: P-value is based from the poisson regression model with interaction of subgroup and treatment added.

Source: biib141/valueaccess/amnog/t-fall-prm-int.sas **Data Tag:** FINAL **Run Date:** 14DEC2023

MOXie Part 2: Number of Falls from Baseline to Week 48 from Poisson Regression Model Analysis by Gender (Female, Male) - ITT Population

Gender: Female

	Placebo (N=17)	Omaveloxolone 150 mg (N=31)
Total number of falls from baseline to week 48		
Mean	14.24	8.71
SD	20.336	10.684
Adjusted incidence rate of falls	0.04	0.03
95% CI	(0.02, 0.08)	(0.02, 0.05)
Rate ratio (Omaveloxolone/Placebo)		0.64
95% CI		(0.29, 1.43)
p-value		0.2769

Note 1: Incidence rate of falls and rate ratio are estimated from the Poisson model with Pes Cavus status as a covariate and the natural logarithm of time on study (days) as an offset term.

Source: biib141/valueaccess/amnog/t-fall-prm-sgrp.sas:t-fall-prm-sgrp-gen.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Number of Falls from Baseline to Week 48 from Poisson Regression Model Analysis by Gender (Female, Male) - ITT Population

Gender: Male

	Placebo (N=35)	Omaveloxolone 150 mg (N=20)
Total number of falls from baseline to week 48		
Mean	15.37	15.15
SD	25.398	27.217
Adjusted incidence rate of falls	0.05	0.05
95% CI	(0.03, 0.07)	(0.03, 0.09)
Rate ratio (Omaveloxolone/Placebo)		1.14
95% CI		(0.59, 2.18)
p-value		0.6999

Note 1: Incidence rate of falls and rate ratio are estimated from the Poisson model with Pes Cavus status as a covariate and the natural logarithm of time on study (days) as an offset term.

Source: biib141/valueaccess/amnog/t-fall-prm-sgrp.sas:t-fall-prm-sgrp-gen.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Number of Falls from Baseline to Week 48 from Poisson Regression Model Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: US

	Placebo (N=35)	Omaveloxolone 150 mg (N=36)
Total number of falls from baseline to week 48		
Mean	16.14	11.69
SD	26.035	17.471
Adjusted incidence rate of falls	0.05	0.04
95% CI	(0.03, 0.07)	(0.03, 0.06)
Rate ratio (Omaveloxolone/Placebo)		0.82
95% CI		(0.46, 1.48)
p-value		0.5175

Note 1: Incidence rate of falls and rate ratio are estimated from the Poisson model with Pes Cavus status as a covariate and the natural logarithm of time on study (days) as an offset term.

Source: biib141/valueaccess/amnog/t-fall-prm-sgrp.sas:t-fall-prm-sgrp-geo.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Number of Falls from Baseline to Week 48 from Poisson Regression Model Analysis by Geographic Location (US, Other) - ITT Population

Geographic location: Other

	Placebo (N=17)	Omaveloxolone 150 mg (N=15)
Total number of falls from baseline to week 48		
Mean	12.65	10.13
SD	18.334	22.825
Adjusted incidence rate of falls	0.04	0.03
95% CI	(0.02, 0.08)	(0.02, 0.07)
Rate ratio (Omaveloxolone/Placebo)		0.80
95% CI		(0.30, 2.10)
p-value		0.6447

Note 1: Incidence rate of falls and rate ratio are estimated from the Poisson model with Pes Cavus status as a covariate and the natural logarithm of time on study (days) as an offset term.

Source: biib141/valueaccess/amnog/t-fall-prm-sgrp.sas:t-fall-prm-sgrp-geo.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Number of Falls from Baseline to Week 48 from Poisson Regression Model Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: Yes

	Placebo (N=21)	Omaveloxolone 150 mg (N=26)
Total number of falls from baseline to week 48		
Mean	19.95	10.81
SD	31.899	13.764
Adjusted incidence rate of falls	0.06	0.03
95% CI	(0.04, 0.09)	(0.02, 0.06)
Rate ratio (Omaveloxolone/Placebo)		0.60
95% CI		(0.30, 1.18)
p-value		0.1391

Note 1: Incidence rate of falls and rate ratio are estimated from the Poisson model with Pes Cavus status as a covariate and the natural logarithm of time on study (days) as an offset term.

Source: biib141/valueaccess/amnog/t-fall-prm-sgrp.sas:t-fall-prm-sgrp-gaa1.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Number of Falls from Baseline to Week 48 from Poisson Regression Model Analysis by GAA1 Repeat Length >=675 (Yes, No) - ITT Population

GAA1 repeat length >=675: No

	Placebo (N=22)	Omaveloxolone 150 mg (N=15)
Total number of falls from baseline to week 48		
Mean	10.41	11.87
SD	11.537	21.560
Adjusted incidence rate of falls	0.03	0.04
95% CI	(0.02, 0.06)	(0.02, 0.08)
Rate ratio (Omaveloxolone/Placebo)		1.26
95% CI		(0.52, 3.07)
p-value		0.6049

Note 1: Incidence rate of falls and rate ratio are estimated from the Poisson model with Pes Cavus status as a covariate and the natural logarithm of time on study (days) as an offset term.

Source: biib141/valueaccess/amnog/t-fall-prm-sgrp.sas:t-fall-prm-sgrp-gaa1.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: SF-36 Mental Component Summary (SF-36 MCS) LS Mean Change From Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	52	51
Mean score	50.22	51.61
SD	9.224	9.715
Week 24		
N	51	45
LS mean change from baseline	-0.06	-0.89
SE	1.031	1.100
95% CI	(-2.11, 1.99)	(-3.08, 1.30)
LS mean difference (Omaveloxolone-Placebo)		-0.83
SE		1.523
95% CI		(-3.86, 2.19)
p-value		0.5856
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.11
95% CI		(-0.51, 0.29)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm.sas:t-sf36-mmrm-mcs.rtf **Data Tag:** FINAL **Run Date:** 08DEC2023

MOXie Part 2: SF-36 Mental Component Summary (SF-36 MCS) LS Mean Change From Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 48		
N	51	44
LS mean change from baseline	-0.18	-1.19
SE	1.039	1.118
95% CI	(-2.24, 1.89)	(-3.41, 1.03)
LS mean difference (Omaveloxolone-Placebo)		-1.01
SE		1.542
95% CI		(-4.08, 2.05)
p-value		0.5132
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-0.54, 0.27)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm.sas:t-sf36-mmrm-mcs.rtf **Data Tag:** FINAL **Run Date:** 08DEC2023

MOXie Part 2: SF-36 Physical Component Summary (SF-36 PCS) LS Mean Change From Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Baseline		
N	52	51
Mean score	39.62	36.76
SD	8.323	8.207
Week 24		
N	51	45
LS mean change from baseline	-0.80	-0.13
SE	0.736	0.786
95% CI	(-2.27, 0.66)	(-1.69, 1.43)
LS mean difference (Omaveloxolone-Placebo)		0.67
SE		1.092
95% CI		(-1.50, 2.84)
p-value		0.5399
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.12
95% CI		(-0.28, 0.52)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm.sas:t-sf36-mmrm-pcs.rtf **Data Tag:** FINAL **Run Date:** 08DEC2023

MOXie Part 2: SF-36 Physical Component Summary (SF-36 PCS) LS Mean Change From Baseline up to Week 48 by Visit from MMRM Analysis - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Week 48		
N	51	44
LS mean change from baseline	-2.08	-1.33
SE	0.893	0.960
95% CI	(-3.85, -0.30)	(-3.23, 0.58)
LS mean difference (Omaveloxolone-Placebo)		0.75
SE		1.326
95% CI		(-1.89, 3.39)
p-value		0.5726
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.11
95% CI		(-0.29, 0.52)

Note 1: LS Mean and Hedges' g are based on the MMRM model that includes site, pes cavus status, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm.sas:t-sf36-mmrm-pcs.rtf **Data Tag:** FINAL **Run Date:** 08DEC2023

MOXie Part 2: Summary of Proportion of Worsening in SF-36 Mental Component Summary (SF-36 MCS) at Week 48 - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Number of subjects with week 48 assessment	51 (100)	44 (100)
Number of subjects with event (%)	3 (5.9)	3 (6.8)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		1.16
95% CI		(0.25, 5.42)
p-value		0.8547
Adjusted OR -Odds Ratio (Omaveloxolone/Placebo)		1.16
95% CI		(0.23, 5.99)
ARR -Absolute Risk Reduction(Omaveloxolone-Placebo)		0.01
95% CI		(-0.09, 0.11)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Worsening if change from baseline at week 48 <= -9.6 in SF-36 MCS.
Source: biib141/valueaccess/amnog/t-sf36-propw.sas:t-sf36-propw-mcs.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Summary of Proportion of Worsening in SF-36 Physical Component Summary (SF-36 PCS) at Week 48 - ITT Population

	Placebo (N=52)	Omaveloxolone 150 mg (N=51)
Number of subjects with week 48 assessment	51 (100)	44 (100)
Number of subjects with event (%)	4 (7.8)	3 (6.8)
Adjusted RR - Relative Risk (Omaveloxolone/Placebo)		0.89
95% CI		(0.21, 3.71)
p-value		0.8712
Adjusted OR -Odds Ratio (Omaveloxolone/Placebo)		0.88
95% CI		(0.18, 4.24)
ARR -Absolute Risk Reduction(Omaveloxolone-Placebo)		-0.01
95% CI		(-0.12, 0.09)

Note 1: Cochran-Mantel-Haenszel adjusted OR and RR are calculated adjusting for pes cavus status.
Note 2: Worsening if change from baseline at week 48 <= -9.4 in SF-36 PCS.
Source: biib141/valueaccess/amnog/t-sf36-propw.sas:t-sf36-propw-pcs.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Summary of Proportion of Worsening in SF-36 Mental Component Summary (SF-36 MCS) at Week 48: Treatment by Subgroup Interaction - ITT Population

Subgroup	p-value based on adjusted RR for Treatment by Subgroup Interaction
Age (<18, >=18)	NA
Gender (female, male)	NA
GAA1 repeat length >=675 (yes, no)	NA
Geographic location (US, other)	NA
Pes Cavus status (yes, no)	NA

Note 1: Cochran-Mantel-Haenszel adjusted RR is calculated adjusting for pes cavus status.
Note 2: Worsening if change from baseline at week 48 <= -9.6 in SF-36 MCS.
Note 3: NA indicates criteria not met for subgroup analysis

Source: biib141/valueaccess/amnog/t-sf36-propw-int.sas:t-sf36-propw-int-mcs.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: Summary of Proportion of Worsening in SF-36 Physical Component Summary (SF-36 PCS) at Week 48: Treatment by Subgroup Interaction - ITT Population

Subgroup	p-value based on adjusted RR for Treatment by Subgroup Interaction
Age (<18, >=18)	NA
Gender (female, male)	NA
GAA1 repeat length >=675 (yes, no)	NA
Geographic location (US, other)	NA
Pes Cavus status (yes, no)	NA

Note 1: Cochran-Mantel-Haenszel adjusted RR is calculated adjusting for pes cavus status.
Note 2: Worsening if change from baseline at week 48 <= -9.4 in SF-36 PCS.
Note 3: NA indicates criteria not met for subgroup analysis.

Source: biib141/valueaccess/amnog/t-sf36-propw-int.sas:t-sf36-propw-int-pcs.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

1. Anzahl der Patient*innen mit mindestens einem UE: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem UE					
n (%)	51 (100 %)	52 (100 %)	1,00 [0,963; 1,038] 0,9931	0,98 [0,019; 50,375] 0,9931	0,00 [0,000; 0,000] NA
Ja (%) Nein (%)	51 (100 %) 0 (0 %)	52 (100 %) 0 (0 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm					
Infektionen und parasitäre Erkrankungen (SOC)	**32 (63 %)**	**30 (58 %)**	**1,09 [0,794; 1,489] 0,6132**	**1,24 [0,560; 2,723] 0,6130**	**0,05 [-0,138; 0,239] 0,6123**
Nasopharyngitis (PT)	7 (14 %)	9 (17 %)	0,79 [0,320; 1,968] 0,6297	0,76 [0,260; 2,224] 0,6291	-0,04 [-0,175; 0,104] 0,6277
Infektion der oberen Atemwege (PT)	14 (27 %)	15 (29 %)	0,95 [0,513; 1,765] 0,8842	0,93 [0,395; 2,204] 0,8841	-0,01 [-0,188; 0,160] 0,8841
Stoffwechsel- und Ernährungsstörungen (SOC)	**8 (16 %)**	**3 (6 %)**	**2,72 [0,764; 9,676] 0,1225**	**3,04 [0,758; 12,184] 0,1167**	**0,10 [-0,019; 0,217] 0,0999**
Appetit vermindert (PT)	6 (12 %)	2 (4 %)	3,06 [0,647; 14,455] 0,1588	3,33 [0,640; 17,360] 0,1532	0,08 [-0,024; 0,182] 0,1309
Psychiatrische Erkrankungen (SOC)	**6 (12 %)**	**9 (17 %)**	**0,68 [0,261; 1,772] 0,4379**	**0,64 [0,209; 1,941] 0,4359**	**-0,06 [-0,191; 0,080] 0,4311**
Erkrankungen des Nervensystems (SOC)	**27 (53 %)**	**22 (42 %)**	**1,25 [0,831; 1,885] 0,2869**	**1,53 [0,705; 3,339] 0,2845**	**0,11 [-0,085; 0,298] 0,2808**
Schwindelgefühl (PT)	3 (6 %)	6 (12 %)	0,51 [0,135; 1,929] 0,3260	0,48 [0,113; 2,030] 0,3227	-0,06 [-0,165; 0,052] 0,3100
Kopfschmerzen (PT)	19 (37 %)	13 (25 %)	1,49 [0,826; 2,689] 0,1865	1,78 [0,764; 4,152] 0,1823	0,12 [-0,055; 0,300] 0,1766
Herzerkrankungen (SOC)	**5 (10 %)**	**7 (13 %)**	**0,73 [0,247; 2,146] 0,5770**	**0,70 [0,207; 2,364] 0,5761**	**-0,04 [-0,160; 0,087] 0,5735**

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Erkrankungen der Atemwege, des Brustraums und Mediastinums (SOC)	**20 (39 %)**	**14 (27 %)**	**1,46 [0,829; 2,558] 0,1919**	**1,75 [0,762; 4,022] 0,1878**	**0,12 [-0,057; 0,303] 0,1824**
Husten (PT)	6 (12 %)	4 (8 %)	1,53 [0,458; 5,102] 0,4994	1,60 [0,424; 6,043] 0,4981	0,04 [-0,074; 0,155] 0,4948
Schmerzen im Oropharynx (PT)	9 (18 %)	3 (6 %)	3,06 [0,878; 10,658] 0,0788	3,50 [0,889; 13,775] 0,0727	0,12 [-0,004; 0,241] 0,0566
Erkrankungen des Gastrointestinaltrakts (SOC)	**34 (67 %)**	**21 (40 %)**	**1,65 [1,125; 2,421] 0,0103**	**2,95 [1,322; 6,594] 0,0083**	**0,26 [0,077; 0,449] 0,0056**
Abdominalschmerz (PT)	11 (22 %)	3 (6 %)	3,74 [1,107; 12,622] 0,0334	4,49 [1,172; 17,209] 0,0281	0,16 [0,029; 0,287] 0,0166
Diarrhö (PT)	10 (20 %)	5 (10 %)	2,04 [0,749; 5,552] 0,1639	2,29 [0,724; 7,258] 0,1588	0,10 [-0,035; 0,235] 0,1480
Übelkeit (PT)	17 (33 %)	7 (13 %)	2,48 [1,123; 5,461] 0,0244	3,21 [1,199; 8,620] 0,0202	0,20 [0,040; 0,358] 0,0143
Erbrechen (PT)	8 (16 %)	6 (12 %)	1,36 [0,507; 3,642] 0,5526	1,43 [0,457; 4,448] 0,5517	0,04 [-0,091; 0,174] 0,5501
Erkrankungen der Haut und des Unterhautgewebes (SOC)	**16 (31 %)**	**7 (13 %)**	**2,33 [1,047; 5,186] 0,0378**	**2,94 [1,090; 7,924] 0,0329**	**0,18 [0,022; 0,337] 0,0257**
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**24 (47 %)**	**21 (40 %)**	**1,17 [0,750; 1,810] 0,5060**	**1,31 [0,601; 2,864] 0,5052**	**0,07 [-0,124; 0,258] 0,5039**
Arthralgie (PT)	9 (18 %)	10 (19 %)	0,92 [0,407; 2,070] 0,8468	0,90 [0,332; 2,439] 0,8467	-0,02 [-0,166; 0,134] 0,8466

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Rückenschmerzen (PT)	7 (14 %)	4 (8 %)	1,78 [0,556; 5,727] 0,3356	1,91 [0,523; 6,969] 0,3327	0,06 [-0,059; 0,179] 0,3253
Muskelspasmen (PT)	8 (16 %)	3 (6 %)	2,72 [0,764; 9,676] 0,1225	3,04 [0,758; 12,184] 0,1167	0,10 [-0,019; 0,217] 0,0999
Erkrankungen der Geschlechtsorgane und der Brustdrüse (SOC)	**7 (14 %)**	**0 (0 %)**	**15,29 [0,896; 260,899] 0,0592**	**17,70 [0,983; 318,559] 0,0510**	**0,14 [0,043; 0,232] 0,0044**
Allgemeine Erkrankungen und Beschwerden am Verabreichungsort (SOC)	**19 (37 %)**	**17 (33 %)**	**1,14 [0,672; 1,932] 0,6406**	**1,22 [0,543; 2,751] 0,6402**	**0,05 [-0,138; 0,230] 0,6398**
Ermüdung (PT)	11 (22 %)	7 (13 %)	1,60 [0,674; 3,807] 0,2894	1,77 [0,626; 4,996] 0,2861	0,08 [-0,065; 0,227] 0,2803
Untersuchungen (SOC)	**27 (53 %)**	**8 (15 %)**	**3,44 [1,730; 6,847] 0,0005**	**6,19 [2,435; 15,724] 0,0001**	**0,38 [0,207; 0,544] < 0,0001**
Alaninaminotransferase erhöht (PT)	19 (37 %)	1 (2 %)	19,37 [2,692; 139,392] 0,0033	30,28 [3,864; 237,321] 0,0012	0,35 [0,215; 0,491] < 0,0001
Aspartataminotransferase erhöht (PT)	11 (22 %)	1 (2 %)	11,22 [1,502; 83,735] 0,0183	14,03 [1,737; 113,227] 0,0131	0,20 [0,078; 0,315] 0,0012
Verletzung, Vergiftung und durch Eingriffe bedingte Komplikationen (SOC)	**33 (65 %)**	**32 (62 %)**	**1,05 [0,783; 1,413] 0,7521**	**1,15 [0,514; 2,553] 0,7521**	**0,03 [-0,155; 0,218] 0,7519**
Kontusion (PT)	17 (33 %)	19 (37 %)	0,91 [0,538; 1,547] 0,7463	0,87 [0,386; 1,954] 0,7461	-0,03 [-0,216; 0,152] 0,7459
Exkoriation (PT)	13 (25 %)	12 (23 %)	1,10 [0,558; 2,187] 0,7878	1,14 [0,463; 2,809] 0,7877	0,02 [-0,141; 0,190] 0,7876

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	**Placebo**			
N	**51**	**52**	**RR**	**OR**	**ARR**
Risswunde (PT)	8 (16 %)	8 (15 %)	1,02 [0,414; 2,509] 0,9694	1,02 [0,352; 2,972] 0,9694	0,00 [-0,137; 0,143] 0,9694
Bänderzerrung (PT)	5 (10 %)	8 (15 %)	0,64 [0,223; 1,818] 0,4069	0,60 [0,182; 1,968] 0,4046	-0,06 [-0,183; 0,072] 0,3983
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

1. Anzahl der Patient*innen mit mindestens einem UE: Interaktionstest

Anzahl der Patient*innen mit mindestens einem UE: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	NA
Region	NA
GAA1 Repeat-Länge	NA
Alter	NB
Pes cavus Status	NA

*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.*
NA: Nicht berechnet, da das Modell nicht konvergiert.
Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.

2. Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Interaktionstest

Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit SOC Erkrankungen des Gastrointestinaltrakts: Interaktionstest der Subgruppen

Subgruppe	**Interaktionstest**
Geschlecht	0,9758
Region	0,4401
GAA1 Repeat-Länge	0,5770
Alter	NB
Pes cavus Status	0,1625

*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.*
NA: Nicht berechnet, da das Modell nicht konvergiert.
Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.

Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit PT Abdominalschmerz: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	NB
Region	NB
GAA1 Repeat-Länge	NB
Alter	NB
Pes cavus Status	NB
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit PT Übelkeit: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	0,0160
Region	0,7127
GAA1 Repeat-Länge	0,7724
Alter	NB
Pes cavus Status	0,4973
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit SOC Erkrankungen der Haut und des Unterhautgewebes: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	0,4348
Region	0,6784
GAA1 Repeat-Länge	NB
Alter	NB
Pes cavus Status	0,7867
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit SOC Erkrankungen der Geschlechtsorgane und der Brustdrüse: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	NB
Region	NB
GAA1 Repeat-Länge	NB
Alter	NB
Pes cavus Status	NB
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit SOC Untersuchungen: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	0,3255
Region	0,1536
GAA1 Repeat-Länge	0,4214
Alter	NB
Pes cavus Status	0,6419
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit PT Alaninaminotransferase erhöht: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	0,3948
Region	0,1248
GAA1 Repeat-Länge	NB
Alter	NB
Pes cavus Status	0,4367
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit PT Aspartataminotransferase erhöht: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	NB
Region	NB
GAA1 Repeat-Länge	NB
Alter	NB
Pes cavus Status	NB
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

1. Anzahl der Patient*innen mit mindestens einem UE: Subgruppenanalyse Geschlecht

408-C-1402-PT2		Behandlungsarm	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem UE			
Geschlecht			
Weiblich	N	31	17
	Ereignisse, n (%)	31 (100 %)	17 (100 %)
	RR [95 %-KI] p-Wert	1,01 [0,926; 1,107] 0,7978	
Männlich	N	20	35
	Ereignisse, n (%)	20 (100 %)	35 (100 %)
	RR [95 %-KI] p-Wert	0,99 [0,916; 1,069] 0,8095	

Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.

2. Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Subgruppenanalyse Geschlecht

408-C-1402-PT2		**Behandlungsarm**	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm			
Geschlecht			
Erkrankungen des Gastrointestinaltrakts (SOC)			
Weiblich	N	31	17
	Ereignisse, n (%)	21 (68 %)	7 (41 %)
	RR [95 %-KI] p-Wert	1,65 [0,887; 3,052] 0,1142	
Männlich	N	20	35
	Ereignisse, n (%)	13 (65 %)	14 (40 %)
	RR [95 %-KI] p-Wert	1,62 [0,968; 2,727] 0,0657	
Erkrankungen des Gastrointestinaltrakts: Übelkeit (PT)			
Weiblich	N	31	17
	Ereignisse, n (%)	16 (52 %)	2 (12 %)
	RR [95 %-KI] p-Wert	4,39 [1,142; 16,850] 0,0310	
Männlich	N	20	35
	Ereignisse, n (%)	1 (5 %)	5 (14 %)
	RR [95 %-KI] p-Wert	0,35 [0,044; 2,789] 0,3264	
Erkrankungen der Haut und des Unterhautgewebes (SOC)			
Weiblich	N	31	17
	Ereignisse, n (%)	11 (35 %)	4 (24 %)
	RR [95 %-KI] p-Wert	1,51 [0,566; 4,017] 0,4188	
Männlich	N	20	35
	Ereignisse, n (%)	5 (25 %)	3 (9 %)
	RR [95 %-KI] p-Wert	2,92 [0,778; 10,937] 0,1123	

408-C-1402-PT2		Behandlungsarm	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm			
Untersuchungen (SOC)			
Weiblich	N	31	17
	Ereignisse, n (%)	14 (45 %)	1 (6 %)
	RR [95 %-KI] p-Wert	7,68 [1,103; 53,458] 0,0392	
Männlich	N	20	35
	Ereignisse, n (%)	13 (65 %)	7 (20 %)
	RR [95 %-KI] p-Wert	3,25 [1,556; 6,788] 0,0018	
Untersuchungen: Alaninaminotransferase erhöht (PT)			
Weiblich	N	31	17
	Ereignisse, n (%)	9 (29 %)	0 (0 %)
	RR [95 %-KI] p-Wert	10,69 [0,660; 173,061] 0,0951	
Männlich	N	20	35
	Ereignisse, n (%)	10 (50 %)	1 (3 %)
	RR [95 %-KI] p-Wert	17,50 [2,414; 126,856] 0,0047	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

1. Anzahl der Patient*innen mit mindestens einem UE: Subgruppenanalyse Region

408-C-1402-PT2		Behandlungsarm	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem UE			
Region			
USA	N	36	35
	Ereignisse, n (%)	36 (100 %)	35 (100 %)
	RR [95 %-KI] p-Wert	1,00 [0,948; 1,056] 0,9901	
Andere	N	15	17
	Ereignisse, n (%)	15 (100 %)	17 (100 %)
	RR [95 %-KI] p-Wert	1,00 [0,886; 1,121] 0,9568	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

1. Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Subgruppenanalyse Region

408-C-1402-PT2		**Behandlungsarm**	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm			
Region			
Erkrankungen des Gastrointestinaltrakts (SOC)			
USA	N	36	35
	Ereignisse, n (%)	26 (72 %)	14 (40 %)
	RR [95 %-KI] p-Wert	1,81 [1,147; 2,842] 0,0106	
Andere	N	15	17
	Ereignisse, n (%)	8 (53 %)	7 (41 %)
	RR [95 %-KI] p-Wert	1,30 [0,618; 2,713] 0,5030	
Erkrankungen des Gastrointestinaltrakts: Übelkeit (PT)			
USA	N	36	35
	Ereignisse, n (%)	14 (39 %)	6 (17 %)
	RR [95 %-KI] p-Wert	2,27 [0,984; 5,231] 0,0543	
Andere	N	15	17
	Ereignisse, n (%)	3 (20 %)	1 (6 %)
	RR [95 %-KI] p-Wert	3,40 [0,394; 29,307] 0,2687	
Erkrankungen der Haut und des Unterhautgewebes (SOC)			
USA	N	36	35
	Ereignisse, n (%)	13 (36 %)	5 (14 %)
	RR [95 %-KI] p-Wert	2,53 [1,007; 6,346] 0,0479	
Andere	N	15	17
	Ereignisse, n (%)	3 (20 %)	2 (12 %)
	RR [95 %-KI] p-Wert	1,70 [0,327; 8,843] 0,5392	
Untersuchungen (SOC)			

408-C-1402-PT2		Behandlungsarm	
		Omaveloxolon	Placebo
Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm			
USA	N	36	35
	Ereignisse, n (%)	15 (42 %)	6 (17 %)
	RR [95 %-KI] p-Wert	2,43 [1,066; 5,544] 0,0345	
Andere	N	15	17
	Ereignisse, n (%)	12 (80 %)	2 (12 %)
	RR [95 %-KI] p-Wert	6,80 [1,805; 25,613] 0,0047	
Untersuchungen: Alaninaminotransferase erhöht (PT)			
USA	N	36	35
	Ereignisse, n (%)	9 (25 %)	1 (3 %)
	RR [95 %-KI] p-Wert	8,75 [1,169; 65,495] 0,0344	
Andere	N	15	17
	Ereignisse, n (%)	10 (67 %)	0 (0 %)
	RR [95 %-KI] p-Wert	23,62 [1,501; 371,763] 0,0243	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

1. Anzahl der Patient*innen mit mindestens einem UE: Subgruppenanalyse GAA1 Repeat-Länge

408-C-1402-PT2		**Behandlungsarm**	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem UE			
GAA1 Repeat-Länge			
>=675	N	26	21
	Ereignisse, n (%)	26 (100 %)	21 (100 %)
	RR [95 %-KI] p-Wert	1,00 [0,925; 1,090] 0,9251	
<675	N	15	22
	Ereignisse, n (%)	15 (100 %)	22 (100 %)
	RR [95 %-KI] p-Wert	0,99 [0,890; 1,102] 0,8680	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

2. Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Subgruppenanalyse GAA1 Repeat-Länge

408-C-1402-PT2		**Behandlungsarm**	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm			
GAA1 Repeat-Länge			
Erkrankungen des Gastrointestinaltrakts (SOC)			
>=675	N	26	21
	Ereignisse, n (%)	20 (77 %)	10 (48 %)
	RR [95 %-KI] p-Wert	1,62 [0,984; 2,651] 0,0574	
<675	N	15	22
	Ereignisse, n (%)	8 (53 %)	6 (27 %)
	RR [95 %-KI] p-Wert	1,96 [0,852; 4,487] 0,1134	
Erkrankungen des Gastrointestinaltrakts: Übelkeit (PT)			
>=675	N	26	21
	Ereignisse, n (%)	11 (42 %)	5 (24 %)
	RR [95 %-KI] p-Wert	1,78 [0,732; 4,314] 0,2056	
<675	N	15	22
	Ereignisse, n (%)	2 (13 %)	1 (5 %)
	RR [95 %-KI] p-Wert	2,93 [0,291; 29,522] 0,3671	
Untersuchungen (SOC)			
>=675	N	26	21
	Ereignisse, n (%)	12 (46 %)	4 (19 %)
	RR [95 %-KI] p-Wert	2,42 [0,914; 6,421] 0,0747	
<675	N	15	22
	Ereignisse, n (%)	9 (60 %)	2 (9 %)
	RR [95 %-KI] p-Wert	6,60 [1,653; 26,353] 0,0076	

408-C-1402-PT2	Behandlungsarm	
	Omaveloxolon	Placebo
Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm		
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.		

1. Anzahl der Patient*innen mit mindestens einem milden UE: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem milden UE					
n (%)	51 (100 %)	52 (100 %)	1,04 [0,973; 1,110] 0,2509	5,10 [0,239; 108,866] 0,3011	0,04 [-0,014; 0,091] 0,1497
Ja (%) Nein (%)	51 (100 %) 0 (0 %)	50 (96 %) 2 (4 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm					
Infektionen und parasitäre Erkrankungen (SOC)	**31 (61 %)**	**29 (56 %)**	**1,09 [0,786; 1,512] 0,6187**	**1,23 [0,561; 2,694] 0,6185**	**0,05 [-0,140; 0,240] 0,6178**
Nasopharyngitis (PT)	6 (12 %)	9 (17 %)	0,68 [0,261; 1,772] 0,4379	0,64 [0,209; 1,941] 0,4359	-0,06 [-0,191; 0,080] 0,4311
Infektion der oberen Atemwege (PT)	14 (27 %)	13 (25 %)	1,10 [0,574; 2,100] 0,7899	1,14 [0,471; 2,733] 0,7898	0,02 [-0,145; 0,194] 0,7897
Stoffwechsel- und Ernährungsstörungen (SOC)	**8 (16 %)**	**3 (6 %)**	**2,72 [0,764; 9,676] 0,1225**	**3,04 [0,758; 12,184] 0,1167**	**0,10 [-0,019; 0,217] 0,0999**
Appetit vermindert (PT)	6 (12 %)	2 (4 %)	3,06 [0,647; 14,455] 0,1588	3,33 [0,640; 17,360] 0,1532	0,08 [-0,024; 0,182] 0,1309
Psychiatrische Erkrankungen (SOC)	**4 (8 %)**	**8 (15 %)**	**0,51 [0,164; 1,588] 0,2479**	**0,47 [0,132; 1,665] 0,2434**	**-0,08 [-0,198; 0,047] 0,2306**
Erkrankungen des Nervensystems (SOC)	**25 (49 %)**	**19 (37 %)**	**1,34 [0,852; 2,114] 0,2068**	**1,67 [0,760; 3,670] 0,2033**	**0,12 [-0,065; 0,314] 0,1984**
Kopfschmerzen (PT)	18 (35 %)	12 (23 %)	1,53 [0,823; 2,843] 0,1802	1,82 [0,767; 4,313] 0,1758	0,12 [-0,052; 0,296] 0,1698
Herzerkrankungen (SOC)	**2 (4 %)**	**6 (12 %)**	**0,34 [0,072; 1,606] 0,1741**	**0,31 [0,060; 1,630] 0,1684**	**-0,08 [-0,178; 0,026] 0,1431**
Erkrankungen der Atemwege, des Brustraums und	**18 (35 %)**	**14 (27 %)**	**1,31 [0,733; 2,346] 0,3680**	**1,48 [0,639; 3,429] 0,3659**	**0,08 [-0,094; 0,262] 0,3631**

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Mediastinums (SOC)					
Schmerzen im Oropharynx (PT)	8 (16 %)	3 (6 %)	2,72 [0,764; 9,676] 0,1225	3,04 [0,758; 12,184] 0,1167	0,10 [-0,019; 0,217] 0,0999
Erkrankungen des Gastrointestinaltrakts (SOC)	**34 (67 %)**	**20 (38 %)**	**1,73 [1,168; 2,572] 0,0063**	**3,20 [1,428; 7,171] 0,0048**	**0,28 [0,097; 0,467] 0,0029**
Abdominalschmerz (PT)	10 (20 %)	3 (6 %)	3,40 [0,992; 11,640] 0,0511	3,98 [1,027; 15,448] 0,0453	0,14 [0,012; 0,264] 0,0312
Diarrhö (PT)	9 (18 %)	4 (8 %)	2,29 [0,754; 6,980] 0,1439	2,57 [0,738; 8,961] 0,1384	0,10 [-0,028; 0,227] 0,1253
Übelkeit (PT)	14 (27 %)	7 (13 %)	2,04 [0,897; 4,634] 0,0886	2,43 [0,889; 6,653] 0,0830	0,14 [-0,014; 0,294] 0,0740
Erbrechen (PT)	6 (12 %)	6 (12 %)	1,02 [0,352; 2,954] 0,9742	1,02 [0,307; 3,407] 0,9742	0,00 [-0,122; 0,126] 0,9742
Erkrankungen der Haut und des Unterhautgewebes (SOC)	**14 (27 %)**	**7 (13 %)**	**2,04 [0,897; 4,634] 0,0886**	**2,43 [0,889; 6,653] 0,0830**	**0,14 [-0,014; 0,294] 0,0740**
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**21 (41 %)**	**20 (38 %)**	**1,07 [0,666; 1,722] 0,7909**	**1,12 [0,509; 2,466] 0,7908**	**0,03 [-0,162; 0,216] 0,7907**
Arthralgie (PT)	8 (16 %)	10 (19 %)	0,82 [0,350; 1,900] 0,6497	0,78 [0,281; 2,172] 0,6491	-0,04 [-0,182; 0,111] 0,6480
Rückenschmerzen (PT)	6 (12 %)	3 (6 %)	2,04 [0,539; 7,718] 0,2981	2,18 [0,514; 9,227] 0,2947	0,06 [-0,049; 0,169] 0,2837
Muskelspasmen (PT)	6 (12 %)	3 (6 %)	2,04 [0,539; 7,718] 0,2981	2,18 [0,514; 9,227] 0,2947	0,06 [-0,049; 0,169] 0,2837

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Erkrankungen der Geschlechtsorgane und der Brustdrüse (SOC)	**7 (14 %)**	**0 (0 %)**	**15,29 [0,896; 260,899] 0,0592**	**17,70 [0,983; 318,559] 0,0510**	**0,14 [0,043; 0,232] 0,0044**
Allgemeine Erkrankungen und Beschwerden am Verabreichungsort (SOC)	**15 (29 %)**	**16 (31 %)**	**0,96 [0,530; 1,723] 0,8896**	**0,94 [0,404; 2,177] 0,8895**	**-0,01 [-0,191; 0,164] 0,8895**
Ermüdung (PT)	8 (16 %)	6 (12 %)	1,36 [0,507; 3,642] 0,5526	1,43 [0,457; 4,448] 0,5517	0,04 [-0,091; 0,174] 0,5501
Untersuchungen (SOC)	**21 (41 %)**	**6 (12 %)**	**3,57 [1,570; 8,110] 0,0024**	**5,37 [1,941; 14,841] 0,0013**	**0,30 [0,136; 0,457] 0,0003**
Alaninaminotransferase erhöht (PT)	14 (27 %)	1 (2 %)	14,27 [1,948; 104,598] 0,0089	19,30 [2,429; 153,301] 0,0052	0,26 [0,127; 0,383] 0,0001
Aspartataminotransferase erhöht (PT)	10 (20 %)	1 (2 %)	10,20 [1,354; 76,785] 0,0240	12,44 [1,529; 101,208] 0,0183	0,18 [0,062; 0,292] 0,0027
Verletzung, Vergiftung und durch Eingriffe bedingte Komplikationen (SOC)	**32 (63 %)**	**31 (60 %)**	**1,05 [0,774; 1,432] 0,7575**	**1,14 [0,516; 2,522] 0,7575**	**0,03 [-0,157; 0,219] 0,7573**
Kontusion (PT)	17 (33 %)	18 (35 %)	0,96 [0,562; 1,650] 0,8992	0,94 [0,418; 2,135] 0,8991	-0,01 [-0,196; 0,170] 0,8991
Exkoriation (PT)	13 (25 %)	12 (23 %)	1,10 [0,558; 2,187] 0,7878	1,14 [0,463; 2,809] 0,7877	0,02 [-0,141; 0,190] 0,7876
Risswunde (PT)	6 (12 %)	8 (15 %)	0,76 [0,285; 2,049] 0,6060	0,73 [0,235; 2,286] 0,6052	-0,04 [-0,168; 0,096] 0,6033
Bänderzerrung (PT)	5 (10 %)	6 (12 %)	0,85 [0,277; 2,610] 0,7885	0,83 [0,238; 2,924] 0,7883	-0,02 [-0,137; 0,102] 0,7879

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	51	52	RR	OR	ARR
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

1. Anzahl der Patient*innen mit mindestens einem milden UE: Interaktionstest

Anzahl der Patient*innen mit mindestens einem milden UE: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	0,0385
Region	0,3361
GAA1 Repeat-Länge	0,8087
Alter	NB
Pes cavus Status	0,4935

*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.*
NA: Nicht berechnet, da das Modell nicht konvergiert.
Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.

2. Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Interaktionstest

Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit SOC Erkrankungen des Gastrointestinaltrakts: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	0,8815
Region	0,3539
GAA1 Repeat-Länge	0,4252
Alter	NB
Pes cavus Status	0,1287
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit PT Abdominalschmerz: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	NB
Region	NB
GAA1 Repeat-Länge	NB
Alter	NB
Pes cavus Status	NB
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit SOC Erkrankungen der Geschlechtsorgane und der Brustdrüse: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	NB
Region	NB
GAA1 Repeat-Länge	NB
Alter	NB
Pes cavus Status	NB
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit SOC Untersuchungen: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	0,4529
Region	0,0744
GAA1 Repeat-Länge	NB
Alter	NB
Pes cavus Status	0,0442
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit PT Alaninaminotransferase erhöht: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	NB
Region	NB
GAA1 Repeat-Länge	NB
Alter	NB
Pes cavus Status	0,3722
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm mit PT Aspartataminotransferase erhöht: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	NB
Region	NB
GAA1 Repeat-Länge	NB
Alter	NB
Pes cavus Status	NB
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

1. Anzahl der Patient*innen mit mindestens einem milden UE: Subgruppenanalyse Geschlecht

408-C-1402-PT2		Behandlungsarm	
		Omaveloxolon	Placebo
Anzahl der Patient*innen mit mindestens einem milden UE			
Geschlecht			
Weiblich	N	31	17
	Ereignisse, n (%)	31 (100 %)	15 (88 %)
	RR [95 %-KI] p-Wert	1,14 [0,945; 1,383] 0,1697	
Männlich	N	20	35
	Ereignisse, n (%)	20 (100 %)	35 (100 %)
	RR [95 %-KI] p-Wert	0,99 [0,916; 1,069] 0,8095	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

2. Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Subgruppenanalyse Geschlecht

408-C-1402-PT2		Behandlungsarm	
		Omaveloxolon	Placebo
Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm			
Geschlecht			
Erkrankungen des Gastrointestinaltrakts (SOC)			
Weiblich	N	31	17
	Ereignisse, n (%)	21 (68 %)	7 (41 %)
	RR [95 %-KI] p-Wert	1,65 [0,887; 3,052] 0,1142	
Männlich	N	20	35

408-C-1402-PT2		Behandlungsarm	
		Omaveloxolon	Placebo
Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm			
	Ereignisse, n (%)	13 (65 %)	13 (37 %)
	RR [95 %-KI] p-Wert	1,75 [1,022; 2,996] 0,0410	
Untersuchungen (SOC)			
Weiblich	N	31	17
	Ereignisse, n (%)	12 (39 %)	1 (6 %)
	RR [95 %-KI] p-Wert	6,58 [0,934; 46,362] 0,0582	
Männlich	N	20	35
	Ereignisse, n (%)	9 (45 %)	5 (14 %)
	RR [95 %-KI] p-Wert	3,15 [1,224; 8,106] 0,0172	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

1. Anzahl der Patient*innen mit mindestens einem milden UE: Subgruppenanalyse Region

408-C-1402-PT2		**Behandlungsarm**	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem milden UE			
Region			
USA	N	36	35
	Ereignisse, n (%)	36 (100 %)	33 (94 %)
	RR [95 %-KI] p-Wert	1,06 [0,962; 1,168] 0,2401	
Andere	N	15	17
	Ereignisse, n (%)	15 (100 %)	17 (100 %)
	RR [95 %-KI] p-Wert	1,00 [0,886; 1,121] 0,9568	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

2. Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Subgruppenanalyse Region

408-C-1402-PT2		**Behandlungsarm**	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm			
Region			
Erkrankungen des Gastrointestinaltrakts (SOC)			
USA	N	36	35
	Ereignisse, n (%)	26 (72 %)	13 (37 %)
	RR [95 %-KI] p-Wert	1,94 [1,208; 3,131] 0,0062	
Andere	N	15	17
	Ereignisse, n (%)	8 (53 %)	7 (41 %)
	RR [95 %-KI] p-Wert	1,30 [0,618; 2,713] 0,5030	
Untersuchungen (SOC)			
USA	N	36	35
	Ereignisse, n (%)	11 (31 %)	5 (14 %)
	RR [95 %-KI] p-Wert	2,14 [0,828; 5,526] 0,1164	
Andere	N	15	17
	Ereignisse, n (%)	10 (67 %)	1 (6 %)
	RR [95 %-KI] p-Wert	11,33 [1,637; 78,459] 0,0139	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

1. Anzahl der Patient*innen mit mindestens einem milden UE: Subgruppenanalyse GAA1 Repeat-Länge

408-C-1402-PT2		**Behandlungsarm**	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem milden UE			
GAA1 Repeat-Länge			
>=675	N	26	21
	Ereignisse, n (%)	26 (100 %)	20 (95 %)
	RR [95 %-KI] p-Wert	1,05 [0,930; 1,193] 0,4208	
<675	N	15	22
	Ereignisse, n (%)	15 (100 %)	21 (95 %)
	RR [95 %-KI] p-Wert	1,04 [0,902; 1,191] 0,6281	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

2. Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Subgruppenanalyse GAA1 Repeat-Länge

408-C-1402-PT2		**Behandlungsarm**	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm			
GAA1 Repeat-Länge			
Erkrankungen des Gastrointestinaltrakts (SOC)			
>=675	N	26	21
	Ereignisse, n (%)	20 (77 %)	10 (48 %)
	RR [95 %-KI] p-Wert	1,62 [0,984; 2,651] 0,0574	
<675	N	15	22
	Ereignisse, n (%)	8 (53 %)	5 (23 %)
	RR [95 %-KI] p-Wert	2,35 [0,950; 5,797] 0,0641	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

1. Anzahl der Patient*innen mit mindestens einem moderaten UE: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem moderaten UE					
n (%)	51 (100 %)	52 (100 %)	1,59 [1,013; 2,484] 0,0436	2,30 [1,039; 5,088] 0,0395	0,20 [0,015; 0,391] 0,0342
Ja (%) Nein (%)	28 (55 %) 23 (45 %)	18 (35 %) 34 (65 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem moderaten UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem moderaten UE bei mindestens 10 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm					
Infektionen und parasitäre Erkrankungen (SOC)	**9 (18 %)**	**4 (8 %)**	**2,29 [0,754; 6,980] 0,1439**	**2,57 [0,738; 8,961] 0,1384**	**0,10 [-0,028; 0,227] 0,1253**
Erkrankungen des Nervensystems (SOC)	**4 (8 %)**	**6 (12 %)**	**0,68 [0,204; 2,268] 0,5410**	**0,65 [0,173; 2,464] 0,5398**	**-0,04 [-0,151; 0,077] 0,5359**
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**6 (12 %)**	**1 (2 %)**	**6,12 [0,763; 49,040] 0,0878**	**6,80 [0,788; 58,647] 0,0808**	**0,10 [0,002; 0,194] 0,0441**
Untersuchungen (SOC)	**8 (16 %)**	**2 (4 %)**	**4,08 [0,910; 18,289] 0,0660**	**4,65 [0,937; 23,088] 0,0597**	**0,12 [0,006; 0,231] 0,0391**
Verletzung, Vergiftung und durch Eingriffe bedingte Komplikationen (SOC)	**5 (10 %)**	**6 (12 %)**	**0,85 [0,277; 2,610] 0,7885**	**0,83 [0,238; 2,924] 0,7883**	**-0,02 [-0,137; 0,102] 0,7879**

Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.

1. Anzahl der Patient*innen mit mindestens einem moderaten UE: Interaktionstest

Anzahl der Patient*innen mit mindestens einem moderaten UE: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Geschlecht	0,3855
Region	0,8881
GAA1 Repeat-Länge	0,7235
Alter	NB
Pes cavus Status	0,0503

*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.*
NA: Nicht berechnet, da das Modell nicht konvergiert.
Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.

1. Anzahl der Patient*innen mit mindestens einem moderaten UE: Subgruppenanalyse Geschlecht

408-C-1402-PT2		Behandlungsarm	
		Omaveloxolon	Placebo
Anzahl der Patient*innen mit mindestens einem moderaten UE			
Geschlecht			
Weiblich	N	31	17
	Ereignisse, n (%)	19 (61 %)	9 (53 %)
	RR [95 %-KI] p-Wert	1,16 [0,683; 1,964] 0,5991	
Männlich	N	20	35
	Ereignisse, n (%)	9 (45 %)	9 (26 %)
	RR [95 %-KI] p-Wert	1,75 [0,833; 3,678] 0,1401	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

1. Anzahl der Patient*innen mit mindestens einem moderaten UE: Subgruppenanalyse Region

408-C-1402-PT2		Behandlungsarm	
		Omaveloxolon	Placebo
Anzahl der Patient*innen mit mindestens einem moderaten UE			
Region			
USA	N	36	35
	Ereignisse, n (%)	20 (56 %)	12 (34 %)
	RR [95 %-KI] p-Wert	1,62 [0,941; 2,791] 0,0816	
Andere	N	15	17
	Ereignisse, n (%)	8 (53 %)	6 (35 %)
	RR [95 %-KI] p-Wert	1,51 [0,680; 3,360] 0,3157	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

1. Anzahl der Patient*innen mit mindestens einem moderaten UE: Subgruppenanalyse GAA1 Repeat-Länge

408-C-1402-PT2		**Behandlungsarm**	
		Omaveloxolon	**Placebo**
Anzahl der Patient*innen mit mindestens einem moderaten UE			
GAA1 Repeat-Länge			
>=675	N	26	21
	Ereignisse, n (%)	13 (50 %)	8 (38 %)
	RR [95 %-KI] p-Wert	1,31 [0,674; 2,557] 0,4324	
<675	N	15	22
	Ereignisse, n (%)	7 (47 %)	5 (23 %)
	RR [95 %-KI] p-Wert	2,05 [0,801; 5,264] 0,1344	
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.			

1. Anzahl der Patient*innen mit mindestens einem schweren UE

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem schweren UE					
n (%)	51 (100 %)	52 (100 %)	11,21 [0,636; 197,673] 0,0986	12,42 [0,669; 230,695] 0,0907	0,10 [0,016; 0,180] 0,0184
Ja (%) Nein (%)	5 (10 %) 46 (90 %)	0 (0 %) 52 (100 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem schweren UE: Interaktionstest

Anzahl der Patient*innen mit mindestens einem schweren UE: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Keine Subgruppenanalysen durchgeführt.	
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

1. Anzahl der Patient*innen mit mindestens einem SUE

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem SUE					
n (%)	51 (100 %)	52 (100 %)	1,70 [0,428; 6,743] 0,4597	1,78 [0,401; 7,853] 0,4581	0,04 [-0,063; 0,144] 0,4528
Ja (%) Nein (%)	5 (10 %) 46 (90 %)	3 (6 %) 49 (94 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem SUE bei mindestens 5 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem SUE bei mindestens 5 % der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in mind. einem Arm					
Herzerkrankungen (SOC)	**3 (6 %)**	**1 (2 %)**	**3,06 [0,329; 28,446] 0,3308**	**3,19 [0,320; 31,705] 0,3276**	**0,04 [-0,035; 0,114] 0,3023**
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

1. Anzahl der Patient*innen mit mindestens einem SUE: Interaktionstest

Anzahl der Patient*innen mit mindestens einem SUE: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Keine Subgruppenanalysen durchgeführt.	
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

1. Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**51**	**52**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE					
n (%)	51 (100 %)	52 (100 %)	2,04 [0,391; 10,648] 0,4054	2,13 [0,372; 12,164] 0,4032	0,04 [-0,050; 0,130] 0,3932
Ja (%) Nein (%)	4 (8 %) 47 (92 %)	2 (4 %) 50 (96 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE nach allen SOC und PT – deskriptive Darstellung, n(%)

408-C-1402-PT2	Behandlungsarm	
	Omaveloxolon	**Placebo**
N	**51**	**52**
Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE nach allen SOC und PT – deskriptive Darstellung, n(%)		
Herzerkrankungen (SOC)	**1 (2 %)**	**1 (2 %)**
Vorhofflimmern (PT)	0 (0 %)	1 (2 %)
Tachykardie ventrikulär (PT)	1 (2 %)	0 (0 %)
Erkrankungen der Haut und des Unterhautgewebes (SOC)	**1 (2 %)**	**1 (2 %)**
Erythrose (PT)	0 (0 %)	1 (2 %)
Rosazea (PT)	1 (2 %)	0 (0 %)
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**1 (2 %)**	**0 (0 %)**
Muskelspasmen (PT)	1 (2 %)	0 (0 %)
Untersuchungen (SOC)	**1 (2 %)**	**0 (0 %)**
Alaninaminotransferase erhöht (PT)	1 (2 %)	0 (0 %)
Aspartataminotransferase erhöht (PT)	1 (2 %)	0 (0 %)
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.		

1. Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE: Interaktionstest

Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE: Interaktionstest der Subgruppen	
Subgruppe	**Interaktionstest**
Keine Subgruppenanalysen durchgeführt.	
*NB: Nicht berechnet, da die Subgruppe entweder zu wenige Patient*innen oder zu wenige Ereignisse enthält.* *NA: Nicht berechnet, da das Modell nicht konvergiert.* *Bei Interaktionstests wurde die Interaktion zwischen Subgruppenmerkmal und Behandlung zum Modell hinzugefügt und der entsprechende p-Wert angegeben.*	

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline at Week 48 by Pes Cavus Status (Yes, No) from MMRM Analysis - ITT Population

Pes Cavus status: Yes

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	34.33	41.09
SD	9.353	9.952
Week 48		
N	9	8
LS mean change from baseline	0.35	1.23
SE	1.400	1.458
95% CI	(-2.43, 3.14)	(-1.67, 4.13)
LS mean difference (Omaveloxolone-Placebo)		0.88
SE		2.005
95% CI		(-3.11, 4.86)
p-value		0.6633
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.20
95% CI		(-0.76, 1.15)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp-itt.sas:t-mfars93-mmrm-sgrp-itt-pcavus.rtf **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline at Week 48 by Pes Cavus Status (Yes, No) from MMRM Analysis - ITT Population

