



43rd Annual J.P. Morgan Healthcare Conference

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Forward-Looking Statements

Various statements in this presentation concerning the future expectations, plans and prospects of Scholar Rock Holding Corporation and Scholar Rock, Inc. (collectively, “Scholar Rock”), including without limitation, Scholar Rock’s expectations regarding its strategy, its product candidate selection and development timing, including timing for the initiation of and reporting results from its preclinical studies and clinical trials for apitegromab, SRK-439, linavonkibart and other product candidates and indication selection and development timing, its cash runway, the ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical, preclinical or clinical trial data, and the potential of its product candidates and proprietary platform. The use of words such as “may,” “could,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify such forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. All such forward-looking statements are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, without limitation, that preclinical and clinical data, including the results from the Phase 3 trial of apitegromab or Part A or Part B of the Phase 1 trial of linavonkibart, are not predictive of, may be inconsistent with, or more favorable than, data generated from future or ongoing clinical trials of the same product candidate; Scholar Rock’s ability to provide the financial support, resources and expertise necessary to identify and develop product candidates on the expected timeline; the data generated from Scholar Rock’s nonclinical and preclinical studies and clinical trials, including from the EMBRAZE clinical trial; information provided or decisions made by regulatory authorities; competition from third parties that are developing products for similar uses; Scholar Rock’s ability to obtain, maintain and protect its intellectual property; the success of Scholar Rock’s current and potential future collaborations; Scholar Rock’s dependence on third parties for development and manufacture of product candidates including, without limitation, to supply any clinical trials; Scholar Rock’s ability to manage expenses and to obtain additional funding when needed to support its business activities; its ability to establish or maintain strategic business alliances; its ability to receive priority or expedited regulatory review or to obtain regulatory approval of apitegromab; its ability to expand globally and the anticipated commercial launch in the United States of apitegromab in the fourth quarter of 2025; as well as those risks more fully discussed in the section entitled “Risk Factors” in Scholar Rock’s Form 10-K for the year ended December 31, 2023, and Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, as well as discussions of potential risks, uncertainties, and other important factors in Scholar Rock’s subsequent filings with the Securities and Exchange Commission. Any forward-looking statements represent Scholar Rock’s views only as of today and should not be relied upon as representing its views as of any subsequent date. All information in this press release is as of the date of the release, and Scholar Rock undertakes no duty to update this information unless required by law.

This presentation may also contain estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions, and estimates of our future performance and the future performance of the markets in which we compete are necessarily subject to a high degree of uncertainty and risk.

Apitegromab and SRK-181 are investigational drug candidates under evaluation. Apitegromab, linavonkibart, SRK-256, SRK-373, and SRK-439 have not been approved for any use by the FDA or any other regulatory agency and the safety and efficacy of apitegromab, linavonkibart, SRK-256, SRK-373, and SRK-439 have not been established.



Industry-leading technology, life-changing potential



OUR MISSION

To discover, develop, and deliver life-changing therapies by harnessing cutting-edge science to create new possibilities for people living with serious diseases



TODAY'S FOCUS

Scholar Rock is Moving with a Sense of Urgency To Bring Transformative Medicines to Patients

1 COMMERCIALIZE

Execute a Successful Commercial Launch*



Building off successful Phase 3 SAPHIRE results



BLA and MAA 1Q 2025 submission on track

\$2B+ Opportunity in SMA

2 EXPAND

Apitegromab Development Program: Building a Pipeline in a Product



Initiate earlier treatment with OPAL study for patients under 2 years of age with SMA



Pursuing opportunities for apitegromab in additional rare neuromuscular diseases

3 ADVANCE

Anti-myostatin Program into Cardiometabolic Indications



2Q 2025 EMBRAZE Phase 2 study results expected



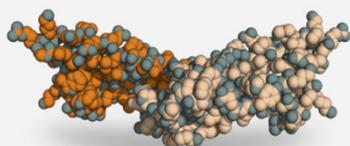
IND submission on track for 3Q 2025 for **SRK-439**, a highly innovative myostatin inhibitor

*Pending regulatory approval.

Scholar Rock Has Succeeded Where Others Have Failed

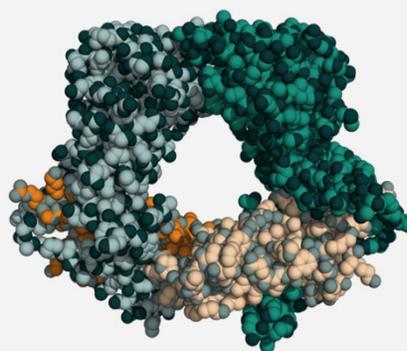
Selectivity Drives Success

Traditional Target
“Mature” Active Growth Factor



Challenging to target because of high homology across super-family

Scholar Rock’s Target
Latent Growth Factor



Targeting the ‘cage’ before growth factor is released allows for exquisite selectivity

