Apitegromab in Spinal Muscular Atrophy (SMA):

Efficacy, Safety and PK/PD Assessments in 24 Months of TOPAZ

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On behalf of the entire TOPAZ Study Team

SMA EU October 21-23, 2022; Barcelona, Spain
TOPAZ (NCT03921528)

Medical Questions should be sent via email to the following address: medicalinquiry@scholarrock.com

Author Disclosures

Dr. Darras is Principal Investigator of the TOPAZ trial

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- Biogen
- Pfizer
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- Roche/Genentech

Study Site Investigations:

- Steering Committee Member: Roche FIREFISH and MANATEE studies
- DSMB member: Amicus Inc.and Lexeo Therapeutics

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- SMA Foundation
- CureSMA
- Working on Walking Fund

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Disclaimers

- The TOPAZ trial is sponsored by Scholar Rock, a biopharmaceutical company developing and investigating apitegromab in a clinical development program for the treatment of Spinal Muscular Atrophy (SMA)
- Apitegromab has not been approved by the US Food and Drug Administration (FDA), the European Commission, or any other health authority
- The safety and effectiveness of apitegromab have not been established

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Types 2/3 SMA: SMN Targeted Therapies Offer Important Benefits, but…

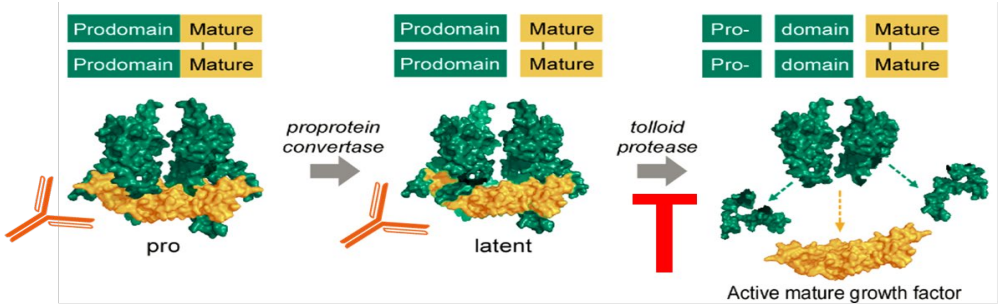
Substantial unmet need remains in this era

- Most patients manifest substantial functional impairment[1,2]
- The greatest improvements with SMN targeted therapies are in the youngest individuals (age < 5)[1,2]
- SMN targeted therapies address neurodegeneration, other approaches to improve motor function are needed[3-5]
- Patients with SMA experience limitations in mobility and daily activities associated with a gradual deterioration in motor function, alongside emotional difficulties, fatigue and a perceived lack of societal support; however, there has been no evidence regarding effective interventions thus far that improve QoL measures* substantially in the symptomatic SMA population[6-7]

SMA, spinal muscular atrophy; SMN, survival motor neuron; QoL, quality of life. 1. Mercuri E. *N Engl J Med.* 2018;378:625-35.DOI: 10.1056/NEJMoa1710504; 2. Mercuri, et al. SUNFISH Part 2: Presented at the AAN 2020. *Neurology.* 2020, 94, 1260. 3. Hua Y et al. *Nature.* 2011 478:123–6. doi: 10.1038/nature10485. 4. Sepulveda, P. et al. *Sci Rep 5*, 17535 (2015). https://doi.org/10.1038/srep17535.; 5. Wang Y, Pessin JE. *Curr Opin Clin Nutr Metab Care.* 2013 May;16(3):243-50. doi. 6. Wan HWY, Carey KA, D'Silva A, et al. *Orphanet J Rare Dis.* 2020;15:70. 7. Yang M. et al. *Adv Ther.* (2022) 39:1915–1958 https://doi.org/10.1007/s12325-022-02089-2; 8. Belter L, Cruz R, Jarecki J. *Orphanet J Rare Dis.* 2020 Aug 24;15(1):217. doi: 10.1186/s13023-020-01498-2. This third-party information is provided for background only and is not intended to convey or imply a comparison to the TOPAZ clinical trial results. *QoL, Quality of Life from this patient population=outcomes that are meaningful from the patient perspective, and which measure the impact of therapies on other dimensions of life other than assessing survival or significant changes in motor milestones such as activities of daily living, work productivity, and fatigue.

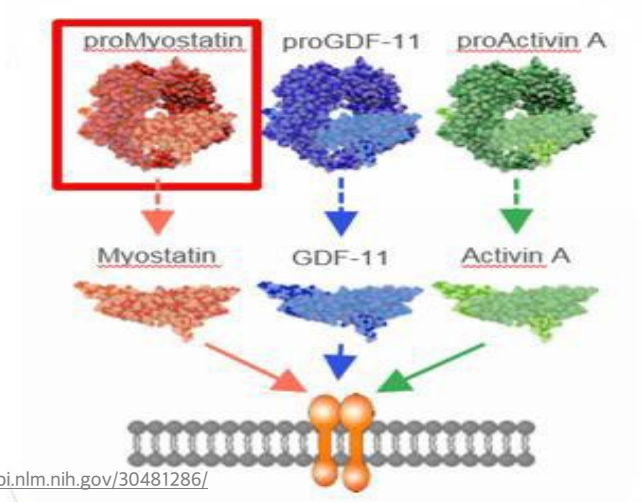
Myostatin is an Important Negative Regulator of Skeletal Muscle Growth Whose Inhibition has the Potential to Lead to Improved Muscle Function in Patients with SMA[1,2]