Pes Cavus status: No

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	38.63	40.57
SD	11.029	10.330
Week 48		
N	41	34
LS mean change from baseline	0.95	-1.58
SE	0.664	0.715
95% CI	(-0.37, 2.27)	(-3.00, -0.16)
LS mean difference (Omaveloxolone-Placebo)		-2.53
SE		0.984
95% CI		(-4.48, -0.57)
p-value		0.0118
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.58
95% CI		(-1.05, -0.12)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp-itt.sas:t-mfars93-mmrm-sgrp-itt-pcavus.rtf **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	0.67	0.76
SD	0.666	0.476
Week 4		
N	42	40
LS mean change from baseline	-0.06	-0.05
SE	0.049	0.050
95% CI	(-0.16, 0.04)	(-0.15, 0.05)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.070
95% CI		(-0.13, 0.15)
p-value		0.9163
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.02
95% CI		(-0.41, 0.46)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-bul-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 12		
N	42	40
LS mean change from baseline	0.02	-0.05
SE	0.043	0.045
95% CI	(-0.07, 0.11)	(-0.14, 0.04)
LS mean difference (Omaveloxolone-Placebo)		-0.07
SE		0.063
95% CI		(-0.19, 0.06)
p-value		0.2838
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.23
95% CI		(-0.67, 0.20)
Week 18		
N	41	38
LS mean change from baseline	0.02	-0.02
SE	0.041	0.042
95% CI	(-0.06, 0.10)	(-0.11, 0.06)
LS mean difference (Omaveloxolone-Placebo)		-0.04
SE		0.060
95% CI		(-0.16, 0.08)
p-value		0.4705
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.16
95% CI		(-0.60, 0.28)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-bul-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 24		
N	41	36
LS mean change from baseline	0.09	-0.06
SE	0.043	0.046
95% CI	(0.00, 0.17)	(-0.15, 0.03)
LS mean difference (Omaveloxolone-Placebo)		-0.15
SE		0.064
95% CI		(-0.27, -0.02)
p-value		0.0241
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.51
95% CI		(-0.97, -0.06)
Week 36		
N	41	35
LS mean change from baseline	0.04	-0.07
SE	0.052	0.056
95% CI	(-0.06, 0.15)	(-0.18, 0.04)
LS mean difference (Omaveloxolone-Placebo)		-0.11
SE		0.077
95% CI		(-0.26, 0.04)
p-value		0.1529
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.32
95% CI		(-0.78, 0.13)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-bul-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 48		
N	41	34
LS mean change from baseline	-0.03	-0.12
SE	0.057	0.062
95% CI	(-0.14, 0.08)	(-0.24, 0.01)
LS mean difference (Omaveloxolone-Placebo)		-0.09
SE		0.085
95% CI		(-0.26, 0.08)
p-value		0.3038
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.23
95% CI		(-0.69, 0.22)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-bul-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	10.22	11.18
SD	3.773	3.726
Week 4		
N	42	40
LS mean change from baseline	-0.60	-0.81
SE	0.344	0.354
95% CI	(-1.29, 0.09)	(-1.52, -0.11)
LS mean difference (Omaveloxolone-Placebo)		-0.21
SE		0.503
95% CI		(-1.22, 0.79)
p-value		0.6717
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.09
95% CI		(-0.52, 0.34)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ul-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 12		
N	42	40
LS mean change from baseline	-0.34	-0.99
SE	0.318	0.327
95% CI	(-0.97, 0.29)	(-1.64, -0.34)
LS mean difference (Omaveloxolone-Placebo)		-0.65
SE		0.466
95% CI		(-1.58, 0.28)
p-value		0.1683
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.30
95% CI		(-0.73, 0.14)
Week 18		
N	41	38
LS mean change from baseline	-0.35	-0.93
SE	0.369	0.383
95% CI	(-1.09, 0.38)	(-1.69, -0.17)
LS mean difference (Omaveloxolone-Placebo)		-0.58
SE		0.540
95% CI		(-1.66, 0.50)
p-value		0.2878
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.23
95% CI		(-0.68, 0.21)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ul-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 24		
N	41	36
LS mean change from baseline	-0.37	-0.69
SE	0.318	0.338
95% CI	(-1.01, 0.26)	(-1.37, -0.02)
LS mean difference (Omaveloxolone-Placebo)		-0.32
SE		0.472
95% CI		(-1.26, 0.62)
p-value		0.5026
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.15
95% CI		(-0.60, 0.30)
Week 36		
N	41	35
LS mean change from baseline	-0.36	-1.04
SE	0.352	0.376
95% CI	(-1.06, 0.34)	(-1.79, -0.29)
LS mean difference (Omaveloxolone-Placebo)		-0.68
SE		0.522
95% CI		(-1.72, 0.36)
p-value		0.1961
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.29
95% CI		(-0.75, 0.16)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ul-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 48		
N	41	34
LS mean change from baseline	0.14	-1.15
SE	0.414	0.445
95% CI	(-0.68, 0.97)	(-2.03, -0.26)
LS mean difference (Omaveloxolone-Placebo)		-1.29
SE		0.615
95% CI		(-2.51, -0.06)
p-value		0.0397
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.47
95% CI		(-0.93, -0.01)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ul-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	6.36	6.30
SD	2.394	2.752
Week 4		
N	42	40
LS mean change from baseline	-0.66	-0.23
SE	0.279	0.286
95% CI	(-1.22, -0.11)	(-0.80, 0.34)
LS mean difference (Omaveloxolone-Placebo)		0.43
SE		0.407
95% CI		(-0.38, 1.24)
p-value		0.2901
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.23
95% CI		(-0.21, 0.66)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ll-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 12		
N	42	40
LS mean change from baseline	-0.95	-0.22
SE	0.268	0.275
95% CI	(-1.49, -0.42)	(-0.77, 0.33)
LS mean difference (Omaveloxolone-Placebo)		0.73
SE		0.391
95% CI		(-0.04, 1.51)
p-value		0.0643
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.40
95% CI		(-0.03, 0.84)
Week 18		
N	41	38
LS mean change from baseline	-0.45	-0.24
SE	0.304	0.315
95% CI	(-1.06, 0.15)	(-0.87, 0.39)
LS mean difference (Omaveloxolone-Placebo)		0.22
SE		0.444
95% CI		(-0.67, 1.10)
p-value		0.6290
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.11
95% CI		(-0.34, 0.55)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ll-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 24		
N	41	36
LS mean change from baseline	-0.44	-0.62
SE	0.279	0.296
95% CI	(-0.99, 0.12)	(-1.21, -0.03)
LS mean difference (Omaveloxolone-Placebo)		-0.19
SE		0.413
95% CI		(-1.01, 0.64)
p-value		0.6506
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.10
95% CI		(-0.55, 0.35)
Week 36		
N	41	35
LS mean change from baseline	-0.42	-0.39
SE	0.291	0.311
95% CI	(-1.00, 0.16)	(-1.01, 0.23)
LS mean difference (Omaveloxolone-Placebo)		0.03
SE		0.433
95% CI		(-0.83, 0.89)
p-value		0.9448
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.02
95% CI		(-0.44, 0.47)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ll-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 48		
N	41	34
LS mean change from baseline	-0.11	-0.32
SE	0.326	0.351
95% CI	(-0.76, 0.54)	(-1.02, 0.38)
LS mean difference (Omaveloxolone-Placebo)		-0.21
SE		0.485
95% CI		(-1.17, 0.76)
p-value		0.6701
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.10
95% CI		(-0.55, 0.36)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ll-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	21.38	22.33
SD	7.289	6.475
Week 4		
N	42	40
LS mean change from baseline	0.28	-0.40
SE	0.263	0.270
95% CI	(-0.24, 0.81)	(-0.94, 0.14)
LS mean difference (Omaveloxolone-Placebo)		-0.68
SE		0.384
95% CI		(-1.45, 0.08)
p-value		0.0796
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.38
95% CI		(-0.82, 0.05)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-us-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 12		
N	42	40
LS mean change from baseline	0.22	0.11
SE	0.310	0.318
95% CI	(-0.40, 0.84)	(-0.53, 0.74)
LS mean difference (Omaveloxolone-Placebo)		-0.11
SE		0.451
95% CI		(-1.01, 0.79)
p-value		0.8084
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.05
95% CI		(-0.49, 0.38)
Week 18		
N	41	38
LS mean change from baseline	0.08	-0.16
SE	0.322	0.334
95% CI	(-0.56, 0.72)	(-0.82, 0.51)
LS mean difference (Omaveloxolone-Placebo)		-0.24
SE		0.470
95% CI		(-1.17, 0.70)
p-value		0.6168
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.11
95% CI		(-0.55, 0.33)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-us-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 24		
N	41	36
LS mean change from baseline	0.12	-0.17
SE	0.342	0.364
95% CI	(-0.56, 0.81)	(-0.89, 0.56)
LS mean difference (Omaveloxolone-Placebo)		-0.29
SE		0.505
95% CI		(-1.30, 0.72)
p-value		0.5662
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-0.58, 0.32)
Week 36		
N	41	35
LS mean change from baseline	0.64	-0.09
SE	0.319	0.341
95% CI	(0.00, 1.27)	(-0.77, 0.59)
LS mean difference (Omaveloxolone-Placebo)		-0.72
SE		0.473
95% CI		(-1.67, 0.22)
p-value		0.1308
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.34
95% CI		(-0.80, 0.11)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-us-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 48		
N	41	34
LS mean change from baseline	0.82	0.10
SE	0.320	0.347
95% CI	(0.18, 1.46)	(-0.59, 0.79)
LS mean difference (Omaveloxolone-Placebo)		-0.72
SE		0.479
95% CI		(-1.67, 0.24)
p-value		0.1385
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.34
95% CI		(-0.80, 0.12)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-us-itt-npc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Summary of Proportion of Worsening in Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score at Week 48 with Imputation - ITT without Pes Cavus population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Number of subjects with week 48 assessment (%)	41 (97.6)	34 (82.9)
Number of subjects with event (%)	19 (45.2)	14 (34.1)
RR - Relative Risk (Omaveloxolone/Placebo)		0.75
95% CI		(0.44, 1.30)
p-value		0.3071
OR - Odds Ratio (Omaveloxolone/Placebo)		0.63
95% CI		(0.26, 1.52)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.11
95% CI		(-0.32, 0.10)

NOTE 1: Worsening if change from baseline at Week 48 >= 1.9 in mFARS Total Score. Subject without week 48 assessment will be imputed as responder with a worsening event.

Source: biib141/valueaccess/amnog/t-mfars93-propw-npc-imp.sas **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	9.87	10.74
SD	4.834	4.707
Week 24		
N	41	36
LS mean change from baseline	0.53	-0.36
SE	0.411	0.439
95% CI	(-0.29, 1.35)	(-1.24, 0.52)
LS mean difference (Omaveloxolone-Placebo)		-0.89
SE		0.614
95% CI		(-2.12, 0.33)
p-value		0.1509
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.32
95% CI		(-0.77, 0.13)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-adl-mmrm-pcp.sas:t-adl-mmrm-npcp.rtf **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 36		
N	41	36
LS mean change from baseline	0.65	0.31
SE	0.426	0.456
95% CI	(-0.20, 1.50)	(-0.60, 1.22)
LS mean difference (Omaveloxolone-Placebo)		-0.34
SE		0.637
95% CI		(-1.61, 0.93)
p-value		0.5934
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.12
95% CI		(-0.57, 0.33)
Week 48		
N	41	36
LS mean change from baseline	1.14	-0.17
SE	0.421	0.450
95% CI	(0.30, 1.98)	(-1.07, 0.73)
LS mean difference (Omaveloxolone-Placebo)		-1.30
SE		0.629
95% CI		(-2.56, -0.05)
p-value		0.0420
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.46
95% CI		(-0.91, -0.01)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-adl-mmrm-pcp.sas:t-adl-mmrm-npcp.rtf **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis - ITT without Pes Cavus Population

Visit Statistic	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 12		
Number of observations per imputation	42 (100)	40 (97.6)
Number of imputed values per imputation	0	0
LS mean	3.71	3.73
SE	0.157	0.153
95% CI	(3.40, 4.02)	(3.42, 4.03)
LS mean difference (Omaveloxolone-Placebo)		0.02
SE		0.208
95% CI		(-0.40, 0.43)
p-value		0.9329
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.02
95% CI		(-0.41, 0.45)
Week 24		
Number of observations per imputation	41 (97.6)	36 (87.8)
Number of imputed values per imputation	1 (2.4)	4 (9.8)
LS mean	3.91	3.75
SE	0.192	0.193
95% CI	(3.54, 4.29)	(3.37, 4.13)
LS mean difference (Omaveloxolone-Placebo)		-0.17
SE		0.258
95% CI		(-0.67, 0.34)
p-value		0.5215
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.14
95% CI		(-0.59, 0.30)

NOTE 1: LS Mean and Hedges' g are based on the ANCOVA model that includes site, treatment group.
NOTE 2: Multiple imputation including treatment, site, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-npcp.sas **Data Tag:** FINAL **Run Date:** 22MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis - ITT without Pes Cavus Population

Visit Statistic	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 36		
Number of observations per imputation	41 (97.6)	36 (87.8)
Number of imputed values per imputation	1 (2.4)	4 (9.8)
LS mean	3.97	3.67
SE	0.196	0.197
95% CI	(3.58, 4.35)	(3.28, 4.06)
LS mean difference (Omaveloxolone-Placebo)		-0.30
SE		0.263
95% CI		(-0.82, 0.22)
p-value		0.2565
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.26
95% CI		(-0.71, 0.19)
Week 48		
Number of observations per imputation	41 (97.6)	36 (87.8)
Number of imputed values per imputation	1 (2.4)	4 (9.8)
LS mean	4.32	3.90
SE	0.211	0.213
95% CI	(3.91, 4.74)	(3.48, 4.32)
LS mean difference (Omaveloxolone-Placebo)		-0.42
SE		0.286
95% CI		(-0.98, 0.14)
p-value		0.1373
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.34
95% CI		(-0.79, 0.11)

NOTE 1: LS Mean and Hedges' g are based on the ANCOVA model that includes site, treatment group.
NOTE 2: Multiple imputation including treatment, site, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-npcp.sas **Data Tag:** FINAL **Run Date:** 22MAY2025

MOXie Part 2: Summary of Proportion of Improvement in Patient Global Impression of Change (PGI-C) at Week 48 by Pes Cavus Status (Yes, No) - ITT Population

Pes Cavus status: No

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Number of subjects with week 48 assessment (%)	41 (100)	36 (100)
Number of subjects with event (%)	11 (26.8)	16 (44.4)
RR - Relative Risk (Omaveloxolone/Placebo)		1.66
95% CI		(0.89, 3.09)
p-value		0.1127
OR - Odds Ratio (Omaveloxolone/Placebo)		2.18
95% CI		(0.84, 5.66)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.18
95% CI		(-0.04, 0.39)

Note 1: Improvement if PGI-C value at Week 48 in (1,2,3).

Source: biib141/valueaccess/amnog/t-pgi-propi-sgrp.sas:t-pgi-propi-sgrp-pcavus.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement and Stable in Patient Global Impression of Change (PGI-C) at Week 48 by Pes Cavus Status (Yes, No) - ITT Population

Pes Cavus status: No

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Number of subjects with week 48 assessment (%)	41 (100)	36 (100)
Number of subjects with event (%)	24 (58.5)	25 (69.4)
RR - Relative Risk (Omaveloxolone/Placebo)		1.19
95% CI		(0.85, 1.66)
p-value		0.3198
OR - Odds Ratio (Omaveloxolone/Placebo)		1.61
95% CI		(0.63, 4.13)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.11
95% CI		(-0.10, 0.32)

Note 1: Improvement and stable if PGI-C value at Week 48 in (1,2,3,4).

Source: biib141/valueaccess/amnog/t-pgi-propis-sgrp.sas:t-pgi-propis-sgrp-pcavus.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Patient Global Impression of Change (PGI-C) at Week 48 by Pes Cavus Status (Yes, No) - ITT Population

Pes Cavus status: No

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Number of subjects with week 48 assessment (%)	41 (100)	36 (100)
Number of subjects with event (%)	17 (41.5)	11 (30.6)
RR - Relative Risk (Omaveloxolone/Placebo)		0.74
95% CI		(0.40, 1.36)
p-value		0.3284
OR - Odds Ratio (Omaveloxolone/Placebo)		0.62
95% CI		(0.24, 1.59)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.11
95% CI		(-0.32, 0.10)

Note 1: Worsening if PGI-C value at Week 48 in (5,6,7).

Source: biib141/valueaccess/amnog/t-pgi-propw-sgrp.sas:t-pgi-propw-sgrp-pcavus.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	0.02	0.02
SD	0.007	0.007
Week 24		
N	40	36
LS mean change from baseline	-0.0001	-0.0004
SE	0.00047	0.00050
95% CI	(-0.0011, 0.0008)	(-0.0013, 0.0006)
LS mean difference (Omaveloxolone-Placebo)		-0.0002
SE		0.00070
95% CI		(-0.0016, 0.0012)
p-value		0.7576
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.0692
95% CI		(-0.5196, 0.3812)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-dh.sas:t-9hpt-mmrm-dh-npcp.rtf **Data Tag:** FINAL **Run Date:** 27MAY2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 48		
N	41	36
LS mean change from baseline	-0.0009	-0.0001
SE	0.00052	0.00056
95% CI	(-0.0019, 0.0002)	(-0.0013, 0.0010)
LS mean difference (Omaveloxolone-Placebo)		0.0007
SE		0.00078
95% CI		(-0.0008, 0.0023)
p-value		0.3659
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.2022
95% CI		(-0.2466, 0.6510)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-dh.sas:t-9hpt-mmrm-dh-npcp.rtf **Data Tag:** FINAL **Run Date:** 27MAY2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	0.02	0.02
SD	0.007	0.008
Week 24		
N	41	36
LS mean change from baseline	-0.0004	-0.0017
SE	0.00056	0.00060
95% CI	(-0.0015, 0.0007)	(-0.0029, -0.0005)
LS mean difference (Omaveloxolone-Placebo)		-0.0013
SE		0.00084
95% CI		(-0.0030, 0.0004)
p-value		0.1282
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.3420
95% CI		(-0.7929, 0.1090)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-ndh.sas:t-9hpt-mmrm-ndh-npcp.rtf **Data Tag:** FINAL **Run Date:** 27MAY2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 48		
N	41	36
LS mean change from baseline	-0.0001	-0.0014
SE	0.00064	0.00068
95% CI	(-0.0014, 0.0011)	(-0.0028, -0.0001)
LS mean difference (Omaveloxolone-Placebo)		-0.0013
SE		0.00095
95% CI		(-0.0032, 0.0006)
p-value		0.1833
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.2985
95% CI		(-0.7487, 0.1516)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-ndh.sas:t-9hpt-mmrm-ndh-npcp.rtf **Data Tag:** FINAL **Run Date:** 27MAY2025

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) at Week 48 by Pes Cavus Status (Yes, No) from MMRM Analysis - ITT Population

Pes Cavus status: No

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	37	37
Mean score	0.13	0.14
SD	0.062	0.067
Week 48		
N	34	30
LS mean change from baseline	-0.02	-0.02
SE	0.006	0.006
95% CI	(-0.03, -0.01)	(-0.03, 0.00)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.008
95% CI		(-0.01, 0.02)
p-value		0.5116
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.16
95% CI		(-0.33, 0.65)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-sgrp.sas:t-ftwk-mmrm-sgrp-pcavus.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Number of Falls from Baseline to Week 48 from Poisson Regression Model Analysis by Pes Cavus Status (Yes, No) - ITT Population

Pes Cavus status: No

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Total number of falls from baseline to week 48		
Mean	16.52	11.02
SD	25.694	19.929
Adjusted incidence rate of falls	0.05	0.04
95% CI	(0.04, 0.07)	(0.02, 0.06)
Rate ratio (Omaveloxolone/Placebo)		0.73
95% CI		(0.42, 1.26)
p-value		0.2523

Note 1: Incidence rate of falls and rate ratio are estimated from the Poisson model with Pes Cavus status as a covariate and the natural logarithm of time on study (days) as an offset term.

Source: biib141/valueaccess/amnog/t-fall-prm-sgrp.sas:t-fall-prm-sgrp-pcavus.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: SF-36 Mental Component Summary (SF-36 MCS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	49.59	50.58
SD	9.232	10.162
Week 24		
N	41	36
LS mean change from baseline	0.38	-0.68
SE	0.986	1.055
95% CI	(-1.59, 2.35)	(-2.79, 1.43)
LS mean difference (Omaveloxolone-Placebo)		-1.06
SE		1.476
95% CI		(-4.01, 1.89)
p-value		0.4747
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.16
95% CI		(-0.61, 0.29)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-mcs.sas:t-sf36-mmrm-mcs-npcp.rtf **Data Tag:** FINAL **Run Date:** 23MAY2025

MOXie Part 2: SF-36 Mental Component Summary (SF-36 MCS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 48		
N	41	36
LS mean change from baseline	-0.11	-1.08
SE	1.219	1.303
95% CI	(-2.55, 2.32)	(-3.68, 1.52)
LS mean difference (Omaveloxolone-Placebo)		-0.96
SE		1.810
95% CI		(-4.57, 2.65)
p-value		0.5966
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.12
95% CI		(-0.57, 0.33)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-mcs.sas:t-sf36-mmrm-mcs-npcp.rtf **Data Tag:** FINAL **Run Date:** 23MAY2025

MOXie Part 2: SF-36 Physical Component Summary (SF-36 PCS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	39.15	37.11
SD	8.739	8.002
Week 24		
N	41	36
LS mean change from baseline	-0.87	-0.50
SE	0.821	0.879
95% CI	(-2.51, 0.77)	(-2.26, 1.25)
LS mean difference (Omaveloxolone-Placebo)		0.36
SE		1.232
95% CI		(-2.09, 2.82)
p-value		0.7683
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.07
95% CI		(-0.38, 0.51)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-pcs.sas:t-sf36-mmrm-pcs-npcp.rtf **Data Tag:** FINAL **Run Date:** 23MAY2025

MOXie Part 2: SF-36 Physical Component Summary (SF-36 PCS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Week 48		
N	41	36
LS mean change from baseline	-1.73	-0.71
SE	0.932	0.997
95% CI	(-3.59, 0.12)	(-2.70, 1.28)
LS mean difference (Omaveloxolone-Placebo)		1.02
SE		1.391
95% CI		(-1.75, 3.80)
p-value		0.4646
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.16
95% CI		(-0.29, 0.61)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-pcs.sas:t-sf36-mmrm-pcs-npcp.rtf **Data Tag:** FINAL **Run Date:** 23MAY2025

MOXie Part 2: Summary of Proportion of Worsening in SF-36 Mental Component Summary (SF-36 MCS) at Week 48 with Imputation - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Number of subjects with week 48 assessment (%)	41 (97.6)	36 (87.8)
Number of subjects with event (%)	4 (9.5)	7 (17.1)
RR - Relative Risk (Omaveloxolone/Placebo)		1.79
95% CI		(0.57, 5.67)
p-value		0.3201
OR - Odds Ratio (Omaveloxolone/Placebo)		1.96
95% CI		(0.53, 7.27)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.08
95% CI		(-0.07, 0.22)

NOTE 1: Worsening if change from baseline at week 48 <= -9.6 in SF-36 MCS. Subject without week 48 assessment will be imputed as responder with a worsening event.

