

Omaveloxolon (Skyclarys™)

Biogen GmbH

Anhang 4-G zu Modul 4A und 4B

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

Stand: 01.07.2025

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1. Gait-Score > 2

1.1 Morbidität - ITT-Population ohne schweren Pes cavus

1.1.1 Krankheitsschwere (mFARS)

MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Greater Than 2

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Baseline		
N	16	16
Mean score	48.36	50.06
SD	9.355	6.474
Week 4		
N	16	16
LS mean change from baseline	-1.19	-2.64
SE	1.143	1.132
95% CI	(-3.55, 1.17)	(-4.99, -0.30)
LS mean difference (Omaveloxolone-Placebo)		-1.45
SE		1.692
95% CI		(-4.94, 2.03)
p-value		0.3986
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.28
95% CI		(-0.98, 0.41)
Week 12		
N	16	16
LS mean change from baseline	-0.12	-1.65
SE	1.283	1.274
95% CI	(-2.79, 2.55)	(-4.30, 1.01)
LS mean difference (Omaveloxolone-Placebo)		-1.53
SE		1.883
95% CI		(-5.44, 2.38)
p-value		0.4259
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.26
95% CI		(-0.96, 0.43)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-itt-npc-byvis-gait.sas:t-mfars93-mmrm-itt-npc-byvis-gait-gt2.rtf Data Tag: FINAL Run Date: 03JAN2025

MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Greater Than 2

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 18		
N	15	15
LS mean change from baseline	1.13	-2.67
SE	1.376	1.365
95% CI	(-1.72, 3.97)	(-5.49, 0.16)
LS mean difference (Omaveloxolone-Placebo)		-3.79
SE		2.008
95% CI		(-7.94, 0.35)
p-value		0.0711
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.64
95% CI		(-1.37, 0.10)
Week 24		
N	15	14
LS mean change from baseline	0.57	-2.65
SE	1.061	1.080
95% CI	(-1.66, 2.81)	(-4.92, -0.37)
LS mean difference (Omaveloxolone-Placebo)		-3.22
SE		1.598
95% CI		(-6.57, 0.13)
p-value		0.0587
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.68
95% CI		(-1.43, 0.07)
Week 36		
N	15	14
LS mean change from baseline	0.27	-2.61
SE	0.896	0.922
95% CI	(-1.64, 2.18)	(-4.57, -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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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	Placebo (N=16)	Omaperoxolone 150 mg (N=16)
LS mean difference (Omaperoxolone-Placebo)		-2.88
SE		1.382
95% CI		(-5.80, 0.04)
p-value		0.0529
Hedge's g standardized mean difference (Omaperoxolone-Placebo)		-0.70
95% CI		(-1.45, 0.05)
Week 48		
N	15	14
LS mean change from baseline	1.50	-3.28
SE	0.883	0.902
95% CI	(-0.36, 3.37)	(-5.18, -1.38)
LS mean difference (Omaperoxolone-Placebo)		-4.78
SE		1.361
95% CI		(-7.64, -1.92)
p-value		0.0025
Hedge's g standardized mean difference (Omaperoxolone-Placebo)		-1.18
95% CI		(-1.97, -0.39)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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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 Population of Gait Score Greater Than 2

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Baseline		
N	16	16
Mean score	0.83	0.69
SD	0.617	0.403
Week 4		
N	16	16
LS mean change from baseline	0.00	-0.14
SE	0.101	0.100
95% CI	(-0.21, 0.21)	(-0.35, 0.07)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.149
95% CI		(-0.45, 0.17)
p-value		0.3676
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.30
95% CI		(-1.00, 0.40)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 12		
N	16	16
LS mean change from baseline	0.00	-0.11
SE	0.066	0.065
95% CI	(-0.13, 0.14)	(-0.25, 0.02)
LS mean difference (Omaveloxolone-Placebo)		-0.12
SE		0.101
95% CI		(-0.33, 0.10)
p-value		0.2663
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.37
95% CI		(-1.07, 0.33)
Week 18		
N	15	15
LS mean change from baseline	0.07	-0.12
SE	0.079	0.079
95% CI	(-0.09, 0.24)	(-0.29, 0.04)
LS mean difference (Omaveloxolone-Placebo)		-0.20
SE		0.119
95% CI		(-0.45, 0.05)
p-value		0.1170
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.55
95% CI		(-1.28, 0.18)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 24		
N	15	14
LS mean change from baseline	0.24	-0.20
SE	0.073	0.077
95% CI	(0.09, 0.39)	(-0.36, -0.04)
LS mean difference (Omaveloxolone-Placebo)		-0.44
SE		0.114
95% CI		(-0.68, -0.20)
p-value		0.0009
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-1.31
95% CI		(-2.12, -0.51)
Week 36		
N	15	14
LS mean change from baseline	-0.09	-0.12
SE	0.100	0.102
95% CI	(-0.30, 0.12)	(-0.33, 0.09)
LS mean difference (Omaveloxolone-Placebo)		-0.03
SE		0.149
95% CI		(-0.33, 0.28)
p-value		0.8583
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.06
95% CI		(-0.79, 0.67)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 48		
N	15	14
LS mean change from baseline	-0.12	-0.21
SE	0.099	0.104
95% CI	(-0.33, 0.09)	(-0.42, 0.01)
LS mean difference (Omaveloxolone-Placebo)		-0.08
SE		0.150
95% CI		(-0.40, 0.23)
p-value		0.5792
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.19
95% CI		(-0.92, 0.54)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Coordination Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Greater Than 2

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	Placebo (N=16)	Omaperoxolone 150 mg (N=16)
Baseline		
N	16	16
Mean score	11.67	12.83
SD	4.936	3.676
Week 4		
N	16	16
LS mean change from baseline	-0.92	-1.35
SE	0.684	0.679
95% CI	(-2.33, 0.50)	(-2.76, 0.06)
LS mean difference (Omaperoxolone-Placebo)		-0.44
SE		1.015
95% CI		(-2.54, 1.66)
p-value		0.6696
Hedge's g standardized mean difference (Omaperoxolone-Placebo)		-0.14
95% CI		(-0.84, 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Coordination Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Greater Than 2