Apitegromab: A Fully Human Monoclonal Antibody That Blocks Cleavage of the Myostatin Prodomain, Thereby Inhibiting Myostatin Activation[2]



- Two distinct proteolysis events necessary to generate active mature myostatin
- Apitegromab binds to both proMyostatin and latent myostatin and inhibits tolloid-mediated cleavage of latent myostatin
- **Apitegromab bound latent myostatin is proposed to be pulled from muscle into systemic circulation, and can be measured as part of total circulating myostatin[3]**

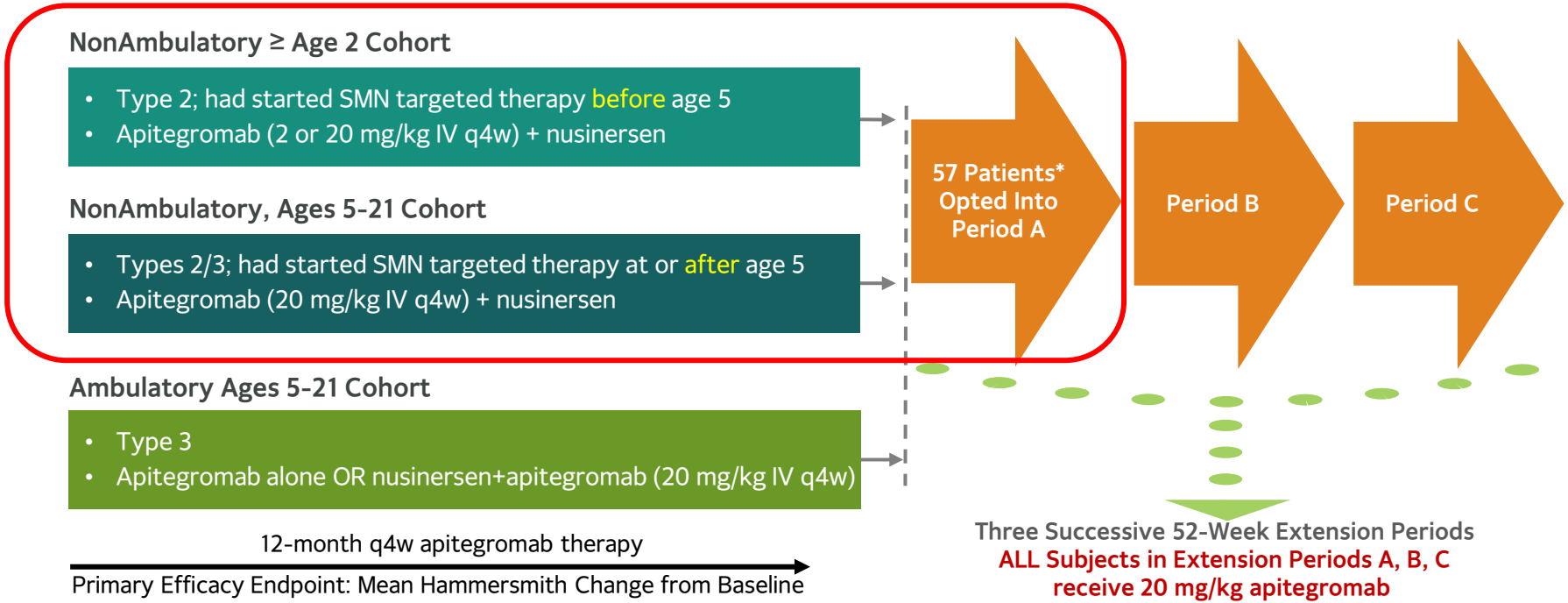
Selective Targeting of preproMyostatin, the Myostatin Precursor: Apitegromab Does Not Bind to Mature Myostatin or Any Form of GDF11, Activin A, or other TGF-β Family[2,3] Members



SMA, spinal muscular atrophy. 1. Long KK, O'Shea KM, Khairallah RJ, et al. *Hum Mol Genet.* 2019;28:1076-1089. Available: https://pubmed.ncbi.nlm.nih.gov/30481286/ 2. Pirruccello-Straub, et al. *Sci Rep.* 2018;8:2292. 3. Dagbay KB, et al. *J Biol Chem.* 2020;295(16):5404-5418.

TOPAZ Phase 2 Trial Design, Including Open Label Extension Periods: Three Pilot Cohorts to Identify Therapeutic Opportunities

All SMA Types 2/3, cohorts defined by age and ambulatory status at time of enrollment



*Excludes one patient from Cohort 1 who discontinued from the trial; 57/58 Completed Treatment Period and Enrolled in Extension Period A; 2 Withdrew Consent in Extension Period A; 55 Completed Extension Period A and Enrolled into Extension Period B Place A, et al. *Eu J Neurol.* 2021;28(Suppl1) 207–334:(EPR-184).
SMA, spinal muscular atrophy; SMN, survival motor neuron.

