Apitegromab Update

Anti-Myostatin Antibody With Transformative Potential in Patients with Type 2 and Type 3 SMA

Data extracted April 7, 2022

ScholarRock

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Agenda

SMA Disease Background & Current Treatment Landscape

Apitegromab TOPAZ Extension Period: 24-Month Data

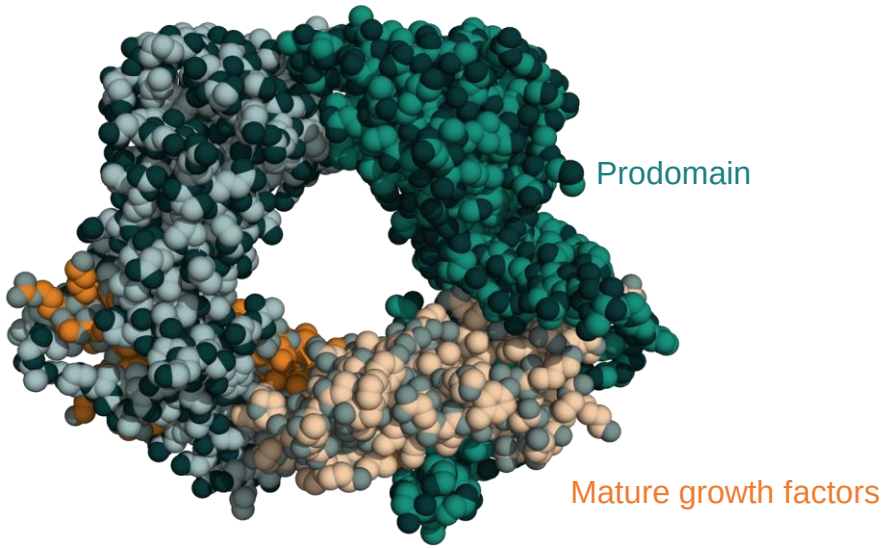
Apitegromab SAPPHIRE Phase 3 Trial Overview

Business Update

Revolutionary Approach to Regulating Highly Sought-After Growth Factors Implicated in Devastating Diseases

Scholar Rock's Target

Growth Factor Precursor (Latent Form)



Scholar Rock's R&D Platform

Transform Medical Practice

- Pursue important targets with well-validated biology but are difficult to drug
- Apply revolutionary approach to tough targets
  - Leverage deep insights into structure and function
  - Engineer antibodies to deliver differentiated therapeutic profiles (i.e. exquisite selectivity)

TOPAZ demonstrated the therapeutic potential of inhibiting the latent forms of growth factors

Apitegromab Shows Transformative Potential in Patients with Type 2 and 3 SMA

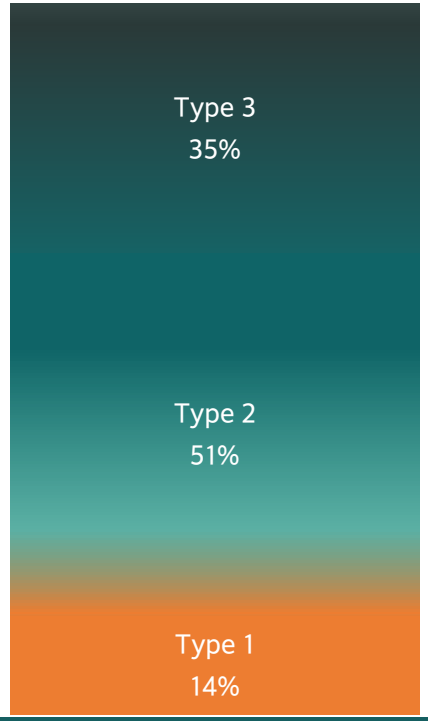
- Spinal Muscular Atrophy (SMA) remains a devastating and debilitating disease despite the utilization of SMN therapies
  - Motor neuron defect with loss of muscle mass
- Current therapies target motor neuron deterioration; muscle-directed therapy may further improve motor function
- Apitegromab targets myostatin for muscle function
  - Transformative potential in patients with non-ambulatory Type 2/3 SMA thru the TOPAZ Phase 2 trial
- Exciting potential path forward for apitegromab with the ongoing SAPPHIRE Phase 3 trial
- Further development in Type 1 SMA planned

Apitegromab is an investigational drug candidate being evaluated for the treatment of spinal muscular atrophy. Apitegromab has not been approved for any use by the FDA or any other regulatory agency and its safety and efficacy have not been established.

Spinal Muscular Atrophy Overview

Global disease with 30,000-35,000 affected in U.S. and Europe alone [1,2,3]

- Significant, progressive motor function impairment; many lose ambulation
- Severe, progressive disabilities and unable to walk independently
- Infantile onset; unable to sit up independently



TOPAZ* 12-month results showed transformative potential in non-ambulatory Types 2 and 3 patients

Represents ~2/3 of overall patient population

Motor neuron impairment and loss due to SMN genetic deficiency, leading to muscle atrophy and weakness

*TOPAZ Phase 2 trial evaluated patients with Type 2 and 3 SMA (did not include Type 1)

1. Lally et al, Orphanet Journal of Rare Diseases, 2017
2. SMA Europe. SMATracker. About SMA. Accessed January 24, 2022. https://smatracker.eu/what-is-spinal-muscular-atrophy.
3. National Organization for Rare Disorders. Spinal muscular atrophy. Accessed January 24, 2022. https://rarediseases.org/rare-diseases/spinal-muscular-atrophy/.

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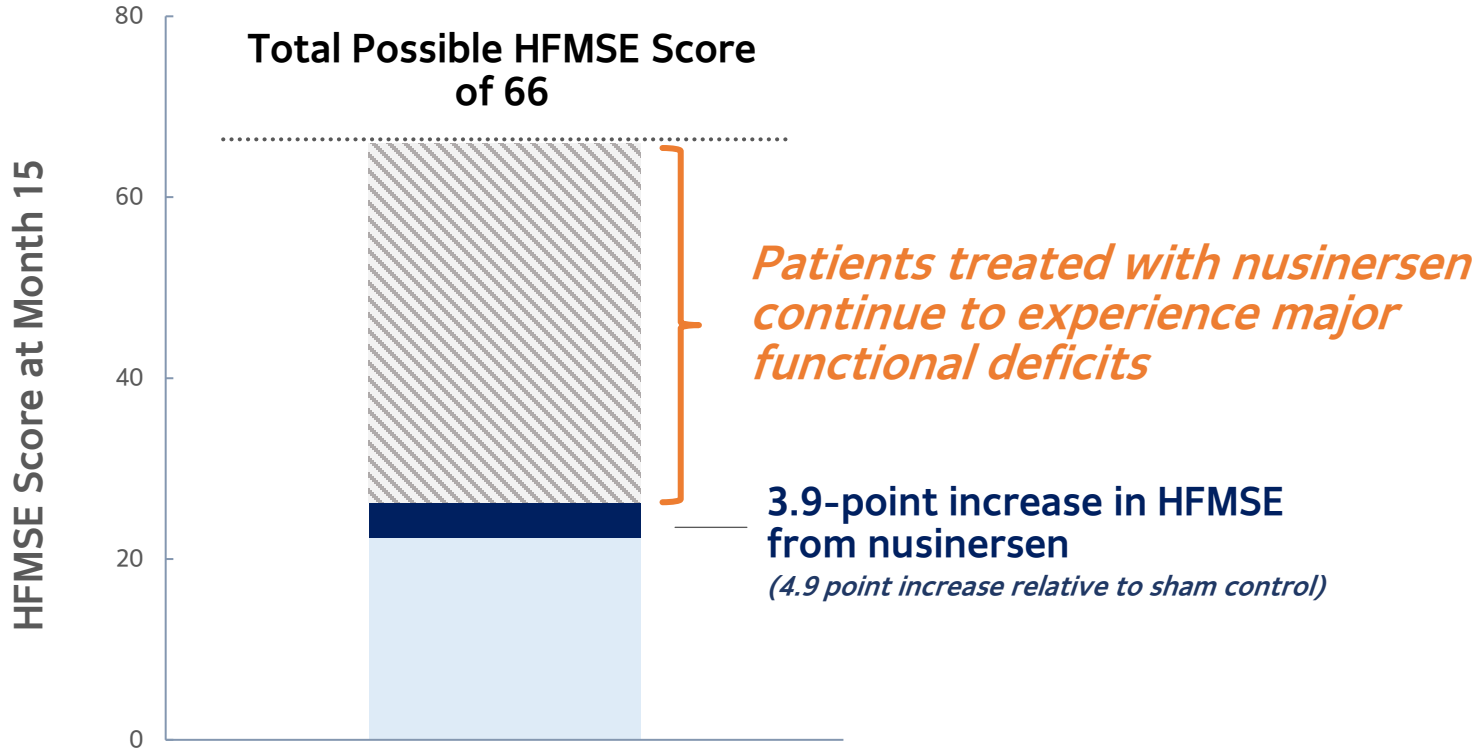
Patients with Types 2 and 3 SMA Continue to Experience Major Functional Deficits Despite Improvement from Nusinersen



Mean improvement in HFMSE experienced by patients with non-ambulatory Types 2/3 SMA in nusinersen Phase 3 CHERISH trial