RIGHT TARGET → Validated Biology

RIGHT TIME → Latent Form

Growing Pipeline Across High Value Therapeutic Areas

Industry-leading Anti-myostatin Programs

Our Differentiated Approach: Target Latent Growth Factor

THERAPEUTIC AREA	PRODUCT (<i>target</i>)	Discovery/ Preclinical	PHASE 1	PHASE 2	PHASE 3	Commercial	
NEUROMUSCULAR	Apitegromab (<i>latent myostatin</i>)	SPINAL MUSCULAR ATROPHY					
	(<i>undisclosed</i>)	*Indication					
CARDIOMETABOLIC	Apitegromab (<i>latent myostatin</i>)	OBESITY					
	SRK-439 (<i>novel latent myostatin</i>)	OBESITY					
IMMUNO-ONCOLOGY	Linavonkibart (SRK-181) (<i>latent TGFβ1</i>)	UC, ccRCC**					
HEMATOLOGY	SRK-256 (<i>RGMc</i>)	ANEMIA					
FIBROSIS	SRK-373 (<i>LTBP1/3</i>)	Fibrotic indications					

*undisclosed indication

**UC=urothelial cell carcinoma; RCC=renal cell carcinoma.

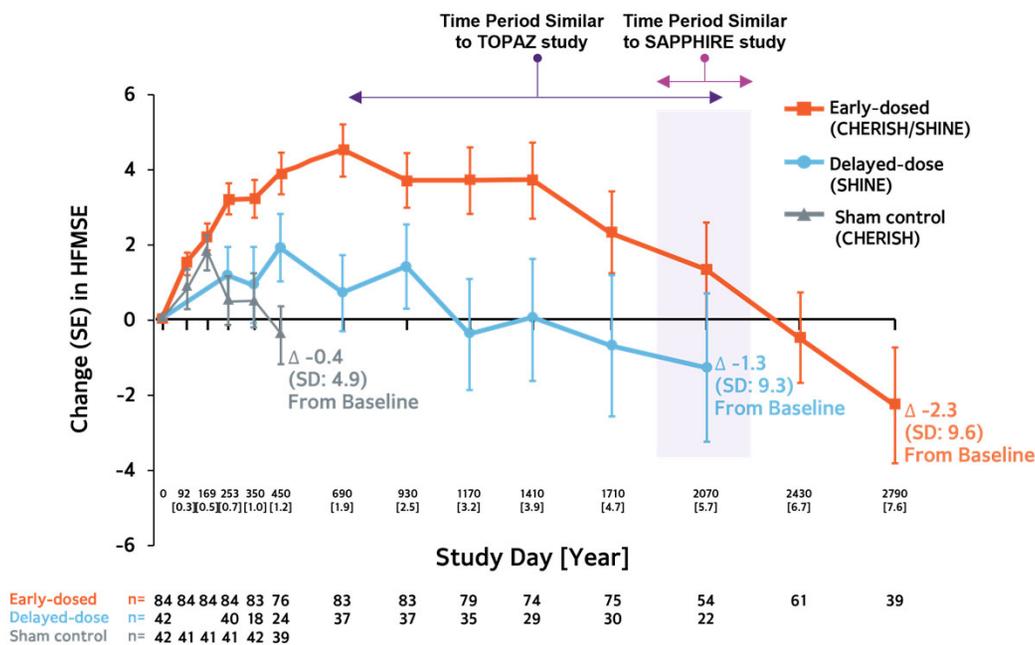


Transforming the Treatment of SMA

Commercializing Apitegromab

Progressive Muscle Weakness Remains Core Unmet Need in SMA

Motor Function Over Time of Patients Treated with Nusinersen¹



What do patients seek most from a new treatment in SMA?²

97% said

Improvement in muscle strength

90% said

Achieving new motor function

88% said

Stabilizing motor function

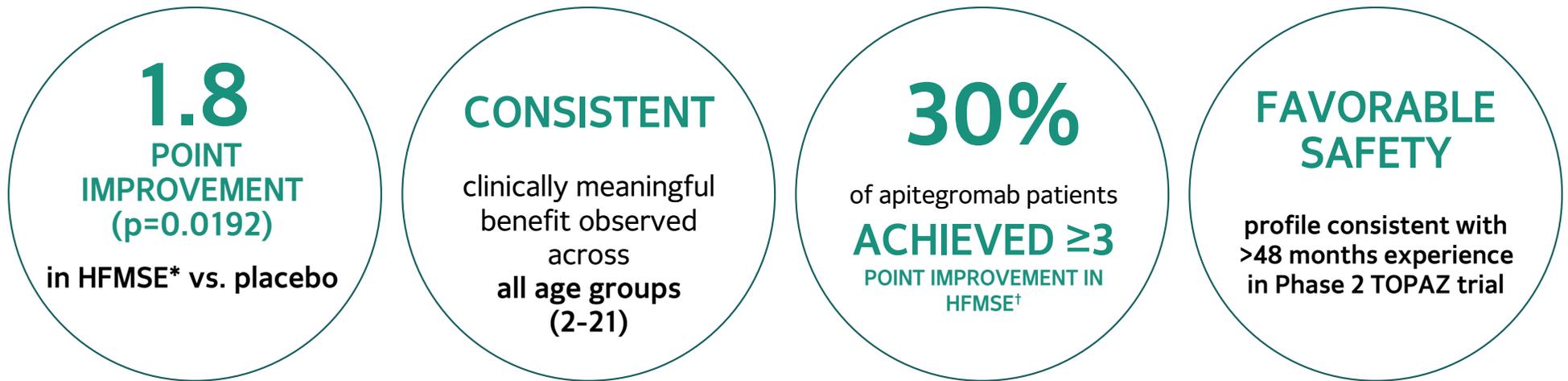