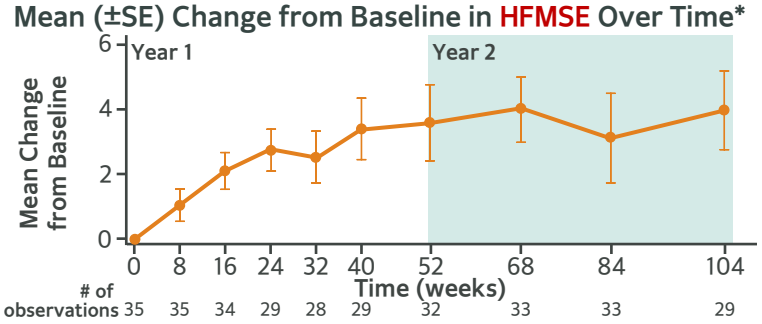
TOPAZ Subject Disposition, Demographics and Baseline Characteristics

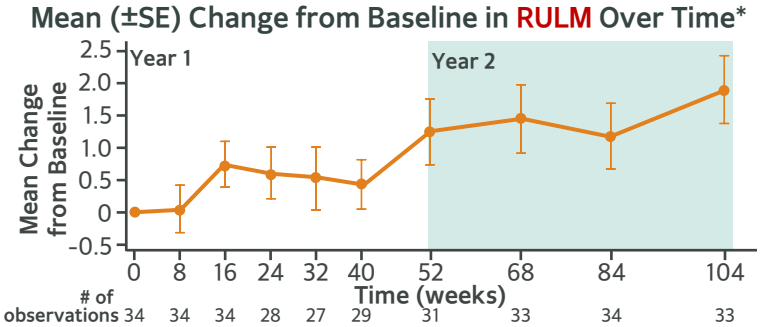
		Age ≥ 2 Type 2		Age 5-21 Type 2/3		Age 5-21 Type 3	
		2 mg/kg + nusinersen	20 mg/kg + nusinersen	20 mg/kg + nusinersen		20 mg/kg monotherapy	20 mg/kg + nusinersen
N (dosed)	NonAmbulatory Patients	10	10	15	Ambulatory Patients	11	12
Mean age at screening (min, max)		4.1 (2, 6)	3.8 (2, 6)	11.7 (8, 19)		12.1 (7, 19)	13.1 (7, 21)
Mean age at SMA diagnosis (min, max)		1.2 (1, 2)	1.2 (1, 3)	3.1 (1, 16)		5.9 (2, 15)	4.5 (2, 15)
Female (%)		30	50	53		73	58
SMN2 Gene Copy* (#, %)							
2		1 (10)	1 (10)			1 (9)	0 (0)
3		8 (80)	8 (80)	11 (73)		4 (36)	9 (75)
4		1 (10)	0 (0)	2 (13)		4 (36)	1 (8)
Months of prior treatment of nusinersen at baseline (min, max)		26.6 (12,36)		25.2 (12, 39)		N/A	26.0 (9, 40)
Discontinuation(s)		0	0	1†		0	2†
Scoliosis (#, %)		4 (40)	3 (30)	11 (73.3)		7 (63.6)	4 (33.3)
Contracture(s) (#, %)		8 (80)	4 (40)	13 (86.7)		6 (54.5)	7 (58.3)
Mean RHS score (min, max)						47.6 (26, 63)	51.3 (43, 62)
Mean HFMSE score (min, max)		26.1 (12, 44)	23.5 (14, 42)	22.7 (13, 39)			

*1 patient answered 3-4, 1 patient answered >4 , both patients are in Cohort 1 treated with 20 mg/kg + nusinersen; data not available for all patients.
†1 cohort 1 patient discontinued study in 12M Treatment Period, 1 cohort 1 patient and 1 cohort 2 patient discontinued during 24M Extension Period A. All discontinuations were for reasons unrelated to study drug. HFMSE=Hammersmith Functional Motor Scale Expanded; RHS=Revised Hammersmith Scale; SMA, spinal muscular atrophy; SMN, survival motor neuron. Data on File. Scholar Rock, Inc. Cambridge, MA.

NonAmbulatory Patients Continue to Improve in Motor Function Over 24 Months of Apitegromab Treatment- Observed Case Analysis

	At 12 months*	At 24 months*
Mean Change in **HFMSE** (95% CI):	Increase of 3.6 (1.2, 6.0)	Increase of 4.0 (1.5, 6.5)
≥3 pt point change in HFMSE:	Achieved in 42% (13/32) of patients	Achieved in 45% (13/29) of patients
Mean Change in **RULM** score (95% CI):	Increase of 1.3 (0.2, 2.3)	Increase of 1.9 (0.8, 3.0)
≥2 pt point change in RULM:	Achieved in 40% (12/31) of patients	Achieved in 45% (15/33) of patients