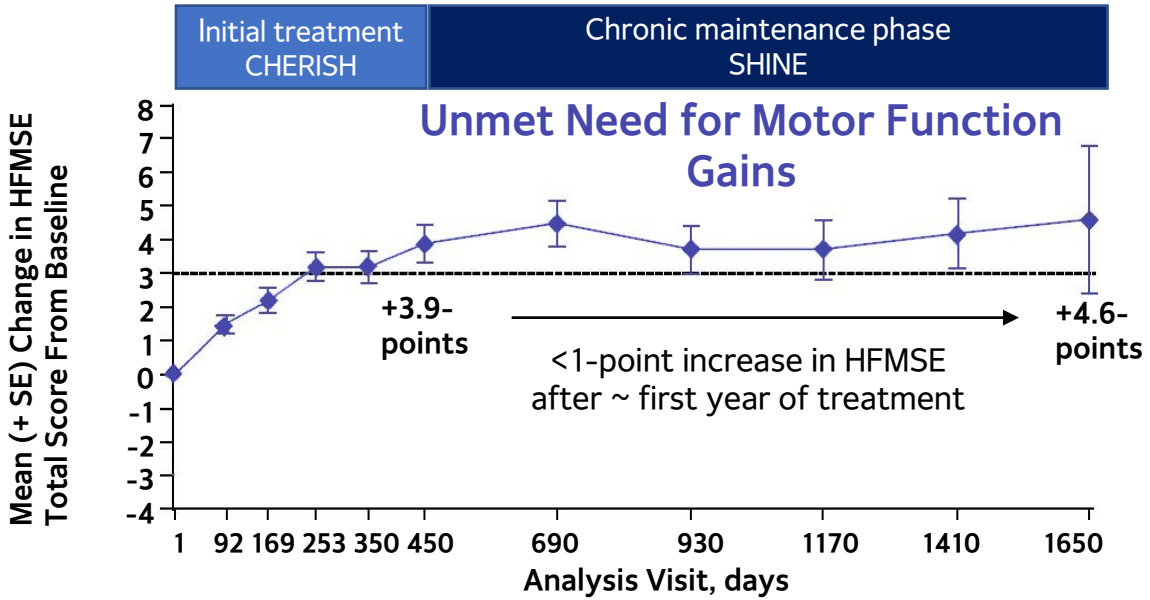
HFMSE=Hammersmith Functional Motor Scale-Expanded

Darras, B., et.al. Nusinersen in later-onset spinal muscular atrophy. Neurology. May 2019; 92 (21) e2492-e2506.

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.

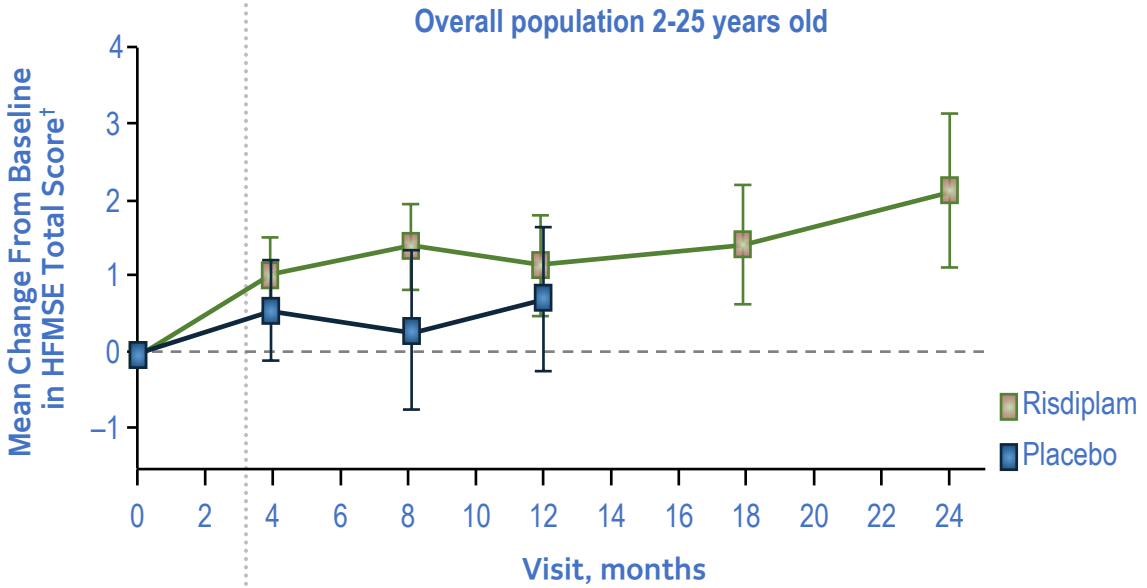
Motor Gains in Patients with Types 2 and 3 SMA on SMN Therapies Appear to Plateau After Initial Gains

Plateauing of HFMSE increases observed following initial treatment effects for nusinersen



NUSINERSEN[1]

Plateau of HFMSE increases observed following initial treatment effect of risdiplam, although longer timeframes currently under investigation [2]



RISDIPLAM[2]

HFMSE=Hammersmith Functional Motor Scale-Expanded.

1. Mercuri E, et al. Presented at: World Muscle Society Congress 2020, P. 257
2. Oskoui M, et al. Presented at: 2021 Muscular Dystrophy Association Clinical & Scientific Conference; March 15-18, 2021. Poster 80.

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.

Summary of TOPAZ Extension Period: 24-Month Data
Sustained Benefit and Evidence of Continued Improvement

❖ Non-Ambulatory Types 2/3 SMA

- **Motor function associated with activities of daily living improved with apitegromab + nusinersen at 24 months:**
  - Sizable and sustained gains in HFMSE scores observed
  - Continued increase in Revised Upper Limb Module (RULM) scores observed
  - Dose response observed over 24 months, with evidence of further motor function gains as low-dose patients switch to high-dose in extension period (2 mg/kg to 20 mg/kg)

❖ Ambulatory Type 3 SMA

- **Stability of Revised Hammersmith Scale (RHS) in patients receiving 20 mg/kg of apitegromab + nusinersen**
- **Potential motor function gains in subgroups**

❖ No serious safety risks identified to date

- **Five most common treatment-emergent adverse events (TEAEs) were headache, pyrexia, upper respiratory tract infection, cough, and nasopharyngitis**
- **Incidence and types of TEAEs were consistent with underlying disease or nusinersen therapy**

❖ Detailed analyses to be presented at Cure SMA (June 2022)

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TOPAZ Phase 2 Trial Design

	Ambulatory Patients (Revised Hammersmith Scale)	Non-Ambulatory Patients (Hammersmith Functional Motor Scale Expanded)	
	Cohort 1	**Cohort 2**	**Cohort 3**
Design	• N= 23; ages 5-21 • Open-label, single-arm • 20 mg/kg apitegromab IV Q4W • 12-month treatment period	• N= 15; ages 5-21 • Open-label, single-arm • 20 mg/kg apitegromab IV Q4W • 12-month treatment period	• N= 20; ages ≥2 • Double-blind, randomized (1:1) to 2 mg/kg or 20 mg/kg apitegromab IV Q4W • 12-month treatment period
Patients	• **Ambulatory Type 3 SMA** • Two subgroups: 1) Receiving nusinersen 2) Apitegromab monotherapy	• **Type 2 or Type 3 SMA** • **Receiving nusinersen (initiated at age 5 or older)**	• **Type 2 SMA** • **Receiving nusinersen (initiated before age 5)**
Primary Objectives	• Safety • Mean change from baseline in RHS	• Safety • Mean change from baseline in HFMSE	• Safety • Mean change from baseline in HFMSE

Apitegromab = nonproprietary name for SRK-015
HFMSE=Hammersmith Functional Motor Scale Expanded; RHS=Revised Hammersmith Scale
Data on file. Scholar Rock, Inc. Cambridge, MA

TOPAZ Subject Disposition, Demographics and Baseline Characteristics

		Cohort 1			Cohort 2	Cohort 3	
		20 mg/kg monotherapy	20 mg/kg + nusinersen		20 mg/kg + nusinersen	2 mg/kg + nusinersen	20 mg/kg + nusinersen
N (dosed)	Ambulatory Patients	11	12	NonAmbulatory Patients	15	10	10
Mean age at screening (min, max)		12.1 (7, 19)	13.1 (7, 21)		11.7 (8, 19)	4.1 (2, 6)	3.8 (2, 6)
Mean age at SMA diagnosis (min, max)		5.9 (2, 15)	4.5 (2, 15)		3.1 (1, 16)	1.2 (1, 2)	1.2 (1, 3)
Female (%)		73%	58%		53%	30%	50%
SMN2 Gene Copy* (#, %)							
2		1 (9%)	0 (0%)			1 (10%)	1 (10%)
3		4 (36%)	9 (75%)		11 (73%)	8 (80%)	8 (80%)
4		4 (36%)	1 (8%)		2 (13%)	1 (10%)	0 (0%)
# of maintenance doses of nusinersen at baseline (min, max)		N/A	5.6 (2, 8)		5.1 (2, 9)	5.5 (2, 9)	5.4 (3, 8)
Discontinuation(s)		0	2†		1†	0	0
Scoliosis (#, %)		7 (63.6)	4 (33.3)		11 (73.3)	4 (40%)	3 (30%)
Contracture(s) (#, %)		6 (54.5)	7 (58.3)		13 (86.7)	8 (80%)	4 (40%)
Mean RHS score (min, max)		47.6 (26, 63)	51.3 (43, 62)				
Mean HFMSE score (min, max)					22.7 (13, 39)	26.1 (12, 44)	23.5 (14, 42)

*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.
Data on File. Scholar Rock, Inc. Cambridge, MA.