Source: biib141/valueaccess/amnog/t-sf36-propw-mcs-imp.sas:t-sf36-propw-mcs-imp-npcp.rtf **Data Tag:** FINAL **Run Date:** 13MAY2025

MOXie Part 2: Summary of Proportion of Worsening in SF-36 Physical Component Summary (SF-36 PCS) at Week 48 with Imputation - ITT without Pes Cavus Population

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Number of subjects with week 48 assessment (%)	41 (97.6)	36 (87.8)
Number of subjects with event (%)	3 (7.1)	7 (17.1)
RR - Relative Risk (Omaveloxolone/Placebo)		2.39
95% CI		(0.66, 8.62)
p-value		0.1829
OR - Odds Ratio (Omaveloxolone/Placebo)		2.68
95% CI		(0.64, 11.17)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.10
95% CI		(-0.04, 0.24)

Source: biib141/valueaccess/amnog/t-sf36-propw-pcs-imp.sas:t-sf36-propw-pcs-imp-npcp.rtf **Data Tag:** FINAL **Run Date:** 13MAY2025

1. Anzahl der Patient*innen mit mindestens einem UE - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem UE - Safety Population ohne schweren Pes Cavus					
n (%)	41 (100 %)	42 (100 %)	1,00 [0,954; 1,047] 0,9915	0,98 [0,019; 50,369] 0,9915	0,00 [0,000; 0,000] NA
Ja (%) Nein (%)	41 (100 %) 0 (0 %)	42 (100 %) 0 (0 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population ohne schweren Pes Cavus					
Infektionen und parasitäre Erkrankungen (SOC)	**29 (71 %)**	**23 (55 %)**	**1,29 [0,921; 1,811] 0,1382**	**2,00 [0,806; 4,942] 0,1352**	**0,16 [-0,045; 0,365] 0,1270**
Grippe (PT)	5 (12 %)	2 (5 %)	2,56 [0,526; 12,463] 0,2467	2,78 [0,507; 15,213] 0,2415	0,07 [-0,045; 0,193] 0,2232
Nasopharyngitis (PT)	7 (17 %)	7 (17 %)	1,02 [0,394; 2,663] 0,9642	1,03 [0,326; 3,248] 0,9642	0,00 [-0,157; 0,165] 0,9642
Infektion der oberen Atemwege (PT)	13 (32 %)	10 (24 %)	1,33 [0,659; 2,690] 0,4327	1,49 [0,564; 3,911] 0,4308	0,08 [-0,113; 0,271] 0,4282
Stoffwechsel- und Ernährungsstörungen (SOC)	**8 (20 %)**	**2 (5 %)**	**4,10 [0,925; 18,155] 0,0629**	**4,85 [0,963; 24,416] 0,0552**	**0,15 [0,010; 0,285] 0,0350**
Appetit vermindert (PT)	6 (15 %)	2 (5 %)	3,07 [0,658; 14,355] 0,1539	3,43 [0,650; 18,093] 0,1469	0,10 [-0,027; 0,225] 0,1244
Psychiatrische Erkrankungen (SOC)	**5 (12 %)**	**7 (17 %)**	**0,73 [0,253; 2,120] 0,5767**	**0,69 [0,201; 2,396] 0,5756**	**-0,04 [-0,195; 0,106] 0,5728**
Erkrankungen des Nervensystems (SOC)	**19 (46 %)**	**16 (38 %)**	**1,22 [0,733; 2,020] 0,4576**	**1,40 [0,585; 3,365] 0,4563**	**0,08 [-0,129; 0,294] 0,4542**
Kopfschmerzen (PT)	14 (34 %)	11 (26 %)	1,30 [0,672; 2,528] 0,4407	1,46 [0,569; 3,753] 0,4389	0,08 [-0,117; 0,276] 0,4365
Herzerkrankungen (SOC)	**5 (12 %)**	**6 (14 %)**	**0,85 [0,282; 2,580] 0,7916**	**0,83 [0,233; 2,978] 0,7914**	**-0,02 [-0,167; 0,125] 0,7910**

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Erkrankungen der Atemwege, des Brustraums und Mediastinums (SOC)	**16 (39 %)**	**13 (31 %)**	**1,26 [0,698; 2,279] 0,4516**	**1,43 [0,577; 3,534] 0,4500**	**0,08 [-0,124; 0,285] 0,4478**
Schmerzen im Oropharynx (PT)	8 (20 %)	3 (7 %)	2,73 [0,779; 9,584] 0,1166	3,15 [0,773; 12,851] 0,1093	0,12 [-0,020; 0,268] 0,0923
Erkrankungen des Gastrointestinaltrakts (SOC)	**28 (68 %)**	**15 (36 %)**	**1,91 [1,212; 3,018] 0,0054**	**3,88 [1,558; 9,647] 0,0036**	**0,33 [0,123; 0,529] 0,0017**
Abdominalschmerz (PT)	9 (22 %)	1 (2 %)	9,22 [1,222; 69,547] 0,0309	11,53 [1,388; 95,788] 0,0234	0,20 [0,061; 0,331] 0,0045
Diarrhö (PT)	7 (17 %)	5 (12 %)	1,43 [0,495; 4,156] 0,5169	1,52 [0,442; 5,257] 0,5156	0,05 [-0,100; 0,203] 0,5132
Übelkeit (PT)	14 (34 %)	5 (12 %)	2,87 [1,136; 7,241] 0,0255	3,84 [1,233; 11,941] 0,0201	0,22 [0,047; 0,398] 0,0127
Erbrechen (PT)	7 (17 %)	4 (10 %)	1,79 [0,567; 5,665] 0,3249	1,96 [0,526; 7,269] 0,3213	0,08 [-0,070; 0,221] 0,3134
Erkrankungen der Haut und des Unterhautgewebes (SOC)	**13 (32 %)**	**6 (14 %)**	**2,22 [0,933; 5,279] 0,0709**	**2,79 [0,940; 8,253] 0,0641**	**0,17 [-0,003; 0,352] 0,0539**
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**18 (44 %)**	**17 (40 %)**	**1,08 [0,655; 1,796] 0,7649**	**1,15 [0,481; 2,752] 0,7649**	**0,03 [-0,178; 0,247] 0,7647**
Arthralgie (PT)	7 (17 %)	9 (21 %)	0,80 [0,327; 1,938] 0,6291	0,75 [0,252; 2,263] 0,6283	-0,04 [-0,213; 0,126] 0,6267
Muskelspasmen (PT)	6 (15 %)	2 (5 %)	3,07 [0,658; 14,355] 0,1539	3,43 [0,650; 18,093] 0,1469	0,10 [-0,027; 0,225] 0,1244

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Erkrankungen der Geschlechtsorgane und der Brustdrüse (SOC)	**5 (12 %)**	**0 (0 %)**	**11,26 [0,643; 197,372] 0,0972**	**12,81 [0,685; 239,544] 0,0876**	**0,12 [0,022; 0,222] 0,0169**
Allgemeine Erkrankungen und Beschwerden am Verabreichungsort (SOC)	**16 (39 %)**	**11 (26 %)**	**1,49 [0,789; 2,814] 0,2208**	**1,80 [0,711; 4,576] 0,2162**	**0,13 [-0,072; 0,328] 0,2100**
Ermüdung (PT)	9 (22 %)	3 (7 %)	3,07 [0,895; 10,554] 0,0741	3,66 [0,913; 14,646] 0,0667	0,15 [-0,001; 0,297] 0,0506
Untersuchungen (SOC)	**22 (54 %)**	**7 (17 %)**	**3,22 [1,546; 6,705] 0,0018**	**5,79 [2,093; 16,015] 0,0008**	**0,37 [0,180; 0,560] 0,0001**
Alaninaminotransferase erhöht (PT)	15 (37 %)	1 (2 %)	15,37 [2,126; 111,066] 0,0068	23,65 [2,946; 189,910] 0,0030	0,34 [0,188; 0,497] < 0,0001
Aspartataminotransferase erhöht (PT)	9 (22 %)	1 (2 %)	9,22 [1,222; 69,547] 0,0309	11,53 [1,388; 95,788] 0,0234	0,20 [0,061; 0,331] 0,0045
Verletzung, Vergiftung und durch Eingriffe bedingte Komplikationen (SOC)	**28 (68 %)**	**26 (62 %)**	**1,10 [0,804; 1,513] 0,5536**	**1,33 [0,536; 3,279] 0,5534**	**0,06 [-0,141; 0,268] 0,5518**
Kontusion (PT)	15 (37 %)	15 (36 %)	1,02 [0,578; 1,815] 0,9398	1,04 [0,424; 2,543] 0,9398	0,01 [-0,198; 0,215] 0,9398
Exkoriation (PT)	11 (27 %)	9 (21 %)	1,25 [0,580; 2,701] 0,5784	1,34 [0,490; 3,692] 0,5776	0,05 [-0,130; 0,238] 0,5765
Risswunde (PT)	7 (17 %)	6 (14 %)	1,20 [0,439; 3,255] 0,7404	1,24 [0,377; 4,048] 0,7402	0,03 [-0,129; 0,184] 0,7399
Bänderzerrung (PT)	3 (7 %)	7 (17 %)	0,44 [0,122; 1,583] 0,2100	0,39 [0,095; 1,647] 0,2036	-0,09 [-0,232; 0,045] 0,1855

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

1. Anzahl der Patient*innen mit mindestens einem milden UE - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem milden UE - Safety Population ohne schweren Pes Cavus					
n (%)	41 (100 %)	42 (100 %)	1,05 [0,967; 1,138] 0,2509	5,12 [0,239; 110,046] 0,3006	0,05 [-0,017; 0,112] 0,1477
Ja (%) Nein (%)	41 (100 %) 0 (0 %)	40 (95 %) 2 (5 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population ohne schweren Pes Cavus					
Infektionen und parasitäre Erkrankungen (SOC)	**28 (68 %)**	**23 (55 %)**	**1,25 [0,883; 1,761] 0,2115**	**1,78 [0,727; 4,357] 0,2089**	**0,14 [-0,072; 0,343] 0,2021**
Nasopharyngitis (PT)	6 (15 %)	7 (17 %)	0,88 [0,322; 2,391] 0,8111	0,86 [0,262; 2,809] 0,8110	-0,02 [-0,177; 0,136] 0,8107
Infektion der oberen Atemwege (PT)	13 (32 %)	10 (24 %)	1,33 [0,659; 2,690] 0,4327	1,49 [0,564; 3,911] 0,4308	0,08 [-0,113; 0,271] 0,4282
Stoffwechsel- und Ernährungsstörungen (SOC)	**8 (20 %)**	**2 (5 %)**	**4,10 [0,925; 18,155] 0,0629**	**4,85 [0,963; 24,416] 0,0552**	**0,15 [0,010; 0,285] 0,0350**
Appetit vermindert (PT)	6 (15 %)	2 (5 %)	3,07 [0,658; 14,355] 0,1539	3,43 [0,650; 18,093] 0,1469	0,10 [-0,027; 0,225] 0,1244
Psychiatrische Erkrankungen (SOC)	**3 (7 %)**	**6 (14 %)**	**0,51 [0,137; 1,912] 0,3244**	**0,47 [0,110; 2,038] 0,3202**	**-0,07 [-0,202; 0,063] 0,3069**
Erkrankungen des Nervensystems (SOC)	**17 (41 %)**	**13 (31 %)**	**1,34 [0,750; 2,392] 0,3280**	**1,58 [0,641; 3,895] 0,3251**	**0,11 [-0,101; 0,311] 0,3212**
Kopfschmerzen (PT)	13 (32 %)	10 (24 %)	1,33 [0,659; 2,690] 0,4327	1,49 [0,564; 3,911] 0,4308	0,08 [-0,113; 0,271] 0,4282
Herzerkrankungen (SOC)	**2 (5 %)**	**5 (12 %)**	**0,41 [0,084; 1,994] 0,2724**	**0,38 [0,069; 2,078] 0,2672**	**-0,07 [-0,188; 0,048] 0,2460**
Erkrankungen der Atemwege, des Brustraums und	**15 (37 %)**	**13 (31 %)**	**1,18 [0,645; 2,165] 0,6005**	**1,29 [0,517; 3,204] 0,5999**	**0,06 [-0,147; 0,260] 0,5990**

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Mediastinums (SOC)					
Schmerzen im Oropharynx (PT)	7 (17 %)	3 (7 %)	2,39 [0,663; 8,616] 0,1840	2,68 [0,641; 11,168] 0,1777	0,10 [-0,040; 0,238] 0,1622
Erkrankungen des Gastrointestinaltrakts (SOC)	**28 (68 %)**	**14 (33 %)**	**2,05 [1,273; 3,297] 0,0032**	**4,31 [1,719; 10,797] 0,0019**	**0,35 [0,148; 0,551] 0,0007**
Abdominalschmerz (PT)	8 (20 %)	1 (2 %)	8,20 [1,072; 62,638] 0,0423	9,94 [1,183; 83,537] 0,0342	0,17 [0,042; 0,301] 0,0097
Diarrhö (PT)	6 (15 %)	4 (10 %)	1,54 [0,468; 5,050] 0,4888	1,63 [0,424; 6,256] 0,4872	0,05 [-0,089; 0,191] 0,4837
Übelkeit (PT)	12 (29 %)	5 (12 %)	2,46 [0,950; 6,360] 0,0632	3,06 [0,969; 9,680] 0,0563	0,17 [0,003; 0,344] 0,0453
Erbrechen (PT)	6 (15 %)	4 (10 %)	1,54 [0,468; 5,050] 0,4888	1,63 [0,424; 6,256] 0,4872	0,05 [-0,089; 0,191] 0,4837
Erkrankungen der Haut und des Unterhautgewebes (SOC)	**11 (27 %)**	**6 (14 %)**	**1,88 [0,766; 4,605] 0,1692**	**2,20 [0,728; 6,652] 0,1632**	**0,13 [-0,047; 0,297] 0,1535**
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**15 (37 %)**	**17 (40 %)**	**0,90 [0,524; 1,559] 0,7293**	**0,85 [0,350; 2,056] 0,7290**	**-0,04 [-0,248; 0,170] 0,7286**
Arthralgie (PT)	6 (15 %)	9 (21 %)	0,68 [0,267; 1,747] 0,4342	0,63 [0,202; 1,960] 0,4316	-0,07 [-0,233; 0,097] 0,4265
Erkrankungen der Geschlechtsorgane und der Brustdrüse (SOC)	**5 (12 %)**	**0 (0 %)**	**11,26 [0,643; 197,372] 0,0972**	**12,81 [0,685; 239,544] 0,0876**	**0,12 [0,022; 0,222] 0,0169**

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Allgemeine Erkrankungen und Beschwerden am Verabreichungsort (SOC)	**12 (29 %)**	**11 (26 %)**	**1,12 [0,557; 2,241] 0,7671**	**1,17 [0,446; 3,052] 0,7670**	**0,03 [-0,162; 0,223] 0,7669**
Ermüdung (PT)	6 (15 %)	3 (7 %)	2,05 [0,549; 7,650] 0,2897	2,23 [0,518; 9,588] 0,2854	0,07 [-0,058; 0,208] 0,2741
Untersuchungen (SOC)	**16 (39 %)**	**6 (14 %)**	**2,73 [1,187; 6,288] 0,0180**	**3,84 [1,320; 11,173] 0,0135**	**0,25 [0,064; 0,430] 0,0081**
Alaninaminotransferase erhöht (PT)	10 (24 %)	1 (2 %)	10,24 [1,372; 76,460] 0,0231	13,23 [1,607; 108,860] 0,0163	0,22 [0,081; 0,359] 0,0020
Aspartataminotransferase erhöht (PT)	8 (20 %)	1 (2 %)	8,20 [1,072; 62,638] 0,0423	9,94 [1,183; 83,537] 0,0342	0,17 [0,042; 0,301] 0,0097
Verletzung, Vergiftung und durch Eingriffe bedingte Komplikationen (SOC)	**28 (68 %)**	**25 (60 %)**	**1,15 [0,829; 1,588] 0,4150**	**1,46 [0,595; 3,607] 0,4142**	**0,09 [-0,118; 0,293] 0,4109**
Kontusion (PT)	15 (37 %)	14 (33 %)	1,10 [0,610; 1,975] 0,7690	1,15 [0,468; 2,847] 0,7689	0,03 [-0,173; 0,238] 0,7687
Exkoriation (PT)	11 (27 %)	9 (21 %)	1,25 [0,580; 2,701] 0,5784	1,34 [0,490; 3,692] 0,5776	0,05 [-0,130; 0,238] 0,5765
Risswunde (PT)	6 (15 %)	6 (14 %)	1,02 [0,360; 2,917] 0,9673	1,03 [0,303; 3,496] 0,9673	0,00 [-0,148; 0,155] 0,9673
Bänderzerrung (PT)	3 (7 %)	6 (14 %)	0,51 [0,137; 1,912] 0,3244	0,47 [0,110; 2,038] 0,3202	-0,07 [-0,202; 0,063] 0,3069

Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.

1. Anzahl der Patient*innen mit mindestens einem moderaten UE - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem moderaten UE - Safety Population ohne schweren Pes Cavus					
n (%)	41 (100 %)	42 (100 %)	1,97 [1,178; 3,293] 0,0097	3,49 [1,408; 8,629] 0,0070	0,30 [0,096; 0,505] 0,0041
Ja (%) Nein (%)	25 (61 %) 16 (39 %)	13 (31 %) 29 (69 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem moderaten UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem moderaten UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population ohne schweren Pes Cavus					
Infektionen und parasitäre Erkrankungen (SOC)	**9 (22 %)**	**2 (5 %)**	**4,61 [1,059; 20,059] 0,0413**	**5,62 [1,134; 27,893] 0,0342**	**0,17 [0,030; 0,314] 0,0176**
Erkrankungen des Nervensystems (SOC)	**3 (7 %)**	**5 (12 %)**	**0,61 [0,157; 2,407] 0,4945**	**0,58 [0,130; 2,621] 0,4926**	**-0,05 [-0,172; 0,080] 0,4860**
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**5 (12 %)**	**0 (0 %)**	**11,26 [0,643; 197,372] 0,0972**	**12,81 [0,685; 239,544] 0,0876**	**0,12 [0,022; 0,222] 0,0169**
Untersuchungen (SOC)	**8 (20 %)**	**1 (2 %)**	**8,20 [1,072; 62,638] 0,0423**	**9,94 [1,183; 83,537] 0,0342**	**0,17 [0,042; 0,301] 0,0097**
Alaninaminotransferase erhöht (PT)	5 (12 %)	0 (0 %)	11,26 [0,643; 197,372] 0,0972	12,81 [0,685; 239,544] 0,0876	0,12 [0,022; 0,222] 0,0169

Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.

