Apitegromab Positioned to be the Potential Next Transformative Therapy for Patients Suffering with SMA

KOL Event and Panel Discussion

June 15, 2021

ScholarRock™

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Agenda

Opening Remarks and Spinal Muscular Atrophy Treatment Landscape	Tony Kingsley President & CEO
Unmet Medical Needs of Individuals with SMA	Jill Jarecki, Ph.D. CSO of Cure SMA
Apitegromab TOPAZ Phase 2 Proof-of-Concept Trial Results Apitegromab Phase 3 Trial Design Considerations	Yung Chyung M.D. CMO
Panel Discussion with TOPAZ Trial Investigators: • Thomas Crawford, M.D. - Johns Hopkins Medicine • Basil Darras, M.D. – Boston Children's Hospital & Harvard Medical School	Moderated by Yung Chyung, M.D.

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

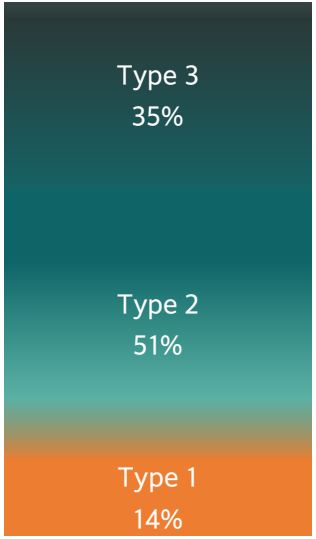
- Spinal Muscular Atrophy (SMA) remains a devastating and debilitating disease despite the utilization of SMN upregulators
- Multi-pronged approach may be needed in treating SMA; muscle-directed therapy may further improve motor function
- Apitegromab showed transformative potential in patients with Type 2/3 SMA thru the TOPAZ Phase 2 trial
- Exciting potential path forward for apitegromab in a rational, targeted, and efficient Phase 3 trial

Apitegromab is an investigational product candidate that is currently being evaluated in a clinical development program for the treatment of SMA. Apitegromab has not been approved by the U.S. Food and Drug Administration (FDA), the European Commission, or any other health or regulatory authority. The safety and effectiveness of this molecule have not been established.

Spinal Muscular Atrophy Overview

Global disease with 30,000-35,000 affected in U.S. and Europe alone

- 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 Type 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)*
Lallyet al, OrphanetJournal of Rare Diseases, 2017

Potential to Pioneer a New Treatment Era: Opportunity for Muscle-Directed Therapy to Complement SMN Upregulators

	SPINRAZA® (nusinersen) injection 12 mg/5 mL	Evrysdi™ risdiplam 60 mg powder for oral solution	zolgensma® (onasemnogene abeparvovec-xioi) suspension for intravenous infusion
Phase 3 Trial Design	• Non-ambulatory Type 2/3 • 2-12 years of age • **Primary endpoint:** Mean change from baseline in HFMSE at 15 months	• Non-ambulatory Type 2/3 • 2-25 years of age • **Primary endpoint:** Mean change from baseline in MFM-32 at 12 months	• Infantile-onset Type 1 • <6 months of age • **Primary endpoints:** Ability to sit independently and event-free survival
Indication	• Type 1, 2, and 3 SMA in pediatric and adult patients	• Type 1, 2, 3 SMA in patients 2 months of age and older	• SMA in patients less than 2 years of age
Market Penetration	• >11,000* patients treated WW • $2+ billion in revenues (LTM)	• ~3,000** patients treated WW • ~CHF135 million in revenues (LTM)	• ~1,200*** patients treated WW • ~$1.1 billion in revenues (LTM)

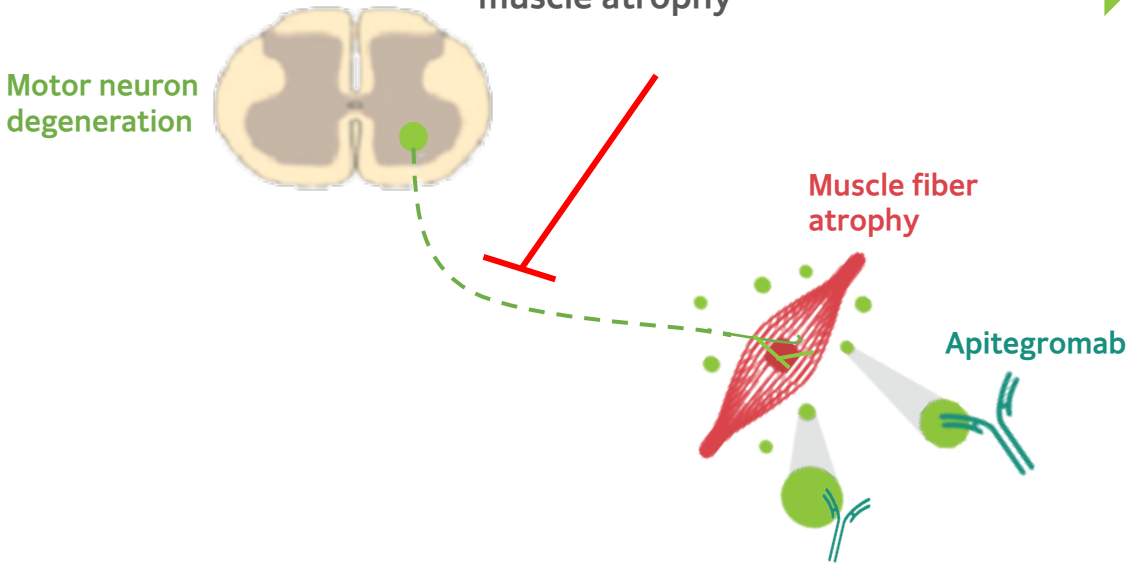
Patients continue to experience major functional impairments despite utilization of SMN upregulators

*As of 1Q21 financial update on 4/22/21; includes patients treated worldwide in post-marketing setting, expanded access program, and clinical trials.
**As of 1Q21 financial update on 4/21/21; includes patients treated worldwide between clinical trials, commercial, and compassionate use program.
***As of 1Q21 financial update on 4/27/21; commercially, via managed access programs and in clinical trials
HFMSE = Hammersmith Functional Motor Scale Expanded; MFM-32 = Motor Function Measure – 32 items

Apitegromab: Muscle-Directed Therapy Aimed at Complementing SMN Upregulators

SMN upregulators prevent further degeneration of motor neurons

...but do not directly address muscle atrophy



Apitegromab is a muscle-directed approach aimed at improving motor function

- Myostatin is a negative regulator of skeletal muscle growth
- Apitegromab is a fully human, mAb that specifically binds to proforms of myostatin and inhibits myostatin activation

Adapted from images courtesy of the SMA Foundation

Cure SMA

Jill Jarecki, PhD
CSO

June 15, 2021

Make today a breakthrough.

Cure SMA

We fund groundbreaking research and provide families the support they need for today.

