TOPAZ Phase 2 Trial Top-Line Results

Improvements in Motor Function with Apitegromab for Patients with Spinal Muscular Atrophy (SMA)

April 6, 2021

ScholarRock™

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Agenda

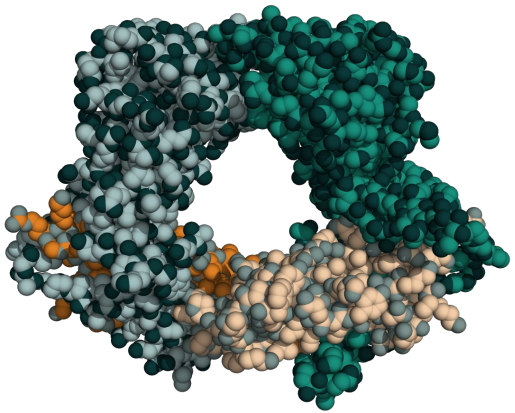
Opening Remarks	Tony Kingsley, President & CEO
Trial Design and Baseline Characteristics **Top-line Safety and Efficacy Results**	Yung Chyung M.D., Chief Medical Officer
Next Steps for Apitegromab Program	Tony Kingsley, President & CEO Ted Myles, Chief Financial Officer

Questions and Answers

Bringing a Revolutionary Approach to 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 demonstrates the therapeutic potential of inhibiting the latent forms of growth factors

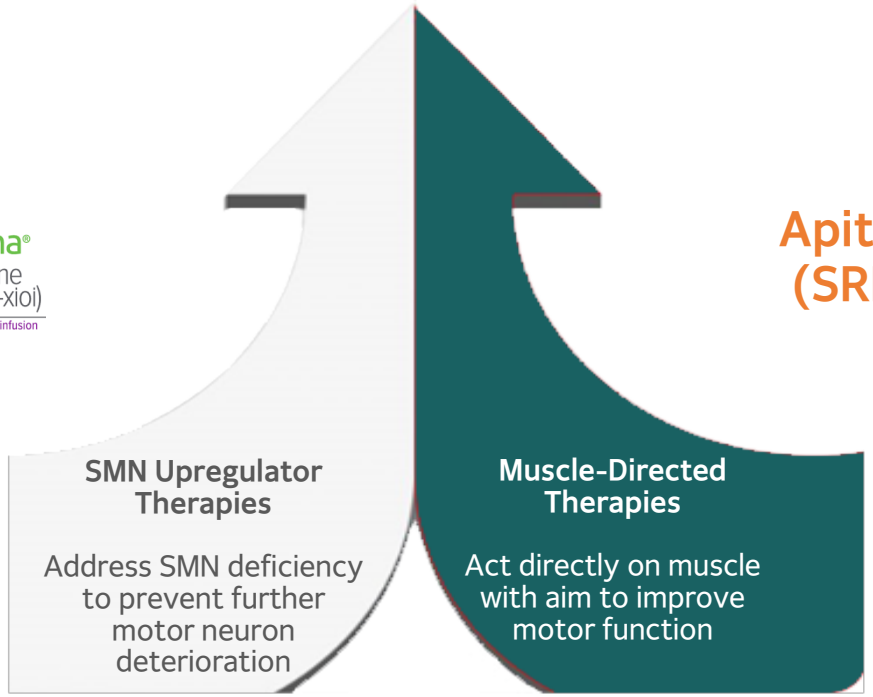
Apitegromab Offers Potential to Pioneer a New Treatment Era to Improve Motor Function in Patients with SMA

SMN Upregulator Therapies + Muscle-Directed Therapy (apitegromab)*
Potential for Enhanced Outcomes for Patients

SPINRAZA® (nusinersen) injection 12 mg/5 mL

zolgensma® (onasemnogene abeparvovec-xioi) suspension for intravenous infusion

Evrysdi™ risdiplam 60 mg powder for oral solution



Apitegromab (SRK-015)**

SMN = survival motor neuron.
**Also referred to as SMN correctors.*
***Apitegromab (SRK-015) is an investigational product candidate under development.*

TOPAZ 12-Month Data Further Support the Potential of Apitegromab in Patients with Type 2 and Type 3 SMA

Adverse events were consistent with the underlying patient population and background therapy

	Cohort 1 Ambulatory Type 3	Cohort 2 Type 2 & non-ambulatory Type 3 (initiated nusinersen ≥5 yrs)	Cohort 3 Type 2 (initiated nusinersen <5 yrs)
TOPAZ 6-month interim results*	• Mean RHS increase from baseline • Majority of patients maintained or improved (≥0-pt change from baseline)	• Mean HFMSE increase from baseline • Majority of patients improved (≥1-pt increase from baseline)	• Mean HFMSE increases from baseline • Majority of patients achieved ≥3-pt increase • Dose response observed
TOPAZ 12-month top-line results	• Mean RHS decline from baseline • Majority of patients maintained or improved (≥0-pt change from baseline)	• Mean HFMSE increase from baseline • Majority of patients improved (≥1-pt increase from baseline) • Sizeable % of patients achieved ≥3-pt increase (29%)	• Further HFMSE increases observed vs. 6-month interim analysis • Majority of patients achieved ≥5-pt increase • Dose response continues to be observed

*Database for HFMSE and RHS scores for the 12-month topline analysis are locked. The 6-month interim analysis was a snapshot and subsequent adjustments by sites investigators resulted in the following changes to the 6-month interim results:

- Cohort 2: Mean change in HFMSE score from baseline was updated to +1.1-points (from +1.4-points). Proportion of patients with ≥3-point increases was updated to 2/14 (from 3/14) and updated to 1/14 (from 2/14) for patients with ≥5-point increases.
- Cohort 3 high dose arm (20 mg/kg): Mean change in HFMSE score from baseline was revised to +5.3-points (from +5.6-points).