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 12		
N	16	16
LS mean change from baseline	0.01	-1.13
SE	0.648	0.642
95% CI	(-1.34, 1.35)	(-2.47, 0.20)
LS mean difference (Omaveloxolone-Placebo)		-1.14
SE		0.965
95% CI		(-3.14, 0.86)
p-value		0.2502
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.38
95% CI		(-1.08, 0.31)
Week 18		
N	15	15
LS mean change from baseline	0.81	-1.24
SE	0.770	0.767
95% CI	(-0.78, 2.40)	(-2.83, 0.34)
LS mean difference (Omaveloxolone-Placebo)		-2.05
SE		1.130
95% CI		(-4.39, 0.28)
p-value		0.0817
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.61
95% CI		(-1.35, 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Coordination Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Greater Than 2

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 24		
N	15	14
LS mean change from baseline	-0.12	-1.10
SE	0.477	0.496
95% CI	(-1.13, 0.88)	(-2.14, -0.06)
LS mean difference (Omaveloxolone-Placebo)		-0.98
SE		0.749
95% CI		(-2.55, 0.59)
p-value		0.2062
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.44
95% CI		(-1.18, 0.29)
Week 36		
N	15	14
LS mean change from baseline	-0.34	-1.42
SE	0.555	0.565
95% CI	(-1.53, 0.85)	(-2.62, -0.22)
LS mean difference (Omaveloxolone-Placebo)		-1.08
SE		0.849
95% CI		(-2.88, 0.72)
p-value		0.2216
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.42
95% CI		(-1.16, 0.31)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Coordination Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Greater Than 2

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 48		
N	15	14
LS mean change from baseline	0.62	-1.98
SE	0.649	0.664
95% CI	(-0.73, 1.96)	(-3.35, -0.61)
LS mean difference (Omaveloxolone-Placebo)		-2.59
SE		0.977
95% CI		(-4.61, -0.58)
p-value		0.0137
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.91
95% CI		(-1.68, -0.15)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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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 of Gait Score Greater Than 2

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Baseline		
N	16	16
Mean score	7.44	7.11
SD	2.087	3.075
Week 4		
N	16	16
LS mean change from baseline	-0.46	-0.48
SE	0.570	0.562
95% CI	(-1.64, 0.72)	(-1.65, 0.69)
LS mean difference (Omaveloxolone-Placebo)		-0.02
SE		0.882
95% CI		(-1.84, 1.81)
p-value		0.9848
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.01
95% CI		(-0.70, 0.69)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-npc-gait.sas:t-mfars93-d-ll-mmrm-itt-npc-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

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 of Gait Score Greater Than 2

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 12		
N	16	16
LS mean change from baseline	-0.25	0.04
SE	0.539	0.531
95% CI	(-1.37, 0.87)	(-1.07, 1.14)
LS mean difference (Omaveloxolone-Placebo)		0.29
SE		0.843
95% CI		(-1.46, 2.04)
p-value		0.7363
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.11
95% CI		(-0.58, 0.80)
Week 18		
N	15	15
LS mean change from baseline	0.05	-0.43
SE	0.641	0.635
95% CI	(-1.27, 1.38)	(-1.74, 0.88)
LS mean difference (Omaveloxolone-Placebo)		-0.48
SE		0.975
95% CI		(-2.49, 1.53)
p-value		0.6263
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.17
95% CI		(-0.88, 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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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 of Gait Score Greater Than 2

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 24		
N	15	14
LS mean change from baseline	0.45	-0.26
SE	0.570	0.587
95% CI	(-0.74, 1.65)	(-1.49, 0.96)
LS mean difference (Omaveloxolone-Placebo)		-0.72
SE		0.896
95% CI		(-2.59, 1.16)
p-value		0.4326
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.27
95% CI		(-1.00, 0.46)
Week 36		
N	15	14
LS mean change from baseline	0.15	0.04
SE	0.642	0.648
95% CI	(-1.18, 1.49)	(-1.31, 1.38)
LS mean difference (Omaveloxolone-Placebo)		-0.11
SE		0.984
95% CI		(-2.15, 1.92)
p-value		0.9087
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.04
95% CI		(-0.77, 0.69)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-npc-gait.sas:t-mfars93-d-ll-mmrm-itt-npc-gait-gt2.rtf Data Tag: FINAL Run Date: 03JAN2025

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 of Gait Score Greater Than 2

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	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 48		
N	15	14
LS mean change from baseline	0.47	-0.36
SE	0.662	0.677
95% CI	(-0.96, 1.90)	(-1.81, 1.10)
LS mean difference (Omaveloxolone-Placebo)		-0.83
SE		1.016
95% CI		(-3.00, 1.35)
p-value		0.4287
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.27
95% CI		(-1.00, 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-npc-gait.sas:t-mfars93-d-ll-mmrm-itt-npc-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

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 of Gait Score Greater Than 2

Page: 1 of 4

	Placebo (N=16)	Omaperoxolone 150 mg (N=16)
Baseline		
N	16	16
Mean score	28.43	29.42
SD	4.909	2.709
Week 4		
N	16	16
LS mean change from baseline	-0.22	-0.31
SE	0.502	0.492
95% CI	(-1.27, 0.83)	(-1.34, 0.72)
LS mean difference (Omaperoxolone-Placebo)		-0.09
SE		0.777
95% CI		(-1.71, 1.54)
p-value		0.9130
Hedge's g standardized mean difference (Omaperoxolone-Placebo)		-0.04
95% CI		(-0.73, 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-npc-gait.sas:t-mfars93-d-us-mmrm-itt-npc-gait-gt2.rtf Data Tag: FINAL Run Date: 03JAN2025

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 of Gait Score Greater Than 2

Page: 2 of 4

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 12		
N	16	16
LS mean change from baseline	-0.18	-0.17
SE	0.575	0.567
95% CI	(-1.38, 1.02)	(-1.35, 1.01)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.874
95% CI		(-1.81, 1.82)
p-value		0.9939
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.00
95% CI		(-0.69, 0.70)
Week 18		
N	15	15
LS mean change from baseline	-0.20	-0.52
SE	0.528	0.524
95% CI	(-1.30, 0.90)	(-1.61, 0.58)
LS mean difference (Omaveloxolone-Placebo)		-0.32
SE		0.814
95% CI		(-2.01, 1.38)
p-value		0.7019
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-0.85, 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-npc-gait.sas:t-mfars93-d-us-mmrm-itt-npc-gait-gt2.rtf Data Tag: FINAL Run Date: 03JAN2025

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 of Gait Score Greater Than 2