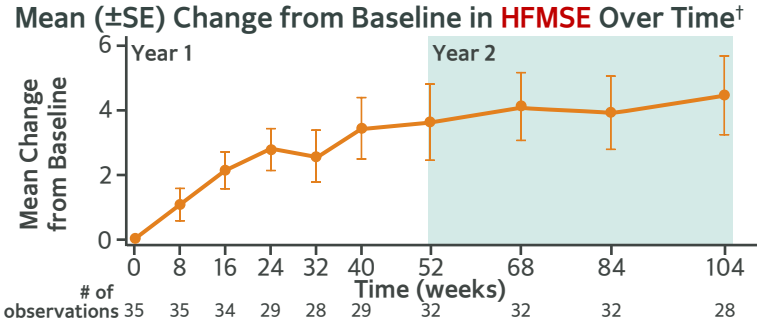


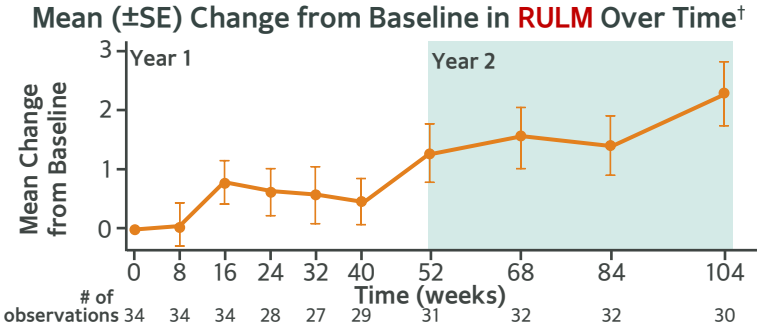
Apitegromab + SMN targeted treatment in NonAmbulatory patients with Types 2 and 3 SMA (age 2-21) for 24 months demonstrated durable improvement in motor function (HFMSE) and continued increases in RULM scores.

*Observed Case (OC) Analysis includes all patients who had a valid measurement at E14 (Day 728).; OC analysis included patients treated with 2 mg/kg as well as 20 mg/kg of apitegromab. This analysis does not exclude any patients who missed doses due to COVID-19 related site access restrictions. Error bars represent SEM. CI, Confidence Interval; 1. Data on File, Scholar Rock, Inc.

NonAmbulatory Patients Continue to Improve in Motor Function Over 24 Months of Apitegromab Treatment- Sensitivity Analysis (Excludes Patients Post Scoliosis Surgery)

	At 12 months*	At 24 months†
Mean Change in **HFMSE** (95% CI):	Increase of 3.6 (1.2, 6.0)	Increase of 4.4 (2.0, 6.9)
≥3 pt point change in **HFMSE**:	Achieved in 42% (13/32) of patients	Achieved in 46% (13/28) of patients
Mean Change in **RULM** score (95% CI):	Increase of 1.3 (0.2, 2.3)	Increase of 2.3 (1.2, 3.4)
≥2 pt point change in **RULM**:	Achieved in 40% (12/31) of patients	Achieved in 50% (15/30) of patients





Apitegromab + SMN targeted treatment in nonambulatory patients with Types 2 and 3 SMA (age 2-21) for 24 months demonstrated durable improvement in motor function (HFMSE) and continued increases in RULM scores

*Observed Case (OC) Analysis includes all patients who had a valid measurement at E14 (Day 728).; OC analysis included patients treated with 2 as well as 20 mg/kg of apitegromab. This analysis does not exclude any patients who missed doses due to COVID-19 related site access restrictions. †24-month Sensitivity Analysis excludes from the OC Analysis any HFMSE data following scoliosis surgery in TOPAZ. Of the three nonambulatory patients who had scoliosis surgery, data from one was excluded and the other two did not have valid HFMSE assessments. Error bars represent SEM. CI, confidence interval; 1. Data on File, Scholar Rock, Inc.

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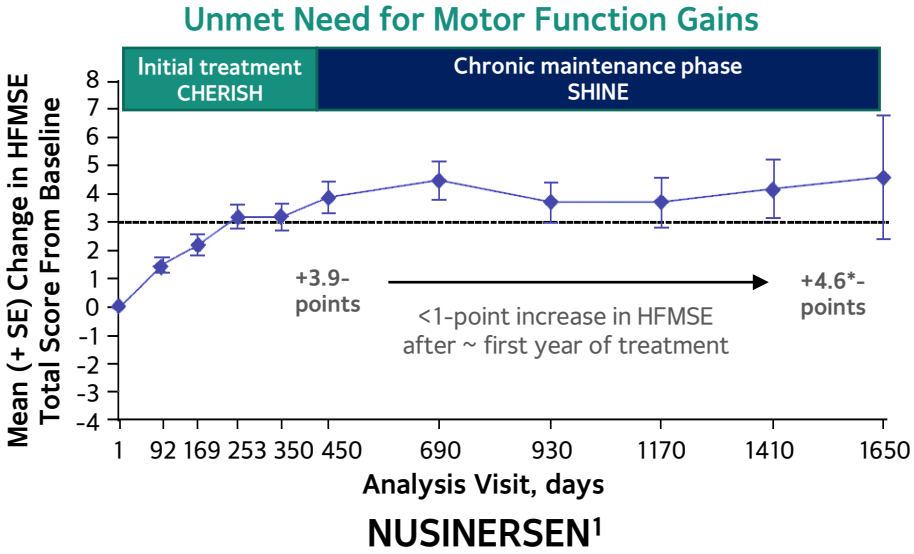
Patients with Types 2 and 3 SMA Continue to Experience Major Functional Deficits Despite Improvement from SMN-Targeted Therapies