Phase 2 Trial Design and 12-Month Top-Line Results

Yung Chyung, M.D.
Chief Medical Officer

ScholarRock

Apitegromab Phase 2 Trial Design

TOPAZ

	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 background nusinersen 2) Apitegromab monotherapy	• Type 2 or non-ambulatory Type 3 SMA • Receiving background nusinersen (initiated ≥5 years of age)	• Type 2 SMA • Receiving background nusinersen (initiated before 5 years of age)
Primary Objectives	• Safety • Mean change from baseline in RHS	• Safety • Mean change from baseline in HFMSE	• Safety • Mean change from baseline in HFMSE

Evaluate potential of apitegromab in improving motor function in patients with Type 2 and Type 3 SMA

HFMSE=Hammersmith Functional Motor Scale Expanded; RHS=Revised Hammersmith Scale

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

Considerations in the Conduct and Design of TOPAZ Proof-of-Concept Study

- Main focus of TOPAZ was to assess the potential additive therapeutic benefit of apitegromab on top of background SMN upregulator therapy.*
  - While the protocol allowed the use of any approved SMN upregulator as background therapy, only nusinersen had widespread use during TOPAZ trial enrollment.
- Specifically designed with 3 distinct cohorts to assess apitegromab's potential across patient populations with varying disease severity and different background expectations for disease course.
- Clinical data for nusinersen and natural history data help inform our background expectations for disease course for the different populations evaluated in TOPAZ.
  - These insights further our understanding as we continue to investigate apitegromab in SMA.
- Cohort 3 evaluated two dose arms as we recognized that complete target saturation may not be necessary to achieve therapeutic effect.
  - Low dose of 2 mg/kg was selected to explore this question by aiming for a high level of target engagement but lower than that of the 20 mg/kg dose.

*An apitegromab monotherapy subgroup was included in Cohort 1.

Baseline Characteristics

	Ambulatory Patients			Non-Ambulatory Patients			
	Cohort 1			Cohort 2	Cohort 3		
	20 mg/kg monotherapy	20 mg/kg +nusinersen	Pooled	20 mg/kg +nusinersen	20 mg/kg +nusinersen	2 mg/kg +nusinersen	Pooled
N	11	12	23	15	10	10	20
Mean age (min, max)	12.1 (7, 19)	13.1 (7, 21)	12.6 (7, 21)	11.7 (8, 19)	3.8 (2, 6)	4.1 (2, 6)	4.0 (2, 6)
Female (%)	73%	58%	65%	53%	50%	30%	40%
SMN2 Gene Copy* (#, %)							
2	1 (9%)	0 (0%)	1 (4%)		1 (10%)	1 (10%)	2 (10%)
3	4 (36%)	9 (75%)	13 (57%)	11 (73%)	8 (80%)	8 (80%)	16 (80%)
4	4 (36%)	1 (8%)	5 (22%)	2 (13%)	0 (0%)	1 (10%)	1 (5%)
Mean # of nusinersen maintenance doses (min, max)	N/A	5.6 (2, 8)	N/A	5.1 (2, 9)	5.4 (3, 8)	5.5 (2, 9)	5.5 (2, 9)
Discontinuation(s)	0	1**	1**	0	0	0	0
Mean RHS score (min, max)	47.6 (26, 63)	51.3 (43, 62)	49.6 (26, 63)				
Mean HFMSE score (min, max)				22.7 (13, 39)	23.5 (14, 42)	26.1 (12, 44)	24.8 (12, 44)

*Data not available for all patients
**Patient who discontinued study for reasons unrelated to study drug
HFMSE=Hammersmith Functional Motor Scale Expanded; RHS=Revised Hammersmith Scale

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

Safety Results from TOPAZ 12-Month Top-Line Analysis Support Evaluation of Apitegromab in Phase 3 Trial

Treatment-emergent adverse events (TEAEs)	Apitegromab 2 mg/kg (n=10)	Apitegromab 20 mg/kg (n=48)	Total (n=58)
Any TEAE	9 (90.0%)	44 (91.7%)	53 (91.4%)
Any Serious TEAE	1 (10.0%)	4 (8.3%)	5 (8.6%)
Any TEAE leading to study drug discontinuation	0 (0.0%)	1 (2.1%)	1 (1.7%)
Any Grade 3 (severe) or higher TEAE	0 (0.0%)	3 (6.2%)	3 (5.2%)

- **Five most frequently reported TEAEs*:** Headache (24%), pyrexia (22%), upper respiratory tract infection (22%), cough (22%), and nasopharyngitis (21%).
- **Anti-drug antibodies (ADA)** were present at low titers following apitegromab treatment in 3 out of 58 enrolled patients. No apparent impact on drug exposure was observed and was not associated with any hypersensitivity reactions.
- No safety signals identified as of the TOPAZ 12-month top-line analysis

Incidence and severity of AEs were consistent with the underlying patient population and background therapy

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.
**TEAE rates are across all patients in TOPAZ trial*
Data on file. Scholar Rock, Inc. Cambridge, MA

Serious and Severe Treatment-Emergent Adverse Events (TEAEs)

Serious TEAEs; *All Assessed by Trial Investigators as Unrelated to Apitegromab*

2 mg/kg:

- **Cohort 3:** 1 patient hospitalized due to adenoidal and tonsillar hypertrophy and scheduled adenotonsillectomy (Grade 2). Resolved without sequelae.

20 mg/kg:

- **Cohort 1:** 2 patients with gait inability considered a significant disability (both Grade 3). Events remain ongoing.
- **Cohort 1:** 1 patient hospitalized with post lumbar puncture syndrome (Grade 2). Resolved without sequelae.
- **Cohort 1:** 1 patient hospitalized due to viral upper respiratory infection (Grade 2/prior history). Resolved without sequelae.

Other Severe TEAE; *Assessed by Trial Investigator as Unrelated to Apitegromab*

- **Cohort 1:** 1 patient presented with post lumbar puncture syndrome (non-serious Grade 3). Resolved without sequelae.

Study Discontinuation; *Assessed by Trial Investigator as Unrelated to Apitegromab*

- **Cohort 1:** 1 patient withdrew consent after ~2 months in the trial. Grade 2 leg muscle fatigue (developed prior to enrollment).