Annual budget of $15M

$85 Million in research funding

36 Chapters in the US

9,000 affected individuals in membership database

300 newly diagnosed contacts annually

- Newly diagnosed care and info packets
- Info on clinical trial recruitment

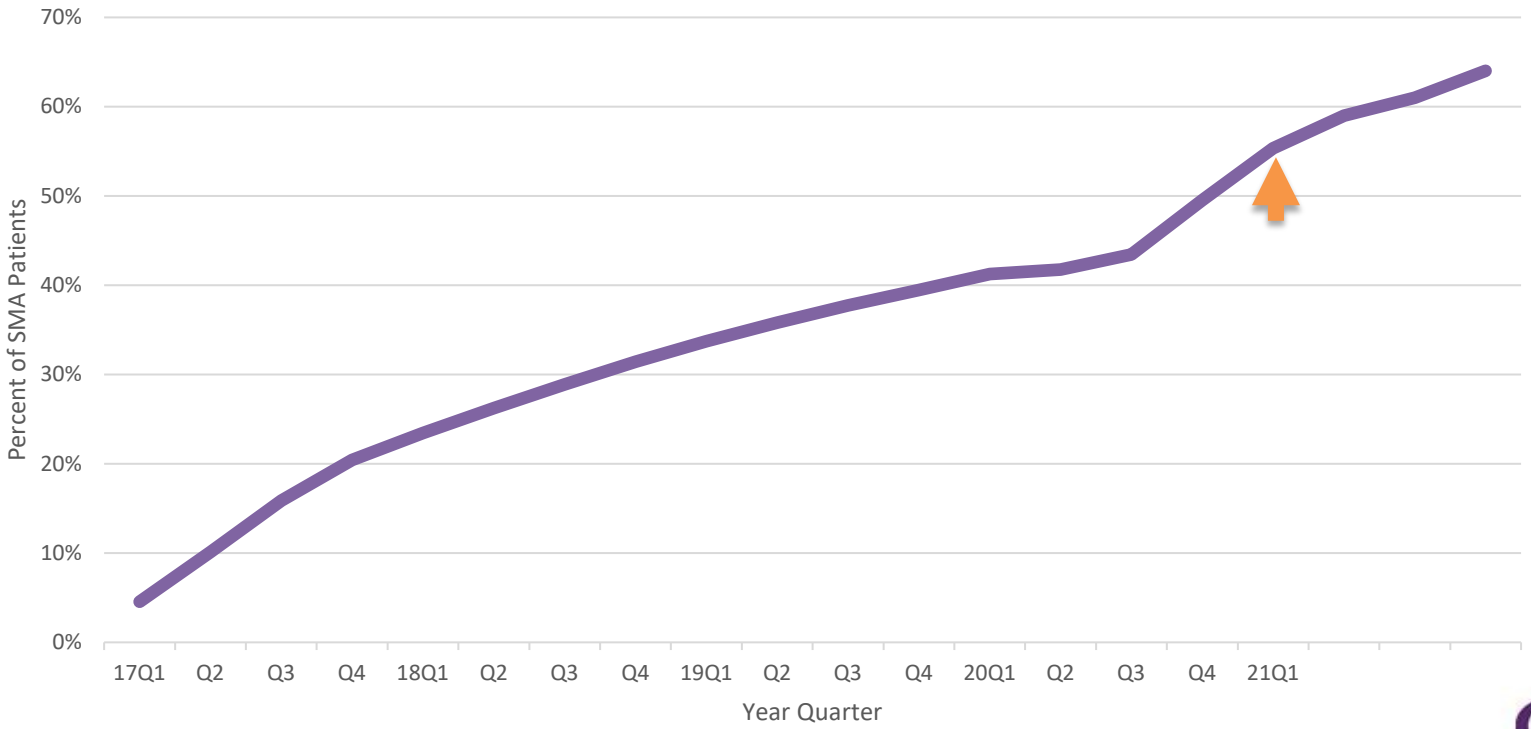
4,000 families obtain services annually

Annual conference, with 2500 attendees

The Evolving Landscape in SMA

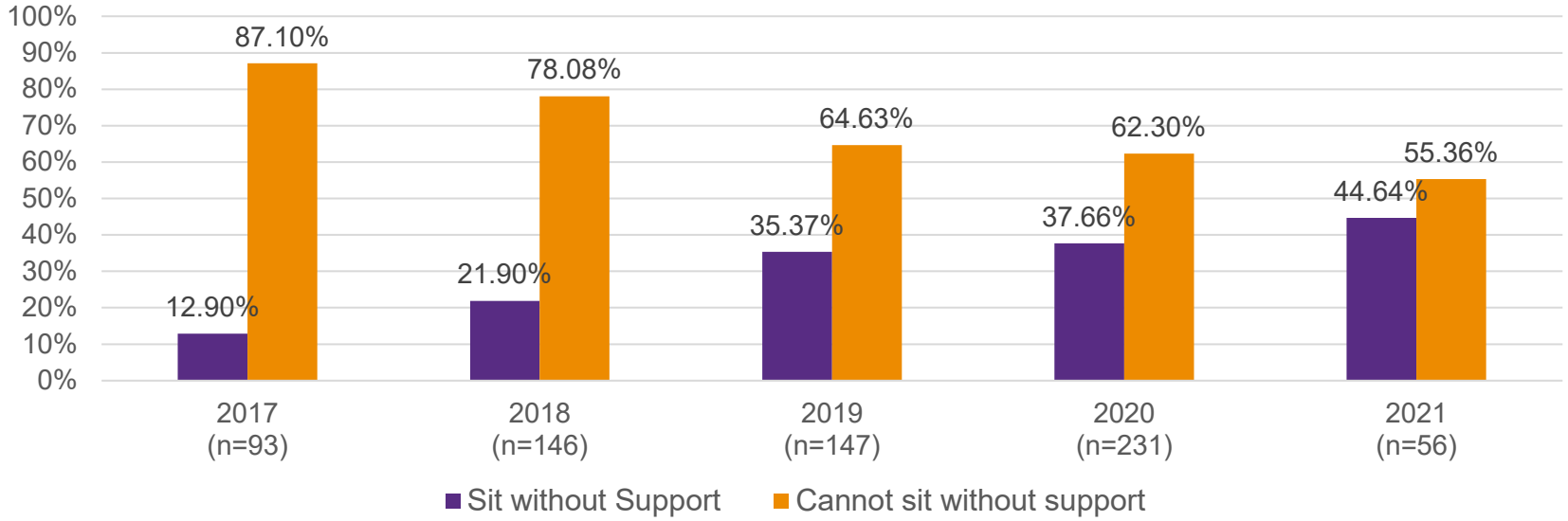
- **The FDA-approval of 3 new therapies** has revolutionized and dramatically changed the natural history of SMA
- Early identification and treatment dramatically alter long-term outcomes, **most strikingly presymptomatically**
- Symptomatic treatment providing improvements through **increases, stabilization, and slowing**
- **Many unmet needs remain** when addressing the complexities of SMA
- **Unmet need is higher in older patients** compared to younger patients.