1. Finkel RS et al. "Final Safety and Efficacy Data From the SHINE Study in Participants With Infantile-Onset and Later-Onset SMA." Presented at Cure SMA Annual Conference, July 2024.
 2. Cure SMA State of SMA Annual Report. Published May 6, 2024; Cure SMA. Education on adult patient expectations according to copy number and disease status at time of report. September 2022.; Internal Scholar Rock market research.

This information from third-party studies is provided for background purposes only and is not intended to convey or imply a comparison to the SAPPHIRE clinical trial results.
 HFMSE=Hammersmith Functional Motor Scale Expanded; SMA=Spinal Muscular Atrophy.

SELECTIVITY DRIVES SUCCESS

The Only Muscle-Targeted Therapy with Clinical Success in SMA

Positive Phase 3 Trial Using Gold Standard SMA Scale



SUCCESSFUL PIVOTAL TRIAL
On Track to Submit BLA and MAA in 1Q 2025

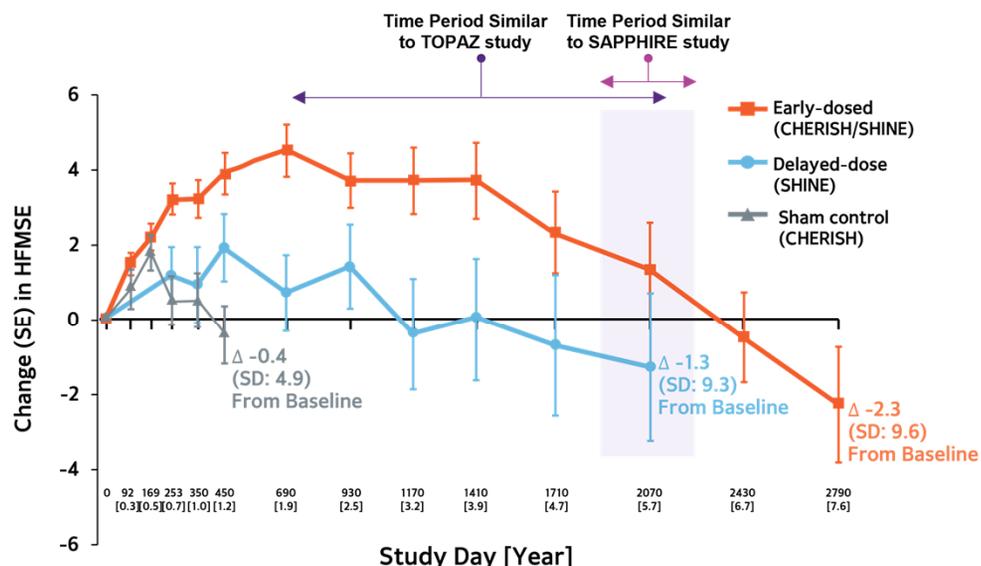
* Based on apitegromab combined dose (10 mg/kg and 20 mg/kg) + SOC versus placebo + SOC (Hochberg multiplicity adjustment).

† 12.5% of patients on placebo + SOC achieved a ≥ 3 -point improvement in HFMSE

SOC=Standard of care (i.e., nusinersen or risdiplam); HFMSE=Hammersmith Functional Motor Scale-Expanded.