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

TOPAZ 12-Month Top-line Results Demonstrate Potential of Apitegromab for Patients with Type 2 and Type 3 SMA

	Ambulatory Patients (Revised Hammersmith Scale)			Non-Ambulatory Patients (Hammersmith Functional Motor Scale Expanded)			
	Cohort 1			Cohort 2*	Cohort 3*		
(Intent-to-Treat Population)	20 mg/kg monotherapy (n=11)	20 mg/kg +nusinersen (n=12)	Pooled (n=23)	20 mg/kg +nusinersen (n=14)	20 mg/kg +nusinersen (n=8)	2 mg/kg +nusinersen (n=9)	Pooled (n=17)
Mean change from baseline (95% CI)	-0.4 (-3.9, 3.1)	-0.3 (-2.0, 1.4)	-0.3 (-2.1, 1.4)	+0.6 (-1.4, 2.7)	+7.1 (1.8, 12.5)	+5.3 (-1.5, 12.2)	+6.2 (2.2, 10.1)
# (%) pts achieving ≥1-pt increase	4/11 (36%)	5/12 (42%)	9/23 (39%)	9/14 (64%)	7/8 (88%)	7/9 (78%)	14/17 (82%)
# (%) pts achieving ≥3-pt increase	3/11 (27%)	2/12 (17%)	5/23 (22%)	4/14 (29%)	5/8 (63%)	5/9 (56%)	10/17 (59%)

✓ **Cohort 1 data suggest potential clinical effect in certain patients in this patient population**
- Mean decline in RHS from baseline
- Majority (57%) maintained or improved in RHS (≥0-point change from baseline) and 22% achieved ≥3-point increase

✓ **Cohort 2 observed improvement of motor function from baseline**
- Mean improvement in HFMSE from baseline; potential durability of improvement apparent up to 12-months
- Majority (64%) achieved ≥1-point increase in HFMSE and sizeable subset (29%) achieved ≥3-point increase

✓ **Cohort 3 observed further improvements in motor function and continued dose response vs. 6-month interim analysis**
- Large mean improvement in HFMSE from baseline in both dose arms; high dose numerically outperformed low dose
- Majority (59%) achieved ≥5-point increase and sizeable subset (35%) achieved >10-point increase in HFMSE

**4 patients (1 in Cohort 2 and 3 in Cohort 3) each missed 3 doses of apitegromab due to COVID-19-related site access restrictions and were not included in the primary (intent-to-treat) analysis.*

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

Cohort 1

Background Insights into Ambulatory Type 3 SMA Patients

Coratti et, al. Natural History Study of Ambulatory Type 3 SMA

Baseline characteristics

- 130 patients with ambulatory Type 3 SMA (some patients were lost to follow-up over time)
- Mean age at baseline of 10.05
- Mean HFMSE score of 52.81

12-month assessments

- Mean change in HFMSE from baseline was -0.79 points
- 11 patients lost ambulation - mean age at ambulation loss was 10.21 years (SD±6.43)

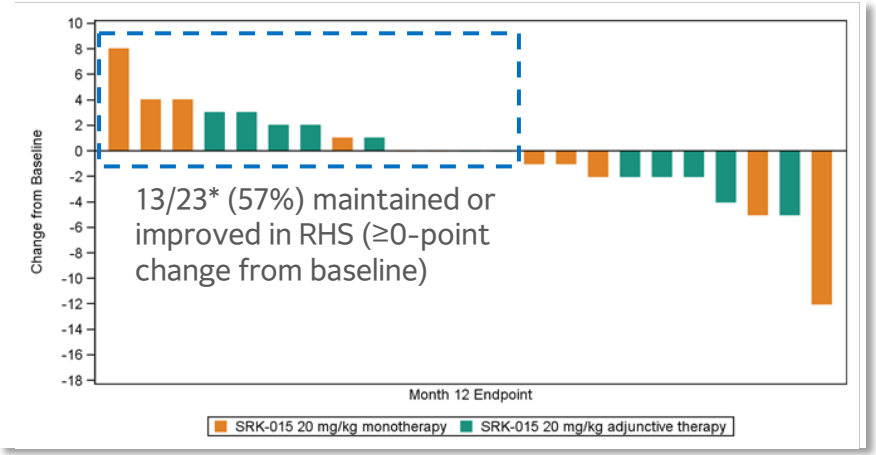
Motor function decline is common in ambulatory Type 3 SMA and can be severe in a subset of patients

Source: Coratti, et, al. Annals of Neurology (2020; 88:1109-1117) DOI: 10.1002/ana.25900
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.

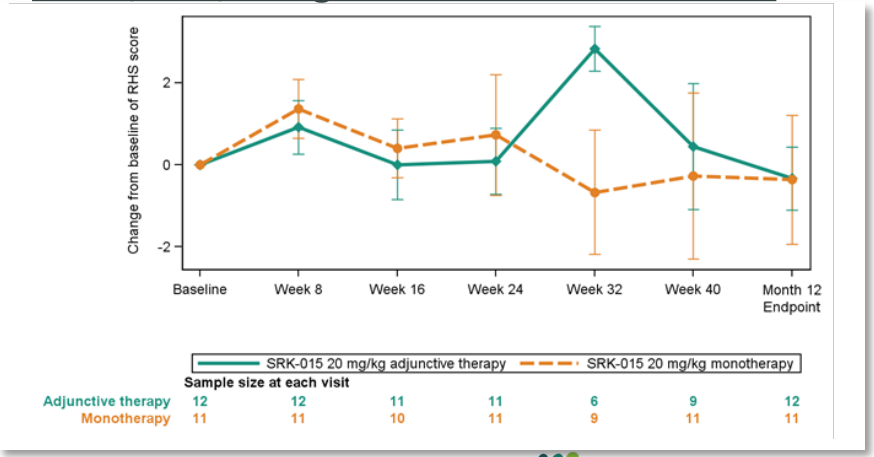
Cohort 1: Mean Decline in RHS at 12-Months but Majority of Patients Maintained or Improved in RHS Score from Baseline

Ambulatory Type 3 SMA (Intent-to-Treat Population)	Apitegromab (20 mg/kg) monotherapy (n=11)	Apitegromab (20 mg/kg) + nusinersen (n=12)	Pooled (n=23)
Mean change from baseline in RHS (95% CI)	-0.4 (-3.9, 3.1)	-0.3 (-2.0, 1.4)	-0.3 (-2.1, 1.4)
# (%) patients achieving ≥1-pt increase in RHS	4/11 (36%)	5/12 (42%)	9/23 (39%)
# (%) patients achieving ≥3-pt increase in RHS	3/11 (27%)	2/12 (17%)	5/23 (22%)
# (%) patients achieving ≥5-pt increase in RHS	1/11 (9%)	0/12 (0%)	1/23 (4%)