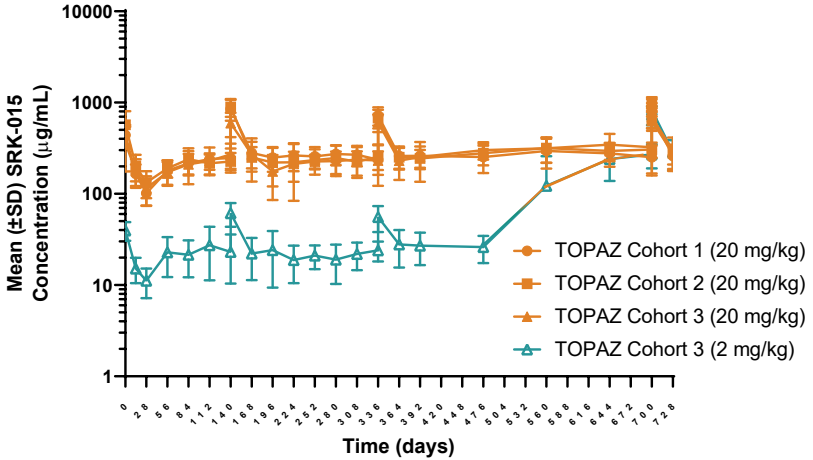
Plateauing of HFMSE increases observed following initial treatment effects for nusinersen



*Relative to sham control, treatment led to a 4.9-point higher HFMSE change from baseline, as sham control patients had –1.0 point change. †±95% confidence interval. Baseline is defined as the last measurement prior to the first treatment dose with risdiplam or placebo.; HFMSE, Hammersmith Functional Motor Scale-Expanded; SMA, spinal muscular atrophy; SMN, survival motor neuron; 1. Mercuri E, et al. Presented at: World Muscle Society Congress 2020, P. 257. This third-party information is provided for background only and is not intended to convey or imply a comparison to the TOPAZ clinical trial results.

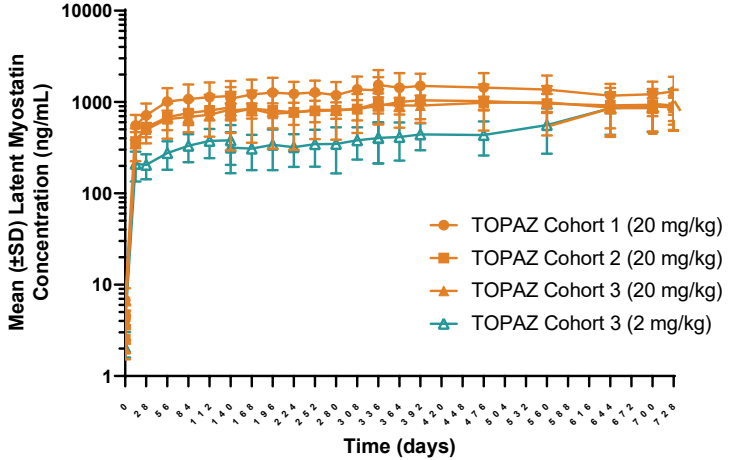
Apitegromab PK and PD Data are Consistent with Clinically Observed Dose Response Through 24 Months

Pharmacokinetics* (PK)



- Well-behaved PK profile consistent with that commonly observed with monoclonal antibodies
- Drug exposure was dose proportional

Target Engagement [Pharmacodynamics (PD)]



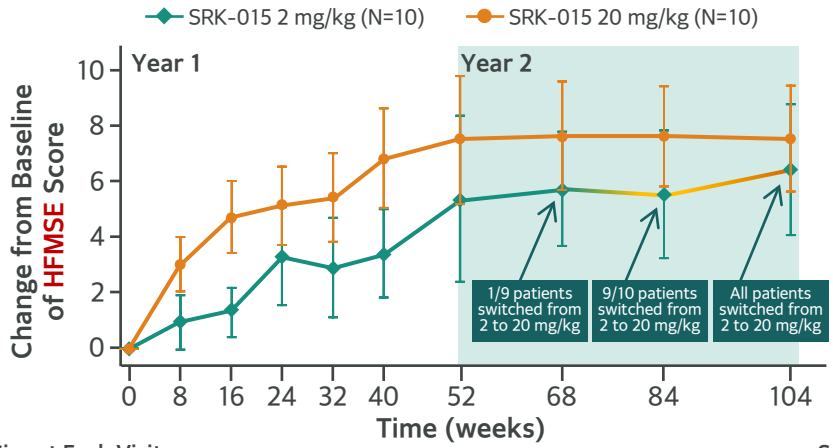
- Target engagement by apitegromab was confirmed
- Low-dose (2 mg/kg) yielded lower level of target engagement and did not achieve full target saturation

Higher Drug Exposure and Target Engagement Reached When Cohort 3 Low-dose Patients Switched from 2 mg/kg to 20 mg/kg

*Starting at day 28, measures are predose trough levels. Data on File. Scholar Rock, Inc. Cambridge, MA.