Page: 3 of 4

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 24		
N	15	14
LS mean change from baseline	-0.38	-0.90
SE	0.612	0.610
95% CI	(-1.65, 0.90)	(-2.17, 0.37)
LS mean difference (Omaveloxolone-Placebo)		-0.53
SE		0.925
95% CI		(-2.45, 1.39)
p-value		0.5740
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.19
95% CI		(-0.92, 0.54)
Week 36		
N	15	14
LS mean change from baseline	0.17	-0.71
SE	0.548	0.553
95% CI	(-0.99, 1.33)	(-1.87, 0.46)
LS mean difference (Omaveloxolone-Placebo)		-0.88
SE		0.846
95% CI		(-2.65, 0.89)
p-value		0.3122
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.35
95% CI		(-1.08, 0.38)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-npc-gait.sas:t-mfars93-d-us-mmrm-itt-npc-gait-gt2.rtf Data Tag: FINAL Run Date: 03JAN2025

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 of Gait Score Greater Than 2

Page: 4 of 4

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 48		
N	15	14
LS mean change from baseline	0.22	-0.36
SE	0.502	0.515
95% CI	(-0.83, 1.26)	(-1.43, 0.71)
LS mean difference (Omaveloxolone-Placebo)		-0.58
SE		0.790
95% CI		(-2.22, 1.06)
p-value		0.4710
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.25
95% CI		(-0.98, 0.48)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-npc-gait.sas:t-mfars93-d-us-mmrm-itt-npc-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

1.1.2 Einschränkungen in Alltagsaktivitäten (FA-ADL)

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 of Gait Score Greater Than 2

Page: 1 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Baseline		
N	16	16
Mean score	12.97	14.25
SD	4.570	3.322
Week 24		
N	15	14
LS mean change from baseline	0.39	-0.85
SE	1.198	1.247
95% CI	(-2.32, 3.11)	(-3.67, 1.98)
LS mean difference (Omaveloxolone-Placebo)		-1.24
SE		1.871
95% CI		(-5.40, 2.92)
p-value		0.5222
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.21
95% CI		(-0.94, 0.52)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-npc-gait.sas:t-adl-mmrm-itt-npc-gait-gt2.rtf Data Tag: FINAL Run Date: 17JAN2025

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 of Gait Score Greater Than 2

Page: 2 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 36		
N	15	14
LS mean change from baseline	0.39	0.51
SE	1.240	1.291
95% CI	(-2.37, 3.15)	(-2.36, 3.38)
LS mean difference (Omaveloxolone-Placebo)		0.12
SE		1.928
95% CI		(-4.11, 4.34)
p-value		0.9534
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.02
95% CI		(-0.71, 0.75)
Week 48		
N	15	14
LS mean change from baseline	1.18	-0.30
SE	1.011	1.055
95% CI	(-0.97, 3.32)	(-2.54, 1.94)
LS mean difference (Omaveloxolone-Placebo)		-1.47
SE		1.625
95% CI		(-4.91, 1.97)
p-value		0.3779
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.30
95% CI		(-1.04, 0.43)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-npc-gait.sas:t-adl-mmrm-itt-npc-gait-gt2.rtf Data Tag: FINAL Run Date: 17JAN2025

1.1.3 Patientenberichteter Gesundheitszustand (PGI-C)

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 without Pes Cavus Population of Gait Score Greater Than 2

Page: 1 of 2

Visit Statistic	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 12		
Number of observations per imputation	16 (100)	16 (100)
Number of imputed values per imputation	0	0
LS mean change	3.65	3.51
SE	0.288	0.275
95% CI	(3.05, 4.25)	(2.93, 4.08)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.397
95% CI		(-0.97, 0.69)
p-value		0.7228
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.11
95% CI		(-0.81, 0.58)
Week 24		
Number of observations per imputation	15 (93.8)	14 (87.5)
Number of imputed values per imputation	1 (6.3)	2 (12.5)
LS mean change	3.72	2.97
SE	0.359	0.352
95% CI	(3.02, 4.43)	(2.28, 3.66)
LS mean difference (Omaveloxolone-Placebo)		-0.75
SE		0.480
95% CI		(-1.69, 0.19)
p-value		0.1168
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.57
95% CI		(-1.31, 0.18)

NOTE 1: LS Mean and Hedge's g are based on the ANCOVA model that includes site, baseline, treatment group.

NOTE 2: Multiple imputation including treatment, site, post-baseline for the endpoint

NOTE 3: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-npc-gait.sas:t-pgi-anc-mi-itt-npc-gait-gt2.rtf Data Tag: FINAL Run Date: 14JAN2025

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 without Pes Cavus Population of Gait Score Greater Than 2

Page: 2 of 2

Visit Statistic	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 36		
Number of observations per imputation	15 (93.8)	14 (87.5)
Number of imputed values per imputation	1 (6.3)	2 (12.5)
LS mean change	3.86	3.14
SE	0.428	0.416
95% CI	(3.03, 4.70)	(2.33, 3.96)
LS mean difference (Omaveloxolone-Placebo)		-0.72
SE		0.577
95% CI		(-1.85, 0.41)
p-value		0.2122
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.45
95% CI		(-1.19, 0.29)
Week 48		
Number of observations per imputation	15 (93.8)	14 (87.5)
Number of imputed values per imputation	1 (6.3)	2 (12.5)
LS mean change	4.03	3.48
SE	0.494	0.485
95% CI	(3.06, 5.00)	(2.53, 4.43)
LS mean difference (Omaveloxolone-Placebo)		-0.55
SE		0.679
95% CI		(-1.88, 0.78)
p-value		0.4198
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.29
95% CI		(-1.02, 0.44)

NOTE 1: LS Mean and Hedge's g are based on the ANCOVA model that includes site, baseline, treatment group.