45% of SMA Patients Remain Untreated at End of Q1 2021

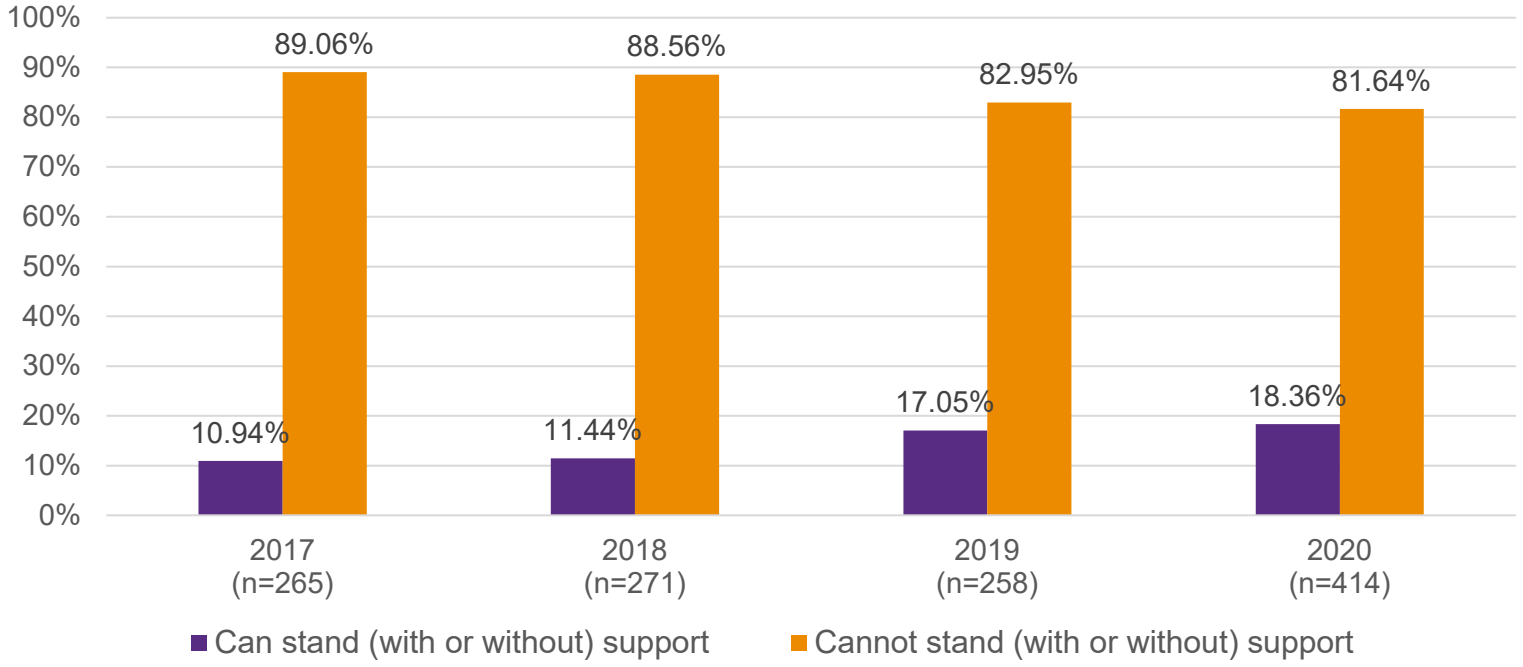


Actuals through the end of Q1 2021, with projections for the three quarters after.

Almost Half of Those with SMA Type I Currently Sit without Support



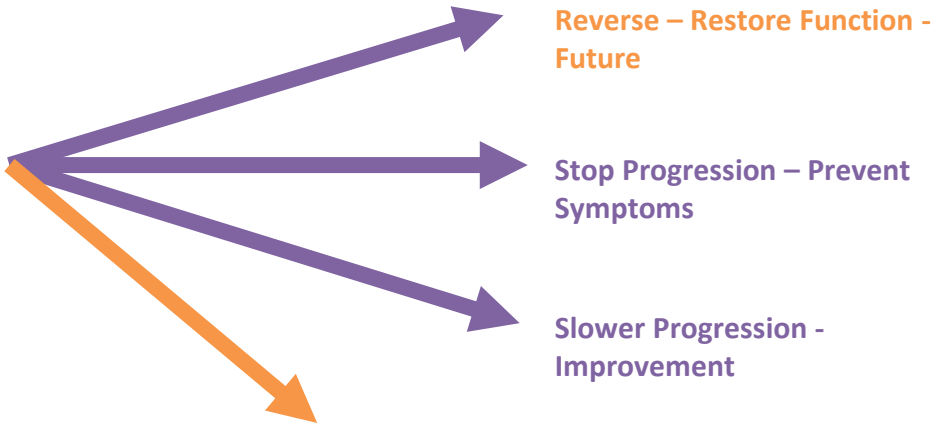
Can Stand With or Without Support Currently among SMA Type II



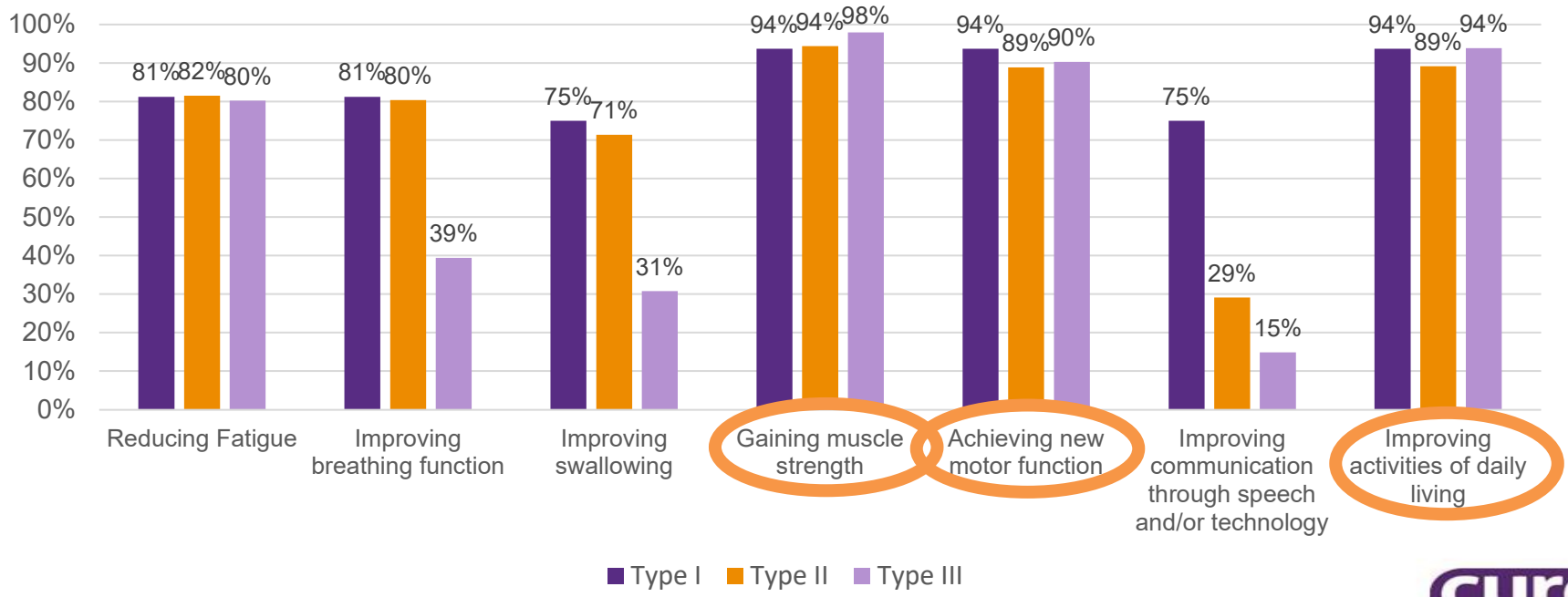
2017 & 2018: Question was what is your maximum current motor function. Assumed able to stand with or without support if any of the following choices were picked: *walk alone, walk with assist, cruise along furniture, stand without support, or stand with support;*
2019 & 2020: Respondents were asked to answer yes or no if affected child could currently can stand with assistance or stand alone.