In the Younger NonAmbulatory Group, the High Dose Arm of Apitegromab Treatment Outperformed the Low Dose Arm, But the Gap is Closed When Switchover to High Dose Occurs

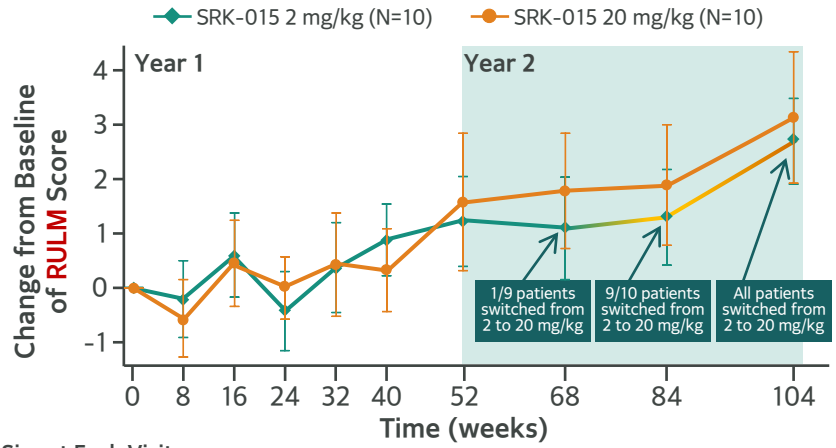
Mean (±SE) Change From Baseline in HFMSE Score Over Time*



Sample Size at Each Visit

2 mg/kg	10	10	10	7	8	9	9	9	10	10
20 mg/kg	10	10	10	8	8	10	8	10	10	8

Mean (±SE) Change From Baseline in RULM Score Over Time*



Sample Size at Each Visit

2 mg/kg	10	10	10	7	8	9	9	10	10	10
20 mg/kg	9	9	9	7	7	9	7	9	9	8

Dose Response Observed Over 24 Months

*24-month Sensitivity Analysis excludes from the OC Analysis any HFMSE data following scoliosis surgery in TOPAZ. Of the three nonambulatory patients who had scoliosis surgery, data from one was excluded and the other two did not have valid HFMSE assessments. Error bars represent SEM. Data on File, Scholar Rock, Inc.

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Activities of Daily Living and Fatigue: Assessed by Three Measures

Fatigue, muscle endurance, and ADL, were assessed using PEDI-CAT, PROMIS, and ESBBT

PEDI-CAT – measure of activities of daily living: Measures pediatric abilities through 3 functional domains, daily activities, mobility, and social cognitive[1]

- 4-point scale **(1=unable to 4=easy)** assessment of various activities, **higher scores reflect improved abilities**[1,2]
- PEDI-CAT has been validated in SMA, but alone cannot identify small changes in function across all types of SMA[3]

PROMIS (Fatigue) – measure of patient fatigue: PRO measurement tool[4]

- Measures mild subjective feelings of tiredness to debilitating and sustained feelings of exhaustion, **with lower scores reflecting less fatigue**[4,5]
- Has been utilized to assess fatigue and fatigability in the Cure SMA database, but has not been fully validated in SMA[5]

ESBBT (Fatigability) – measure of how fast a patient fatigues: Muscle endurance measurement tool[6]

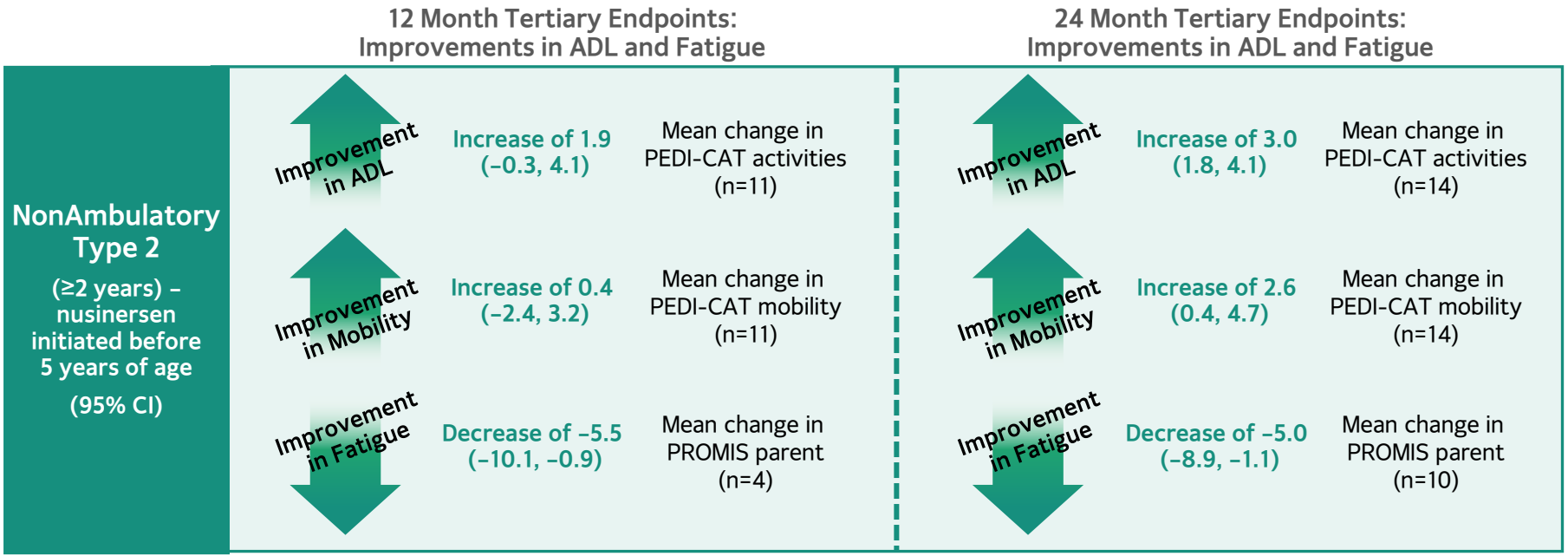
- Part of a series of endurance shuttle tests that include: nine-hole peg test, box and block test, and walk test (ESNHPT, ESBBT, and ESWT)[6]
- Patients are asked to move blocks individually from one box to another in one minute, with **higher numbers of blocks suggesting higher muscle endurance**[6]
- The endurance shuttle tests have been validated for use in patients with SMA[7]