NOTE 2: Multiple imputation including treatment, site, post-baseline for the endpoint

NOTE 3: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-pgi-anc-mi-itt-npc-gait.sas:t-pgi-anc-mi-itt-npc-gait-gt2.rtf Data Tag: FINAL Run Date: 14JAN2025

1.1.4 Feinmotorik der oberen Gliedmaßen Nine-Hole Peg Test (9-HPT)

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 of Gait Score Greater Than 2

Page: 1 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Baseline		
N	16	16
Mean score	0.0180	0.0179
SD	0.00885	0.00553
Week 24		
N	15	14
LS mean change from baseline	-0.0005	-0.0001
SE	0.00054	0.00056
95% CI	(-0.0016, 0.0006)	(-0.0013, 0.0011)
LS mean difference (Omaveloxolone-Placebo)		0.0004
SE		0.00085
95% CI		(-0.0014, 0.0022)
p-value		0.6498
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.1548
95% CI		(-0.5746, 0.8842)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-npc-dh-gait.sas:t-9hpt-mmrm-itt-npc-dh-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 22JAN2025

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 of Gait Score Greater Than 2

Page: 2 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 48		
N	15	14
LS mean change from baseline	-0.0009	0.0000
SE	0.00075	0.00078
95% CI	(-0.0025, 0.0006)	(-0.0016, 0.0017)
LS mean difference (Omaveloxolone-Placebo)		0.0010
SE		0.00114
95% CI		(-0.0014, 0.0033)
p-value		0.3934
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.2976
95% CI		(-0.4347, 1.0300)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-npc-dh-gait.sas:t-9hpt-mmrm-itt-npc-dh-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 22JAN2025

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 of Gait Score Greater Than 2

Page: 1 of 2

	Placebo (N=16)	Omaproloxolone 150 mg (N=16)
Baseline		
N	16	16
Mean score	0.0162	0.0158
SD	0.00835	0.00490
Week 24		
N	15	14
LS mean change from baseline	-0.0001	-0.0006
SE	0.00055	0.00057
95% CI	(-0.0012, 0.0011)	(-0.0018, 0.0006)
LS mean difference (Omaproloxolone-Placebo)		-0.0005
SE		0.00087
95% CI		(-0.0023, 0.0013)
p-value		0.5711
Hedge's g standardized mean difference (Omaproloxolone-Placebo)		-0.1939
95% CI		(-0.9240, 0.5361)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-npc-ndh-gait.sas:t-9hpt-mmrm-itt-npc-ndh-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 21JAN2025

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 of Gait Score Greater Than 2

Page: 2 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 48		
N	15	14
LS mean change from baseline	0.0004	-0.0001
SE	0.00073	0.00076
95% CI	(-0.0011, 0.0019)	(-0.0016, 0.0015)
LS mean difference (Omaveloxolone-Placebo)		-0.0005
SE		0.00111
95% CI		(-0.0028, 0.0018)
p-value		0.6571
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.1541
95% CI		(-0.8835, 0.5753)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-npc-ndh-gait.sas:t-9hpt-mmrm-itt-npc-ndh-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 21JAN2025

1.1.5 Beinfunktion (T25-FWT)

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 - ITT without Pes Cavus Population of Gait Score Greater Than 2

Page: 1 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Baseline		
N	11	12
Mean score	0.07	0.06
SD	0.053	0.042
Week 24		
N	8	10
LS mean change from baseline	-0.01	-0.01
SE	0.007	0.006
95% CI	(-0.03, 0.00)	(-0.02, 0.01)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.010
95% CI		(-0.02, 0.03)
p-value		0.5939
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.21
95% CI		(-0.72, 1.14)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-itt-npc-gait.sas:t-ftwk-mmrm-itt-npc-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 21JAN2025

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 - ITT without Pes Cavus Population of Gait Score Greater Than 2

Page: 2 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 48		
N	8	8
LS mean change from baseline	-0.03	-0.02
SE	0.008	0.008
95% CI	(-0.04, -0.01)	(-0.03, 0.00)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.012
95% CI		(-0.02, 0.04)
p-value		0.5422
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.26
95% CI		(-0.72, 1.24)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-itt-npc-gait.sas:t-ftwk-mmrm-itt-npc-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 21JAN2025

1.2. Gesundheitsbezogene Lebensqualität - ITT-Population ohne schweren Pes cavus

1.2.1 Gesundheitsbezogene Lebensqualität (SF-36)

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 of Gait Score Greater Than 2

Page: 1 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Baseline		
N	16	16
Mean score	51.27	49.36
SD	7.679	11.509
Week 24		
N	15	14
LS mean change from baseline	-2.07	-0.57
SE	1.720	1.790
95% CI	(-5.68, 1.54)	(-4.33, 3.19)
LS mean difference (Omaveloxolone-Placebo)		1.50
SE		2.671
95% CI		(-4.10, 7.10)
p-value		0.5811
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.19
95% CI		(-0.54, 0.92)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-mcs-itt-npc-gait.sas:t-sf36-mmrm-mcs-itt-npc-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 16JAN2025

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 of Gait Score Greater Than 2

Page: 2 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 48		
N	15	14
LS mean change from baseline	-0.24	2.48
SE	1.737	1.808
95% CI	(-3.88, 3.41)	(-1.32, 6.27)
LS mean difference (Omaveloxolone-Placebo)		2.71
SE		2.694
95% CI		(-2.93, 8.35)
p-value		0.3265
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.34
95% CI		(-0.39, 1.07)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-mcs-itt-npc-gait.sas:t-sf36-mmrm-mcs-itt-npc-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 16JAN2025

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 of Gait Score Greater Than 2

Page: 1 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Baseline		
N	16	16
Mean score	34.57	34.05
SD	9.203	7.238
Week 24		
N	15	14
LS mean change from baseline	1.20	0.34
SE	1.410	1.471
95% CI	(-1.79, 4.19)	(-2.77, 3.46)
LS mean difference (Omaveloxolone-Placebo)		-0.86
SE		2.245
95% CI		(-5.61, 3.90)
p-value		0.7081
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-0.86, 0.60)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-pcs-itt-npc-gait.sas:t-sf36-mmrm-pcs-itt-npc-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 16JAN2025

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 of Gait Score Greater Than 2

Page: 2 of 2

	Placebo (N=16)	Omaveloxolone 150 mg (N=16)
Week 48		
N	15	14
LS mean change from baseline	0.91	-1.21
SE	2.139	2.221
95% CI	(-3.54, 5.37)	(-5.83, 3.42)
LS mean difference (Omaveloxolone-Placebo)		-2.12
SE		3.225
95% CI		(-8.81, 4.57)
p-value		0.5179
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.22
95% CI		(-0.95, 0.51)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-pcs-itt-npc-gait.sas:t-sf36-mmrm-pcs-itt-npc-gait-gt2.rtf **Data Tag:** FINAL **Run Date:** 16JAN2025