Extension Period Overview

57 of 58 patients completed primary treatment period and enrolled in second year of therapy

- **55 of 57 patients completed**
- High dose (20 mg/kg) patients stayed on high dose
- Low dose (2 mg/kg) patients switched to high dose (20 mg/kg)*
- Four patients had scoliosis surgery during year 2**

54 of 55 patients remain on treatment as of June 1, 2022

*All ten patients switched from low dose to high dose. Time points varied across individual patients.
** Three non-ambulatory patients and one ambulatory patient

Significance of Hammersmith Functional Motor Scale Expanded (HFMSE)

Validated measure assessing the physical abilities of patients with Types 2/3 SMA

Examples of HFMSE items

Able to touch head above ear level whilst maintaining stable trunk and head position

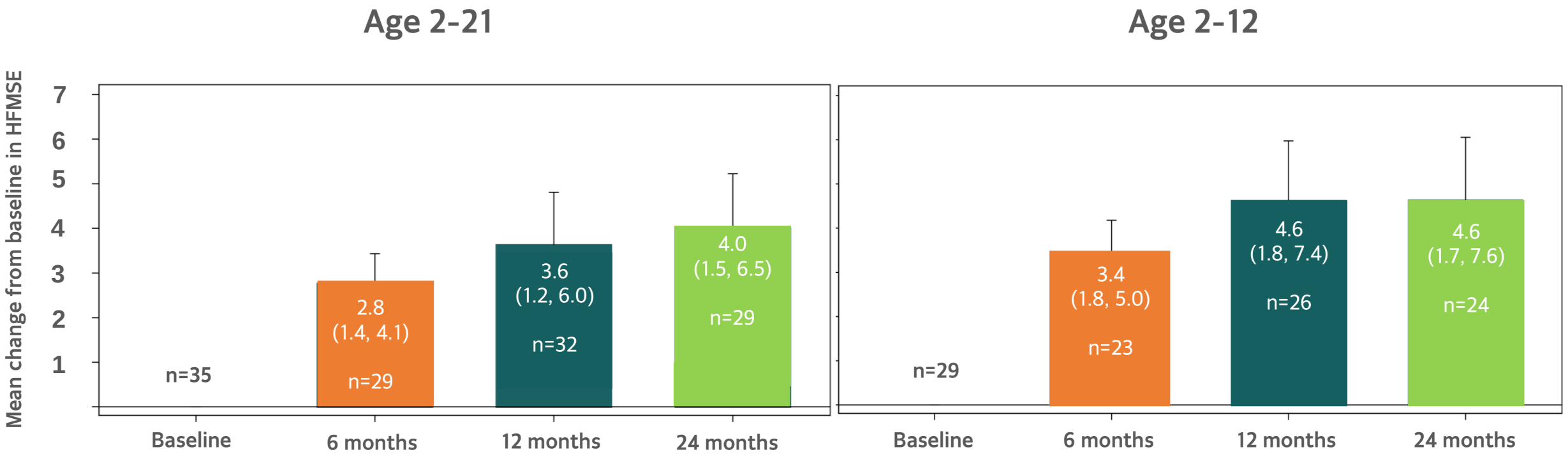
Able to roll from supine to prone over the right side without pulling/pushing on hands

- Consists of 33 items graded on a scale of 0 to 2: 0 denotes unable; 1 denotes performed with modification or adaptation; and 2 denotes without modification or adaptation.
- Item scores are summed to give a total score with a maximum of 66. The higher the total score, the greater the patient's motor function.
- Examples of items:
  - One hand to head in sitting
  - Rolls supine to prone
  - Lying to sitting
  - Four-point kneeling
  - Supported standing
  - Stepping
  - Ascends 4 stairs with railing

O'Hagen et al. 2007; Glanzman et al. 2011; Hammersmith Functional Motor Scale Expanded for SMA (HFMSE) Manual, 2019

Sizable, Sustained Increases in HFMSE Observed At 24 Months of Apitegromab

Pooled Non-Ambulatory Patients



Observed Case Analysis is based upon data available for a given timepoint, and this analysis population includes patients treated with the lower dose 2 mg/kg and does not exclude any patients who missed apitegromab doses due to COVID-19 site access restrictions. Error bars represent standard error of the mean (SEM). Values in parentheticals represent 95% confidence interval.

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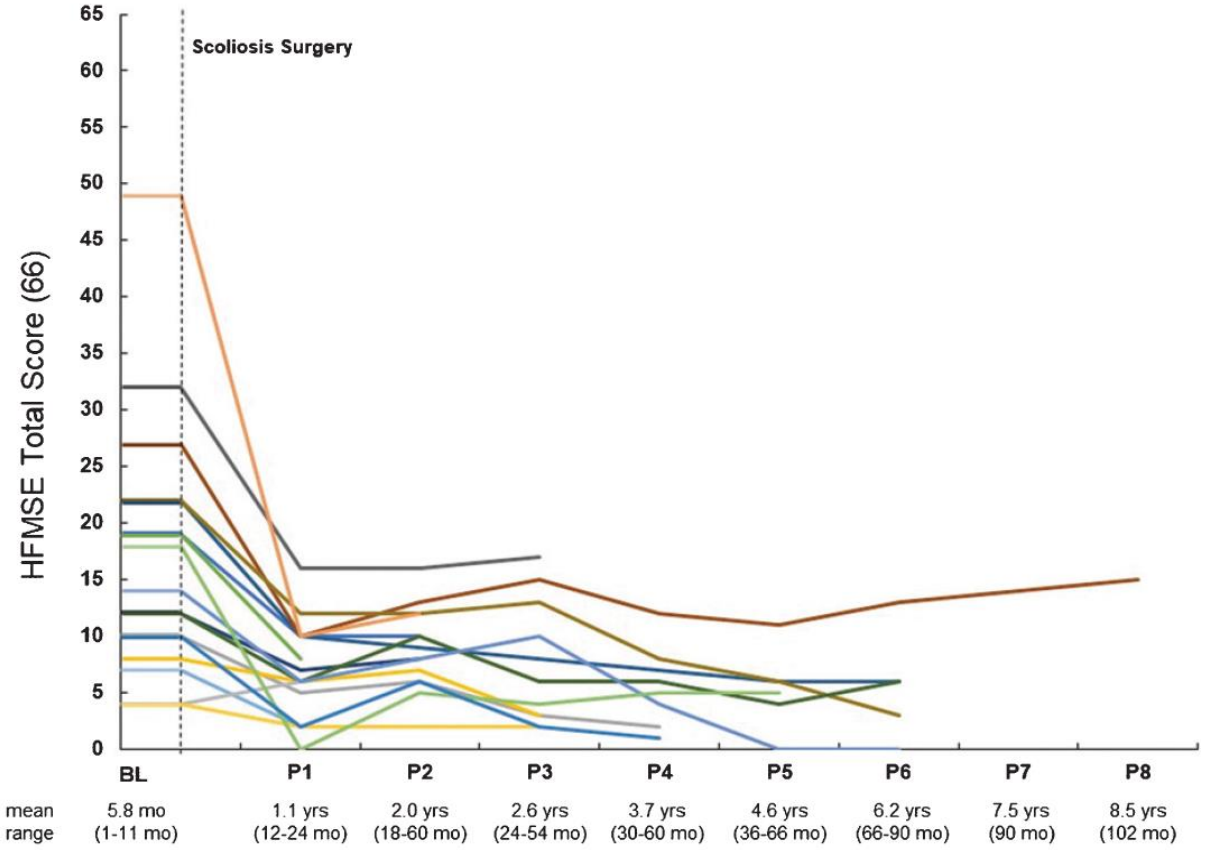
Data on File. Scholar Rock, Inc. Cambridge, MA.

Reported Impact of Scoliosis Surgery on Motor Abilities in SMA

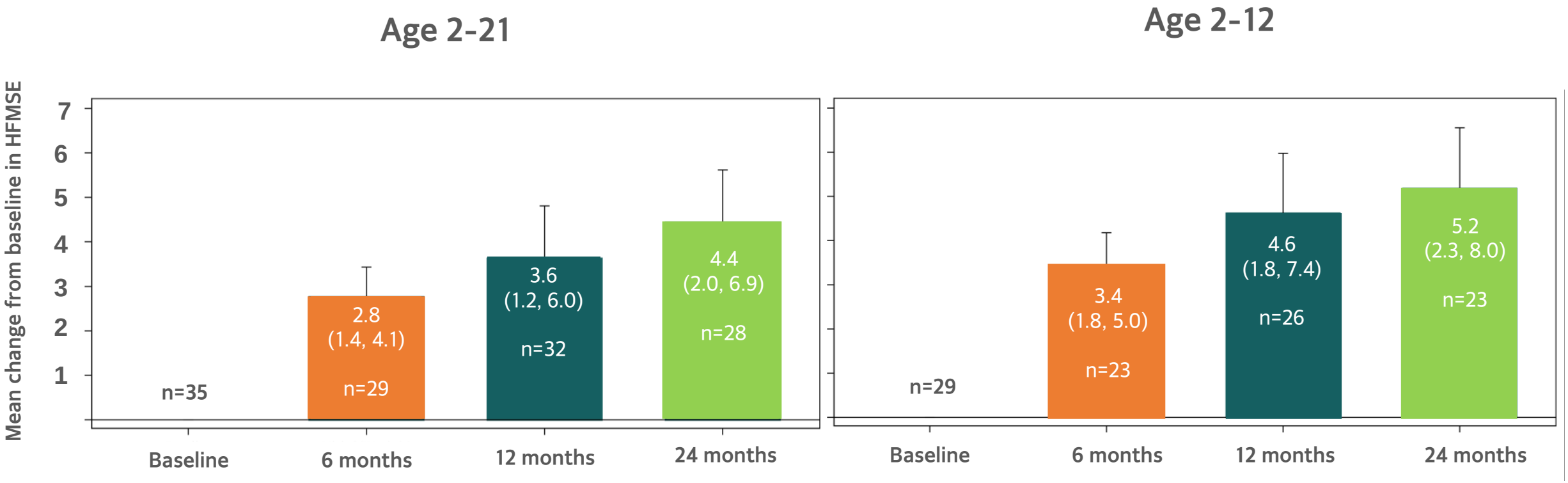
Post-Surgery HFMSE scores Type 2/3 SMA (*Dunaway Young et al. 2020)*



- The vast majority of patients in this peer-reviewed study lost >3 pts on the HFMSE as assessed at least 3 months post-surgery
  - 14/17 participants lost >3 points (mean change = - 12.1, SD = 8.9) on the HFMSE, representing a functionally meaningful change
  - 3/17 participants had minimal HFMSE changes within ±2 points (mean change = - 0.7), representing no change or stability
  - 0/17 participants had improvement greater than 2 points post-surgery

Sizable, Sustained Increases in HFMSE Observed At 24 Months of Apitegromab

Pooled Non-Ambulatory Patients Excluding Data Post Scoliosis Surgery



This analysis excludes from the Observed Case Analysis any HFMSE data following scoliosis surgery in TOPAZ. Of the three non-ambulatory patients who had scoliosis surgery, data from one was excluded and the other two did not have valid HFMSE assessments. Error bars represent SEM. Values in parentheticals represent 95% confidence interval.