Apitegromab: Potential to Transform the Standard of Care in SMA

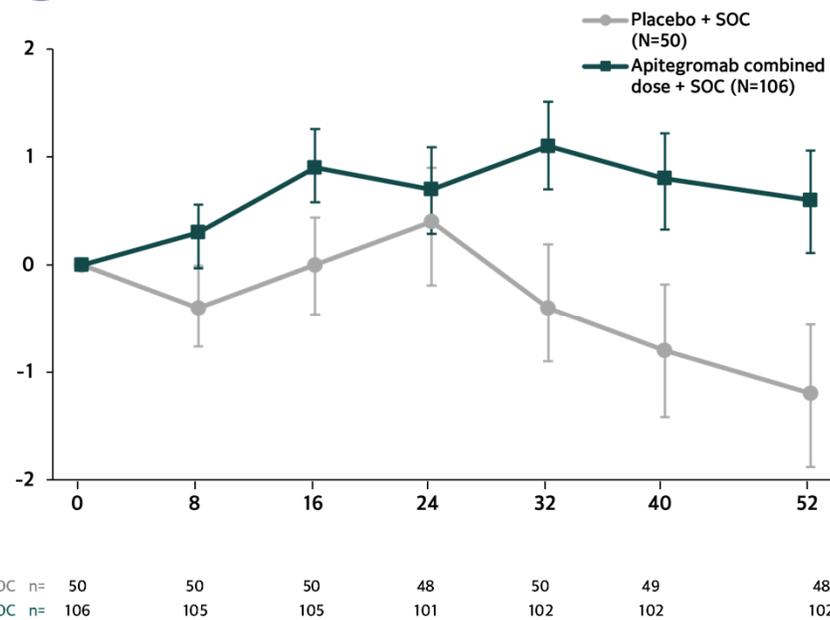
Motor Function Over Time of Patients Treated with Nusinersen



Study Day [Year]	Early-dosed n=	Delayed-dose n=	Sham control n=
0	84	42	42
92	84	41	41
169	84	41	41
253	84	41	41
350	84	18	42
450	83	24	39
690	83	37	37
930	83	37	37
1170	79	35	35
1410	74	29	29
1710	75	30	30
2070	54	22	22
2430	61	22	22
2790	39	22	22



Motor Function Over Time of Patients in SAPHIRE



KEY TAKEAWAYS

- Despite effective SMN-targeted therapy, long-term trajectory of SMA patients remains that of progressive decline in motor function
- Treatment with apitegromab has improved motor function vs. placebo

Finkel RS et al. "Final Safety and Efficacy Data From the SHINE Study in Participants With Infantile-Onset and Later-Onset SMA." Presented at Cure SMA Annual Conference, July 2024

*Patient age based on those received active treatment (mean or median)

1. This information from third-party studies is provided for background purposes only and is not intended to convey or imply a comparison to the SAPHIRE clinical trial results.

CI=Confidence Interval; EXP=Exploration Subpopulation; HFMSSE=Hammersmith Functional Motor Scale Expanded; LS=Least Squares; MEP=Main Efficacy Population; SOC=standard of care.

Apitegromab: Shifting the Treatment Paradigm in SMA



Transformative Therapeutic Potential

- Validated with a rigorous approach:
Strong trial design using a gold-standard SMA scale
- Potential to transform standard of care in SMA



Long-Term Treatment Experience

- >4 years treatment in SMA in Phase 2 TOPAZ trial
- >90% retention in TOPAZ
- 98% continuation from SAPPHIRE to ONYX long-term extension



Suitable for a Broad SMA Population

- Consistency of effect seen across age groups studied
- Long-term tolerability shown to date



Expanding our Impact: Initiating Phase 2 OPAL Trial in mid-2025

Studying apitegromab in patients under 2 years old



TIME Is Muscle

Reaching patients earlier in their treatment journey



EXPANDING Our Impact

Including patients who received gene therapy



CHANGING More Lives

Potential to alter the course of SMA in a broad population

Scholar Rock is Positioned for a Successful Commercial Launch

THE RIGHT...