2. Gait-Score ≤ 2

2.1 Morbidität - ITT-Population ohne schweren Pes cavus

2.1.1 Krankheitsschwere (mFARS)

MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Baseline		
N	26	25
Mean score	32.65	34.50
SD	7.009	7.298
Week 4		
N	26	24
LS mean change from baseline	-0.37	-1.16
SE	0.674	0.710
95% CI	(-1.73, 0.99)	(-2.60, 0.27)
LS mean difference (Omaveloxolone-Placebo)		-0.80
SE		1.017
95% CI		(-2.85, 1.25)
p-value		0.4378
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.21
95% CI		(-0.77, 0.34)
Week 12		
N	26	24
LS mean change from baseline	-1.07	-1.22
SE	0.721	0.759
95% CI	(-2.53, 0.39)	(-2.76, 0.31)
LS mean difference (Omaveloxolone-Placebo)		-0.16
SE		1.083
95% CI		(-2.34, 2.03)
p-value		0.8847
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.04
95% CI		(-0.59, 0.52)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-itt-npc-byvis-gait.sas:t-mfars93-mmrm-itt-npc-byvis-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Less and Equal Than 2

Page: 2 of 3

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 18		
N	26	23
LS mean change from baseline	-1.23	-0.99
SE	0.843	0.900
95% CI	(-2.93, 0.48)	(-2.81, 0.82)
LS mean difference (Omaveloxolone-Placebo)		0.23
SE		1.267
95% CI		(-2.32, 2.79)
p-value		0.8549
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.05
95% CI		(-0.51, 0.61)
Week 24		
N	26	22
LS mean change from baseline	-0.80	-1.29
SE	0.822	0.897
95% CI	(-2.46, 0.86)	(-3.10, 0.52)
LS mean difference (Omaveloxolone-Placebo)		-0.50
SE		1.249
95% CI		(-3.02, 2.02)
p-value		0.6925
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.11
95% CI		(-0.68, 0.46)
Week 36		
N	26	21
LS mean change from baseline	0.14	-1.45
SE	0.875	0.963
95% CI	(-1.62, 1.91)	(-3.39, 0.49)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-itt-npc-byvis-gait.sas:t-mfars93-mmrm-itt-npc-byvis-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
LS mean difference (Omaveloxolone-Placebo)		-1.59
SE		1.332
95% CI		(-4.28, 1.09)
p-value		0.2381
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.34
95% CI		(-0.91, 0.24)
Week 48		
N	26	20
LS mean change from baseline	0.91	-0.73
SE	0.871	0.968
95% CI	(-0.85, 2.67)	(-2.68, 1.22)
LS mean difference (Omaveloxolone-Placebo)		-1.64
SE		1.332
95% CI		(-4.32, 1.05)
p-value		0.2264
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.35
95% CI		(-0.94, 0.24)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-mmrm-itt-npc-byvis-gait.sas:t-mfars93-mmrm-itt-npc-byvis-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

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 Population of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Baseline		
N	26	25
Mean score	0.57	0.80
SD	0.688	0.520
Week 4		
N	26	24
LS mean change from baseline	-0.08	-0.01
SE	0.058	0.061
95% CI	(-0.20, 0.04)	(-0.13, 0.12)
LS mean difference (Omaveloxolone-Placebo)		0.07
SE		0.087
95% CI		(-0.10, 0.25)
p-value		0.4076
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.23
95% CI		(-0.33, 0.78)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-npc-gait.sas:t-mfars93-d-bul-mmrm-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

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 Population of Gait Score Less and Equal Than 2

Page: 2 of 4

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 12		
N	26	24
LS mean change from baseline	0.05	-0.02
SE	0.066	0.069
95% CI	(-0.09, 0.18)	(-0.16, 0.12)
LS mean difference (Omaveloxolone-Placebo)		-0.06
SE		0.098
95% CI		(-0.26, 0.13)
p-value		0.5223
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.17
95% CI		(-0.73, 0.38)
Week 18		
N	26	23
LS mean change from baseline	0.01	0.02
SE	0.054	0.058
95% CI	(-0.10, 0.12)	(-0.09, 0.14)
LS mean difference (Omaveloxolone-Placebo)		0.01
SE		0.082
95% CI		(-0.15, 0.18)
p-value		0.8591
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.05
95% CI		(-0.51, 0.61)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-npc-gait.sas:t-mfars93-d-bul-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

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 Population of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 24		
N	26	22
LS mean change from baseline	0.02	0.00
SE	0.047	0.051
95% CI	(-0.08, 0.11)	(-0.10, 0.10)
LS mean difference (Omaveloxolone-Placebo)		-0.02
SE		0.073
95% CI		(-0.16, 0.13)
p-value		0.8168
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.06
95% CI		(-0.63, 0.50)
Week 36		
N	26	21
LS mean change from baseline	0.14	-0.05
SE	0.063	0.070
95% CI	(0.01, 0.26)	(-0.19, 0.09)
LS mean difference (Omaveloxolone-Placebo)		-0.19
SE		0.097
95% CI		(-0.38, 0.01)
p-value		0.0626
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.54
95% CI		(-1.12, 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-npc-gait.sas:t-mfars93-d-bul-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

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 Population of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 48		
N	26	20
LS mean change from baseline	0.05	-0.09
SE	0.068	0.077
95% CI	(-0.09, 0.19)	(-0.24, 0.07)
LS mean difference (Omaveloxolone-Placebo)		-0.14
SE		0.104
95% CI		(-0.35, 0.07)
p-value		0.1941
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.37
95% CI		(-0.96, 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-bul-mmrm-itt-npc-gait.sas:t-mfars93-d-bul-mmrm-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Coordination Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaperoxolone 150 mg (N=25)
Baseline		
N	26	25
Mean score	9.33	10.12
SD	2.560	3.422
Week 4		
N	26	24
LS mean change from baseline	-0.14	-0.64
SE	0.361	0.380
95% CI	(-0.87, 0.58)	(-1.41, 0.13)
LS mean difference (Omaperoxolone-Placebo)		-0.50
SE		0.546
95% CI		(-1.60, 0.61)
p-value		0.3690
Hedge's g standardized mean difference (Omaperoxolone-Placebo)		-0.25
95% CI		(-0.80, 0.31)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-npc-gait.sas:t-mfars93-d-ul-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Coordination Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 12		
N	26	24
LS mean change from baseline	-0.32	-1.03
SE	0.371	0.391
95% CI	(-1.07, 0.43)	(-1.82, -0.24)
LS mean difference (Omaveloxolone-Placebo)		-0.71
SE		0.561
95% CI		(-1.84, 0.42)
p-value		0.2135
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.34
95% CI		(-0.90, 0.22)
Week 18		
N	26	23
LS mean change from baseline	-0.82	-0.88
SE	0.413	0.440
95% CI	(-1.66, 0.01)	(-1.77, 0.01)
LS mean difference (Omaveloxolone-Placebo)		-0.06
SE		0.624
95% CI		(-1.32, 1.20)
p-value		0.9280
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.02
95% CI		(-0.59, 0.54)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-npc-gait.sas:t-mfars93-d-ul-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Coordination Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 24		
N	26	22
LS mean change from baseline	-0.38	-0.60
SE	0.415	0.452
95% CI	(-1.22, 0.46)	(-1.52, 0.31)
LS mean difference (Omaveloxolone-Placebo)		-0.23
SE		0.632
95% CI		(-1.50, 1.05)
p-value		0.7231
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.10
95% CI		(-0.67, 0.47)
Week 36		
N	26	21
LS mean change from baseline	-0.17	-0.96
SE	0.458	0.506
95% CI	(-1.10, 0.75)	(-1.98, 0.05)
LS mean difference (Omaveloxolone-Placebo)		-0.79
SE		0.700
95% CI		(-2.20, 0.62)
p-value		0.2635
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.32
95% CI		(-0.90, 0.26)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-npc-gait.sas:t-mfars93-d-ul-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