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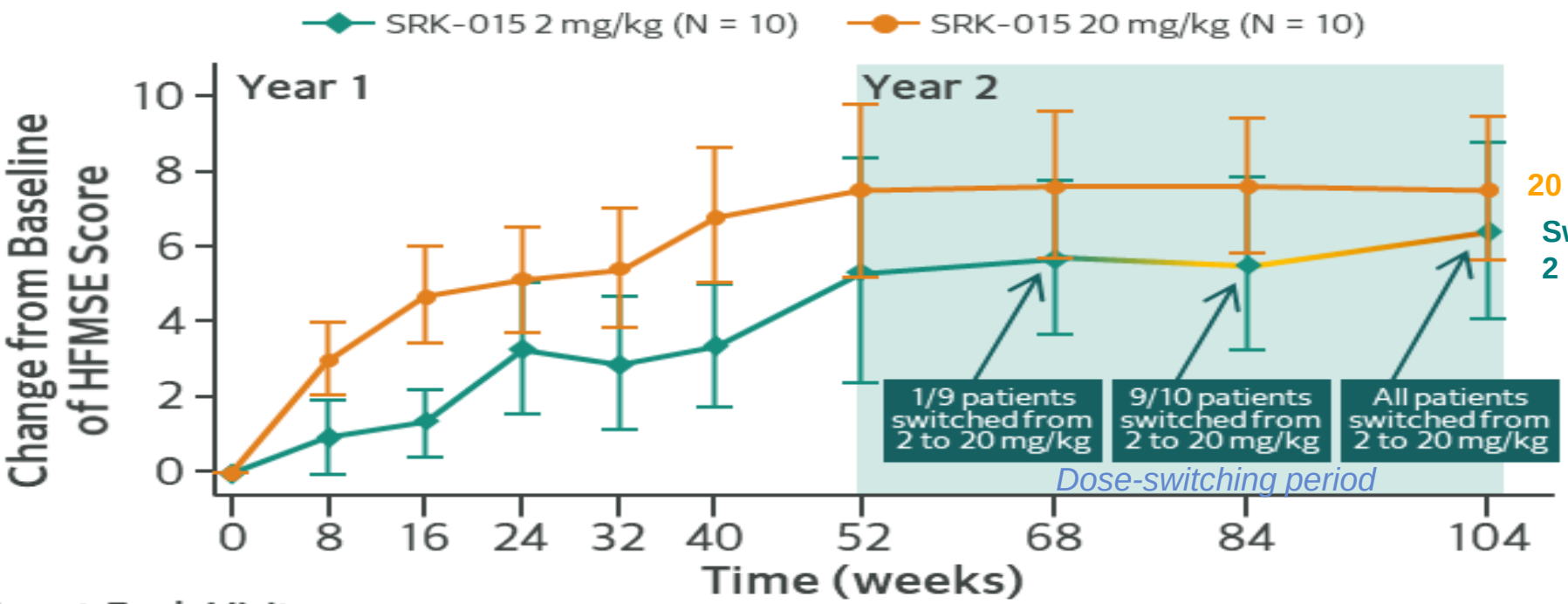
Data on File. Scholar Rock, Inc. Cambridge, MA.

Strong Evidence of Dose Response Observed Over 24 Months
Further Supported by Data from Low Dose to High Dose Switch

Mean Change from Baseline in HFSME Scores Over Time (Cohort 3)
Excludes data after scoliosis surgery



20 mg/kg throughout

Switched from 2 mg/kg to 20 mg/kg

Sample Size at Each Visit

Cohort 3 SRK 2 mg/kg	10	10	10	7	8	9	9	9	10	10
Cohort 3 SRK 20 mg/kg	10	10	10	8	8	10	8	10	10	8

This analysis excludes from the Observed Case Analysis the HFMSE data attained post-scoliosis surgery during TOPAZ. Error bars represent SEM.

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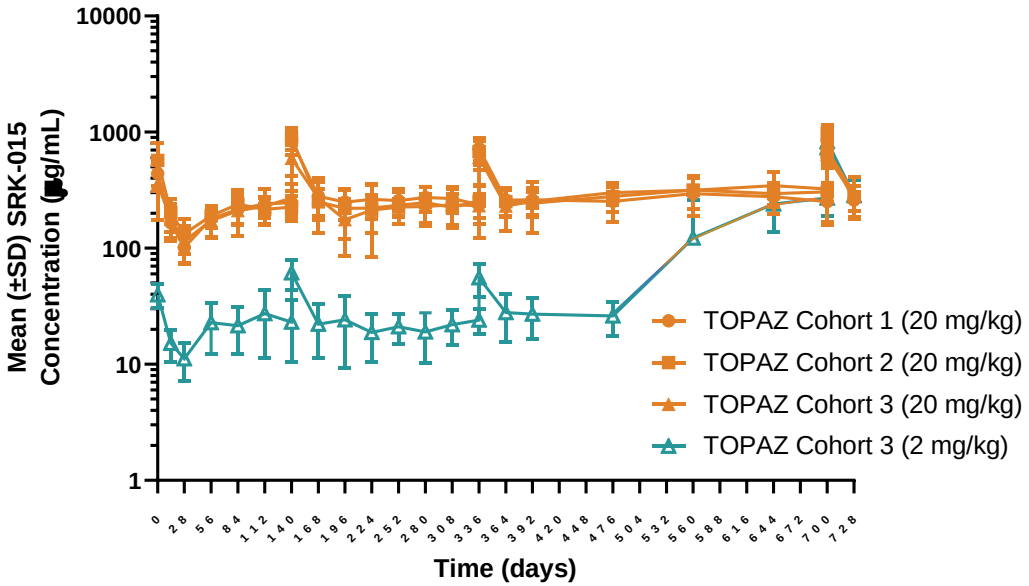
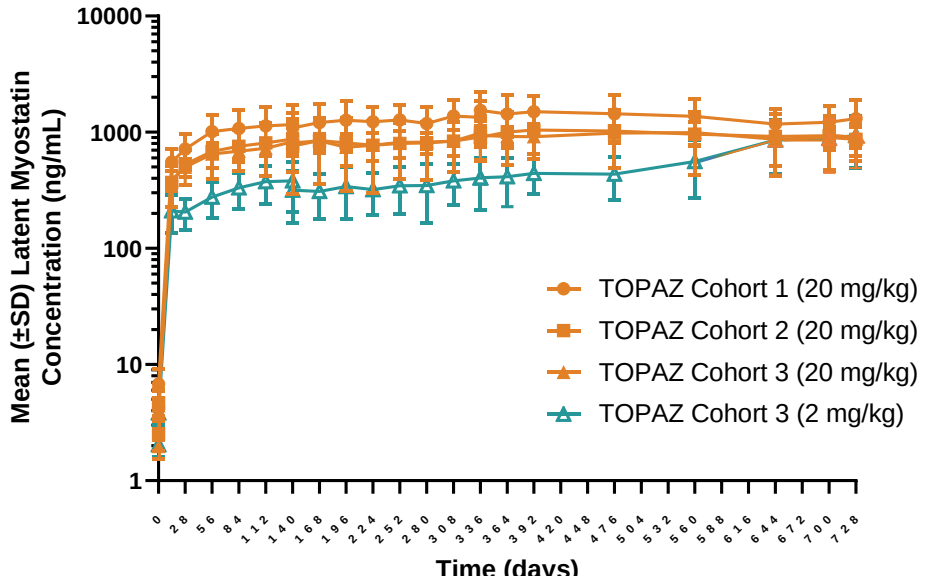
PK and PD Data are Consistent With Clinically Observed Dose Response

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 dose levels of drug exposure and target engagement were 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.

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Significance of the Revised Upper Limb Module (RULM)
Evaluates Motor Performance in Upper Limbs

Examples of RULM items

Able to bring token to cup placed vertically at shoulder height

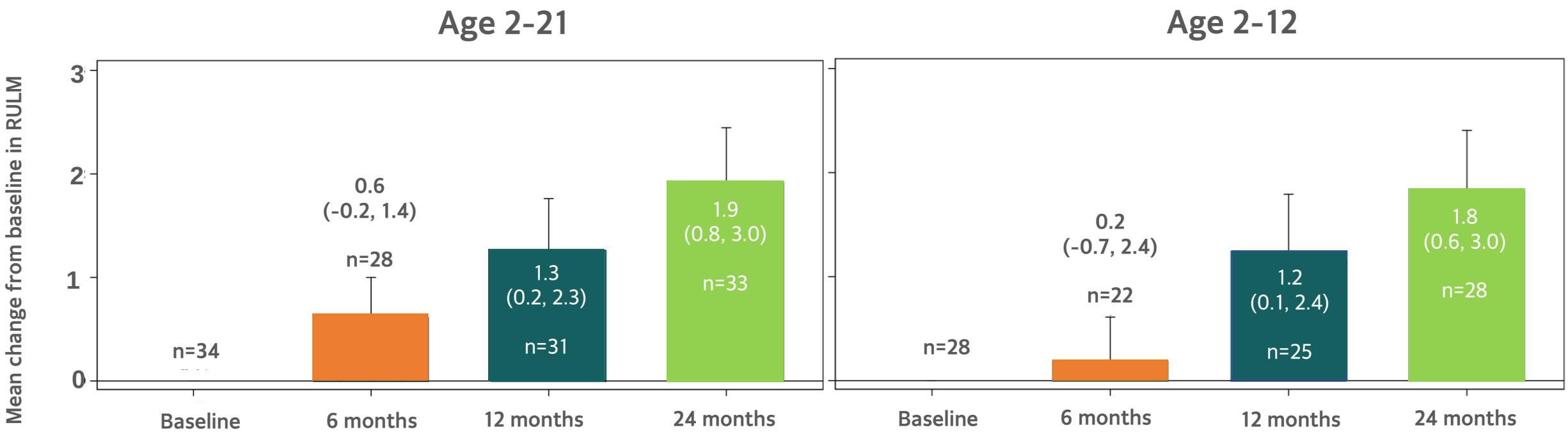
Brings weight at eye level using two hands

- Evaluated upper limb tasks correspond to ability to perform everyday activities
- Except for 1 activity with a binary score, the items are scored 0 to 2: 0 denotes unable; 1 denotes able with modification; and 2 denotes able with no difficulty
- Maximum score of 37 points (19 task items)
- Examples of items:
  - Putting a coin into a cup
  - Elevating a cup to mouth
  - Picking up a coin
  - Bringing hand to shoulder
  - Lifting up weighted objects
  - Opening a zip lock bag
  - Drawing a line on paper

Mazzone et al. 2017; Pierzchlewicz et al. 2021;
Revised Upper Limb Module for SMA Manual, 2014

Continued Increase in RULM Observed at 24 Months of Apitegromab

Pooled Non-Ambulatory Patients



Observed Case Analysis is based upon data available for a given timepoint, and this analysis population includes patients treated with the lower dose 2 mg/kg and does not exclude any patients who missed apitegromab doses due to COVID-19 site access restrictions. Error bars represent SEM. Values in parentheticals represent 95% confidence interval.