What Comes Next - SMA is Not Cured Yet

- Need More Impact for older ages and stage of SMA
- Different for symptomatic vs NBS patients
- Slow to Stop to Reverse

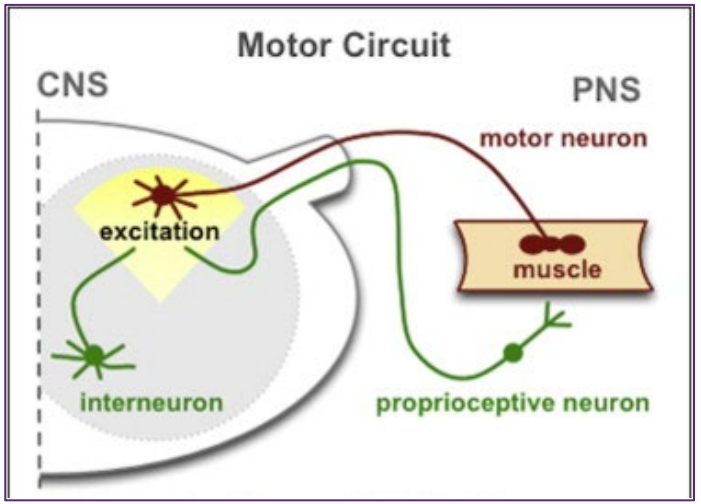


Unmet Needs that Future Therapies will Address – From Adults in the 2020 Community Update Survey



Therapeutic Strategies – SMN Independent

- Neuroprotection
- Neural transmission
- Regenerative targets
- Muscle enhancement
  - Apitegromab - Scholar Rock
- Genetic modifiers
- Combinations of above with SMN enhancer



Thank You!

Cure SMA

Jill Jarecki, PhD
CSO

June 15, 2021

Make today a breakthrough.

Apitegromab Showed Transformative Potential in SMA Phase 2 Trial

Yung Chyung, M.D.
Chief Medical Officer

TOPAZ

ScholarRock

TOPAZ: Evolving Understanding of Apitegromab's Transformative Potential in Type 2 and Type 3 SMA

October 2020
6-month interim ✓

Showed Initial Proof-of-Concept

- Interim data showed the therapeutic potential of apitegromab in Type 2 and 3 SMA as add-on therapy to background nusinersen
- HFMSE increases in patients who had already received on average ~2 years of background nusinersen therapy
- Meaningful HFMSE gains over 6-month time frame

April 2021
12-month top-line ✓

Further Support for Efficacy Signal

- Largest HFMSE increases in non-ambulatory Type 2 and 3 patients; represents ~2/3 of SMA prevalence
- Durability of effect with continued improvement in a subset of patients
- Supports advancing to a Phase 3 trial
  - Anticipated focus on patients with non-ambulatory Type 2 and 3

June 2021
Additional Analyses ✓

Further Support Therapeutic Potential and Phase 3 Plans

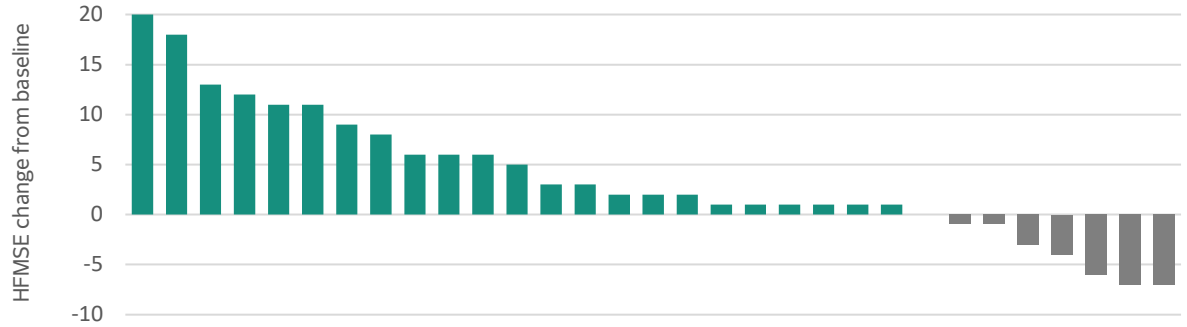
- Additional insights from exploratory analyses offer deeper understanding of apitegromab's potential profile:
  - Age of patients
  - Duration of prior background therapy
  - Secondary efficacy endpoint (WHO Motor Milestones)

2021

Additional exploratory analyses to be presented at future medical congresses…

TOPAZ Top-Line Data Showed Apitegromab's Transformative Potential in Patients with Type 2/3 SMA

✓ **Majority of non-ambulatory patients observed a clinical improvement in HFMSE***



✓ **Apitegromab treatment (as add-on to background nusinersen) led to improvements in HFMSE in both non-ambulatory cohorts**

At 12 months	Mean HFMSE increase	≥1-point increase	≥3-point increase
Initiated background nusinersen age <5**	+7.1 points	88% (7/8) of patients	63% (5/8) of patients
Initiated background nusinersen age ≥5	+0.6 points	64% (9/14) of patients	29% (4/14) of patients

** Pooled cohorts of non-ambulatory patients treated with apitegromab 20 mg/kg and 2 mg/kg*
***Non-ambulatory patients who initiated background nusinersen at a young age of <5 years and treated with apitegromab 20 mg/kg dose*

Additional TOPAZ Analyses Further Support Apitegromab's Potential to Improve Motor Function

Evaluating range of exploratory analyses to better understand the therapeutic response

Initial findings from non-ambulatory cohorts:

1. **HFMSE improvements observed across age range with relatively larger gains from earlier treatment**
2. **Duration of prior nusinersen treatment not correlated with HFMSE increase**
   - Provides further support that improvements may be attributable to apitegromab
   - Patients were already in chronic maintenance phase of nusinersen at enrollment
3. **Achievement by some patients of WHO motor milestones (additional high bar efficacy endpoint) further shows apitegromab's potential**
   - Total of 7 patients gained new WHO motor milestones across both non-ambulatory cohorts
   - 1 patient* gained 3 milestones (hands/knees crawling, standing with assistance, walking with assistance)
   - 1 patient** gained 2 new milestones (hands & knees crawling, standing with assistance)

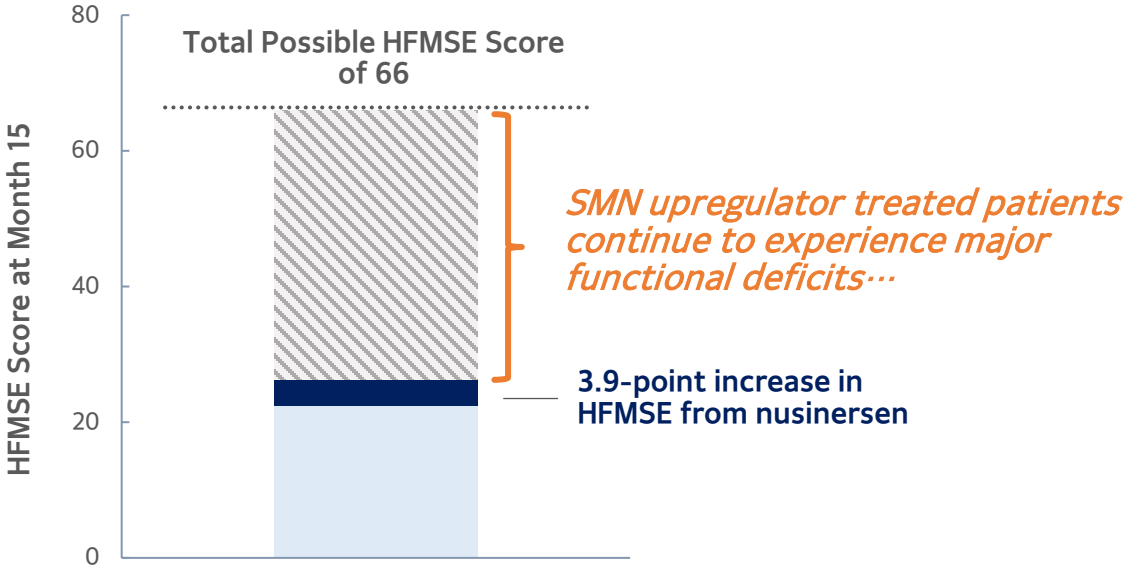
Phase 3 trial in patients with non-ambulatory Type 2 and 3 anticipated to initiate by end of 2021