MEDICINE

Potential to alter the course of SMA



MARKET

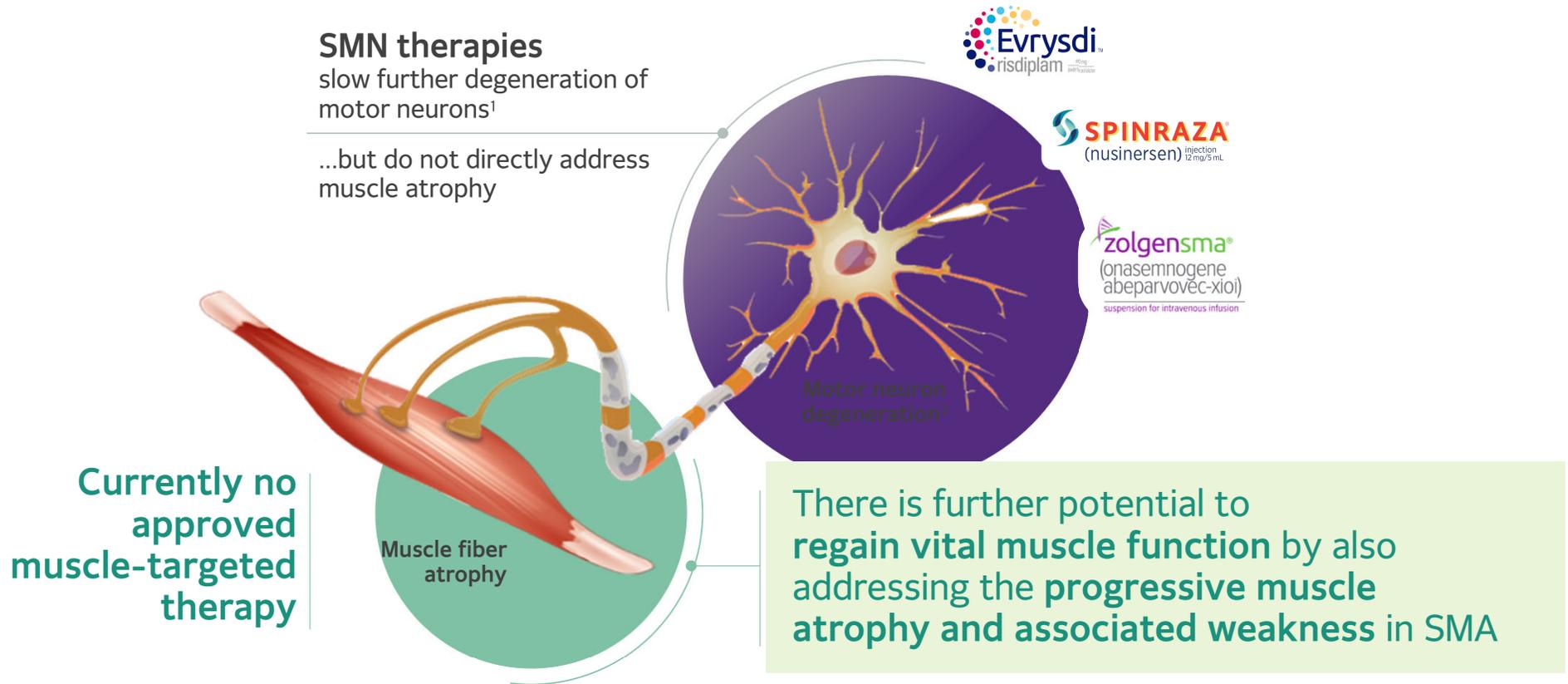
Clear unmet need and first and only muscle-targeted therapy



PLAN

Engagement, patient focus & execution

Existing Therapies Generate ~\$4.5B in Annual Revenues Addressing the Motor Neuron



1. Hua Y, et al. *Nature*. 2011;478(7367):123-6.

2. Figure adapted from: SMA Foundation Overview. <http://www.smafoundation.org/wp-content/uploads/2012/03/SMA-Overview.pdf>; Accessed April 18, 2021.
SMA=Spinal muscular atrophy; SMN=Survival motor neuron.

\$2B+ Global Opportunity for Apitegromab in SMA

~\$4.5B¹

Global revenue for three SMN-targeted therapies

SPINRAZA
(nusinersen)
injection, 12 mg/0.5 mL

Evrysdi
risdiplam
suspension for intravenous infusion

zolgensma
(onasemnogene
abeparvovec-xiol)
suspension for intravenous infusion

1st

And only muscle-targeted treatment to show clinical benefit in SMA

Apitegromab global revenue potential

\$2B+²



SMA patients are diagnosed, treated, and still need more to continue to improve their lives

¹ Revenue as of Biogen 4Q23 financial update, Roche 4Q23 financial update, and Novartis 4Q23 financial update.

² Scholar Rock internal estimates as of December 2024.

SMA=Spinal muscular atrophy; SMN=Survival motor neuron.

SMA is a Defined Market and Optimal For Apitegromab Launch

Patients are diagnosed and treated, but still need more



1. Patients Identified and Treated

~25K patients in US and EU
100% US newborn screening
2/3 of US patients on treatment



2. Clear Unmet Need

Patient, HCP, and payers recognize remaining need to improve function



3. Engaged Patient Community

Organized community aligned on need for muscle targeted therapy



4. Payer Receptivity

Established value for improving function

The SMA community is calling for new treatments to improve function

Advantaged by Deep Rare Disease Commercial Experience



Leadership

- ✓ Seasoned executive team with average of **20+ years industry experience**



Launches

- ✓ Commercial, medical, and advocacy teams with **deep rare disease launch experience**