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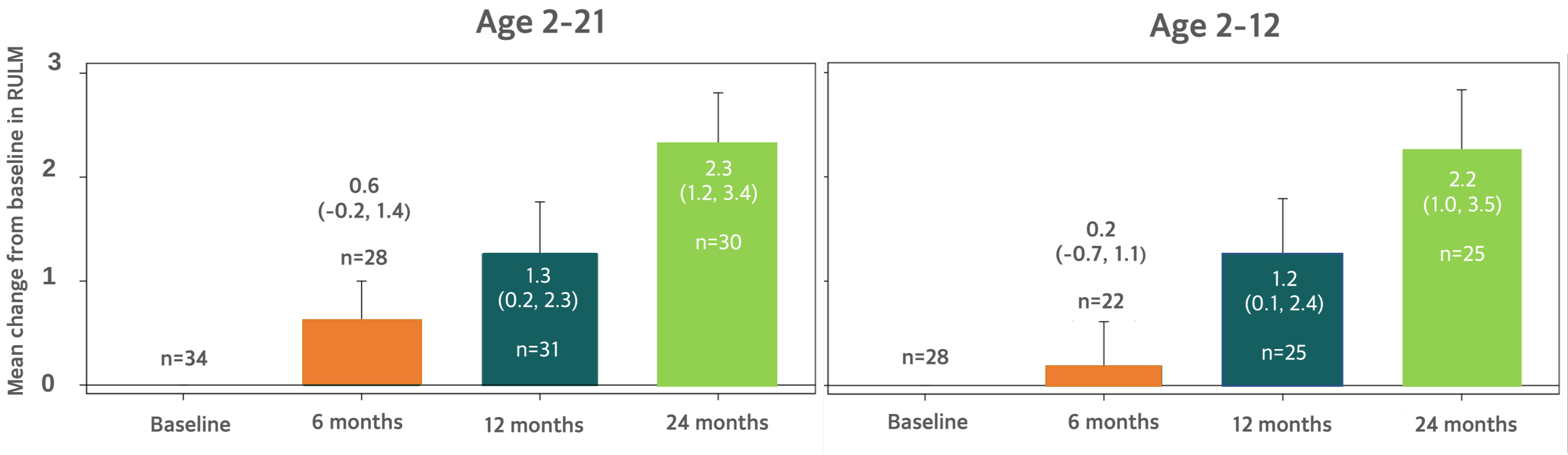
Data on File. Scholar Rock, Inc. Cambridge, MA.

Continued Increase in RULM Observed at 24 Months of Apitegromab

Pooled Non-Ambulatory Patients Excluding Data Post Scoliosis Surgery



This analysis excludes data from 3 non-ambulatory patients after their scoliosis surgery during TOPAZ from the Observed Case Analysis. Error bars represent SEM. Values in parentheticals represent 95% confidence interval.

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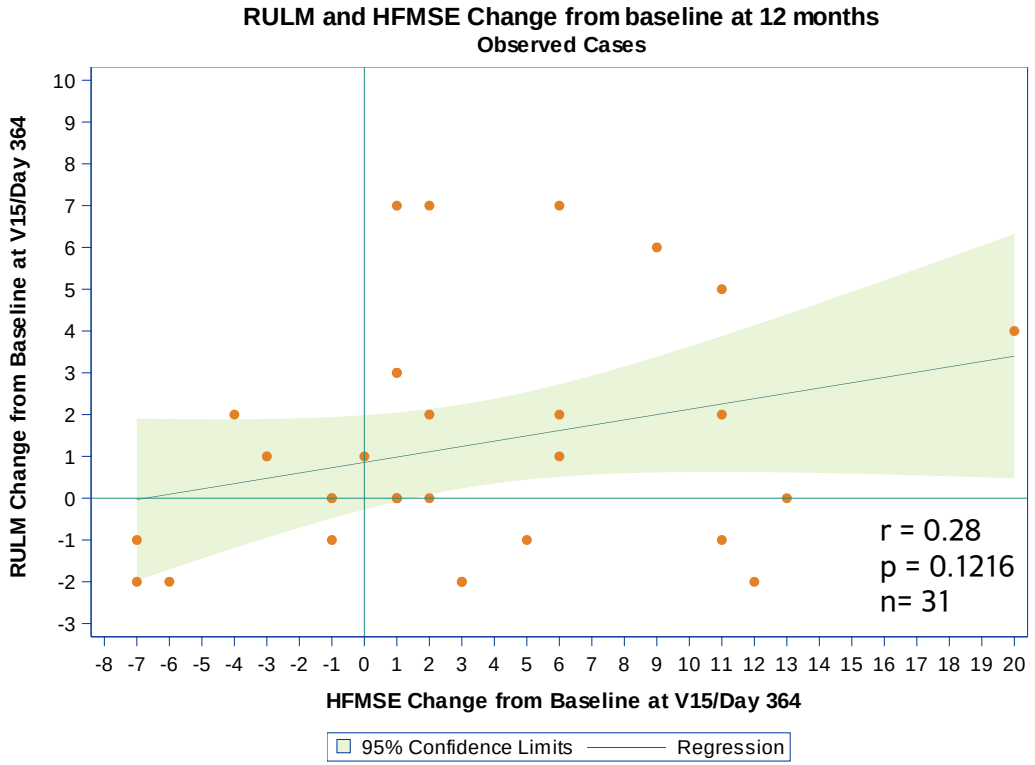
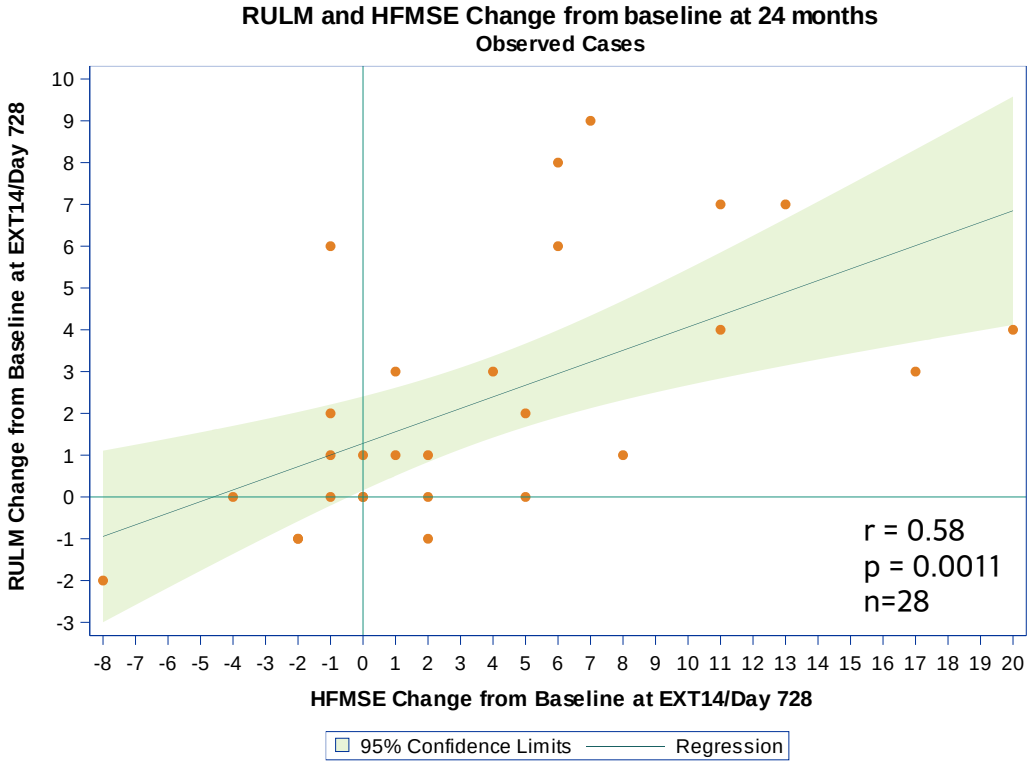
Data on File. Scholar Rock, Inc. Cambridge, MA.

Correlation of HFMSE to RULM Increased Over 24 Months
Pooled Non-Ambulatory Patients

> **"The observation that the majority of patients in this analysis experienced gains in both the HFMSE and RULM over 24 months further supports the therapeutic potential of apitegromab."**

-Basil Darras, MD *Associate Neurologist-in-Chief, Boston Children's Hospital; Professor of Neurology, Harvard Medical School; TOPAZ trial Investigator*





This analysis is based on the Observed Case Analysis population. The 12-month graph displays all patients who had a valid measurement at visit 15 (Day 364) and the 24-month graph displays all patients who had a valid measurement at extension visit 14 (Day 728).