**initiated nusinersen age <5, apitegromab 20 mg/kg*
***initiated nusinersen age ≥5*

Expectations for Patients on Background SMN Upregulator Therapy

Yung Chyung, M.D.
Chief Medical Officer

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

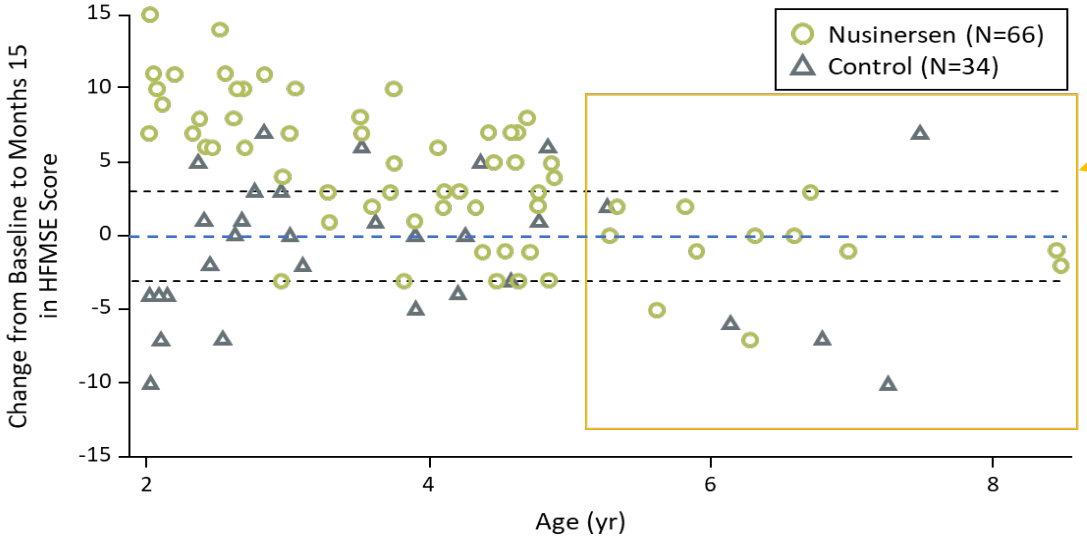


Mean improvement in HFMSE experienced by patients with non-ambulatory Type 2/3 SMA in nusinersen Phase 3 CHERISH trial

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.

Nusinersen Does Not Increase HFMSE on Average in Children Who Initiate Treatment After the Age of 5 Years

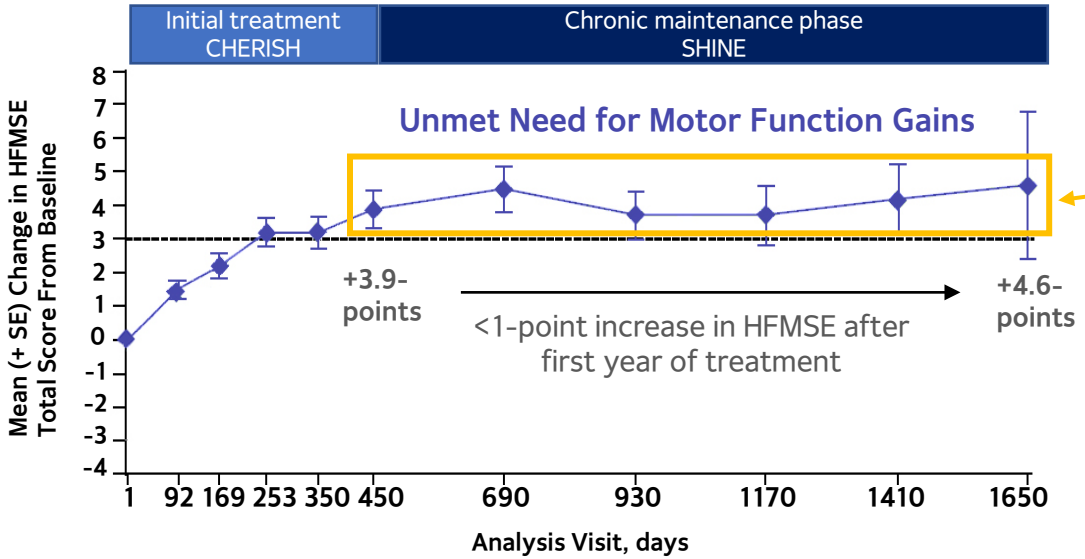
CHERISH Trial in Non-Ambulatory Type 2/3 SMA†



Majority of patients treated with nusinersen after the age of 5 did not observe an improvement

†Mercuri E, et.al. Nusinersen versus sham control in later-onset spinal muscular atrophy. *N Engl J Med*. 2018;378:625-635.
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.

Plateauing of HFMSE Increases Observed After First 15 Months of Nusinersen Treatment in Type 2 and 3 SMA



Nusinersen observed plateauing of improvement during chronic maintenance phase…

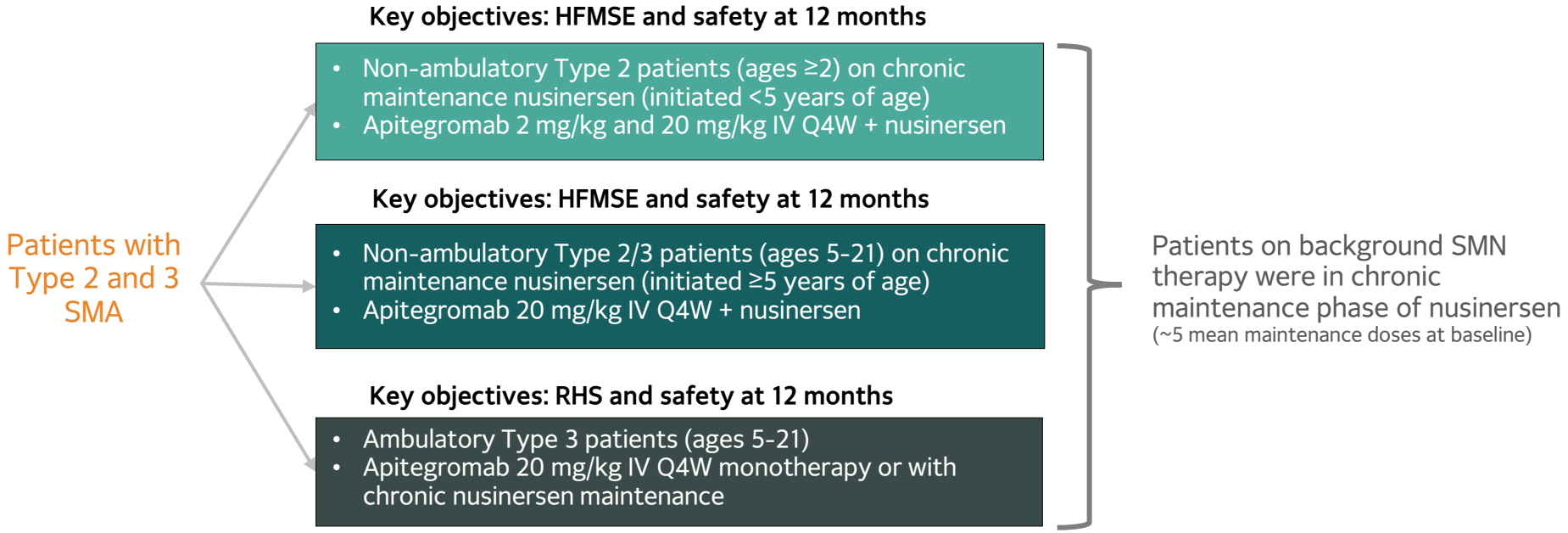
Most nusinersen-treated patients in CHERISH were <5 years of age at therapy initiation

"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.