ADL, activities of daily living; ESBBT, endurance shuttle box and block test; ESNHPT, endurance shuttle nine-hole peg test; ESWT, endurance shuttle walk test; PEDI-CAT, pediatric evaluation of disability inventory computer adaptive test; PROMIS, patient-reported outcomes measurement information system; PRO(s), patient-reported outcome(s); SMA, spinal muscular atrophy.

1. Cre Care. PEDI-CAT. Accessed April 26, 2022. https://www.pedicat.com/. 2. Data on file; Scholar Rock. 2022. 3. Pasternak A, et al. *Muscle Nerve*. 2016;54(6):1097-1107. 4. NIH. PROMIS. Accessed April 26, 2022. https://commonfund.nih.gov/promis/index. 5. Belter L, et al. *Orphanet Journal of Rare Diseases*. 2020;15:217. 6. Cure SMA. Best Practices for Physical Therapists and Clinical Evaluators in Spinal Muscular Atrophy (SMA). 2021. Available at: https://www.curesma.org/wp-content/uploads/2021/09/Clinical-Evaluators-Best-Practices-13-August-2021.pdf. 7. Bartels B, et al. *Orphanet Journal of Rare Diseases*. 2020;15:75.

NonAmbulatory Patients Indicate Maintenance or Continuous Improvements in ADL and Fatigue Measures Over 24 Months of Apitegromab Treatment

Apitegromab treatment in nonambulatory type 2 resulted in improvements in patient-reported outcomes related to self-sufficiency and fatigue



ADL, activities of daily living; PEDI-CAT, the Pediatric Evaluation of Disability Inventory computer adaptive test; PROMIS, Patient-Reported Outcome Measurement Information System. 1. Data on file; Scholar Rock. 2022.

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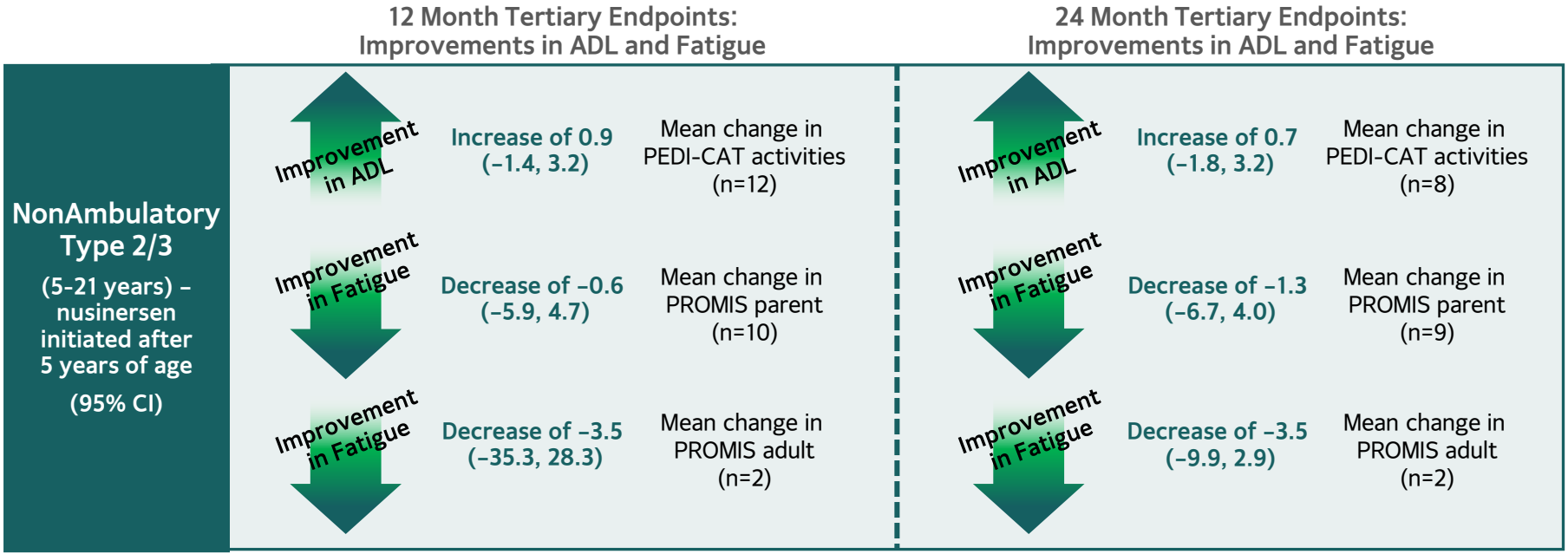
NonAmbulatory Patients Indicate Stabilization or Improvements in ADL and Fatigue Measures over 24 Months of Apitegromab Treatment

Apitegromab treatment in nonambulatory types 2 and 3 resulted in improvements in patient-reported outcomes related to self-sufficiency and fatigue



ADL, activities of daily living; PEDI-CAT, the Pediatric Evaluation of Disability Inventory computer adaptive test; PROMIS, Patient-Reported Outcome Measurement Information System. 1. Data on file; Scholar Rock. 2022.