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Therapeutic Potential of Apitegromab Observed in the Ambulatory Type 3 SMA Cohort at 24 Months

	Ambulatory Patients (Revised Hammersmith Scale; RHS)		
24 Month Analysis	Cohort 1		
	20 mg/kg pooled (n=21)	20 mg/kg monotherapy (n=11)	20 mg/kg + nusinersen (n=10)
Mean change from baseline (95% CI)	-1.8 (-4.7, 1.1)	-2.8 (-8.4, 2.8)	-0.7 (-3.1, 1.7)
# (%) pts achieving ≥1-pt increase	9/21 (42.9%)	5/11 (45.5%)	4/10 (40%)
# (%) pts achieving ≥3-pt increase	5/21 (23.8%)	3/11 (27.3%)	2/10 (20%)

Observed Case Analysis includes all patients who had a valid measurement at E14 (Day 728).
Inclusive of data from 3 patients in apitegromab monotherapy who lost ability to ambulate.

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No Serious Safety Risks Identified Over 24 Months of Apitegromab Treatment

Treatment-Emergent Adverse Events (TEAEs)*	2 mg/kg dose (N=10) n (%)	20 mg/kg dose (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 (0.0)	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 treatment-emergent AEs were consistent with the underlying disease or nusinersen therapy**
- **The 5 most common treatment-emergent AEs were headache, pyrexia, upper respiratory tract infection, cough, and nasopharyngitis**
- **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 in the 12-month analysis**
- **No serious safety risks identified to date**

*Notes: % = 100 x n/N (n=incidence)
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 collected for safety events over the 24-month period and includes patients who switched from 2 mg/kg to 20 mg/kg. Data on file, extracted on April 7, 2022. Scholar Rock, Inc. Cambridge, MA

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Summary of TOPAZ Extension Period: 24-Month Data
Sustained Benefit and Evidence of Continued Improvement

❖ Non-Ambulatory Types 2/3 SMA

- **Motor function associated with activities of daily living improved with apitegromab + nusinersen at 24 months:**
  - Sizable and sustained gains in HFMSE scores observed
  - Continued increase in Revised Upper Limb Module (RULM) scores observed
  - Dose response observed over 24 months, with evidence of further motor function gains as low-dose patients switch to high-dose in extension period (2 mg/kg to 20 mg/kg)

❖ Ambulatory Type 3 SMA

- **Stability of Revised Hammersmith Scale (RHS) in patients receiving 20 mg/kg of apitegromab + nusinersen**
- **Potential motor function gains in subgroups**

❖ No serious safety risks identified to date

- **Five most common treatment-emergent adverse events (TEAEs) were headache, pyrexia, upper respiratory tract infection, cough, and nasopharyngitis**
- **Incidence and types of TEAEs were consistent with underlying disease or nusinersen therapy**

❖ Detailed analyses to be presented at Cure SMA (June 2022)

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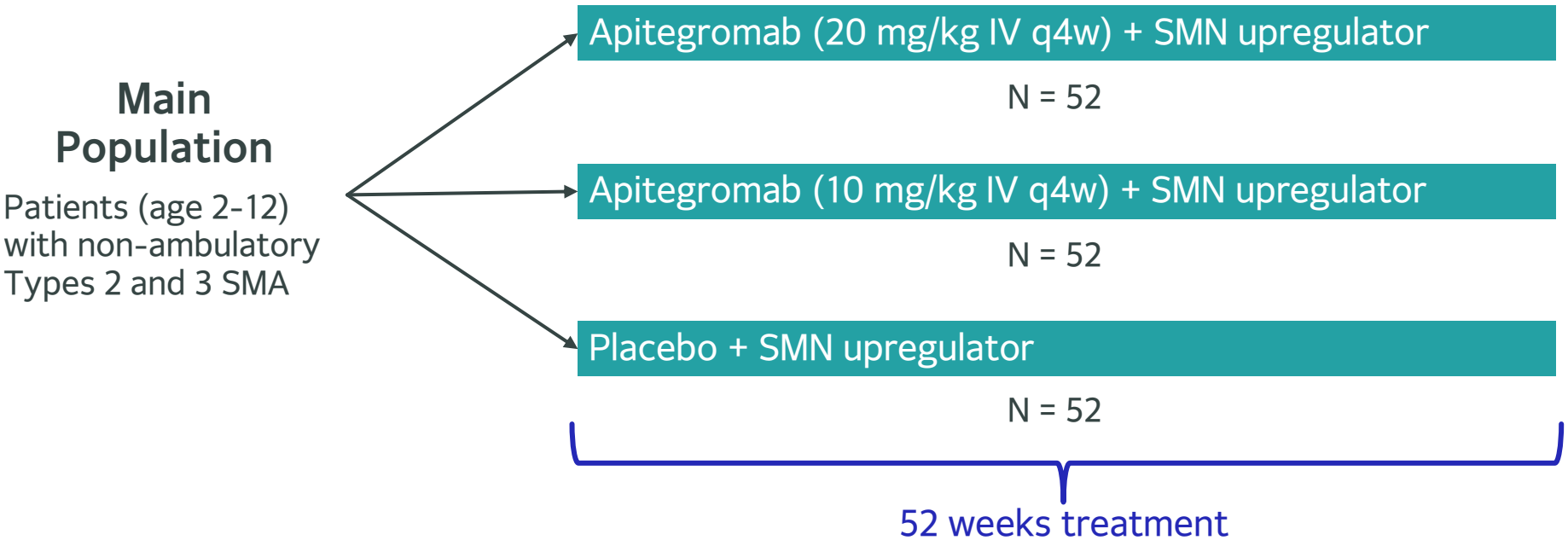
Ongoing SAPPHIRE Phase 3 Trial Overview



SAPPHIRE

- Randomized, double-blind, placebo-controlled, parallel arm design
- Enrolling patients on SMN therapy (nusinersen or risdiplam)
- Primary efficacy endpoint: mean HFMSE change from baseline at 12 months
- Study start-up activities commenced

SAPPHIRE Details

Main population

- Age 2-12, non-ambulatory Type 2 and Type 3 SMA
- Maintenance phase of SMN therapy (nusinersen ≥10 months; risdiplam ≥ 6 months)
- Stratified randomization to ensure balanced allocation: 1) age at SMN Rx initiation (age < 5 vs age ≥ 5) 2) SMN Rx (nusinersen vs. risdiplam)

Endpoints

- Primary efficacy: HFMSE
- Additional efficacy measures: RULM, WHO, other outcome measures
- Safety, PK/PD, ADA

Analysis

- Topline readout based of main efficacy population (age 2-12) is apitegromab 20 mg/kg* vs. placebo
- Interim analysis opportunity when ≥ 50% of patients in main efficacy population have completed 12 months

Additional Data Opportunities

- Exploratory population (age 13-21), in patients using SMN therapy; focused upon safety & exploratory efficacy (n=48; 2:1 randomization between apitegromab 20 mg/kg vs placebo)
- Separate open-label extension study (after patients complete 12-month period); focused upon safety & exploratory long-term efficacy

*To control type I error caused by multiple comparisons, the efficacy analysis will first compare the apitegromab 20 mg/kg arm against placebo before any testing of apitegromab 10 mg/kg against placebo.
PK/PD=pharmacokinetics and pharmacodynamics; ADA=anti-drug antibodies

Focused Pipeline of Novel Product Candidates

Potential to transform the lives of patients suffering from a wide range of serious diseases, including neuromuscular disorders, cancer, and fibrosis

APITEGROMAB

A selective inhibitor of myostatin activation being developed as the potential first muscle-directed therapy for the treatment of spinal muscular atrophy (SMA) in multiple clinical trials

- Phase 2 TOPAZ study (extension portion ongoing)
- Phase 3 SAPPHIRE trial

SAPPHIRE

Phase 3 Pivotal Trial

SRK-181

A selective inhibitor of latent TGFβ1 activation being developed with the aim of overcoming primary resistance to and increasing the number of patients who may benefit from checkpoint inhibitor therapy

- Potential to become the next transformative therapy for cancer patients
- Phase 1 DRAGON (Part B ongoing)

DRAGON

Phase 1 Proof of Concept Trial

Discovery-stage pipeline focused on the selective modulation of growth factor signaling

Key Investment Highlights

Proprietary Platform

- **Designed to discover and develop** monoclonal antibodies that can modulate growth factors with extraordinary selectivity
- **Designed to overcome the challenges** that have plagued traditional approaches by targeting the precursor or latent forms of growth factors

Robust Pipeline

- **Apitegromab (Phase 3)**: potentially transformative therapy to improve motor function for patients with SMA
- **SRK-181 (Phase 1)**: potential to shift the treatment landscape for cancer patients with CPI resistance
- **Discovery-stage pipeline**: focus on myostatin and TGFβ

SAPPHIRE

Phase 3 Pivotal Trial

DRAGON

Phase 1 PoC Trial

Financing Announced Today:

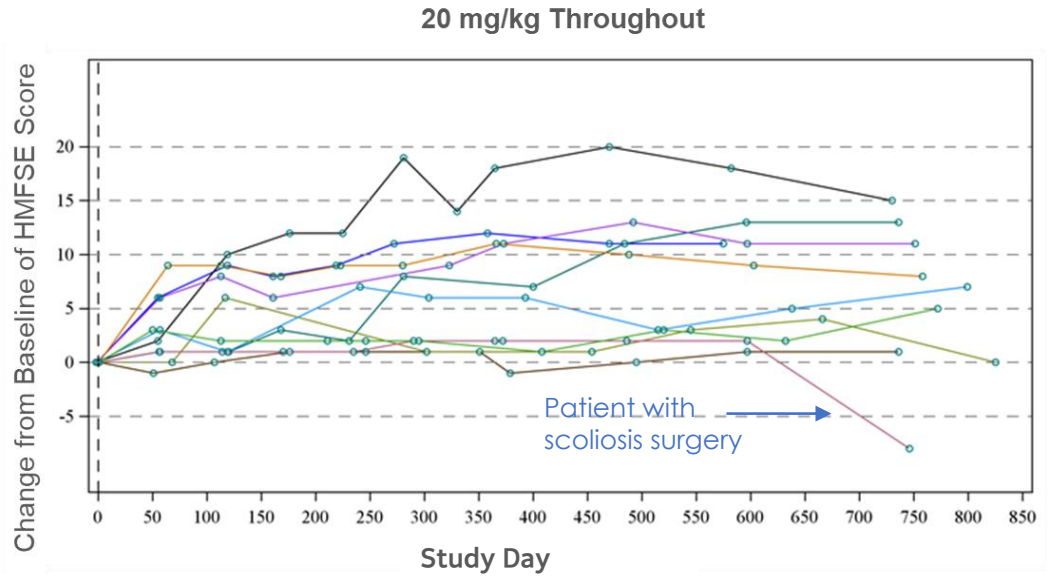
-Fully Funds Sapphire, Phase 3 Pivotal Trial, Continued Advancement of Part B Dragon Trial