1. Anzahl der Patient*innen mit mindestens einem schweren UE - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem schweren UE - Safety Population ohne schweren Pes Cavus					
n (%)	41 (100 %)	42 (100 %)	11,26 [0,643; 197,372] 0,0972	12,81 [0,685; 239,544] 0,0876	0,12 [0,022; 0,222] 0,0169
Ja (%) Nein (%)	5 (12 %) 36 (88 %)	0 (0 %) 42 (100 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

1. Anzahl der Patient*innen mit mindestens einem SUE - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem SUE - Safety Population ohne schweren Pes Cavus					
n (%)	41 (100 %)	42 (100 %)	2,56 [0,526; 12,463] 0,2467	2,78 [0,507; 15,213] 0,2415	0,07 [-0,045; 0,193] 0,2232
Ja (%) Nein (%)	5 (12 %) 36 (88 %)	2 (5 %) 40 (95 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem SUE bei mindestens 5% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem SUE bei mindestens 5% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population ohne schweren Pes Cavus					
Herzerkrankungen (SOC)	**3 (7 %)**	**1 (2 %)**	**3,07 [0,333; 28,349] 0,3269**	**3,24 [0,323; 32,473] 0,3229**	**0,05 [-0,043; 0,141] 0,2975**
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

1. Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**41**	**42**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE - Safety Population ohne schweren Pes Cavus					
n (%)	41 (100 %)	42 (100 %)	1,54 [0,271; 8,725] 0,6406	1,58 [0,250; 9,976] 0,6400	0,03 [-0,077; 0,128] 0,6378
Ja (%) Nein (%)	3 (7 %) 38 (93 %)	2 (5 %) 40 (95 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE nach allen SOC und PT – deskriptive Darstellung, n(%) - Safety Population ohne schweren Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm	
	Omaveloxolon	**Placebo**
N	**41**	**42**
Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE nach allen SOC und PT – deskriptive Darstellung, n(%) - Safety Population ohne schweren Pes Cavus		
Herzerkrankungen (SOC)	**1 (2 %)**	**1 (2 %)**
Vorhofflimmern (PT)	0 (0 %)	1 (2 %)
Tachykardie ventrikulär (PT)	1 (2 %)	0 (0 %)
Erkrankungen der Haut und des Unterhautgewebes (SOC)	**1 (2 %)**	**1 (2 %)**
Erythrose (PT)	0 (0 %)	1 (2 %)
Rosazea (PT)	1 (2 %)	0 (0 %)
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**1 (2 %)**	**0 (0 %)**
Muskelspasmen (PT)	1 (2 %)	0 (0 %)
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.		

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline at Week 48 by Pes Cavus Status (Yes, No) from MMRM Analysis - ITT Population

Pes Cavus status: Yes

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	34.33	41.09
SD	9.353	9.952
Week 48		
N	9	8
LS mean change from baseline	0.35	1.23
SE	1.400	1.458
95% CI	(-2.43, 3.14)	(-1.67, 4.13)
LS mean difference (Omaveloxolone-Placebo)		0.88
SE		2.005
95% CI		(-3.11, 4.86)
p-value		0.6633
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.20
95% CI		(-0.76, 1.15)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp-itt.sas:t-mfars93-mmrm-sgrp-itt-pcavus.rtf **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score LS Mean Change from Baseline at Week 48 by Pes Cavus Status (Yes, No) from MMRM Analysis - ITT Population

Pes Cavus status: No

	Placebo (N=42)	Omaveloxolone 150 mg (N=41)
Baseline		
N	42	41
Mean score	38.63	40.57
SD	11.029	10.330
Week 48		
N	41	34
LS mean change from baseline	0.95	-1.58
SE	0.664	0.715
95% CI	(-0.37, 2.27)	(-3.00, -0.16)
LS mean difference (Omaveloxolone-Placebo)		-2.53
SE		0.984
95% CI		(-4.48, -0.57)
p-value		0.0118
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.58
95% CI		(-1.05, -0.12)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)
Note 2: This analysis is based on mFARS using the 93-point scale. The primary analyses are based on mFARS using the 99-point scale.
Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp-itt.sas:t-mfars93-mmrm-sgrp-itt-pcavus.rtf **Data Tag:** FINAL **Run Date:** 25JUN2024

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	0.48	0.60
SD	0.399	0.603
Week 4		
N	10	10
LS mean change from baseline	0.08	-0.07
SE	0.138	0.139
95% CI	(-0.22, 0.39)	(-0.38, 0.23)
LS mean difference (Omaveloxolone-Placebo)		-0.15
SE		0.199
95% CI		(-0.59, 0.28)
p-value		0.4553
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.30
95% CI		(-1.18, 0.59)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-bul-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 12		
N	10	10
LS mean change from baseline	-0.14	-0.05
SE	0.082	0.083
95% CI	(-0.33, 0.04)	(-0.24, 0.14)
LS mean difference (Omaveloxolone-Placebo)		0.10
SE		0.121
95% CI		(-0.18, 0.37)
p-value		0.4481
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.29
95% CI		(-0.59, 1.17)
Week 18		
N	10	9
LS mean change from baseline	-0.04	0.13
SE	0.157	0.163
95% CI	(-0.39, 0.30)	(-0.22, 0.49)
LS mean difference (Omaveloxolone-Placebo)		0.18
SE		0.229
95% CI		(-0.32, 0.68)
p-value		0.4558
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.30
95% CI		(-0.60, 1.21)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-bul-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 24		
N	10	9
LS mean change from baseline	0.10	0.33
SE	0.179	0.183
95% CI	(-0.29, 0.49)	(-0.08, 0.73)
LS mean difference (Omaveloxolone-Placebo)		0.23
SE		0.258
95% CI		(-0.34, 0.79)
p-value		0.4016
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.34
95% CI		(-0.57, 1.25)
Week 36		
N	10	9
LS mean change from baseline	-0.04	0.14
SE	0.159	0.164
95% CI	(-0.39, 0.30)	(-0.21, 0.50)
LS mean difference (Omaveloxolone-Placebo)		0.19
SE		0.231
95% CI		(-0.32, 0.69)
p-value		0.4328
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.32
95% CI		(-0.59, 1.23)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-bul-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Bulbar Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 48		
N	9	8
LS mean change from baseline	-0.05	0.09
SE	0.137	0.141
95% CI	(-0.35, 0.25)	(-0.22, 0.40)
LS mean difference (Omaveloxolone-Placebo)		0.14
SE		0.200
95% CI		(-0.30, 0.58)
p-value		0.4964
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.29
95% CI		(-0.67, 1.25)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-bul-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	8.55	9.03
SD	1.840	3.271
Week 4		
N	10	10
LS mean change from baseline	-0.67	-0.77
SE	0.660	0.661
95% CI	(-2.12, 0.79)	(-2.23, 0.69)
LS mean difference (Omaveloxolone-Placebo)		-0.10
SE		0.953
95% CI		(-2.20, 2.00)
p-value		0.9184
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.04
95% CI		(-0.92, 0.84)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ul-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 12		
N	10	10
LS mean change from baseline	-1.51	0.46
SE	0.690	0.691
95% CI	(-3.13, 0.11)	(-1.16, 2.09)
LS mean difference (Omaveloxolone-Placebo)		1.98
SE		0.994
95% CI		(-0.35, 4.30)
p-value		0.0849
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.71
95% CI		(-0.19, 1.62)
Week 18		
N	10	9
LS mean change from baseline	-1.69	-0.08
SE	0.713	0.732
95% CI	(-3.25, -0.14)	(-1.67, 1.51)
LS mean difference (Omaveloxolone-Placebo)		1.61
SE		1.040
95% CI		(-0.64, 3.87)
p-value		0.1456
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.62
95% CI		(-0.31, 1.54)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ul-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 24		
N	10	9
LS mean change from baseline	-1.03	0.52
SE	0.773	0.810
95% CI	(-2.81, 0.74)	(-1.31, 2.36)
LS mean difference (Omaveloxolone-Placebo)		1.56
SE		1.138
95% CI		(-1.03, 4.14)
p-value		0.2052
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.52
95% CI		(-0.40, 1.43)
Week 36		
N	10	9
LS mean change from baseline	0.08	1.11
SE	0.854	0.877
95% CI	(-1.82, 1.98)	(-0.83, 3.04)
LS mean difference (Omaveloxolone-Placebo)		1.02
SE		1.238
95% CI		(-1.71, 3.76)
p-value		0.4257
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.32
95% CI		(-0.58, 1.23)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ul-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 48		
N	9	8
LS mean change from baseline	-0.30	0.89
SE	0.633	0.672
95% CI	(-1.65, 1.06)	(-0.55, 2.32)
LS mean difference (Omaveloxolone-Placebo)		1.19
SE		0.961
95% CI		(-0.86, 3.23)
p-value		0.2361
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.52
95% CI		(-0.44, 1.49)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ul-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	5.80	6.28
SD	1.790	1.812
Week 4		
N	10	10
LS mean change from baseline	-0.91	-0.31
SE	0.445	0.443
95% CI	(-1.87, 0.05)	(-1.27, 0.65)
LS mean difference (Omaveloxolone-Placebo)		0.60
SE		0.657
95% CI		(-0.82, 2.02)
p-value		0.3773
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.36
95% CI		(-0.53, 1.24)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ll-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 12		
N	10	10
LS mean change from baseline	-1.08	-0.35
SE	0.613	0.613
95% CI	(-2.43, 0.26)	(-1.70, 1.00)
LS mean difference (Omaveloxolone-Placebo)		0.74
SE		0.890
95% CI		(-1.21, 2.69)
p-value		0.4243
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.32
95% CI		(-0.56, 1.20)
Week 18		
N	10	9
LS mean change from baseline	-1.12	-0.01
SE	0.622	0.646
95% CI	(-2.46, 0.23)	(-1.40, 1.38)
LS mean difference (Omaveloxolone-Placebo)		1.11
SE		0.923
95% CI		(-0.87, 3.09)
p-value		0.2507
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.48
95% CI		(-0.43, 1.39)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ll-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 24		
N	10	9
LS mean change from baseline	-0.60	-0.09
SE	0.688	0.712
95% CI	(-2.10, 0.90)	(-1.64, 1.45)
LS mean difference (Omaveloxolone-Placebo)		0.51
SE		1.013
95% CI		(-1.69, 2.70)
p-value		0.6262
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.20
95% CI		(-0.70, 1.10)
Week 36		
N	10	9
LS mean change from baseline	-1.28	0.53
SE	0.652	0.681
95% CI	(-2.72, 0.16)	(-0.96, 2.02)
LS mean difference (Omaveloxolone-Placebo)		1.80
SE		0.966
95% CI		(-0.31, 3.91)
p-value		0.0874
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.73
95% CI		(-0.20, 1.66)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ll-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Lower Limb Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 48		
N	9	8
LS mean change from baseline	-1.15	0.77
SE	0.524	0.552
95% CI	(-2.32, 0.02)	(-0.44, 1.99)
LS mean difference (Omaveloxolone-Placebo)		1.92
SE		0.788
95% CI		(0.19, 3.66)
p-value		0.0327
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		1.00
95% CI		(-0.01, 2.01)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-ll-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	19.48	25.18
SD	6.511	6.558
Week 4		
N	10	10
LS mean change from baseline	0.67	-1.44
SE	0.688	0.690
95% CI	(-0.79, 2.13)	(-2.90, 0.02)
LS mean difference (Omaveloxolone-Placebo)		-2.11
SE		1.033
95% CI		(-4.29, 0.07)
p-value		0.0572
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.81
95% CI		(-1.72, 0.10)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-us-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 12		
N	10	10
LS mean change from baseline	-0.85	-0.75
SE	0.796	0.798
95% CI	(-2.54, 0.83)	(-2.44, 0.94)
LS mean difference (Omaveloxolone-Placebo)		0.10
SE		1.191
95% CI		(-2.41, 2.62)
p-value		0.9330
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.03
95% CI		(-0.84, 0.91)
Week 18		
N	10	9
LS mean change from baseline	0.70	-2.85
SE	0.600	0.643
95% CI	(-0.59, 1.99)	(-4.23, -1.48)
LS mean difference (Omaveloxolone-Placebo)		-3.56
SE		0.942
95% CI		(-5.57, -1.55)
p-value		0.0019
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-1.52
95% CI		(-2.54, -0.50)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-us-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 24		
N	10	9
LS mean change from baseline	0.79	-2.05
SE	0.724	0.757
95% CI	(-0.77, 2.35)	(-3.67, -0.43)
LS mean difference (Omaveloxolone-Placebo)		-2.84
SE		1.112
95% CI		(-5.23, -0.46)
p-value		0.0228
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-1.03
95% CI		(-1.98, -0.07)
Week 36		
N	10	9
LS mean change from baseline	1.28	-1.32
SE	1.246	1.313
95% CI	(-1.36, 3.92)	(-4.09, 1.45)
LS mean difference (Omaveloxolone-Placebo)		-2.60
SE		1.909
95% CI		(-6.63, 1.43)
p-value		0.1907
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.56
95% CI		(-1.47, 0.36)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-us-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upright Stability Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 48		
N	9	8
LS mean change from baseline	2.44	-1.95
SE	1.071	1.136
95% CI	(0.18, 4.70)	(-4.35, 0.45)
LS mean difference (Omaveloxolone-Placebo)		-4.39
SE		1.651
95% CI		(-7.87, -0.90)
p-value		0.0167
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-1.14
95% CI		(-2.16, -0.11)

NOTE 1: LS Mean and Hedges' g are based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-sgrp2.sas:t-mfars93-mmrm-sgrp2-d-us-itt-pc.rtf **Data Tag:** FINAL **Run Date:** 06JUN2025

MOXie Part 2: Summary of Proportion of Worsening in Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Total Score at Week 48 with Imputation -ITT with Pes Cavus population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Number of subjects with week 48 assessment (%)	9 (90.0)	8 (80.0)
Number of subjects with event (%)	4 (40.0)	7 (70.0)
RR - Relative Risk (Omaveloxolone/Placebo)		1.75
95% CI		(0.74, 4.14)
p-value		0.2025
OR - Odds Ratio (Omaveloxolone/Placebo)		3.50
95% CI		(0.55, 22.30)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.30
95% CI		(-0.12, 0.72)

NOTE 1: Worsening if change from baseline at Week 48 >= 1.9 in mFARS Total Score. Subject without week 48 assessment will be imputed as responder with a worsening event.

Source: biib141/valueaccess/amnog/t-mfars93-propw-pcp-imp.sas **Data Tag:** FINAL **Run Date:** 22MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	9.75	12.20
SD	4.424	3.385
Week 24		
N	10	9
LS mean change from baseline	-0.16	1.73
SE	0.870	0.942
95% CI	(-2.07, 1.76)	(-0.34, 3.79)
LS mean difference (Omaveloxolone-Placebo)		1.88
SE		1.384
95% CI		(-1.13, 4.90)
p-value		0.1983
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.54
95% CI		(-0.38, 1.45)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-adl-mmrm-pcp.sas:t-adl-mmrm-pcp.rtf **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Activities of Daily Living (ADL) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 36		
N	10	9
LS mean change from baseline	-0.05	2.81
SE	0.870	0.942
95% CI	(-1.98, 1.88)	(0.73, 4.89)
LS mean difference (Omaveloxolone-Placebo)		2.86
SE		1.384
95% CI		(-0.18, 5.90)
p-value		0.0625
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.81
95% CI		(-0.13, 1.75)
Week 48		
N	10	8
LS mean change from baseline	0.54	2.31
SE	1.155	1.256
95% CI	(-2.03, 3.11)	(-0.47, 5.09)
LS mean difference (Omaveloxolone-Placebo)		1.77
SE		1.795
95% CI		(-2.19, 5.72)
p-value		0.3462
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.40
95% CI		(-0.54, 1.33)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-adl-mmrm-pcp.sas:t-adl-mmrm-pcp.rtf **Data Tag:** FINAL **Run Date:** 20MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis - ITT with Pes Cavus Population

Visit Statistic	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 12		
Number of observations per imputation	10 (100)	10 (100)
Number of imputed values per imputation	0	0
LS mean	3.48	4.04
SE	0.302	0.296
95% CI	(2.83, 4.14)	(3.40, 4.68)
LS mean difference (Omaveloxolone-Placebo)		0.56
SE		0.393
95% CI		(-0.29, 1.41)
p-value		0.1778
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.55
95% CI		(-0.34, 1.45)
Week 24		
Number of observations per imputation	10 (100)	9 (90.0)
Number of imputed values per imputation	0	1 (10.0)
LS mean	3.77	3.77
SE	0.396	0.424
95% CI	(3.00, 4.55)	(2.94, 4.60)
LS mean difference (Omaveloxolone-Placebo)		-0.01
SE		0.533
95% CI		(-1.05, 1.04)
p-value		0.9896
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.01
95% CI		(-0.91, 0.89)

NOTE 1: LS Mean and Hedges' g are based on the ANCOVA model that includes site, treatment group.
NOTE 2: Multiple imputation including treatment, site, Pes cavus status, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-pcp.sas **Data Tag:** FINAL **Run Date:** 22MAY2025

MOXie Part 2: Patient Global Impression of Change (PGI-C) LS Mean up to Week 48 by Visit from ANCOVA+MI Analysis - ITT with Pes Cavus Population

Visit Statistic	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 36		
Number of observations per imputation	10 (100)	9 (90.0)
Number of imputed values per imputation	0	1 (10.0)
LS mean	4.09	3.87
SE	0.429	0.449
95% CI	(3.25, 4.93)	(2.99, 4.75)
LS mean difference (Omaveloxolone-Placebo)		-0.23
SE		0.573
95% CI		(-1.35, 0.90)
p-value		0.6928
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.17
95% CI		(-1.08, 0.73)
Week 48		
Number of observations per imputation	10 (100)	8 (80.0)
Number of imputed values per imputation	0	2 (20.0)
LS mean	4.63	3.96
SE	0.505	0.553
95% CI	(3.64, 5.62)	(2.87, 5.04)
LS mean difference (Omaveloxolone-Placebo)		-0.67
SE		0.697
95% CI		(-2.04, 0.70)
p-value		0.3352
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.44
95% CI		(-1.38, 0.51)

NOTE 1: LS Mean and Hedges' g are based on the ANCOVA model that includes site, treatment group.
NOTE 2: Multiple imputation including treatment, site, Pes cavus status, postbaseline for the endpoint.
NOTE 3: Subject 1949208 is not included in the analysis set due to missing assessments on week 12, 24, 36, 48.
Source: biib141/valueaccess/amnog/t-pgi-anc-mi-pcp.sas **Data Tag:** FINAL **Run Date:** 22MAY2025

MOXie Part 2: Summary of Proportion of Improvement in Patient Global Impression of Change (PGI-C) at Week 48 by Pes Cavus Status (Yes, No) - ITT Population

Pes Cavus status: Yes

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Number of subjects with week 48 assessment (%)	10 (100)	8 (100)
Number of subjects with event (%)	2 (20.0)	3 (37.5)
RR - Relative Risk (Omaveloxolone/Placebo)		1.88
95% CI		(0.41, 8.65)
p-value		0.4203
OR - Odds Ratio (Omaveloxolone/Placebo)		2.40
95% CI		(0.29, 19.78)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.18
95% CI		(-0.24, 0.59)

Note 1: Improvement if PGI-C value at Week 48 in (1,2,3).

Source: biib141/valueaccess/amnog/t-pgi-propi-sgrp.sas:t-pgi-propi-sgrp-pcavus.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Improvement and Stable in Patient Global Impression of Change (PGI-C) at Week 48 by Pes Cavus Status (Yes, No) - ITT Population

Pes Cavus status: Yes

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Number of subjects with week 48 assessment (%)	10 (100)	8 (100)
Number of subjects with event (%)	4 (40.0)	6 (75.0)
RR - Relative Risk (Omaveloxolone/Placebo)		1.88
95% CI		(0.79, 4.42)
p-value		0.1510
OR - Odds Ratio (Omaveloxolone/Placebo)		4.50
95% CI		(0.59, 34.61)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.35
95% CI		(-0.08, 0.78)

Note 1: Improvement and stable if PGI-C value at Week 48 in (1,2,3,4).