Individual RHS responses



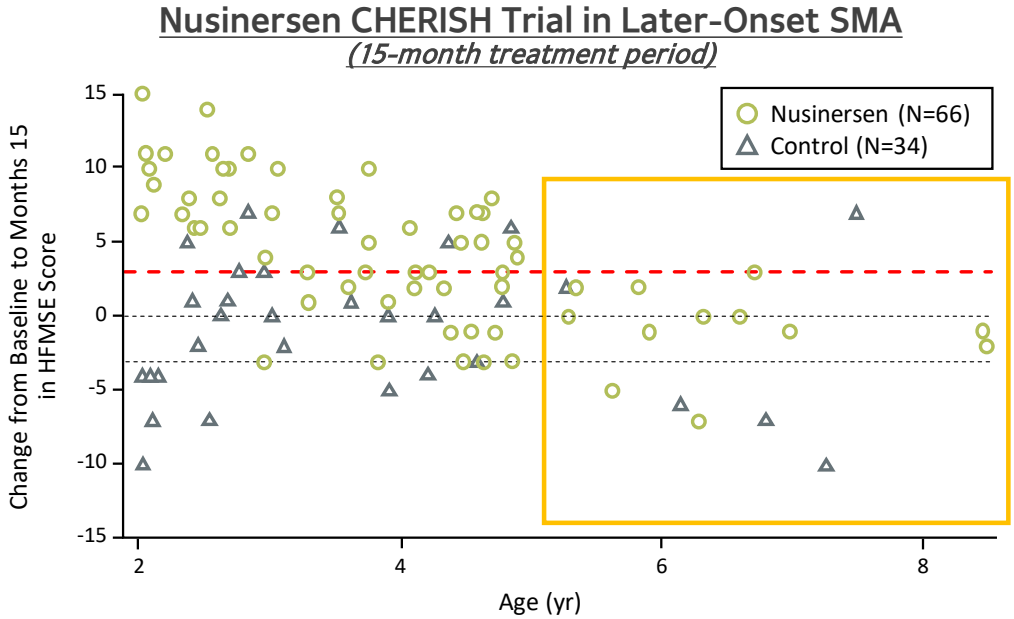
Mean (±SEM) change from baseline in RHS scores



*Includes 2 patients in monotherapy and 2 patients in apitegromab + nusinersen subgroup who maintained RHS score (0-point change from baseline)
Per protocol and sensitivity (all patients) analyses showed similar results to primary intent-to-treat analysis
apitegromab = SRK-015
Data on file. Scholar Rock, Inc. Cambridge, MA

Cohort 2

Background Insights Into Non-Ambulatory Later-Onset SMA ≥5 Years of Age



CHERISH clinical trial data*

After 15 months of treatment in patients who started on nusinersen at age ≥5....

- Mean HFMSE decline of >0.5-points
- <15% with ≥3-point increase

Natural history study**

After 12-month follow-up in patients age ≥5...

- Mean HFMSE decline
- <5% with ≥3-point increase

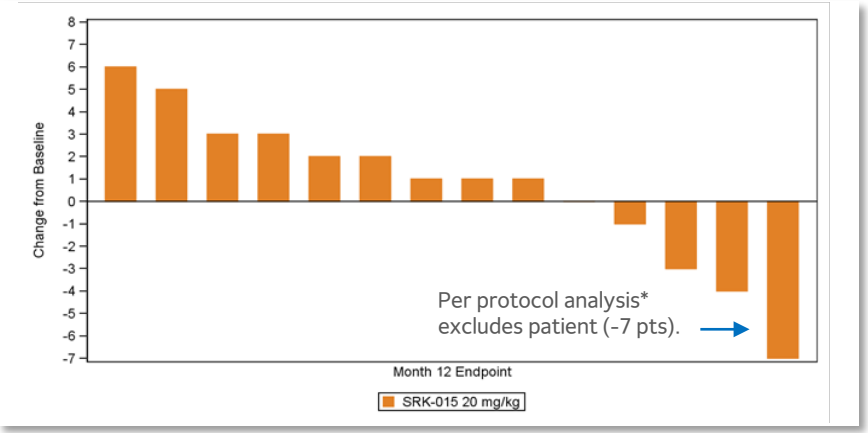
Majority of patients in this age range do not experience HFMSE improvements and rarely achieve a ≥3-point increase

*Mercuri E, et.al. Nusinersen versus sham control in later-onset spinal muscular atrophy. *N Engl J Med.* 2018;378:625-635.
**Mercuri E. et al. Patterns of disease progression in type 2 and 3 SMA: implications for clinical trials. https://doi.org/10.1016/j.nmd.2015.10.006
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.

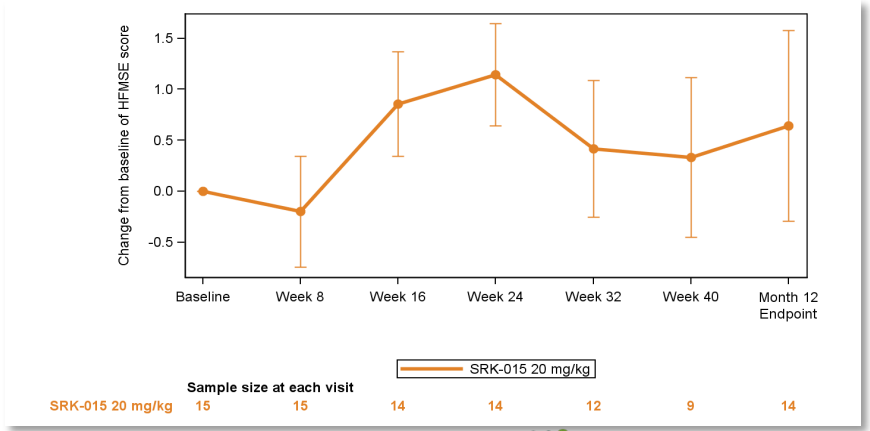
Cohort 2: Improvement in Mean HFMSE at 12-Months with Majority of Patients Achieving ≥1-point Increase

Type 2 and Non-Ambulatory Type 3 SMA	Apitegromab (20 mg/kg) + nusinersen	
	Intent-to-Treat Population (n=14)	Per Protocol Population* (n=13)
Mean change from baseline in HFMSE (95% CI)	+0.6 (-1.4, 2.7)	+1.2 (-0.5, 2.9)
# (%) patients achieving ≥1-pt increase in HFMSE	9/14 (64%)	9/13 (69%)
# (%) patients achieving ≥3-pt increase in HFMSE	4/14 (29%)	4/13 (31%)
# (%) patients achieving ≥5-pt increase in HFMSE	2/14 (14%)	2/13 (15%)