MOXIe Part 2: Modified Friedreich's Ataxia 93-points Rating Scale (mFARS) Upper Limb Coordination Domain LS Mean Change from Baseline up to Week 48 by Visit from MMRM Analysis - ITT without Pes Cavus Population of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 48		
N	26	20
LS mean change from baseline	0.06	-0.79
SE	0.521	0.582
95% CI	(-0.99, 1.11)	(-1.96, 0.38)
LS mean difference (Omaveloxolone-Placebo)		-0.85
SE		0.795
95% CI		(-2.45, 0.75)
p-value		0.2890
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.31
95% CI		(-0.89, 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ul-mmrm-itt-npc-gait.sas:t-mfars93-d-ul-mmrm-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

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 of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Baseline		
N	26	25
Mean score	5.69	5.78
SD	2.363	2.449
Week 4		
N	26	24
LS mean change from baseline	-0.68	-0.12
SE	0.324	0.341
95% CI	(-1.33, -0.03)	(-0.80, 0.57)
LS mean difference (Omaveloxolone-Placebo)		0.56
SE		0.483
95% CI		(-0.41, 1.54)
p-value		0.2493
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.32
95% CI		(-0.24, 0.87)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-npc-gait.sas:t-mfars93-d-ll-mmrm-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

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 of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 12		
N	26	24
LS mean change from baseline	-1.27	-0.44
SE	0.303	0.319
95% CI	(-1.88, -0.66)	(-1.08, 0.21)
LS mean difference (Omaveloxolone-Placebo)		0.83
SE		0.454
95% CI		(-0.08, 1.75)
p-value		0.0731
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.50
95% CI		(-0.07, 1.06)
Week 18		
N	26	23
LS mean change from baseline	-0.65	-0.20
SE	0.343	0.367
95% CI	(-1.34, 0.05)	(-0.94, 0.54)
LS mean difference (Omaveloxolone-Placebo)		0.45
SE		0.515
95% CI		(-0.60, 1.49)
p-value		0.3924
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.24
95% CI		(-0.33, 0.80)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-npc-gait.sas:t-mfars93-d-ll-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

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 of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 24		
N	26	22
LS mean change from baseline	-0.90	-0.91
SE	0.286	0.311
95% CI	(-1.48, -0.32)	(-1.53, -0.28)
LS mean difference (Omaveloxolone-Placebo)		-0.01
SE		0.435
95% CI		(-0.89, 0.87)
p-value		0.9870
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.00
95% CI		(-0.57, 0.56)
Week 36		
N	26	21
LS mean change from baseline	-0.69	-0.66
SE	0.317	0.353
95% CI	(-1.33, -0.05)	(-1.38, 0.05)
LS mean difference (Omaveloxolone-Placebo)		0.03
SE		0.487
95% CI		(-0.96, 1.01)
p-value		0.9564
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.02
95% CI		(-0.56, 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-npc-gait.sas:t-mfars93-d-ll-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

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 of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 48		
N	26	20
LS mean change from baseline	-0.40	-0.28
SE	0.337	0.381
95% CI	(-1.08, 0.28)	(-1.05, 0.49)
LS mean difference (Omaveloxolone-Placebo)		0.12
SE		0.520
95% CI		(-0.93, 1.17)
p-value		0.8133
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.07
95% CI		(-0.52, 0.65)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-ll-mmrm-itt-npc-gait.sas:t-mfars93-d-ll-mmrm-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

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 of Gait Score Less and Equal Than 2

Page: 1 of 4

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Baseline		
N	26	25
Mean score	17.05	17.80
SD	4.627	3.231
Week 4		
N	26	24
LS mean change from baseline	0.66	-0.56
SE	0.368	0.387
95% CI	(-0.09, 1.40)	(-1.34, 0.22)
LS mean difference (Omaveloxolone-Placebo)		-1.21
SE		0.551
95% CI		(-2.33, -0.10)
p-value		0.0330
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.60
95% CI		(-1.16, -0.03)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-npc-gait.sas:t-mfars93-d-us-mmrm-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

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 of Gait Score Less and Equal Than 2

Page: 2 of 4

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 12		
N	26	24
LS mean change from baseline	0.53	0.19
SE	0.414	0.434
95% CI	(-0.31, 1.37)	(-0.69, 1.07)
LS mean difference (Omaveloxolone-Placebo)		-0.34
SE		0.616
95% CI		(-1.58, 0.90)
p-value		0.5827
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.15
95% CI		(-0.71, 0.41)
Week 18		
N	26	23
LS mean change from baseline	0.32	-0.04
SE	0.438	0.468
95% CI	(-0.56, 1.21)	(-0.99, 0.90)
LS mean difference (Omaveloxolone-Placebo)		-0.37
SE		0.656
95% CI		(-1.69, 0.96)
p-value		0.5776
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.15
95% CI		(-0.72, 0.41)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-npc-gait.sas:t-mfars93-d-us-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

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 of Gait Score Less and Equal Than 2