Review of Positive TOPAZ Top-Line Results

Yung Chyung, M.D.
Chief Medical Officer

Apitegromab Phase 2 Trial Design



All 57* patients who completed the 12-month trial elected to opt into the extension period

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

Baseline Characteristics

Nusinersen-treated patients well into chronic maintenance phase

TOPAZ

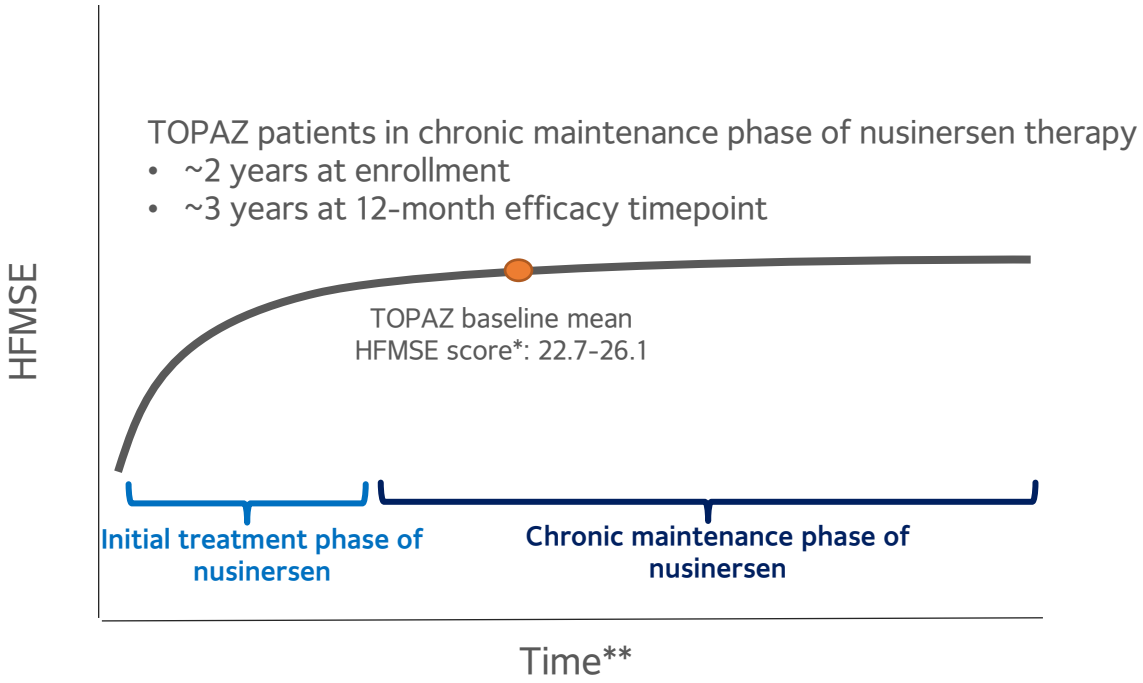
	Non-Ambulatory, Ages ≥2 and initiated nusinersen <5 years			Non-Ambulatory, Ages 5-21	Ambulatory, Ages 5-21		
	20 mg/kg +nusinersen	2 mg/kg +nusinersen	Pooled	20 mg/kg +nusinersen	20 mg/kg monotherapy	20 mg/kg +nusinersen	Pooled
N	10	10	20	15	11	12	23
Mean age at baseline (min, max)	3.8 (2, 6)	4.1 (2,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

*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

Patients Enrolled in TOPAZ Already in Chronic Maintenance Phase of Nusinersen Treatment

CONCEPTUAL



*Reflects non-ambulatory cohorts in TOPAZ

**The HFMSE time course plot for background nusinersen effect is hypothetical and intended for illustrative purposes only. The data presented here do not reflect any cross-trial comparisons. TOPAZ was not a placebo-controlled trial, it is not possible to draw direct conclusions in relation to background nusinersen effects alone.

Non-Ambulatory Type 2 Cohort:
Initiated nusinersen age <5

Apitegromab (20 mg/kg) + nusinersen	n=8
Mean change from baseline in HFMSE (95% CI)	+7.1 (1.8, 12.5)
# (%) patients achieving:	
≥1-pt increase in HFMSE	7/8 (88%)
≥3-pt increase in HFMSE	5/8 (63%)
≥5-pt increase in HFMSE	5/8 (63%)
Baseline characteristics: mean (min, max)	n=10
Age	3.8 (2, 6)
HFMSE score	23.5 (14, 42)
# of nusinersen maintenance doses	5.4 (3, 8)

Sizable increases in HFMSE observed in patients already treated with chronic maintenance nusinersen

- 88% (7/8) improved
- 63% (5/8) with ≥5-point increase
- 38% (3/8) with >10-point increase
- Continuous and durable improvements observed through 12-months of treatment

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

Non-Ambulatory Type 2/3 Cohort:
Initiated nusinersen age ≥5

Apitegromab (20 mg/kg) + nusinersen	Per Protocol Population* (n=13)	Intent-to-Treat Population (n=14)
Mean change from baseline in HFMSE (95% CI)	+1.2 (-0.5, 2.9)	+0.6 (-1.4, 2.7)
# (%) patients achieving:		
≥1-pt increase in HFMSE	9/13 (69%)	9/14 (64%)
≥3-pt increase in HFMSE	4/13 (31%)	4/14 (29%)
≥5-pt increase in HFMSE	2/13 (15%)	2/14 (14%)
Baseline characteristics: mean (min, max)	n=15	
Age	11.7 (8, 19)	
HFMSE score	22.7 (13, 39)	
# of nusinersen maintenance doses	5.1 (2, 9)	

Majority of patients improved in HFMSE (despite initiating background nusinersen age ≥5)

- ~2/3 with ≥1-point increase
- ~30% with ≥3-point increase
- Durability of effect observed through 12-months of treatment

**Patient had concomitant exposure to an acetylcholinesterase inhibitor, which was not permitted per the TOPAZ trial protocol*

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

TOPAZ Results 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%).
- SAEs, Grade 3 AEs and AE leading to early study discontinuation were all assessed by investigators as unrelated to study drug
- **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.