Individual HFMSE responses



Mean (±SEM) change from baseline in HFMSE scores



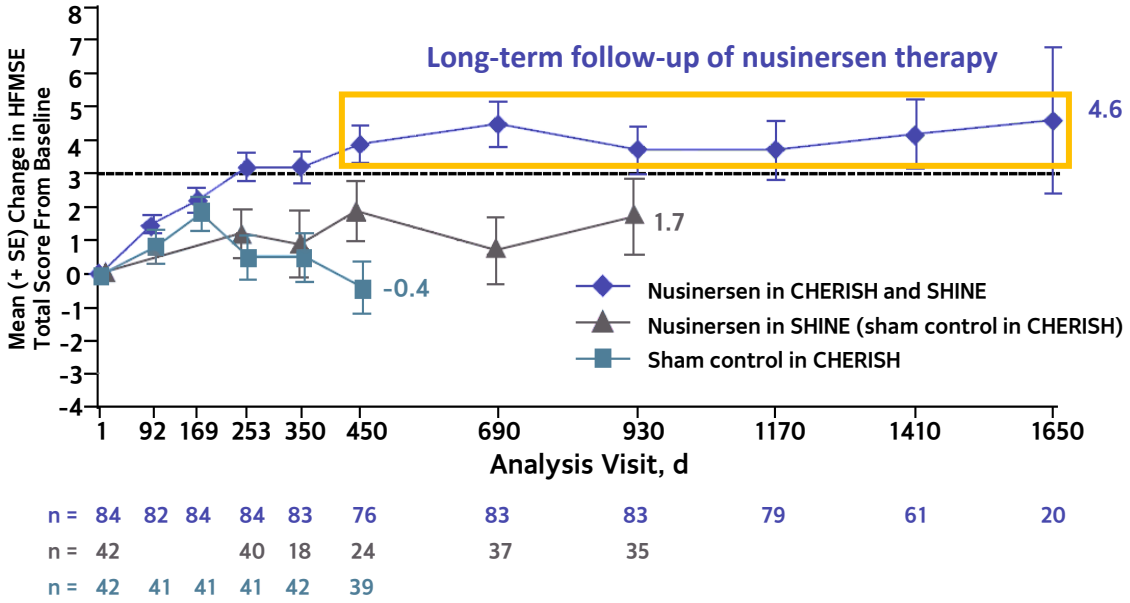
*Patient had concomitant exposure to an acetylcholinesterase inhibitor, which is not permitted per the TOPAZ trial protocol
Sensitivity analysis (all patients) showed similar results to primary intent-to-treat analysis
apitegromab = SRK-015.

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

Cohort 3

Background Insights Into Non-Ambulatory Later-Onset SMA with Early Initiation of Nusinersen Therapy

Nusinersen SHINE Trial in Later-Onset SMA*



*Most nusinersen-treated patients in CHERISH were under age 5 years at time of therapy initiation

Nusinersen SHINE Trial

SHINE data suggest nusinersen-treated patients primarily stabilize or experience modest and gradual improvement beyond the initial 15 months of therapy

TOPAZ Cohort 3

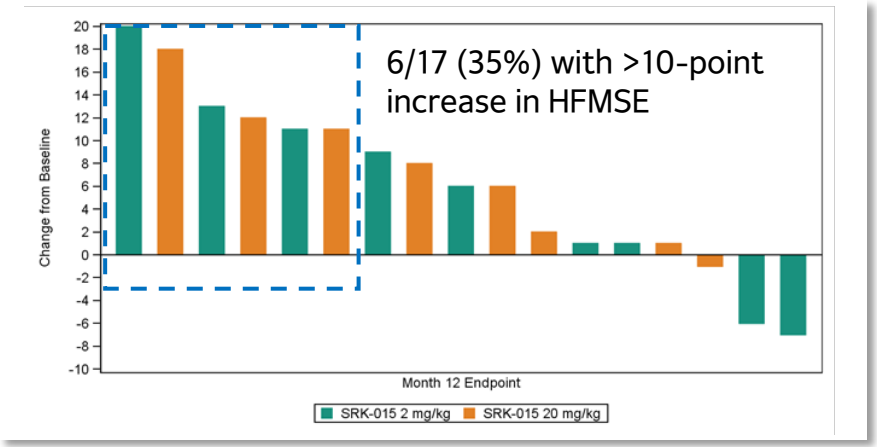
Patients on average had received ~2 years of treatment with nusinersen at baseline and ~3 years by the 12-month analysis timepoint.

Source: Darras, B., et.al. Nusinersen in later-onset spinal muscular atrophy. *Neurology.* May 2019; 92 (21) e2492-e2506.
"Longer-term treatment with nusinersen: results in later-onset spinal muscular atrophy from the SHINE study" P.257, World Muscle Society Congress 2020
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.

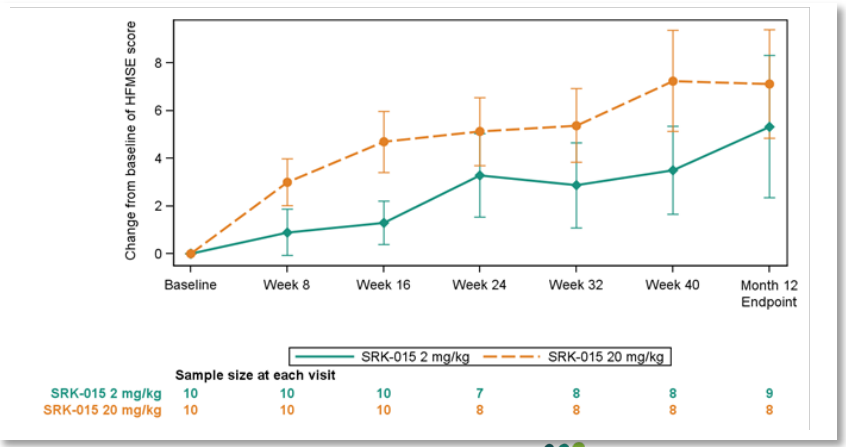
Cohort 3: Sizeable Continued Improvements in Mean HFMSE Observed Across 12 Months

Type 2 SMA (Intent-to-Treat Population)	Apitegromab 20 mg/kg + nusinersen (n=8)	Apitegromab 2 mg/kg + nusinersen (n=9)	Pooled (n=17)
Mean change from baseline in HFMSE (95% CI)	+7.1 (1.8, 12.5)	+5.3 (-1.5, 12.2)	+6.2 (2.2, 10.1)
# (%) patients achieving ≥1-pt increase in HFMSE	7/8 (88%)	7/9 (78%)	14/17 (82%)
# (%) patients achieving ≥3-pt increase in HFMSE	5/8 (63%)	5/9 (56%)	10/17 (59%)
# (%) patients achieving ≥5-pt increase in HFMSE	5/8 (63%)	5/9 (56%)	10/17 (59%)