Page: 3 of 4

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 24		
N	26	22
LS mean change from baseline	0.53	0.20
SE	0.449	0.492
95% CI	(-0.38, 1.43)	(-0.79, 1.19)
LS mean difference (Omaveloxolone-Placebo)		-0.33
SE		0.680
95% CI		(-1.70, 1.04)
p-value		0.6343
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-0.70, 0.44)
Week 36		
N	26	21
LS mean change from baseline	0.95	0.15
SE	0.423	0.465
95% CI	(0.10, 1.81)	(-0.79, 1.09)
LS mean difference (Omaveloxolone-Placebo)		-0.80
SE		0.644
95% CI		(-2.10, 0.50)
p-value		0.2217
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.35
95% CI		(-0.93, 0.23)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-npc-gait.sas:t-mfars93-d-us-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 03JAN2025

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 of Gait Score Less and Equal Than 2

Page: 4 of 4

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 48		
N	26	20
LS mean change from baseline	1.27	0.30
SE	0.439	0.495
95% CI	(0.38, 2.16)	(-0.70, 1.30)
LS mean difference (Omaveloxolone-Placebo)		-0.97
SE		0.675
95% CI		(-2.33, 0.40)
p-value		0.1603
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.41
95% CI		(-0.99, 0.18)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-mfars93-d-us-mmrm-itt-npc-gait.sas:t-mfars93-d-us-mmrm-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 03JAN2025

2.1.2 Einschränkungen in Alltagsaktivitäten (FA-ADL)

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 of Gait Score Less and Equal Than 2

Page: 1 of 2

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Baseline		
N	26	25
Mean score	7.96	8.50
SD	3.982	4.072
Week 24		
N	26	22
LS mean change from baseline	0.44	0.16
SE	0.377	0.412
95% CI	(-0.32, 1.21)	(-0.68, 1.00)
LS mean difference (Omaveloxolone-Placebo)		-0.28
SE		0.581
95% CI		(-1.46, 0.89)
p-value		0.6277
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.13
95% CI		(-0.70, 0.43)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-npc-gait.sas:t-adl-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 17JAN2025

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 of Gait Score Less and Equal Than 2

Page: 2 of 2

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 36		
N	26	22
LS mean change from baseline	0.61	0.41
SE	0.406	0.444
95% CI	(-0.21, 1.43)	(-0.49, 1.31)
LS mean difference (Omaveloxolone-Placebo)		-0.20
SE		0.622
95% CI		(-1.46, 1.05)
p-value		0.7465
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.09
95% CI		(-0.66, 0.48)
Week 48		
N	26	22
LS mean change from baseline	0.94	0.11
SE	0.419	0.458
95% CI	(0.09, 1.79)	(-0.82, 1.04)
LS mean difference (Omaveloxolone-Placebo)		-0.83
SE		0.640
95% CI		(-2.13, 0.47)
p-value		0.2043
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.36
95% CI		(-0.93, 0.22)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-adl-mmrm-itt-npc-gait.sas:t-adl-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 17JAN2025

2.1.3 Patientenberichteter Gesundheitszustand (PGI-C)

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 without Pes Cavus Population of Gait Score Less and Equal Than 2

Page: 1 of 2

Visit Statistic	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 12		
Number of observations per imputation	26 (100)	24 (96.0)
Number of imputed values per imputation	0	0
LS mean change	3.80	4.04
SE	0.212	0.241
95% CI	(3.37, 4.23)	(3.55, 4.53)
LS mean difference (Omaveloxolone-Placebo)		0.24
SE		0.296
95% CI		(-0.36, 0.84)
p-value		0.4215
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.22
95% CI		(-0.34, 0.77)
Week 24		
Number of observations per imputation	26 (100)	22 (88.0)
Number of imputed values per imputation	0	2 (8.0)
LS mean change	4.10	4.29
SE	0.239	0.276
95% CI	(3.63, 4.57)	(3.75, 4.83)
LS mean difference (Omaveloxolone-Placebo)		0.18
SE		0.335
95% CI		(-0.47, 0.84)
p-value		0.5820
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.16
95% CI		(-0.41, 0.73)

NOTE 1: LS Mean and Hedge's g are based on the ANCOVA model that includes site, baseline, treatment group.

NOTE 2: Multiple imputation including treatment, site, post-baseline for the endpoint

NOTE 3: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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-npc-gait.sas:t-pgi-anc-mi-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 14JAN2025

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 without Pes Cavus Population of Gait Score Less and Equal Than 2

Page: 2 of 2

Visit Statistic	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 36		
Number of observations per imputation	26 (100)	22 (88.0)
Number of imputed values per imputation	0	2 (8.0)
LS mean change	4.04	3.96
SE	0.244	0.280
95% CI	(3.57, 4.52)	(3.41, 4.51)
LS mean difference (Omaveloxolone-Placebo)		-0.09
SE		0.341
95% CI		(-0.75, 0.58)
p-value		0.8011
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.07
95% CI		(-0.64, 0.50)
Week 48		
Number of observations per imputation	26 (100)	22 (88.0)
Number of imputed values per imputation	0	2 (8.0)
LS mean change	4.49	4.08
SE	0.262	0.303
95% CI	(3.98, 5.00)	(3.48, 4.67)
LS mean difference (Omaveloxolone-Placebo)		-0.41
SE		0.367
95% CI		(-1.13, 0.30)
p-value		0.2584
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.32
95% CI		(-0.89, 0.25)

NOTE 1: LS Mean and Hedge's g are based on the ANCOVA model that includes site, baseline, treatment group.

NOTE 2: Multiple imputation including treatment, site, post-baseline for the endpoint

NOTE 3: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

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-npc-gait.sas:t-pgi-anc-mi-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 14JAN2025

2.1.4 Feinmotorik der oberen Gliedmaßen Nine-Hole Peg Test (9-HPT)

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 of Gait Score Less and Equal Than 2

Page: 1 of 2

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Baseline		
N	26	25
Mean score	0.0251	0.0265
SD	0.00469	0.00603
Week 24		
N	25	22
LS mean change from baseline	-0.0002	-0.0002
SE	0.00070	0.00075
95% CI	(-0.0016, 0.0012)	(-0.0017, 0.0013)
LS mean difference (Omaveloxolone-Placebo)		0.0000
SE		0.00108
95% CI		(-0.0022, 0.0021)
p-value		0.9753
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.0087
95% CI		(-0.5816, 0.5643)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-npc-dh-gait.sas:t-9hpt-mmrm-itt-npc-dh-gait-le2.rtf Data Tag: FINAL Run Date: 22JAN2025

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 of Gait Score Less and Equal Than 2