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*

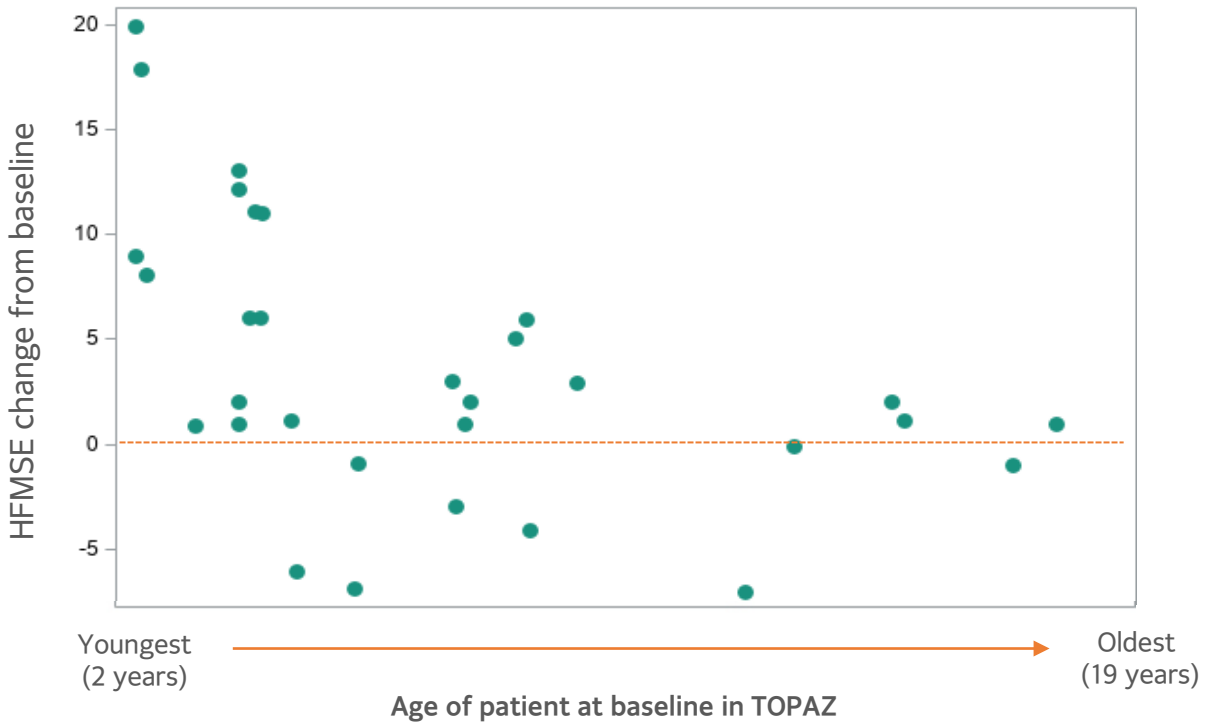
- **Cohort 1:** 1 patient withdrew consent after ~2 months in the trial. Grade 2 leg muscle fatigue (developed prior to enrollment).

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

Additional Exploratory Analyses Further Support Apitegromab's Potential Additive Benefit on Top of Nusinersen

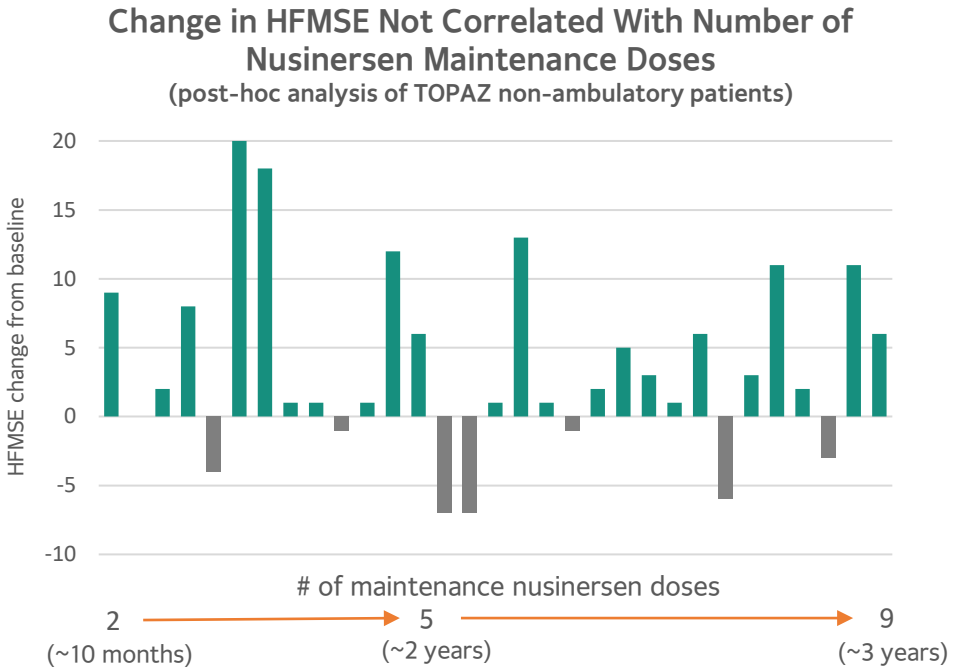
Yung Chyung, M.D.
Chief Medical Officer

HFMSE Improvements Observed Across Age Range of Non-Ambulatory Patients with Relatively Larger Gains from Earlier Treatment



**Pooled cohorts of non-ambulatory patients treated with apitegromab 20 mg/kg and 2 mg/kg; excludes 4 patients who 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

Increases in HFMSE Not Correlated with Duration of Prior Nusinersen Treatment



Further data suggesting increases in HFMSE may be attributable to apitegromab

- No correlation between duration of prior nusinersen treatment and change in HFMSE
- Patients in TOPAZ were already in chronic maintenance phase of nusinersen (mean of ~2 years at enrollment)

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

Clinical Outcome Measures: Progressive Levels of Difficulty in Measuring Gross Motor Functions

HFMSE - Validated Regulatory Endpoint Used in SMA Trials

33 distinct measures of an individual's ability to perform various activities

- Total achievable score of 66
- Quality and execution of each movement is ranked on a scale of 0, 1, 2
  - 0 - cannot perform the task
  - 1 – can perform task but with adaptation
  - 2 – can perform task
- Examples of HFMSE tasks:
  - Raising hand(s) to head in sitting
  - Lifting head from prone
  - Rolls prone to supine
  - High kneeling to half kneel
  - Ascending/descending 4 stairs

WHO Motor Milestones – Different and More Challenging Tests of Self-Sufficient Locomotion

6 gross motor skills considered to be universal*

- Sitting without support – head erect for at least 10 seconds; no use of arms or hands to balance
- Hands and knees crawling – at least 3 movements in a row and stomach does not touch supporting surface
- Standing with assistance – upright on both feet for at least 10 seconds without leaning on any object
- Walking with assistance – takes at least 5 steps holding a stable object
- Standing alone – at least 10 seconds with no contact with person or object
- Walking alone – takes at least 5 steps independently

For complete descriptions of the 6 WHO motor development milestones, please refer to https://cdn.who.int/media/docs/default-source/child-growth/child-growth-standards/indicators/motor-development-milestones/assessment-of-gross-motor-development-in-the-who-multicentre-growth-reference-study.pdf?sfvrsn=81277ea7_0

WHO Motor Development Milestone Achievements Further Support Apitegromab's Potential to Improve Motor Function

Non-ambulatory Type 2/3 Patients	Pooled, 20 mg/kg	Initiated nusinersen age <5	Initiated nusinersen age ≥5
# of patients gaining ≥1 WHO motor milestone(s)	7/35	4/10	3/15

Following 12 months of apitegromab treatment...

Sitting without support

Hands & knees crawling

Standing with assistance

Walking with assistance

Standing alone

Walking alone

WHO motor milestone analysis included all patients who completed the 12-month treatment period, including 4 patients who missed 3 doses of apitegromab due to COVID-19-related site access restrictions. Median baseline score for both non-ambulatory cohorts was 1.0.

1 patient (initiated nusinersen age ≥5) gained 2 new motor milestones and 1 patient (initiated nusinersen age <5, 20 mg/kg) gained 3 new motor milestones

Pictures are not of patients with SMA and are not meant to be representative of patients with SMA. Data on file. Scholar Rock, Inc. Cambridge, MA.