Individual HFMSE responses



Mean (±SEM) change from baseline in HFMSE scores

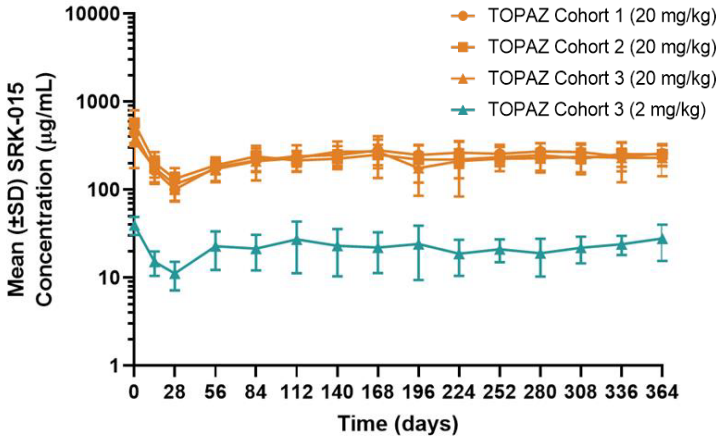


Per protocol and sensitivity (all patients) analyses showed similar results to primary intent-to-treat analysis
apitegromab = SRK-015

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

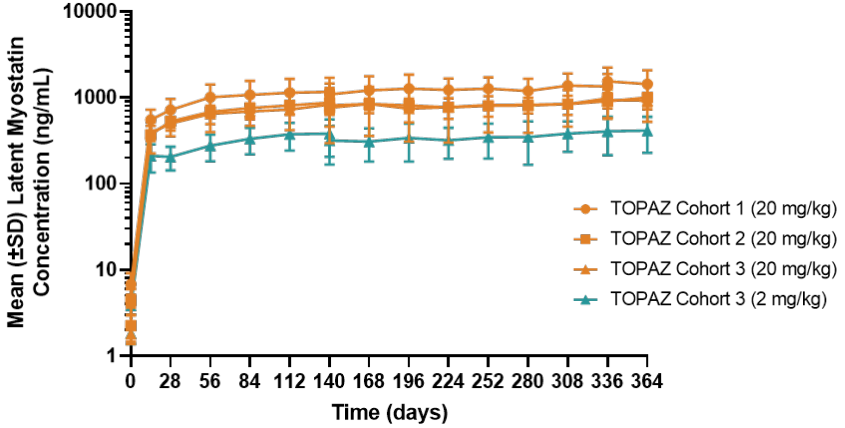
Pharmacokinetic and Pharmacodynamic Data are Supportive of Clinically Observed Effects

Pharmacokinetics* (PK)



- Dose-proportional and sustained drug exposure following chronic administration of apitegromab

Pharmacodynamics (PD)



- Both 2 mg/kg and 20 mg/kg doses yielded high levels of target engagement (>100-fold increase from baseline)
- 20 mg/kg dose offers relatively higher magnitude of target engagement than 2 mg/kg dose

High levels of target engagement achieved by both doses, with relatively higher absolute levels with high dose

*Starting at day 28, measures are pre-dose trough levels

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

12-Month Top-line Results Support the Therapeutic Potential of Apitegromab and Further Development

Cohort 1

- Mean RHS decline from baseline, but majority of patients maintained or improved (≥0-pt change in RHS)
- Potential subset of patients with more pronounced effect (22% with ≥3-pt increase)

Cohort 2

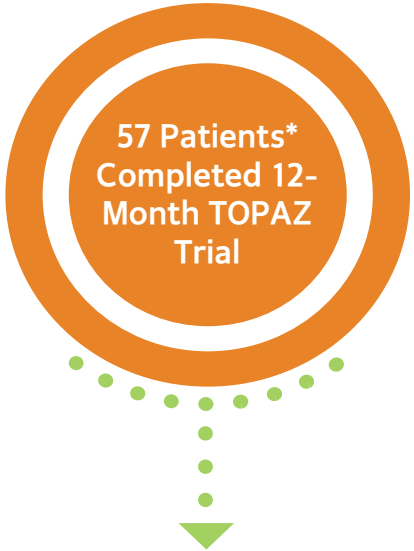
- Mean HFMSE improvement from baseline
- Majority (64%) of patients improved (≥1-pt increase in HFMSE) and sizeable subset (29%) attained ≥3-pt increase in HFMSE

Cohort 3

- Large HFMSE improvements from baseline, with dose response observed
- Majority (59%) of patients attained ≥5-pt increase and sizeable subset (35%) attained >10-pt increase in HFMSE
- PK/PD results support observed dose response

Safety

- No safety signals identified as of the 12-month top-line analysis
- Incidence and severity of AEs were consistent with the underlying patient population and background therapy



All Elected to Opt Into Extension Period

*Excludes one patient from Cohort 1 that discontinued from the trial

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

1. Plinth/chair sitting
2. Long sitting
3. One hand to head in sitting
4. Two hands to head in sitting
5. Supine to side-lying
6. Rolls prone to supine over R
7. Rolls prone to supine over L
8. Rolls supine to prone over R
9. Rolls supine to prone over L
10. Sitting to lying
11. Props on forearms
12. Lifts head from prone
13. Prop on extended arms
14. Lying to sitting
15. Four-point kneeling
16. Crawling
17. Lifts head from supine
18. Supporting standing
19. Stand unsupported
20. Stepping
21. Right hip flexion in supine
22. Left hip flexion in supine
23. High kneeling to right half kneel
24. High kneeling to left half kneel
25. High kneeling to standing leading with left leg (through right half kneel)
26. High kneeling to standing leading with right leg (through left half kneel)
27. Stand to sitting on the floor
28. Squat
29. Jump 12 inches forward
30. Ascends 4 stairs with railing
31. Descends 4 stairs with railing
32. Ascends 4 stairs without arm support
33. Descends 4 stairs without arm support

http://columbiasma.org/docs/HFMSE_2019_Manual.pdf

Hammersmith Functional Motor Scale Expanded for SMA (HFMSE)