Preparing for a Successful Launch



Delivering Apitegromab

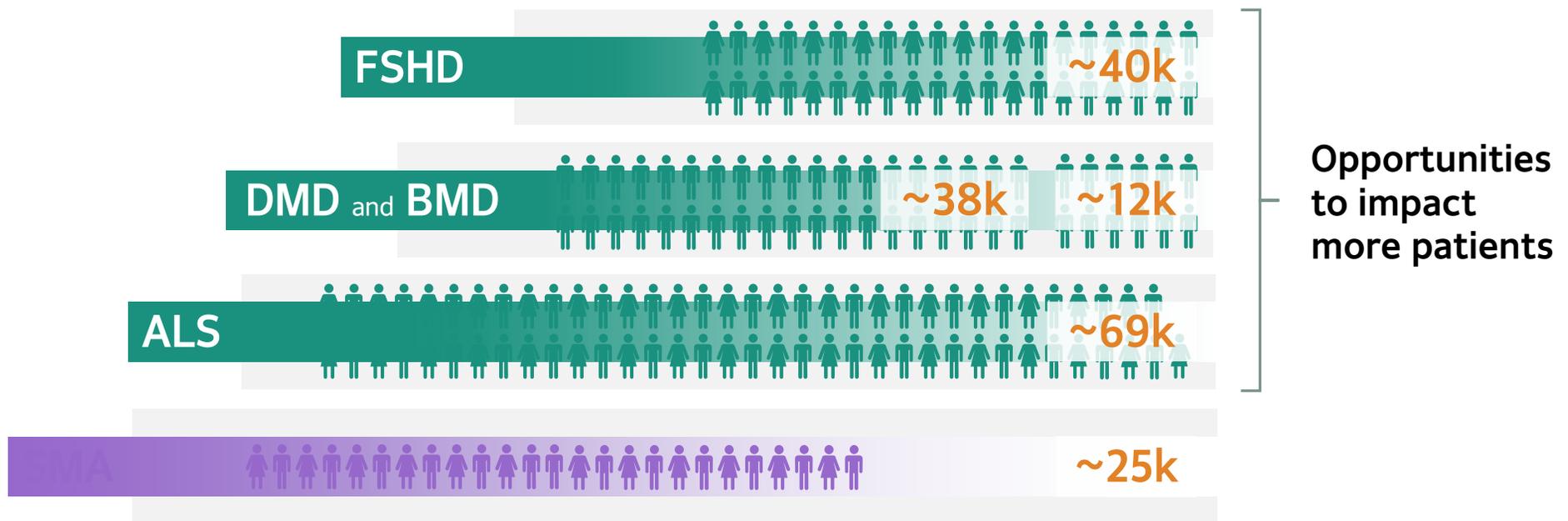
- ✓ Commercial launch supply secured
- ✓ Rare disease distribution partners selected
- ✓ Home infusion at launch



Customer Engagement

- ✓ Account team with average 30 years experience engaging with US commercial and federal payers
- ✓ Efficient US customer-facing footprint of ~50 FTEs planned
- ✓ Establishing European presence

SMA is Only the Beginning: Creating Possibilities with Apitegromab in Additional Neuromuscular Diseases