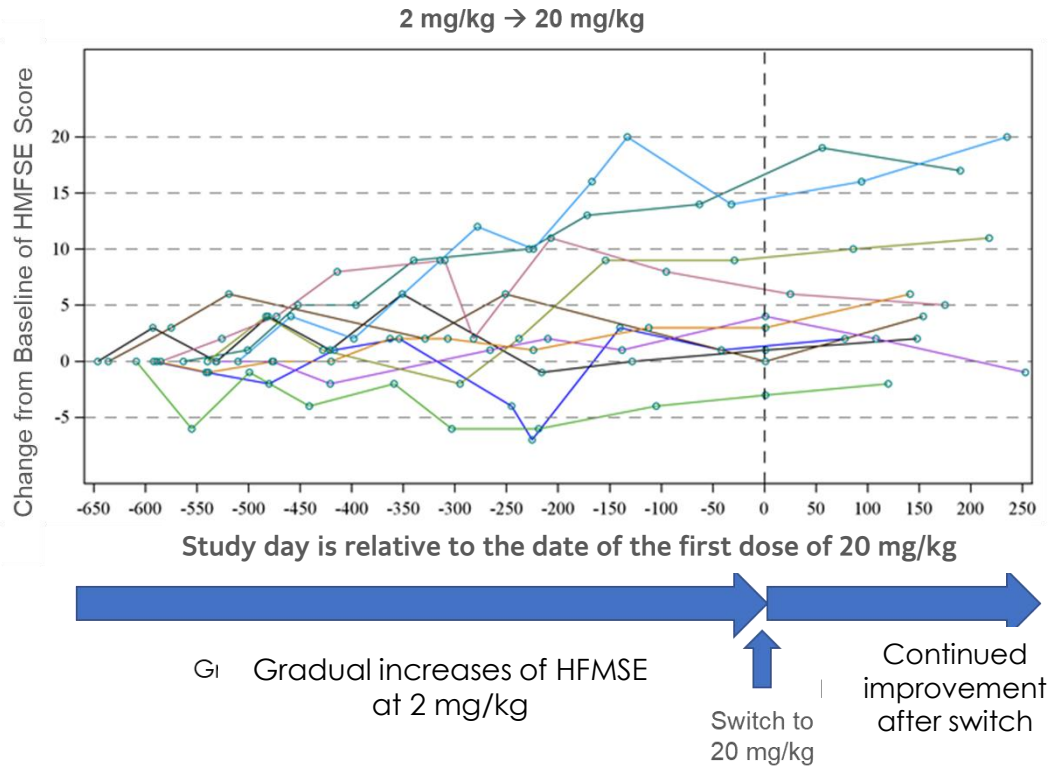
-Runway Extended into 2025

Appendix

Strong Evidence of Dose Response Observed Over 24 Months
Further Supported by Data from Low Dose to High Dose Switch

Most patients in Cohort 3 who switched from
2 mg/kg to 20 mg/kg continued to show HFMSE improvement





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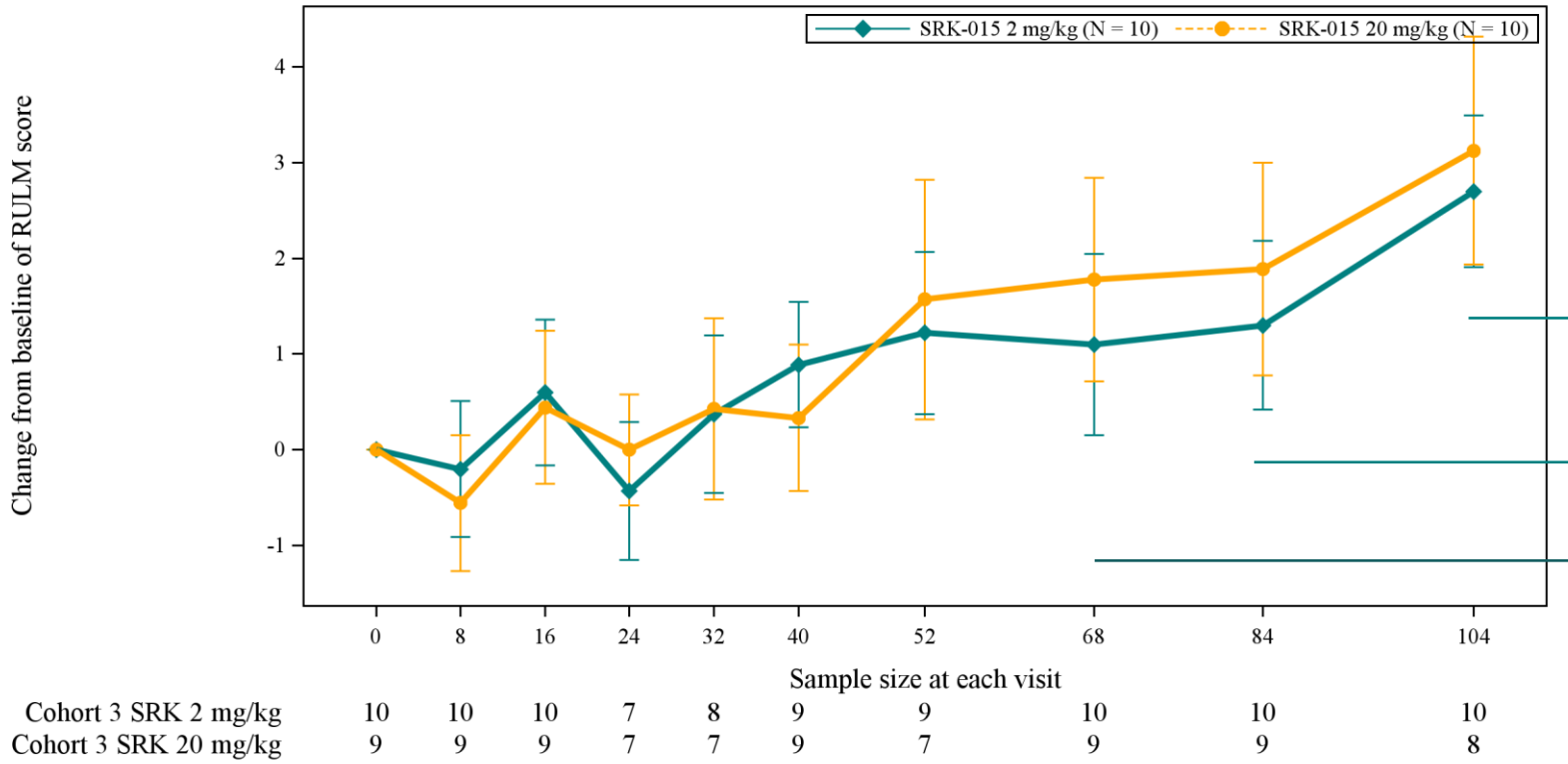
Cohort 3: Mean RULM Score Change Over Time

RULM trended up in low dose arm patients after switch to high dose

Mean Change from Baseline in RULM Scores Over Time (Cohort 3)

Excludes data after scoliosis surgery



Cohort 3 SRK 2 mg/kg	10	10	10	7	8	9	9	10	10	10
Cohort 3 SRK 20 mg/kg	9	9	9	7	7	9	7	9	9	8

All patients in Cohort 3 switched from 2 mg/kg to 20 mg/kg

9 of 10 patients in Cohort 3 switched from to 2 mg/kg to 20 mg/kg

1 of 9 patients in Cohort 3 switched from 2 mg/kg to 20 mg/kg

Observed Case Analysis is based upon data available for a given timepoint, and this analysis population includes patients treated with the lower dose 2 mg/kg and does not exclude any patients who missed apitegromab doses due to COVID-19 site access restrictions. Error bars represent standard error of the mean (SEM). Error bars represent SEM.