Total achievable score of 66

- 33 distinct measures of an individual's ability to perform various activities
- Quality and execution of each movement is ranked on a scale of 0, 1, 2

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Next Steps for Apitegromab Program

Tony Kingsley - President & CEO

Ted Myles - CFO & Head of Business Ops

Additional TOPAZ Data and Analyses Will Further Our Understanding of Apitegromab's Potential in SMA

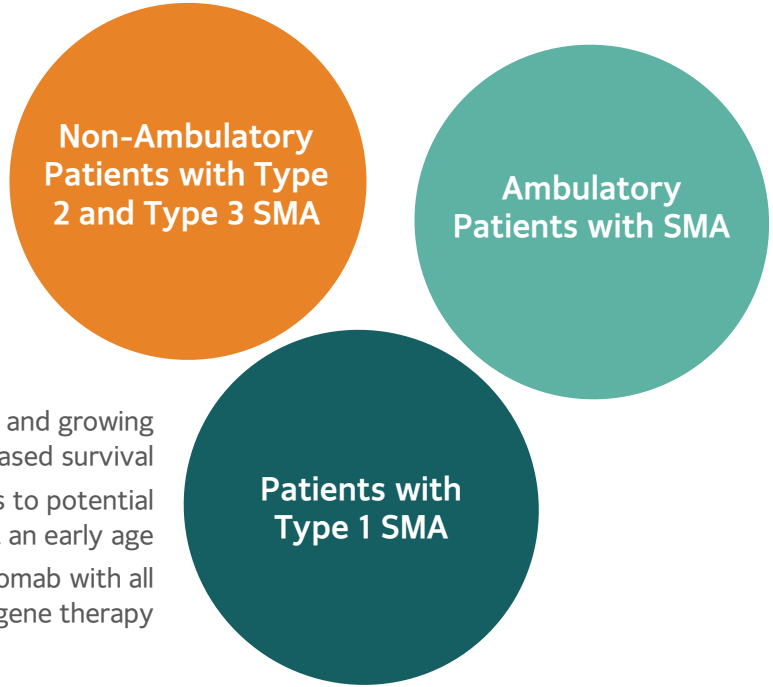
- Exploratory analyses, including patient-level data
- Additional outcome measures
- Additional safety data

Plan to present 12-month top-line data and additional analyses at upcoming medical congresses

Apitegromab Has Broad Potential in SMA…

Global Disease with Overall Prevalence of 30,000-35,000 in U.S. and Europe Alone

Subject to discussions with regulatory authorities; planned Phase 3 trial expected to initiate by year-end



- Most prevalent population
- TOPAZ has shown potential to improve motor function
- Many patients already treated with or eligible to be treated with SMN upregulators

- High unmet medical need; benefits of SMN upregulators not well established
- TOPAZ data suggest potential clinical benefit; possibly more pronounced in subset of patients
- Opportunity for additional exploration of apitegromab, both as monotherapy and in conjunction with SMN upregulators

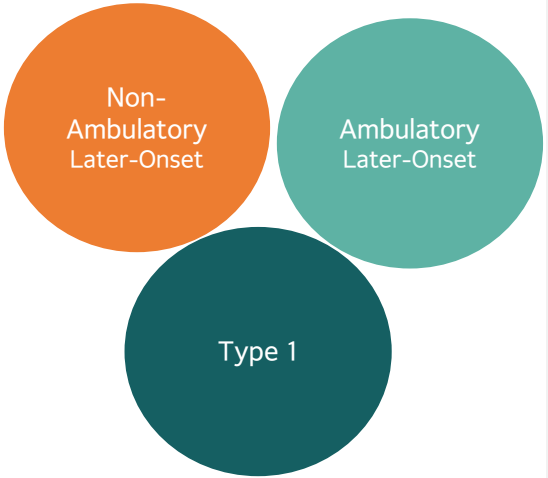
- Highest incidence population and growing prevalence due to increased survival
- TOPAZ Cohort 3 data points to potential benefit of treating at an early age
- Potential to evaluate apitegromab with all SMN upregulators, including gene therapy

http://www.smafoundation.org/wp-content/uploads/2012/03/SMA-Overview.pdf
https://www.curesma.org/wp-content/uploads/2018/01/SMA-VoP-for-publication-1-22-2018.pdf

…as well as Broad Potential Beyond SMA

Spinal Muscular Atrophy

Leverage TOPAZ findings to conduct **further explorations** in Type 1 and other subpopulations



Muscular Dystrophies

Becker Muscular Dystrophy*

- Prevalence of 15,000-25,000, substantially under-diagnosed in earliest stages
- Younger population with less severe dystrophin deficiency and slower progressing muscle damage

Duchenne Muscular Dystrophy*

- Prevalence of 30,000-40,000 with very severe symptoms and high unmet need
- Progress in the development of next-generation disease-stabilizing therapies may enable add-on muscle-directed approach

Other Dystrophies

- Potential for add-on muscle-directed therapy in other rare dystrophies with less severe phenotypes or upon availability of disease-stabilizing therapies

Additional Indications

Late-onset Pompe Disease**

- Large percentage of patients treated with enzyme replacement therapies (ERTs)
- Existing ERTs may address underlying pathology, but muscle strength remains ongoing challenge

Post-cancer muscle recovery in pediatrics***

- Some children may develop severe muscle wasting from chemotherapy

Glucocorticoid induced myopathy

- Potential benefit for subset of patients unable to discontinue steroid therapy

*"Muscular Dystrophy: Disease Landscape and Forecast." DRG Reports, June 19, 2020
**Enzyme replacement therapy in late-onset Pompe disease: a systematic literature review, Journal of Neuology. 2013
***A Systematic Review of Selected Musculoskeletal Late Effects in Survivors of Childhood Cancer, Current Pediatric Reviews. 2014

Potential for Apitegromab in Becker Muscular Dystrophy (BMD); Aim to Initiate Clinical Trial in 2022

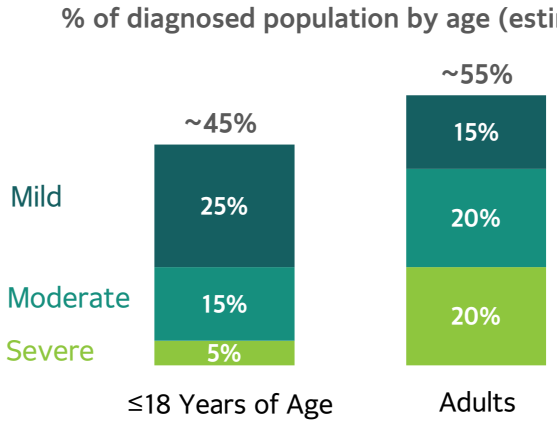
Strong fit for a selective inhibitor of latent myostatin…