Source: biib141/valueaccess/amnog/t-pgi-propis-sgrp.sas:t-pgi-propis-sgrp-pcavus.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Summary of Proportion of Worsening in Patient Global Impression of Change (PGI-C) at Week 48 by Pes Cavus Status (Yes, No) - ITT Population

Pes Cavus status: Yes

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Number of subjects with week 48 assessment (%)	10 (100)	8 (100)
Number of subjects with event (%)	6 (60.0)	2 (25.0)
RR - Relative Risk (Omaveloxolone/Placebo)		0.42
95% CI		(0.11, 1.53)
p-value		0.1877
OR - Odds Ratio (Omaveloxolone/Placebo)		0.22
95% CI		(0.03, 1.71)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		-0.35
95% CI		(-0.78, 0.08)

Note 1: Worsening if PGI-C value at Week 48 in (5,6,7).

Source: biib141/valueaccess/amnog/t-pgi-propw-sgrp.sas:t-pgi-propw-sgrp-pcavus.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	0.02	0.02
SD	0.009	0.010
Week 24		
N	10	9
LS mean change from baseline	-0.0010	0.0000
SE	0.00088	0.00095
95% CI	(-0.0030, 0.0009)	(-0.0021, 0.0021)
LS mean difference (Omaveloxolone-Placebo)		0.0010
SE		0.00139
95% CI		(-0.0020, 0.0041)
p-value		0.4689
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.2934
95% CI		(-0.6120, 1.1987)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-dh.sas:t-9hpt-mmrm-dh-pcp.rtf **Data Tag:** FINAL **Run Date:** 27MAY2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 48		
N	10	8
LS mean change from baseline	-0.0003	-0.0003
SE	0.00097	0.00108
95% CI	(-0.0024, 0.0019)	(-0.0026, 0.0021)
LS mean difference (Omaveloxolone-Placebo)		0.0000
SE		0.00154
95% CI		(-0.0034, 0.0034)
p-value		0.9942
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.0030
95% CI		(-0.9267, 0.9327)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-dh.sas:t-9hpt-mmrm-dh-pcp.rtf **Data Tag:** FINAL **Run Date:** 27MAY2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	0.02	0.02
SD	0.007	0.010
Week 24		
N	10	9
LS mean change from baseline	-0.0012	-0.0006
SE	0.00078	0.00084
95% CI	(-0.0029, 0.0005)	(-0.0024, 0.0012)
LS mean difference (Omaveloxolone-Placebo)		0.0006
SE		0.00119
95% CI		(-0.0020, 0.0031)
p-value		0.6412
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.1911
95% CI		(-0.7115, 1.0937)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-ndh.sas:t-9hpt-mmrm-ndh-pcp.rtf **Data Tag:** FINAL **Run Date:** 27MAY2025

MOXie Part 2: Nine-Hole Peg Test (9-HPT) Reciprocal of Average Time (1/sec) Non-Dominant Hand LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 48		
N	10	8
LS mean change from baseline	0.0000	-0.0001
SE	0.00055	0.00062
95% CI	(-0.0012, 0.0012)	(-0.0014, 0.0013)
LS mean difference (Omaveloxolone-Placebo)		-0.0001
SE		0.00088
95% CI		(-0.0020, 0.0019)
p-value		0.9381
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.0319
95% CI		(-0.9616, 0.8979)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-ndh.sas:t-9hpt-mmrm-ndh-pcp.rtf **Data Tag:** FINAL **Run Date:** 27MAY2025

MOXie Part 2: Timed 25-Foot Walk Test (T25-FWT) LS Mean Change from Baseline of Reciprocal of Average Walk Time (1/sec) at Week 48 by Pes Cavus Status (Yes, No) from MMRM Analysis - ITT Population

Pes Cavus status: Yes

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	8
Mean score	0.15	0.11
SD	0.071	0.068
Week 48		
N	9	7
LS mean change from baseline	-0.02	-0.02
SE	0.011	0.013
95% CI	(-0.04, 0.00)	(-0.04, 0.01)
LS mean difference (Omaveloxolone-Placebo)		0.00
SE		0.016
95% CI		(-0.03, 0.04)
p-value		0.8427
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.09
95% CI		(-0.89, 1.08)

Note 1: LS Mean and Hedges' g are from the MMRM model for ITT population with three way interaction added (subgroup*treatment*time)

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-sgrp.sas:t-ftwk-mmrm-sgrp-pcavus.rtf **Data Tag:** FINAL **Run Date:** 18DEC2023

MOXie Part 2: Number of Falls from Baseline to Week 48 from Poisson Regression Model Analysis by Pes Cavus Status (Yes, No) - ITT Population

Pes Cavus status: Yes

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Total number of falls from baseline to week 48		
Mean	8.60	12.10
SD	10.480	15.300
Adjusted incidence rate of falls	0.03	0.04
95% CI	(0.01, 0.07)	(0.02, 0.09)
Rate ratio (Omaveloxolone/Placebo)		1.53
95% CI		(0.43, 5.49)
p-value		0.5129

Note 1: Incidence rate of falls and rate ratio are estimated from the Poisson model with Pes Cavus status as a covariate and the natural logarithm of time on study (days) as an offset term.

Source: biib141/valueaccess/amnog/t-fall-prm-sgrp.sas:t-fall-prm-sgrp-pcavus.rtf **Data Tag:** FINAL **Run Date:** 15DEC2023

MOXie Part 2: SF-36 Mental Component Summary (SF-36 MCS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	52.86	55.83
SD	9.183	6.386
Week 24		
N	10	9
LS mean change from baseline	-3.06	-0.23
SE	3.842	4.139
95% CI	(-11.23, 5.12)	(-9.02, 8.57)
LS mean difference (Omaveloxolone-Placebo)		2.83
SE		6.006
95% CI		(-9.89, 15.55)
p-value		0.6437
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.19
95% CI		(-0.71, 1.09)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-mcs.sas:t-sf36-mmrm-mcs-pcp.rtf **Data Tag:** FINAL **Run Date:** 23MAY2025

MOXie Part 2: SF-36 Mental Component Summary (SF-36 MCS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 48		
N	10	8
LS mean change from baseline	-1.34	-0.63
SE	2.518	2.802
95% CI	(-6.97, 4.28)	(-6.86, 5.61)
LS mean difference (Omaveloxolone-Placebo)		0.71
SE		4.180
95% CI		(-8.61, 10.04)
p-value		0.8677
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.07
95% CI		(-0.86, 1.00)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-mcs.sas:t-sf36-mmrm-mcs-pcp.rtf **Data Tag:** FINAL **Run Date:** 23MAY2025

MOXie Part 2: SF-36 Physical Component Summary (SF-36 PCS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Baseline		
N	10	10
Mean score	41.59	35.31
SD	6.267	9.311
Week 24		
N	10	9
LS mean change from baseline	-0.09	0.94
SE	1.913	2.083
95% CI	(-4.24, 4.07)	(-3.59, 5.46)
LS mean difference (Omaveloxolone-Placebo)		1.03
SE		3.071
95% CI		(-5.64, 7.69)
p-value		0.7439
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.13
95% CI		(-0.77, 1.03)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-pcs.sas:t-sf36-mmrm-pcs-pcp.rtf **Data Tag:** FINAL **Run Date:** 23MAY2025

MOXie Part 2: SF-36 Physical Component Summary (SF-36 PCS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Week 48		
N	10	8
LS mean change from baseline	-3.05	-4.27
SE	2.517	2.805
95% CI	(-8.50, 2.41)	(-10.34, 1.81)
LS mean difference (Omaveloxolone-Placebo)		-1.22
SE		3.968
95% CI		(-9.78, 7.34)
p-value		0.7634
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-1.06, 0.80)

NOTE 1: LS Mean is based on the MMRM model that includes site, baseline, treatment group, time, the interaction between treatment and time, and the interaction between baseline and time.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-pcs.sas:t-sf36-mmrm-pcs-pcp.rtf **Data Tag:** FINAL **Run Date:** 23MAY2025

MOXie Part 2: Summary of Proportion of Worsening in SF-36 Mental Component Summary (SF-36 MCS) at Week 48 with Imputation - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Number of subjects with week 48 assessment (%)	10 (100)	8 (80.0)
Number of subjects with event (%)	0	3 (30.0)
RR - Relative Risk (Omaveloxolone/Placebo)		7.00
95% CI		(0.41, 120.16)
p-value		0.1797
OR - Odds Ratio (Omaveloxolone/Placebo)		9.80
95% CI		(0.44, 219.25)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.27
95% CI		(-0.03, 0.57)

NOTE 1: Worsening if change from baseline at week 48 <= -9.6 in SF-36 MCS. Subject without week 48 assessment will be imputed as responder with a worsening event.

Source: biib141/valueaccess/amnog/t-sf36-propw-mcs-imp.sas:t-sf36-propw-mcs-imp-pcp.rtf **Data Tag:** FINAL **Run Date:** 13MAY2025

MOXie Part 2: Summary of Proportion of Worsening in SF-36 Physical Component Summary (SF-36 PCS) at Week 48 with Imputation - ITT with Pes Cavus Population

	Placebo (N=10)	Omaveloxolone 150 mg (N=10)
Number of subjects with week 48 assessment (%)	10 (100)	8 (80.0)
Number of subjects with event (%)	2 (20.0)	3 (30.0)
RR - Relative Risk (Omaveloxolone/Placebo)		1.50
95% CI		(0.32, 7.14)
p-value		0.6104
OR - Odds Ratio (Omaveloxolone/Placebo)		1.71
95% CI		(0.22, 13.41)
ARR - Absolute Risk Reduction (Omaveloxolone-Placebo)		0.10
95% CI		(-0.28, 0.48)

Source: biib141/valueaccess/amnog/t-sf36-propw-pcs-imp.sas:t-sf36-propw-pcs-imp-pcp.rtf **Data Tag:** FINAL **Run Date:** 13MAY2025

1. Anzahl der Patient*innen mit mindestens einem UE - Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem UE - Safety Population mit schwerem Pes Cavus					
n (%)	10 (100 %)	10 (100 %)	1,00 [0,833; 1,200] 1,0000	1,00 [0,018; 55,267] 1,0000	0,00 [0,000; 0,000] NA
Ja (%) Nein (%)	10 (100 %) 0 (0 %)	10 (100 %) 0 (0 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population mit schwerem Pes Cavus					
Infektionen und parasitäre Erkrankungen (SOC)	**3 (30 %)**	**7 (70 %)**	**0,43 [0,153; 1,201] 0,1067**	**0,18 [0,027; 1,244] 0,0821**	**-0,40 [-0,802; 0,002] 0,0506**
Bronchitis (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Febrile Infektion (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Gastrointestinalinfektion (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Nasopharyngitis (PT)	0 (0 %)	2 (20 %)	0,20 [0,011; 3,705] 0,2835	0,16 [0,007; 3,847] 0,2630	-0,20 [-0,448; 0,048] 0,1138
Otitis externa (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Sinusitis (PT)	0 (0 %)	2 (20 %)	0,20 [0,011; 3,705] 0,2835	0,16 [0,007; 3,847] 0,2630	-0,20 [-0,448; 0,048] 0,1138
Infektion der oberen Atemwege (PT)	1 (10 %)	5 (50 %)	0,20 [0,028; 1,420] 0,1074	0,11 [0,010; 1,236] 0,0735	-0,40 [-0,761; -0,039] 0,0298
Virusinfektion (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Erkrankungen des	**1 (10 %)**	**0 (0 %)**	**3,00 [0,137; 65,903] 0,4957**	**3,32 [0,120; 91,601] 0,4887**	**0,10 [-0,086; 0,286] 0,2958**

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Immunsystems (SOC)					
Allergie auf Arthropodenstich (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Stoffwechsel- und Ernährungsstörungen (SOC)	**0 (0 %)**	**1 (10 %)**	**0,33 [0,015; 7,323] 0,4957**	**0,30 [0,011; 8,332] 0,4887**	**-0,10 [-0,286; 0,086] 0,2958**
Hypercholesterinämie (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Psychiatrische Erkrankungen (SOC)	**1 (10 %)**	**2 (20 %)**	**0,50 [0,054; 4,672] 0,5545**	**0,44 [0,034; 5,880] 0,5494**	**-0,10 [-0,410; 0,210] 0,5380**
Depressive Verstimmung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Depression (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Schlaflosigkeit (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Erkrankungen des Nervensystems (SOC)	**8 (80 %)**	**6 (60 %)**	**1,33 [0,737; 2,414] 0,3475**	**2,67 [0,361; 19,712] 0,3419**	**0,20 [-0,192; 0,592] 0,3221**
Ataxie (PT)	1 (10 %)	1 (10 %)	1,00 [0,072; 13,868] 1,0000	1,00 [0,054; 18,574] 1,0000	0,00 [-0,263; 0,263] 1,0000
Gleichgewichtsstörung (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Schwindelgefühl (PT)	1 (10 %)	3 (30 %)	0,33 [0,041; 2,686] 0,3064	0,26 [0,022; 3,063] 0,2877	-0,20 [-0,539; 0,139] 0,2509
Kopfschmerzen (PT)	5 (50 %)	2 (20 %)	2,50 [0,625; 9,996] 0,1964	4,00 [0,550; 29,096] 0,1717	0,30 [-0,097; 0,697] 0,1387

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Hypoästhesie (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Migräne (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Spastik (PT)	2 (20 %)	0 (0 %)	5,00 [0,270; 92,622] 0,2835	6,18 [0,260; 146,777] 0,2630	0,20 [-0,048; 0,448] 0,1138
Parästhesie (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Somnolenz (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Synkope (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Tremor (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Erkrankungen des Ohrs und des Labyrinths (SOC)	**1 (10 %)**	**0 (0 %)**	**3,00 [0,137; 65,903] 0,4957**	**3,32 [0,120; 91,601] 0,4887**	**0,10 [-0,086; 0,286] 0,2958**
Vertigo (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Herzerkrankungen (SOC)	**0 (0 %)**	**1 (10 %)**	**0,33 [0,015; 7,323] 0,4957**	**0,30 [0,011; 8,332] 0,4887**	**-0,10 [-0,286; 0,086] 0,2958**
Angina pectoris (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Erkrankungen der Atemwege, des Brustraums und Mediastinums (SOC)	**4 (40 %)**	**1 (10 %)**	**4,00 [0,537; 29,805] 0,1770**	**6,00 [0,532; 67,649] 0,1476**	**0,30 [-0,056; 0,656] 0,0984**

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Husten (PT)	2 (20 %)	1 (10 %)	2,00 [0,214; 18,687] 0,5545	2,25 [0,170; 29,767] 0,5494	0,10 [-0,210; 0,410] 0,5380
Dyspnoe (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Epistaxis (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Nasenverstopfung (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Schmerzen im Oropharynx (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Erkrankungen des Gastrointestinaltrakts (SOC)	**6 (60 %)**	**6 (60 %)**	**1,00 [0,489; 2,046] 1,0000**	**1,00 [0,167; 5,985] 1,0000**	**0,00 [-0,429; 0,429] 1,0000**
Abdominalschmerz (PT)	2 (20 %)	2 (20 %)	1,00 [0,173; 5,772] 1,0000	1,00 [0,112; 8,947] 1,0000	0,00 [-0,351; 0,351] 1,0000
Diarrhö (PT)	3 (30 %)	0 (0 %)	7,00 [0,408; 120,157] 0,1808	9,80 [0,438; 219,247] 0,1505	0,30 [0,016; 0,584] 0,0381
Stuhlverfaerbung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Flatulenz (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Gastritis (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Übelkeit (PT)	3 (30 %)	2 (20 %)	1,50 [0,315; 7,137] 0,6229	1,71 [0,219; 13,406] 0,6200	0,10 [-0,277; 0,477] 0,6156
Erbrechen (PT)	1 (10 %)	2 (20 %)	0,50 [0,054; 4,672] 0,5545	0,44 [0,034; 5,880] 0,5494	-0,10 [-0,410; 0,210] 0,5380

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Erkrankungen der Haut und des Unterhautgewebes (SOC)	**3 (30 %)**	**1 (10 %)**	**3,00 [0,372; 24,171] 0,3064**	**3,86 [0,326; 45,570] 0,2877**	**0,20 [-0,139; 0,539] 0,2509**
Akne (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Ausschlag (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Makulöser Ausschlag (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Urtikaria (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**6 (60 %)**	**4 (40 %)**	**1,50 [0,602; 3,735] 0,3905**	**2,25 [0,376; 13,465] 0,3809**	**0,20 [-0,229; 0,629] 0,3674**
Arthralgie (PT)	2 (20 %)	1 (10 %)	2,00 [0,214; 18,687] 0,5545	2,25 [0,170; 29,767] 0,5494	0,10 [-0,210; 0,410] 0,5380
Rückenschmerzen (PT)	3 (30 %)	2 (20 %)	1,50 [0,315; 7,137] 0,6229	1,71 [0,219; 13,406] 0,6200	0,10 [-0,277; 0,477] 0,6156
Knochenschmerzen (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Muskelspasmen (PT)	2 (20 %)	1 (10 %)	2,00 [0,214; 18,687] 0,5545	2,25 [0,170; 29,767] 0,5494	0,10 [-0,210; 0,410] 0,5380
Schmerz in einer Extremität (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Skoliose (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Erkrankungen der Nieren und Harnwege (SOC)	**2 (20 %)**	**2 (20 %)**	**1,00 [0,173; 5,772] 1,0000**	**1,00 [0,112; 8,947] 1,0000**	**0,00 [-0,351; 0,351] 1,0000**
Pollakisurie (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Harnverhaltung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Harninkontinenz (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Harnfluss vermindert (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Erkrankungen der Geschlechtsorgane und der Brustdrüse (SOC)	**2 (20 %)**	**0 (0 %)**	**5,00 [0,270; 92,622] 0,2835**	**6,18 [0,260; 146,777] 0,2630**	**0,20 [-0,048; 0,448] 0,1138**
Menorrhagie (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Eierstockzyste rupturiert (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Allgemeine Erkrankungen und Beschwerden am Verabreichungsort (SOC)	**3 (30 %)**	**6 (60 %)**	**0,50 [0,171; 1,463] 0,2073**	**0,29 [0,045; 1,821] 0,1860**	**-0,30 [-0,716; 0,116] 0,1579**
Asthenie (PT)	1 (10 %)	1 (10 %)	1,00 [0,072; 13,868] 1,0000	1,00 [0,054; 18,574] 1,0000	0,00 [-0,263; 0,263] 1,0000
Schüttelfrost (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Zyste (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Ermüdung (PT)	2 (20 %)	4 (40 %)	0,50 [0,117; 2,139] 0,3557	0,38 [0,051; 2,772] 0,3419	-0,20 [-0,592; 0,192] 0,3221
Waermegefuehl (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Thoraxschmerz nicht kardialen Ursprungs (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Untersuchungen (SOC)	**5 (50 %)**	**1 (10 %)**	**5,00 [0,704; 35,495] 0,1074**	**9,00 [0,809; 100,139] 0,0735**	**0,40 [0,039; 0,761] 0,0298**
Alaninaminotransferase erhöht (PT)	4 (40 %)	0 (0 %)	9,00 [0,547; 147,952] 0,1240	14,54 [0,667; 316,694] 0,0883	0,40 [0,096; 0,704] 0,0098
Aspartataminotransferase erhöht (PT)	2 (20 %)	0 (0 %)	5,00 [0,270; 92,622] 0,2835	6,18 [0,260; 146,777] 0,2630	0,20 [-0,048; 0,448] 0,1138
Kreatinphosphokinase im Blut erhöht (PT)	1 (10 %)	1 (10 %)	1,00 [0,072; 13,868] 1,0000	1,00 [0,054; 18,574] 1,0000	0,00 [-0,263; 0,263] 1,0000
Eisen im Blut erniedrigt (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Verletzung, Vergiftung und durch Eingriffe bedingte Komplikationen (SOC)	**5 (50 %)**	**6 (60 %)**	**0,83 [0,374; 1,855] 0,6682**	**0,67 [0,113; 3,919] 0,6667**	**-0,10 [-0,534; 0,334] 0,6644**
Knöchelfraktur (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Gehirnerschütterung (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Kontusion (PT)	2 (20 %)	4 (40 %)	0,50 [0,117; 2,139] 0,3557	0,38 [0,051; 2,772] 0,3419	-0,20 [-0,592; 0,192] 0,3221
Exkoriation (PT)	2 (20 %)	3 (30 %)	0,67 [0,140; 3,172] 0,6229	0,58 [0,075; 4,562] 0,6200	-0,10 [-0,477; 0,277] 0,6156
Fraktur des Fußes (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Kopfverletzung (PT)	0 (0 %)	2 (20 %)	0,20 [0,011; 3,705] 0,2835	0,16 [0,007; 3,847] 0,2630	-0,20 [-0,448; 0,048] 0,1138
Gelenksverletzung (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Risswunde (PT)	1 (10 %)	2 (20 %)	0,50 [0,054; 4,672] 0,5545	0,44 [0,034; 5,880] 0,5494	-0,10 [-0,410; 0,210] 0,5380
Bänderzerrung (PT)	2 (20 %)	1 (10 %)	2,00 [0,214; 18,687] 0,5545	2,25 [0,170; 29,767] 0,5494	0,10 [-0,210; 0,410] 0,5380
Verletzung der Gliedmaßen (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Muskelzerrung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Schmerzen während eines Eingriffes (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Zungenverletzung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958

Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.