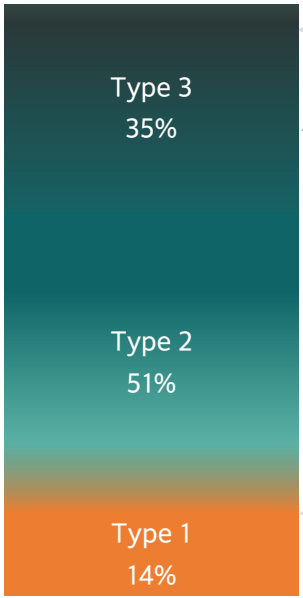
Apitegromab to Advance to Phase 3 Trial in Patients with Non-Ambulatory Type 2/3 SMA

Yung Chyung, M.D.
Chief Medical Officer

ScholarRock

Initial Development Strategy Focuses on Non-Ambulatory Patients on Background SMN Upregulators

Global disease with 30,000-35,000 affected in U.S. and Europe alone



C

Ambulatory patients

- Smaller population but high unmet need as benefits of SMN regulators not well-established
- TOPAZ suggests potential clinical benefit in a subset of patients

A

Apitegromab in non-ambulatory Type 2 and 3 with background SMN upregulators

- Represents 2/3 of overall patients
- Many patients already treated with or are eligible for SMN upregulator therapy
- Improvements in motor function on top of SMN upregulators observed in TOPAZ

Anticipated Focus of Phase 3 Trial

B

Type 1 patients, including those treated with gene therapy

- Highest incidence population and growing prevalence due to SMN upregulator treatment
- TOPAZ showed benefits of early treatment suggesting potential in Type 1 patients

Preliminary Thoughts on Apitegromab Phase 3 Trial Design

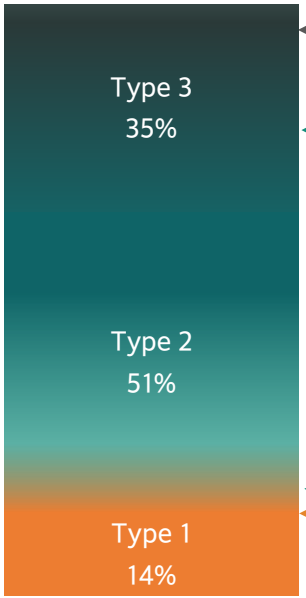
Apitegromab recently received Fast Track (FDA) and PRIME (EMA) designations, recognizing unmet medical needs in SMA

Phase 3 trial design subject to regulator interactions and feedback

Design	• Randomized, double-blind, placebo-controlled • 12-month treatment period • Apitegromab IV Q4W as add-on to nusinersen or risdiplam • TOPAZ data support investigation of up to 20 mg/kg
Subjects	• Non-ambulatory Type 2 and Type 3 SMA • Pediatric population in chronic maintenance phase of SMN therapy
Key Objectives	• HFMSE • Safety
Timeline	• Aim to initiate by end of 2021

Additional Opportunities May Be Pursued With Separate Development Strategies

Global disease with 30,000-35,000 affected in U.S. and Europe alone



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Panel Discussion on Apitegromab's Therapeutic Potential in SMA

Thomas Crawford, M.D.
Johns Hopkins Medicine

Basil Darras, M.D.
Boston Children's Hospital
Harvard Medical School

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Esteemed Panelists: TOPAZ Trial Investigators

Thomas Crawford, MD
Co-Director, MDA Clinic and Professor of Neurology and Pediatrics, Johns Hopkins Medicine
Lead TOPAZ Principal Investigator

- Member of the Department of Neurology at Johns Hopkins since 1987.
- Practice involves general child neurology with a principal interest in caring for children with neuromuscular, neuromotor, and ataxic disorders.
- On the Medical and Scientific Advisory Boards of Families of Spinal Muscular Atrophy, and the Medical Advisory Committee for the Muscular Dystrophy Association.

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

- Chief of the division of clinical neurology in the Department of Neurology at Boston Children's Hospital.
- Director of Neuromuscular Center and Spinal Muscular Atrophy program.
- Special focus is in the care of children with neuromuscular conditions originating from inherited or acquired conditions of the motor unit.

Thank You for Participating!

Pictures of individuals with SMA courtesy of Cure SMA

Appendix

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Majority of Ambulatory Patients Maintained or Improved in RHS Score from Baseline

	Apitegromab 20 mg/kg monotherapy	Apitegromab 20 mg/kg + nusinersen
Mean change from baseline in RHS (95% CI)	-0.4 (-3.9, 3.1)	-0.3 (-2.0, 1.4)
# (%) patients achieving:		
≥0-pt increase in RHS	6/11 (55%)	7/12 (58%)
≥1-pt increase in RHS	4/11 (36%)	5/12 (42%)
≥3-pt increase in RHS	3/11 (27%)	2/12 (17%)
Baseline characteristics: mean (min, max)	n=11	n=12
Age	12.1 (7, 19)	13.1 (7, 21)
HFMSE score	47.6 (26, 63)	51.3 (43, 62)
# of nusinersen maintenance doses	n/a	5.6 (2, 8)

Majority maintained or improved

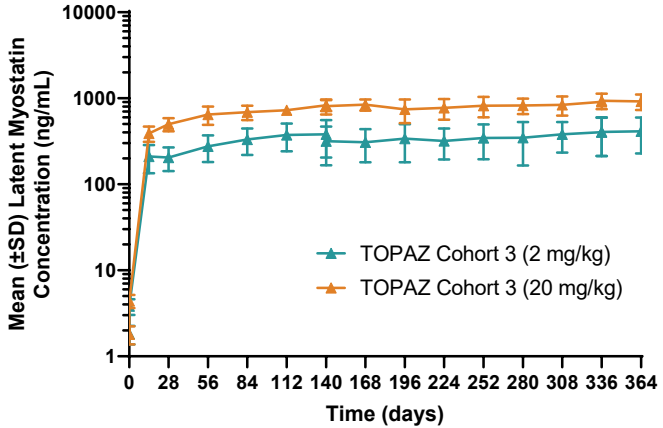
- 57% (13/23) with ≥0-point increase
- 39% (9/23) with ≥1-point increase
- Up to 8-point increase observed
- Results contrast with declines typically observed with natural history of ambulatory patients

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

Non-Ambulatory Type 2 Cohort (Age ≥2 years): Dose Response Observed in PD and Efficacy Data

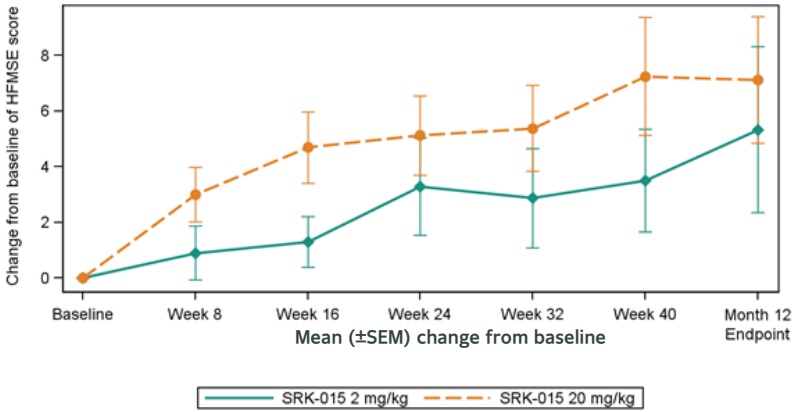
TOPAZ

Apitegromab achieved robust target engagement



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

Sizable increases in HFMSE achieved by patients on chronic maintenance nusinersen



- 20 mg/kg dose numerically offered greater HFMSE increases than 2 mg/kg dose across all timepoints
- Durability of effect observed through 12-months of treatment

Greater target engagement and efficacy observed with 20 mg/kg

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

2021: Potential for Another Transformative Year