NonAmbulatory Patients Show Trends for Improvement in Fatigability and Endurance Measures Which May be Complementary to Upper Limb Function Improvements over 24 Months[1]

Apitegromab treatment in nonambulatory types 2 and 3 (cohort 2) indicate improvements in QoL assessments related to self-sufficiency and endurance

NonAmbulatory Type 2/3 (5-21 years) – nusinersen initiated after 5 years of age (95% CI)	12 Month Tertiary Endpoints: Improvements in Fatigability	24 Month Tertiary Endpoints: Improvements in Fatigability
	Improvement in Endurance	Improvement in Endurance
	Increase of 280.8 (-22.7, 584.2)[1] (n=8)	Increase of 147.9 (0.6, 295.2)[1] (n=8)
	Mean change in ESBBT activities (95% CI)	Mean change in ESBBT activities (95% CI)

- The ESBBT is the first validated and sensitive fatigability test for proximal arm function in SMA and may be complementary to outcome measures that focus on arm motor function such as the RULM, by adding the dimension of endurance[2]
- Trends of improvements with ESBBT are consistent with RULM over 24 months

ESBBT, endurance shuttle box and block test; SMA, spinal muscular atrophy; SMN, survival motor neuron; QoL, quality of life. 1. Data on file; Scholar Rock. 2022. 2. Mazzone ES, et al. RULM for SMA: development of a new module. *Muscle Nerve.* 2017;55(6):869–74. Baseline is defined as the last measurement prior to the first dose of study drug. Subject visits after an intercurrent event of 3 consecutive missed doses during the Extension A period, or after taking nusinersen for SMN up-regulator therapy if in Cohort1, are excluded from the Efficacy Eligible Set.

No Safety Risks Identified to Date Over 2 Years of Treatment

Treatment-Emergent Adverse Events (TEAEs)*	Apitegromab 2 mg/kg N=10 n (%)	Apitegromab 20 mg/kg N=48 n (%)	Total N=58 n (%)
Any TEAE	10 (100)	45 (93.8)	55 (94.8)
Any serious TEAE	3 (30)	11 (22.9)	14 (24.1)
Any TEAE leading to study drug discontinuation	0	1 (2.1)	1 (1.7)
Any grade 3 (severe) or higher TEAE	2 (20)	9 (18.8)	11 (19)

- The incidence and types of TEAEs were consistent with the underlying patient population and nusinersen therapy
- The Five most frequently reported TEAEs*: headache (24%), upper respiratory tract infection (22%), pyrexia (22%), cough (22%), and nasopharyngitis (21%)
- No deaths or suspected unexpected serious adverse reactions (SUSARs) reported
- Adverse events continue to be reported as mostly mild to moderate in severity, as observed during the 12-month analysis
- All patients tested negative for the presence of anti-apitegromab antibodies
- Consistent with the phase 1 trial, no hypersensitivity reactions were identified

*Notes: % = 100 x n/N; % at12 month
Treatment-emergent adverse events (TEAEs) are defined as AEs that start after the first dose of study drug or start prior to the administration of study drug and worsen in severity/grade or relationship to investigational medication after the administration of study drug; Data on File. Scholar Rock, Inc. Cambridge, MA.

Summary of TOPAZ Extension Period: 24-Month Data

- The results of these data are relevant for informing the therapeutic hypotheses being evaluated in the Phase 3 SAPPHIRE trial, a randomized double-blind, placebo-controlled study now in progress
- Nonambulatory types 2/3 SMA: motor function gains with apitegromab treatment show durability at 24 months
  - Sustained increases in motor function (HFMSE & RULM) over 2 years
  - Continued increases in RULM throughout 2 years
  - High dose outperforms low dose in HFMSE & RULM, but switchover to high dose closes the gap
  - Consistent PK & PD dose response and confirmed target engagement
  - **Trends of continuous improvements in ADL, fatigue and endurance measures which may be complementary to upper limb function improvements over 24 months. Further analyses under review**
  - No safety risks have been identified over 2 years of apitegromab treatment
- **In a systemic review of natural history of SMA, there was QoL deterioration corresponding to clinical SMA severity, despite treatment with SMN up-regulators[1]**
- **ScholarRock is the first to show data suggesting sustained improvements of QoL measures over 24 months in SMA clinical trials in the TOPAZ population studied, to date**
- **Many thanks to all the patients who participate in these studies, caregivers/families, healthcare professionals & patient advocacy groups**

ADL, activities of daily living; QoL, quality of life; SMA, spinal muscular atrophy; SMN, survival motor neuron.

1. Yang M, et al. *Adv Ther.* 2022;39:1915-1958; 2. Data on File, Scholar Rock, Inc.