1. Anzahl der Patient*innen mit mindestens einem milden UE - Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem milden UE - Safety Population mit schwerem Pes Cavus					
n (%)	10 (100 %)	10 (100 %)	1,00 [0,833; 1,200] 1,0000	1,00 [0,018; 55,267] 1,0000	0,00 [0,000; 0,000] NA
Ja (%) Nein (%)	10 (100 %) 0 (0 %)	10 (100 %) 0 (0 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem milden UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population mit schwerem Pes Cavus					
Infektionen und parasitäre Erkrankungen (SOC)	**3 (30 %)**	**6 (60 %)**	**0,50 [0,171; 1,463] 0,2073**	**0,29 [0,045; 1,821] 0,1860**	**-0,30 [-0,716; 0,116] 0,1579**
Bronchitis (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Febrile Infektion (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Gastrointestinalinfektion (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Nasopharyngitis (PT)	0 (0 %)	2 (20 %)	0,20 [0,011; 3,705] 0,2835	0,16 [0,007; 3,847] 0,2630	-0,20 [-0,448; 0,048] 0,1138
Otitis externa (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Sinusitis (PT)	0 (0 %)	2 (20 %)	0,20 [0,011; 3,705] 0,2835	0,16 [0,007; 3,847] 0,2630	-0,20 [-0,448; 0,048] 0,1138
Infektion der oberen Atemwege (PT)	1 (10 %)	3 (30 %)	0,33 [0,041; 2,686] 0,3064	0,26 [0,022; 3,063] 0,2877	-0,20 [-0,539; 0,139] 0,2509
Erkrankungen des Immunsystems (SOC)	**1 (10 %)**	**0 (0 %)**	**3,00 [0,137; 65,903] 0,4957**	**3,32 [0,120; 91,601] 0,4887**	**0,10 [-0,086; 0,286] 0,2958**

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Allergie auf Arthropodenstich (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Stoffwechsel- und Ernährungsstörungen (SOC)	**0 (0 %)**	**1 (10 %)**	**0,33 [0,015; 7,323] 0,4957**	**0,30 [0,011; 8,332] 0,4887**	**-0,10 [-0,286; 0,086] 0,2958**
Hypercholesterinämie (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Psychiatrische Erkrankungen (SOC)	**1 (10 %)**	**2 (20 %)**	**0,50 [0,054; 4,672] 0,5545**	**0,44 [0,034; 5,880] 0,5494**	**-0,10 [-0,410; 0,210] 0,5380**
Depressive Verstimmung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Depression (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Schlaflosigkeit (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Erkrankungen des Nervensystems (SOC)	**8 (80 %)**	**6 (60 %)**	**1,33 [0,737; 2,414] 0,3475**	**2,67 [0,361; 19,712] 0,3419**	**0,20 [-0,192; 0,592] 0,3221**
Ataxie (PT)	1 (10 %)	1 (10 %)	1,00 [0,072; 13,868] 1,0000	1,00 [0,054; 18,574] 1,0000	0,00 [-0,263; 0,263] 1,0000
Gleichgewichtsstörung (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Schwindelgefühl (PT)	1 (10 %)	3 (30 %)	0,33 [0,041; 2,686] 0,3064	0,26 [0,022; 3,063] 0,2877	-0,20 [-0,539; 0,139] 0,2509
Kopfschmerzen (PT)	5 (50 %)	2 (20 %)	2,50 [0,625; 9,996] 0,1964	4,00 [0,550; 29,096] 0,1717	0,30 [-0,097; 0,697] 0,1387
Hypoästhesie (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Spastik (PT)	2 (20 %)	0 (0 %)	5,00 [0,270; 92,622] 0,2835	6,18 [0,260; 146,777] 0,2630	0,20 [-0,048; 0,448] 0,1138
Parästhesie (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Somnolenz (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Synkope (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Tremor (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Erkrankungen des Ohrs und des Labyrinths (SOC)	**1 (10 %)**	**0 (0 %)**	**3,00 [0,137; 65,903] 0,4957**	**3,32 [0,120; 91,601] 0,4887**	**0,10 [-0,086; 0,286] 0,2958**
Vertigo (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Herzerkrankungen (SOC)	**0 (0 %)**	**1 (10 %)**	**0,33 [0,015; 7,323] 0,4957**	**0,30 [0,011; 8,332] 0,4887**	**-0,10 [-0,286; 0,086] 0,2958**
Angina pectoris (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Erkrankungen der Atemwege, des Brustraums und Mediastinums (SOC)	**3 (30 %)**	**1 (10 %)**	**3,00 [0,372; 24,171] 0,3064**	**3,86 [0,326; 45,570] 0,2877**	**0,20 [-0,139; 0,539] 0,2509**
Husten (PT)	1 (10 %)	1 (10 %)	1,00 [0,072; 13,868] 1,0000	1,00 [0,054; 18,574] 1,0000	0,00 [-0,263; 0,263] 1,0000
Dyspnoe (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Epistaxis (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Nasenverstopfung (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Schmerzen im Oropharynx (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Erkrankungen des Gastrointestinaltrakts (SOC)	**6 (60 %)**	**6 (60 %)**	**1,00 [0,489; 2,046] 1,0000**	**1,00 [0,167; 5,985] 1,0000**	**0,00 [-0,429; 0,429] 1,0000**
Abdominalschmerz (PT)	2 (20 %)	2 (20 %)	1,00 [0,173; 5,772] 1,0000	1,00 [0,112; 8,947] 1,0000	0,00 [-0,351; 0,351] 1,0000
Diarrhö (PT)	3 (30 %)	0 (0 %)	7,00 [0,408; 120,157] 0,1808	9,80 [0,438; 219,247] 0,1505	0,30 [0,016; 0,584] 0,0381
Stuhlverfaerbung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Flatulenz (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Gastritis (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Übelkeit (PT)	2 (20 %)	2 (20 %)	1,00 [0,173; 5,772] 1,0000	1,00 [0,112; 8,947] 1,0000	0,00 [-0,351; 0,351] 1,0000
Erbrechen (PT)	0 (0 %)	2 (20 %)	0,20 [0,011; 3,705] 0,2835	0,16 [0,007; 3,847] 0,2630	-0,20 [-0,448; 0,048] 0,1138
Erkrankungen der Haut und des Unterhautgewebes (SOC)	**3 (30 %)**	**1 (10 %)**	**3,00 [0,372; 24,171] 0,3064**	**3,86 [0,326; 45,570] 0,2877**	**0,20 [-0,139; 0,539] 0,2509**

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Akne (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Ausschlag (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Makulöser Ausschlag (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Urtikaria (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**6 (60 %)**	**3 (30 %)**	**2,00 [0,684; 5,851] 0,2073**	**3,50 [0,549; 22,304] 0,1860**	**0,30 [-0,116; 0,716] 0,1579**
Arthralgie (PT)	2 (20 %)	1 (10 %)	2,00 [0,214; 18,687] 0,5545	2,25 [0,170; 29,767] 0,5494	0,10 [-0,210; 0,410] 0,5380
Rückenschmerzen (PT)	3 (30 %)	1 (10 %)	3,00 [0,372; 24,171] 0,3064	3,86 [0,326; 45,570] 0,2877	0,20 [-0,139; 0,539] 0,2509
Knochenschmerzen (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Muskelspasmen (PT)	2 (20 %)	1 (10 %)	2,00 [0,214; 18,687] 0,5545	2,25 [0,170; 29,767] 0,5494	0,10 [-0,210; 0,410] 0,5380
Schmerz in einer Extremität (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Erkrankungen der Nieren und Harnwege (SOC)	**2 (20 %)**	**2 (20 %)**	**1,00 [0,173; 5,772] 1,0000**	**1,00 [0,112; 8,947] 1,0000**	**0,00 [-0,351; 0,351] 1,0000**
Pollakisurie (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Harnverhaltung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Harninkontinenz (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Harnfluss vermindert (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Erkrankungen der Geschlechtsorgane und der Brustdrüse (SOC)	**2 (20 %)**	**0 (0 %)**	**5,00 [0,270; 92,622] 0,2835**	**6,18 [0,260; 146,777] 0,2630**	**0,20 [-0,048; 0,448] 0,1138**
Menorrhagie (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Eierstockzyste rupturiert (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Allgemeine Erkrankungen und Beschwerden am Verabreichungsort (SOC)	**3 (30 %)**	**5 (50 %)**	**0,60 [0,194; 1,860] 0,3829**	**0,43 [0,068; 2,684] 0,3716**	**-0,20 [-0,620; 0,220] 0,3569**
Asthenie (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Schüttelfrost (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Zyste (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Ermüdung (PT)	2 (20 %)	3 (30 %)	0,67 [0,140; 3,172] 0,6229	0,58 [0,075; 4,562] 0,6200	-0,10 [-0,477; 0,277] 0,6156

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Waermegefuehl (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Thoraxschmerz nicht kardialen Ursprungs (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Untersuchungen (SOC)	**5 (50 %)**	**0 (0 %)**	**11,00 [0,688; 175,863] 0,0897**	**21,00 [0,972; 453,912] 0,0518**	**0,50 [0,190; 0,810] 0,0016**
Alaninaminotransferase erhöht (PT)	4 (40 %)	0 (0 %)	9,00 [0,547; 147,952] 0,1240	14,54 [0,667; 316,694] 0,0883	0,40 [0,096; 0,704] 0,0098
Aspartataminotransferase erhöht (PT)	2 (20 %)	0 (0 %)	5,00 [0,270; 92,622] 0,2835	6,18 [0,260; 146,777] 0,2630	0,20 [-0,048; 0,448] 0,1138
Kreatinphosphokinase im Blut erhöht (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Eisen im Blut erniedrigt (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Verletzung, Vergiftung und durch Eingriffe bedingte Komplikationen (SOC)	**4 (40 %)**	**6 (60 %)**	**0,67 [0,268; 1,660] 0,3905**	**0,44 [0,074; 2,660] 0,3809**	**-0,20 [-0,629; 0,229] 0,3674**
Gehirnerschütterung (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Kontusion (PT)	2 (20 %)	4 (40 %)	0,50 [0,117; 2,139] 0,3557	0,38 [0,051; 2,772] 0,3419	-0,20 [-0,592; 0,192] 0,3221
Exkoriation (PT)	2 (20 %)	3 (30 %)	0,67 [0,140; 3,172] 0,6229	0,58 [0,075; 4,562] 0,6200	-0,10 [-0,477; 0,277] 0,6156
Fraktur des Fußes (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Kopfverletzung (PT)	0 (0 %)	2 (20 %)	0,20 [0,011; 3,705] 0,2835	0,16 [0,007; 3,847] 0,2630	-0,20 [-0,448; 0,048] 0,1138
Gelenksverletzung (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Risswunde (PT)	0 (0 %)	2 (20 %)	0,20 [0,011; 3,705] 0,2835	0,16 [0,007; 3,847] 0,2630	-0,20 [-0,448; 0,048] 0,1138
Bänderzerrung (PT)	2 (20 %)	0 (0 %)	5,00 [0,270; 92,622] 0,2835	6,18 [0,260; 146,777] 0,2630	0,20 [-0,048; 0,448] 0,1138
Verletzung der Gliedmaßen (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Muskelzerrung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Zungenverletzung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958

Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.

1. Anzahl der Patient*innen mit mindestens einem moderaten UE - Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem moderaten UE - Safety Population mit schwerem Pes Cavus					
n (%)	10 (100 %)	10 (100 %)	0,60 [0,194; 1,860] 0,3829	0,43 [0,068; 2,684] 0,3716	-0,20 [-0,620; 0,220] 0,3569
Ja (%) Nein (%)	3 (30 %) 7 (70 %)	5 (50 %) 5 (50 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem moderaten UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem moderaten UE bei mindestens 10% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population mit schwerem Pes Cavus					
Infektionen und parasitäre Erkrankungen (SOC)	**0 (0 %)**	**2 (20 %)**	**0,20 [0,011; 3,705] 0,2835**	**0,16 [0,007; 3,847] 0,2630**	**-0,20 [-0,448; 0,048] 0,1138**
Infektion der oberen Atemwege (PT)	0 (0 %)	2 (20 %)	0,20 [0,011; 3,705] 0,2835	0,16 [0,007; 3,847] 0,2630	-0,20 [-0,448; 0,048] 0,1138
Virusinfektion (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Erkrankungen des Nervensystems (SOC)	**1 (10 %)**	**1 (10 %)**	**1,00 [0,072; 13,868] 1,0000**	**1,00 [0,054; 18,574] 1,0000**	**0,00 [-0,263; 0,263] 1,0000**
Migräne (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Synkope (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Erkrankungen der Atemwege, des Brustraums und Mediastinums (SOC)	**1 (10 %)**	**0 (0 %)**	**3,00 [0,137; 65,903] 0,4957**	**3,32 [0,120; 91,601] 0,4887**	**0,10 [-0,086; 0,286] 0,2958**
Husten (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Erkrankungen des Gastrointestinaltrakts (SOC)	**1 (10 %)**	**0 (0 %)**	**3,00 [0,137; 65,903] 0,4957**	**3,32 [0,120; 91,601] 0,4887**	**0,10 [-0,086; 0,286] 0,2958**
Übelkeit (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Erbrechen (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Skelettmuskulatur-, Bindegewebs- und Knochenerkrankungen (SOC)	**1 (10 %)**	**1 (10 %)**	**1,00 [0,072; 13,868] 1,0000**	**1,00 [0,054; 18,574] 1,0000**	**0,00 [-0,263; 0,263] 1,0000**
Rückenschmerzen (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Skoliose (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Allgemeine Erkrankungen und Beschwerden am Verabreichungsort (SOC)	**0 (0 %)**	**2 (20 %)**	**0,20 [0,011; 3,705] 0,2835**	**0,16 [0,007; 3,847] 0,2630**	**-0,20 [-0,448; 0,048] 0,1138**
Asthenie (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Ermüdung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Untersuchungen (SOC)	**0 (0 %)**	**1 (10 %)**	**0,33 [0,015; 7,323] 0,4957**	**0,30 [0,011; 8,332] 0,4887**	**-0,10 [-0,286; 0,086] 0,2958**
Kreatinphosphokinase im Blut erhöht (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Verletzung, Vergiftung und durch Eingriffe bedingte Komplikationen (SOC)	**1 (10 %)**	**2 (20 %)**	**0,50 [0,054; 4,672] 0,5545**	**0,44 [0,034; 5,880] 0,5494**	**-0,10 [-0,410; 0,210] 0,5380**
Knöchelfraktur (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Risswunde (PT)	1 (10 %)	0 (0 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Bänderzerrung (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Schmerzen während eines Eingriffes (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958

Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.

1. Anzahl der Patient*innen mit mindestens einem schweren UE – Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem schweren UE – Safety Population mit schwerem Pes Cavus					
Es traten keine Events auf.					
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

1. Anzahl der Patient*innen mit mindestens einem SUE - Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem SUE - Safety Population mit schwerem Pes Cavus					
n (%)	10 (100 %)	10 (100 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Ja (%) Nein (%)	0 (0 %) 10 (100 %)	1 (10 %) 9 (90 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit mindestens einem SUE bei mindestens 5% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit mindestens einem SUE bei mindestens 5% der Patient*innen oder mindestens 10 Patient*innen aufgetretene SOC und PT in einem Arm - Safety Population mit schwerem Pes Cavus					
Verletzung, Vergiftung und durch Eingriffe bedingte Komplikationen (SOC)	**0 (0 %)**	**1 (10 %)**	**0,33 [0,015; 7,323] 0,4957**	**0,30 [0,011; 8,332] 0,4887**	**-0,10 [-0,286; 0,086] 0,2958**
Knöchelfraktur (PT)	0 (0 %)	1 (10 %)	0,33 [0,015; 7,323] 0,4957	0,30 [0,011; 8,332] 0,4887	-0,10 [-0,286; 0,086] 0,2958
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

1. Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE - Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	Behandlungsarm		Omaveloxolon vs. Placebo Effektmaß [95 %-KI] p-Wert		
	Omaveloxolon	Placebo			
N	**10**	**10**	**RR**	**OR**	**ARR**
Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE - Safety Population mit schwerem Pes Cavus					
n (%)	10 (100 %)	10 (100 %)	3,00 [0,137; 65,903] 0,4957	3,32 [0,120; 91,601] 0,4887	0,10 [-0,086; 0,286] 0,2958
Ja (%) Nein (%)	1 (10 %) 9 (90 %)	0 (0 %) 10 (100 %)			
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.					

2. Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE nach allen SOC und PT – deskriptive Darstellung, n(%) - Safety Population mit schwerem Pes Cavus: Analyse

408-C-1402-PT2	**Behandlungsarm**	
	Omaveloxolon	**Placebo**
N	**10**	**10**
Anzahl der Patient*innen mit Therapieabbruch aufgrund von UE nach allen SOC und PT – deskriptive Darstellung, n(%) - Safety Population mit schwerem Pes Cavus		
Untersuchungen (SOC)	**1 (10 %)**	**0 (0 %)**
Alaninaminotransferase erhöht (PT)	1 (10 %)	0 (0 %)
Aspartataminotransferase erhöht (PT)	1 (10 %)	0 (0 %)
Für die Bedeutung von Abkürzungen siehe Abkürzungsverzeichnis.		