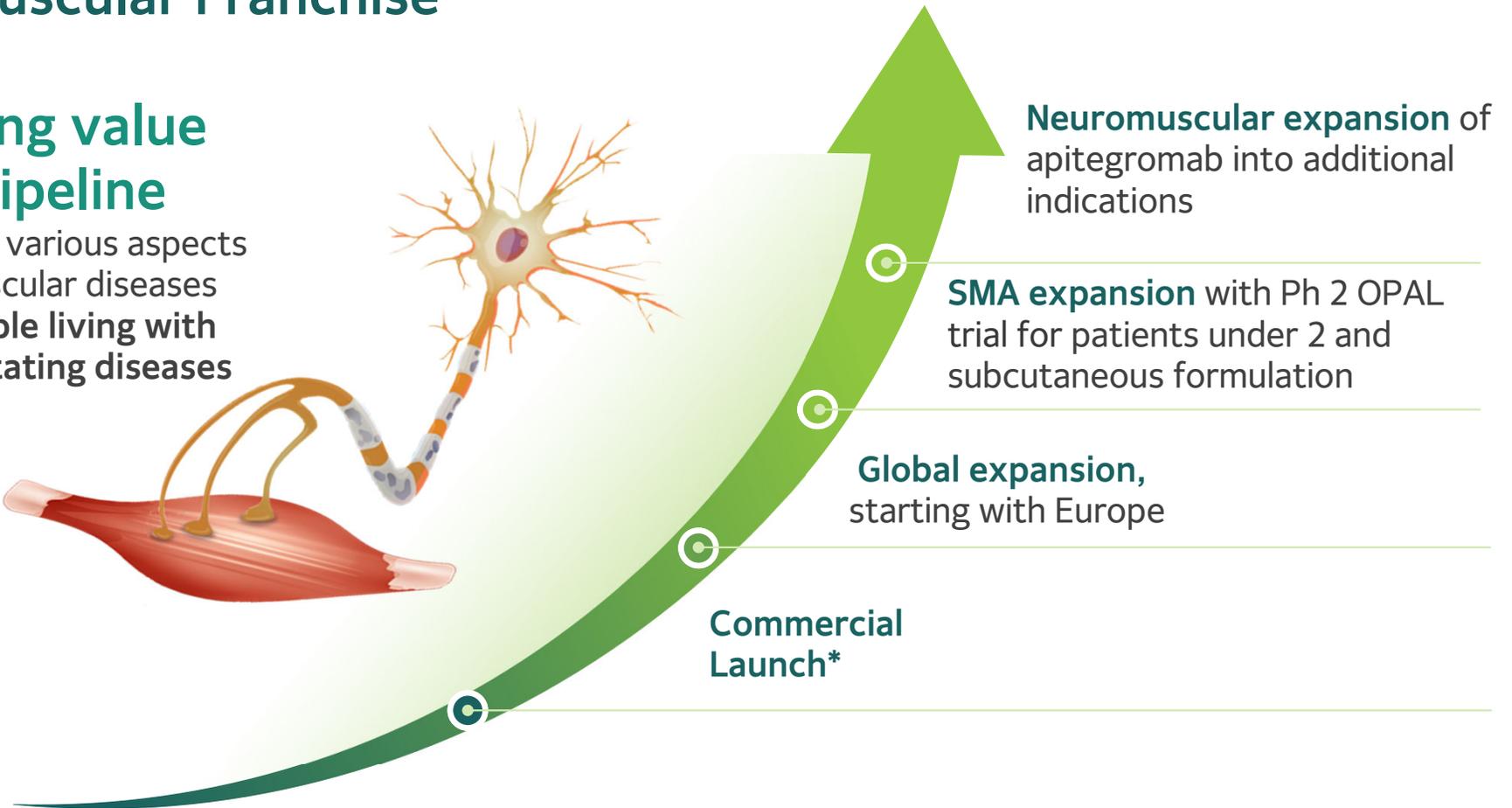
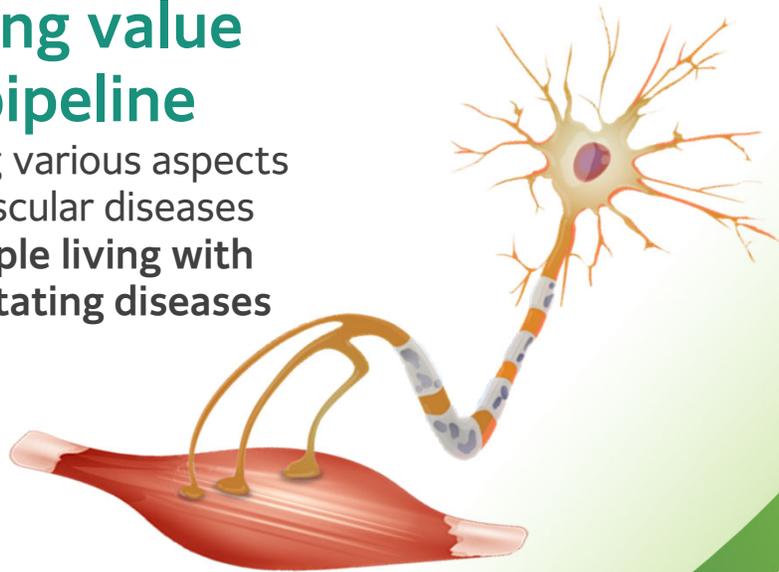
Building a neuromuscular franchise is a key driver towards future growth

*Numbers represent prevalence in the US and Europe based on internal market research.
SM=Spinal Muscular Atrophy; ALS=Amyotrophic Lateral Sclerosis; DMD=Duchenne Muscular Dystrophy; BMD=Becker's Muscular Dystrophy; FSHD=Facioscapulohumeral muscular dystrophy.

Apitegromab is the Foundation of a Future Multi-Billion Dollar Neuromuscular Franchise*

Unlocking value in our pipeline

by targeting various aspects of neuromuscular diseases to help people living with rare, devastating diseases



*Subject to regulatory approval.



Unlocking Value in Our Anti-Myostatin Platform

Cardiometabolic Program Update

GLP-1 RAs are Transforming Weight Loss for Millions of People



Recently approved
GLP-1 RAs are
highly effective
in weight loss &
experiencing
rapid uptake



ONCE-WEEKLY
wegovy[®]
semaglutide injection 2.4 mg



once weekly
zepbound[™]
(tirzepatide) injection 0.5 mL
2.5 mg | 5 mg | 7.5 mg | 10 mg | 12.5 mg | 15 mg



BY
2029...



40 million people on GLP-1 RAs
\$126 billion in Sales*

Source: UBS Bank, GLP-1: A medication worth \$126 billion in sales by 2029? <https://www.ubs.com/global/en/investment-bank/insights-and-data/2024/glp-1-a-medication.html>.
GLP-1 RA=GLP-1 receptor agonist.