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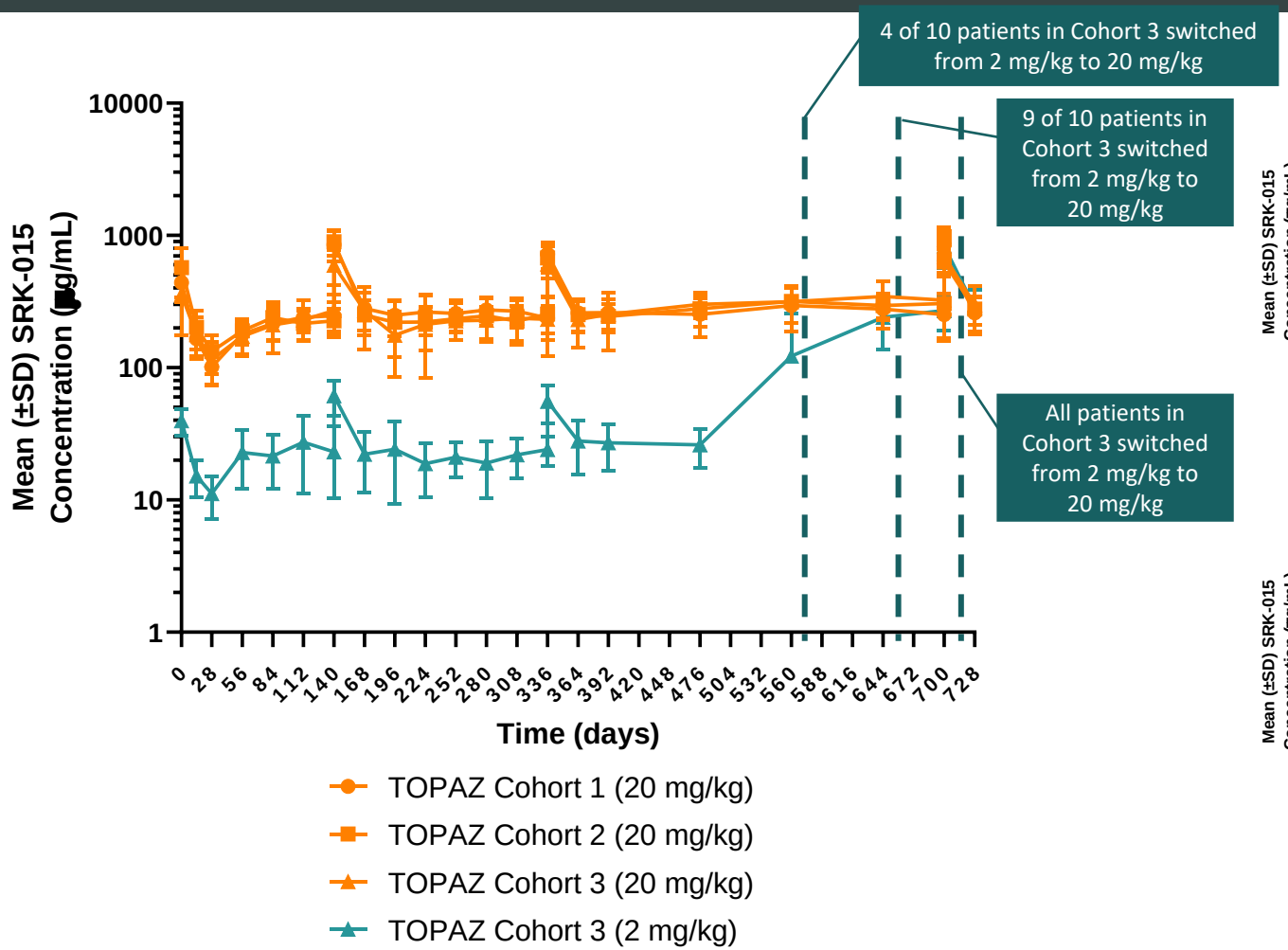
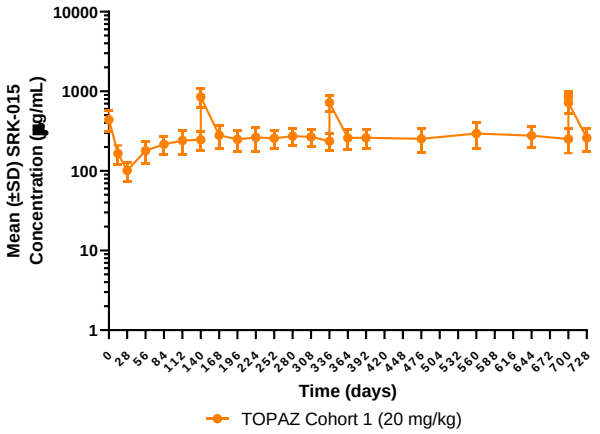
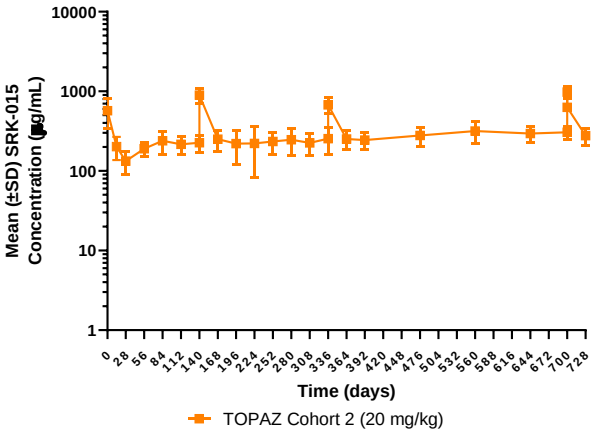
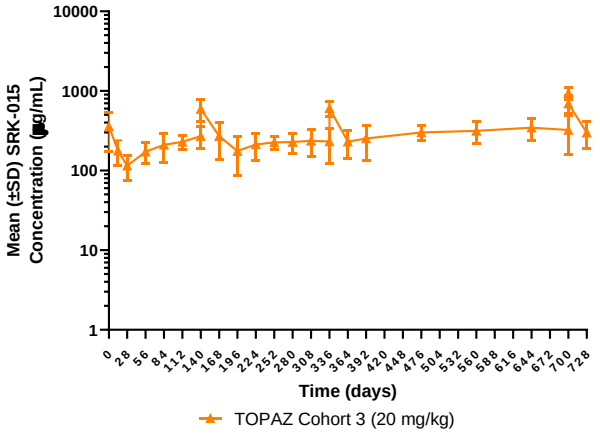
ScholarRock

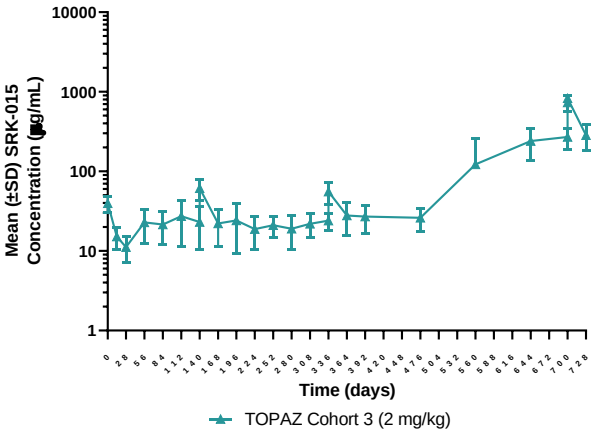
PK Data Consistent With Clinically Observed Dose Response











Cohort 3 2 mg/kg Timing Patient Conversion Timeline to 20 mg/kg:

- Day 560: 105-304, 110-301, 110-302, and 114-301
- Day 644: 105-301, 107-302, 118-301, 302-301, and 501-301
- Day 700: 103-301

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TOPAZ Extension Period: 24-Month Patient Disposition

	Cohort 1 Ambulatory	Non-Ambulatory		
		Cohort 2	Cohort 3	Total
# Non-Ambulatory Patients (2-21)		15	20	35
# Non-Ambulatory Patients (2-12)		9	20	29
Dropped Out (0-12 M)	1	0	0	0
Dropped Out (12-24 M)	1	1		1
Not Having Valid HFMSE testing at Month 24	Not applicable	5*	1**	6
Not Having RULM at Month 24	Not applicable	2***	1****	3
# of patients scoliosis surgery	1	2*****	1	3

**Includes 1 patient who withdrew from study; 1 patient off schedule due to scheduled surgery; 1 patient who had hip pain; 1 patient with femur fracture; and 1 patient who refused to be in supine position.*

***Patient with bilateral lower extremity cast*

****Includes 1 patient withdrew from study, and 1 patient off schedule due to planned surgery.*

*****Patient was too young for RULM at baseline and RULM was not conducted at following visit.*

******Patients did not have valid HFMSE test at 24 months.*

Overall Safety and Tolerability Profile Over 24 Months of Treatment: Serious TEAEs

- Fourteen patients experienced a serious TEAE, all assessed by the respective trial investigator as unrelated to apitegromab:
    - One patient treated with 2 mg/kg dose (Cohort 3) was hospitalized due to adenoidal hypertrophy and tonsillar hypertrophy to perform scheduled adenotonsillectomy (Grade 2). Events resolved without sequelae.
    - Two patients treated with 20 mg/kg dose (both Cohort 1) presented with gait inability considered a significant disability (both Grade 3). Events remain ongoing.
    - One patient treated with 20 mg/kg dose (Cohort 1) was hospitalized with post lumbar puncture syndrome (Grade 2). Event resolved without sequelae.
    - One patient treated with 20 mg/kg dose (Cohort 1) was hospitalized due to viral upper respiratory tract infection (Grade 2). Event resolved without sequelae.
    - Five patients treated with 20 mg/kg dose (one from Cohort 1, three from Cohort 2, and one from Cohort 3) were hospitalized for spinal fusion surgery/ scoliosis/ scoliosis surgery (all Grade 3). All events resolved without sequelae.
    - One patient treated with 20 mg/kg dose (Cohort 1) was hospitalized due to bilateral developmental hip dysplasia and left hip dislocation (both Grade 3). Events resolved without sequelae.
    - One patient treated with 2 mg/kg dose (Cohort 3) was hospitalized due to hip dislocation (Grade 3). Event resolved with sequelae (anxiety and post-operative pain).
    - One patient treated with 20 mg/kg dose (Cohort 3) was hospitalized due to respiratory syncytial virus infection (Grade 2). Events resolved without sequelae.
    - One patient treated with 2 mg/kg dose (Cohort 3) was hospitalized due to vomiting and pneumonia (Grade 3). Events resolved without sequelae.

Overall Safety and Tolerability Profile Over 24 Months of Treatment: Non-Serious Grade 3 Events

- Four patients presented with non-serious Grade 3 events, all assessed by the respective trial investigator as unrelated to apitegromab:
    - One patient treated with 20 mg/kg dose (Cohort 1) presented with post lumbar puncture syndrome. Event resolved without sequelae.
    - One patient treated with 20 mg/kg dose (Cohort 2) presented with worsening of scoliosis. Event resolved (with surgery, reported as serious, above) without sequelae.
    - One patient treated with 20 mg/kg dose (Cohort 2) presented with osteopenia. Events remains ongoing.
    - One patient treated with 2 mg/kg (Cohort 3) presented with two instances of hypoglycemia and one instance of metabolic acidosis. All events resolved without sequelae.
    - One patient (Cohort 1) discontinued from the trial due to Grade 2 muscle fatigue that started prior to initiation of dosing with study drug; assessed by the trial investigator as unrelated to apitegromab.