Key Scientific Question	BMD Fit
Is patient population young?	Genetic disorder present at birth, with majority of patients identified at young age (before 18)
Are muscles structurally intact?	Less severe dystrophin deficiency and muscle disease than DMD with slower progression
Does disease impact fast-twitch fibers?	BMD causes a substantial deficit in fast-twitch muscle fibers
Is there an established endpoint that relies on fast-twitch fibers?	Several endpoints[1] (NSAA, TTSTAND) dependent on fast-twitch fibers have been used in past pivotal studies of muscular dystrophy therapies

(1)NSAA: North Star Ambulatory Assessment. TTSTAND: Time To Stand
Source: KOL Interviews; Dystrophin levels and clinical severity in Becker muscular dystrophy patients, J Neurol Neurosurg Psychiatry. 2014; Functional changes in Becker muscular dystrophy: implications for clinical trials in dystrophinopathies, Scientific Reports, 2016

… with a sizeable unmet need to be addressed

Estimated Prevalence of 15,000 -25,000



- Natural adjacencies to current program in SMA
- Positions apitegromab program for evaluation in a range of other muscular dystrophies (e.g. Duchenne Muscular Dystrophy)

Source: KOL Interviews; Practicing neurologist survey (N=21), 'Muscular Dystrophy: Disease Landscape & Forecast' published Jun 2020

Broad Patent Portfolio Protecting Apitegromab Into Late 2030s; Multiple Designations Granted by FDA/EMA

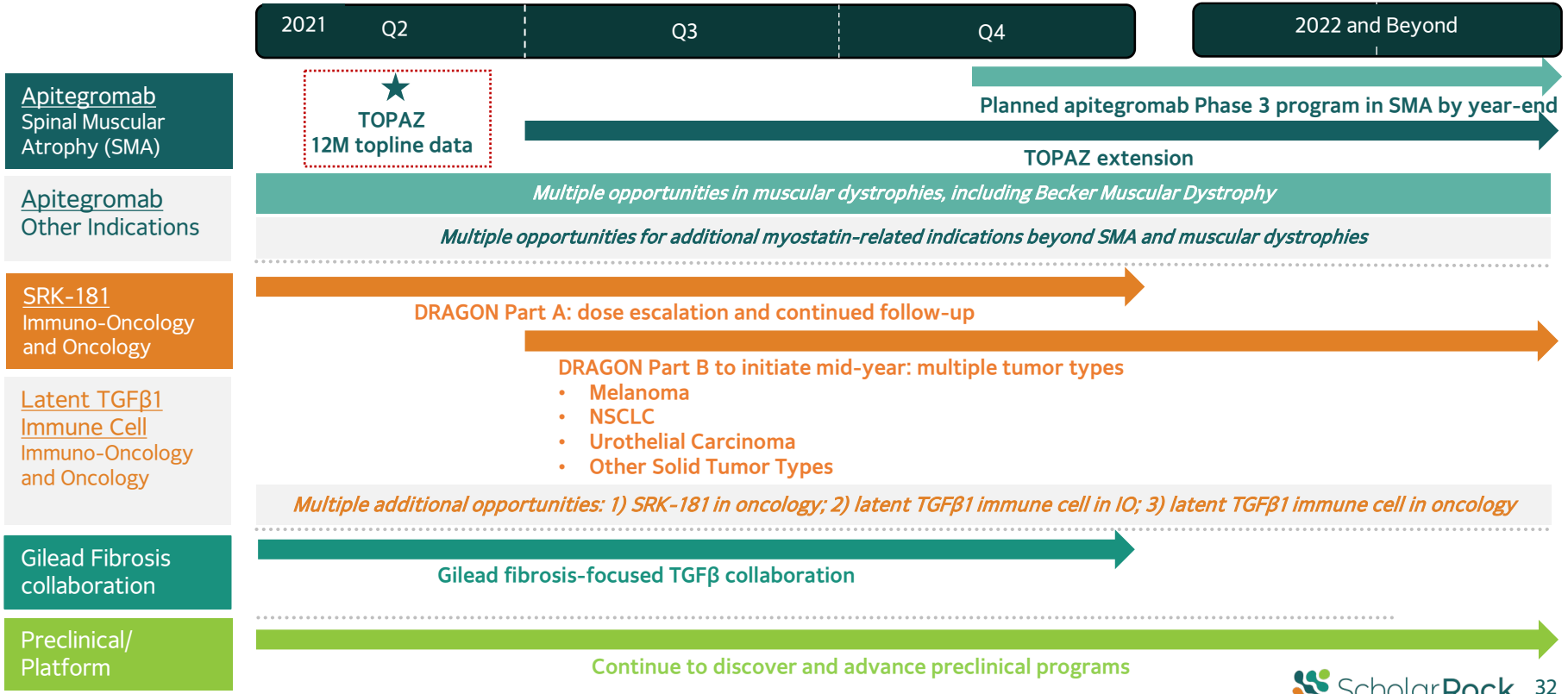
Highlights of apitegromab patent portfolio:

- **US 10,751,413 (2037**): Composition of matter and methods of use for apitegromab
- **US 9,758,576 (2034):** Composition of matter claims to mAbs that inhibit the activation of myostatin precursor
- **US 10,307,480 (2035):** Antibodies that selectively inhibit myostatin activation
- **US 10,287,345 (2037):** Treatment methods for various myostatin-related conditions
- **US 10,946,036 (2037): C**overs both add-on and combination therapy with a myostatin inhibitor and a neuronal corrector therapy
- **US 10,882,904 (2036):** Broadly directed to use of apitegromab to achieve certain therapeutic effects; without limiting to specific indications
- **US 9,399,676 (2034):** Methods of producing antibodies that bind pro/latent myostatin

Multiple designations granted by FDA/EMA recognizing the potential for apitegromab to address unmet medical needs in SMA

Rare Pediatric Disease for SMA granted by FDA
Orphan Drug Designation for SMA granted by FDA
Priority Medicines (PRIME) Designation for SMA granted by EMA
Orphan Medicinal Product Designation for SMA granted by EMA

2021: Potential for Another Transformative Year



TOPAZ

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