Page: 2 of 2

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 48		
N	26	22
LS mean change from baseline	-0.0011	0.0001
SE	0.00077	0.00084
95% CI	(-0.0026, 0.0005)	(-0.0016, 0.0018)
LS mean difference (Omaveloxolone-Placebo)		0.0011
SE		0.00118
95% CI		(-0.0012, 0.0035)
p-value		0.3354
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.2695
95% CI		(-0.3008, 0.8399)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-npc-dh-gait.sas:t-9hpt-mmrm-itt-npc-dh-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 22JAN2025

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 of Gait Score Less and Equal Than 2

Page: 1 of 2

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Baseline		
N	26	25
Mean score	0.0227	0.0241
SD	0.00457	0.00725
Week 24		
N	26	22
LS mean change from baseline	-0.0012	-0.0017
SE	0.00080	0.00088
95% CI	(-0.0029, 0.0004)	(-0.0035, 0.0000)
LS mean difference (Omaveloxolone-Placebo)		-0.0005
SE		0.00125
95% CI		(-0.0031, 0.0020)
p-value		0.6812
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.1140
95% CI		(-0.6822, 0.4542)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-npc-ndh-gait.sas:t-9hpt-mmrm-itt-npc-ndh-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 21JAN2025

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 of Gait Score Less and Equal Than 2

Page: 2 of 2

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 48		
N	26	22
LS mean change from baseline	-0.0011	-0.0016
SE	0.00093	0.00102
95% CI	(-0.0030, 0.0008)	(-0.0036, 0.0005)
LS mean difference (Omaveloxolone-Placebo)		-0.0005
SE		0.00143
95% CI		(-0.0034, 0.0024)
p-value		0.7352
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.0940
95% CI		(-0.6621, 0.4741)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-9hpt-mmrm-itt-npc-ndh-gait.sas:t-9hpt-mmrm-itt-npc-ndh-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 21JAN2025

2.1.5 Beinfunktion (T25-FWT)

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 - ITT without Pes Cavus Population of Gait Score Less and Equal Than 2

Page: 1 of 2

	Placebo (N=26)	Omaprolozolon 150 mg (N=25)
Baseline		
N	26	25
Mean score	0.16	0.17
SD	0.045	0.042
Week 24		
N	26	22
LS mean change from baseline	-0.01	0.00
SE	0.005	0.006
95% CI	(-0.02, 0.00)	(-0.01, 0.01)
LS mean difference (Omaprolozolon-Placebo)		0.01
SE		0.008
95% CI		(-0.01, 0.03)
p-value		0.3475
Hedge's g standardized mean difference (Omaprolozolon-Placebo)		0.26
95% CI		(-0.31, 0.83)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-itt-npc-gait.sas:t-ftwk-mmrm-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 21JAN2025

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 - ITT without Pes Cavus Population of Gait Score Less and Equal Than 2

Page: 2 of 2

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 48		
N	26	22
LS mean change from baseline	-0.02	-0.02
SE	0.007	0.007
95% CI	(-0.03, -0.01)	(-0.03, 0.00)
LS mean difference (Omaveloxolone-Placebo)		0.00
SE		0.010
95% CI		(-0.02, 0.02)
p-value		0.7692
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.08
95% CI		(-0.49, 0.65)

NOTE1: 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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-ftwk-mmrm-itt-npc-gait.sas:t-ftwk-mmrm-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 21JAN2025

2.2. Gesundheitsbezogene Lebensqualität - ITT-Population ohne schweren Pes cavus

2.2.1 Gesundheitsbezogene Lebensqualität (SF-36)

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 of Gait Score Less and Equal Than 2

Page: 1 of 2

	Placebo (N=26)	Omaproloxolone 150 mg (N=25)
Baseline		
N	26	25
Mean score	48.55	51.36
SD	10.073	9.365
Week 24		
N	26	22
LS mean change from baseline	2.08	-1.09
SE	1.174	1.285
95% CI	(-0.30, 4.46)	(-3.70, 1.52)
LS mean difference (Omaproloxolone-Placebo)		-3.17
SE		1.813
95% CI		(-6.84, 0.51)
p-value		0.0890
Hedge's g standardized mean difference (Omaproloxolone-Placebo)		-0.48
95% CI		(-1.06, 0.09)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-mcs-itt-npc-gait.sas:t-sf36-mmrm-mcs-itt-npc-gait-le2.rtf Data Tag: FINAL Run Date: 16JAN2025

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 of Gait Score Less and Equal Than 2

Page: 2 of 2

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 48		
N	26	22
LS mean change from baseline	0.29	-3.73
SE	1.417	1.549
95% CI	(-2.58, 3.16)	(-6.86, -0.59)
LS mean difference (Omaveloxolone-Placebo)		-4.02
SE		2.162
95% CI		(-8.39, 0.36)
p-value		0.0709
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.51
95% CI		(-1.09, 0.06)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-mcs-itt-npc-gait.sas:t-sf36-mmrm-mcs-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 16JAN2025

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 of Gait Score Less and Equal Than 2

Page: 1 of 2

	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Baseline		
N	26	25
Mean score	41.98	39.06
SD	7.258	7.985
Week 24		
N	26	22
LS mean change from baseline	-1.46	-1.75
SE	1.084	1.188
95% CI	(-3.65, 0.74)	(-4.16, 0.65)
LS mean difference (Omaveloxolone-Placebo)		-0.30
SE		1.684
95% CI		(-3.70, 3.11)
p-value		0.8616
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		-0.05
95% CI		(-0.62, 0.52)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-pcs-itt-npc-gait.sas:t-sf36-mmrm-pcs-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 16JAN2025

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 of Gait Score Less and Equal Than 2

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	Placebo (N=26)	Omaveloxolone 150 mg (N=25)
Week 48		
N	26	22
LS mean change from baseline	-2.62	-1.16
SE	1.087	1.192
95% CI	(-4.82, -0.42)	(-3.57, 1.26)
LS mean difference (Omaveloxolone-Placebo)		1.46
SE		1.689
95% CI		(-1.96, 4.88)
p-value		0.3923
Hedge's g standardized mean difference (Omaveloxolone-Placebo)		0.24
95% CI		(-0.33, 0.81)

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.

NOTE 2: Baseline Gait score is the maximum value of screening and Day 1 pre dose.

Source: biib141/valueaccess/amnog/t-sf36-mmrm-pcs-itt-npc-gait.sas:t-sf36-mmrm-pcs-itt-npc-gait-le2.rtf **Data Tag:** FINAL **Run Date:** 16JAN2025