However, Patients Want Healthier Weight Loss*



WEAKNESS is a Concern

Patients complain of reduced strength after GLP-1 RA treatment



Improved LEAN MASS

Patients hope for a combination treatment approach to address this need



Significant Weight REGAIN

in 40-100% of patients after stopping GLP-1 RA treatment



*Patients feel good about the number on the scale, but **there are issues with muscle loss** ... they complain of **weakness or reduced strength**.*

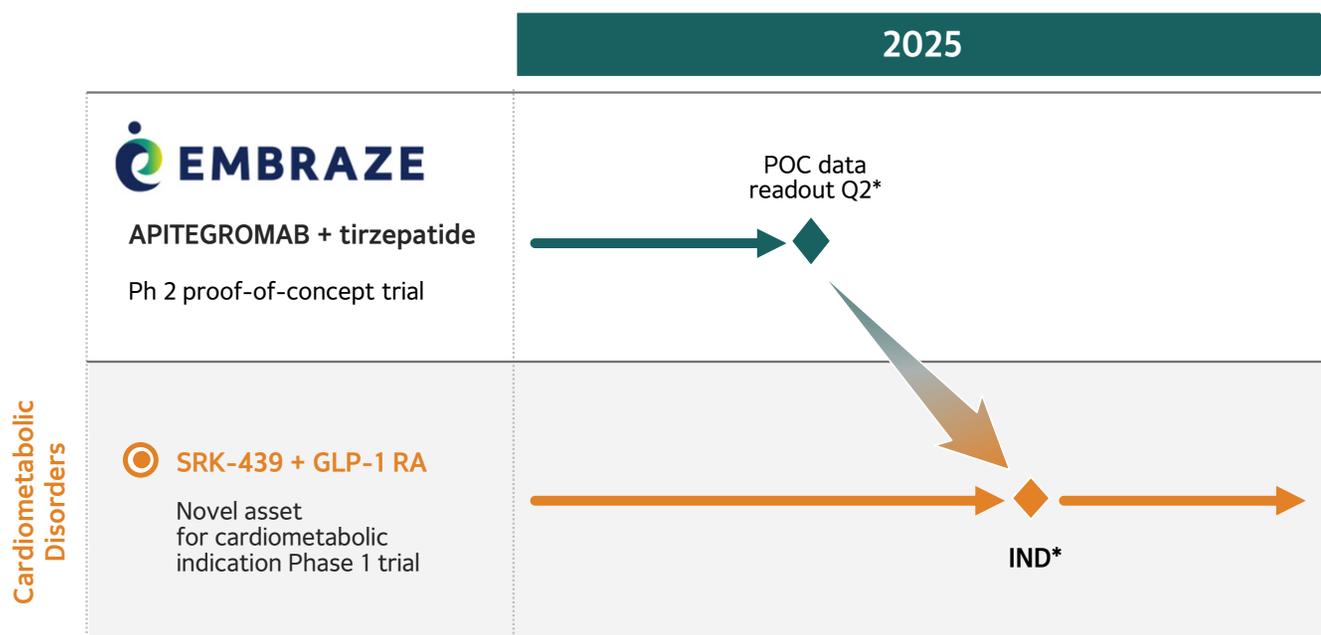
Obesity Clinician



Scholar Rock's unique highly selective approach to targeting latent myostatin has the potential to address these patient needs

*Source: Scholar Rock market research, completed December 2024.
GLP-1 RA=GLP-1 receptor agonist.

Industry-Leading Anti-Myostatin Platform: Leveraging Apitegromab's Success to Advance the Obesity Program



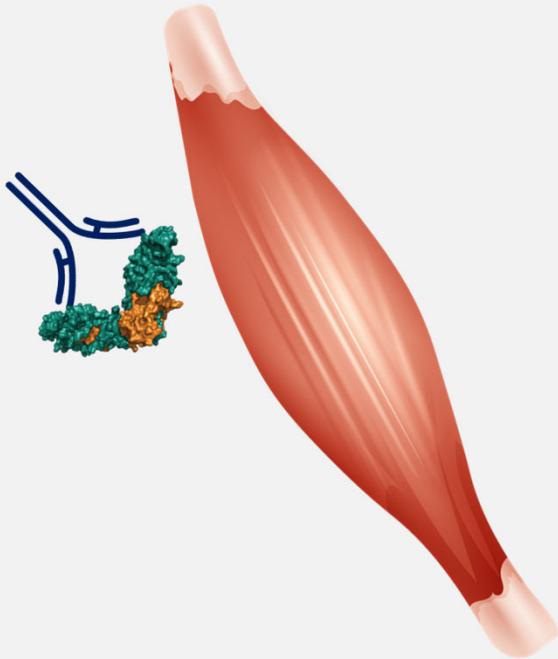
Testing hypothesis of selective anti-myostatin antibody in obese population

- EMBRAZE POC readout in Q2 2025
- SRK-439 IND submission in Q3 2025

*Expected timelines
POC=Proof of Concept; GLP-1 RA=GLP-1 receptor agonist.

Strong Scientific Validation and Promising Preclinical Evidence

SRK-439: Potential Best in Class



GLP-1 RA=GLP-1 receptor agonist.

Preclinical data to date show strong potential to support healthier weight loss in combination with GLP-1 RAs:

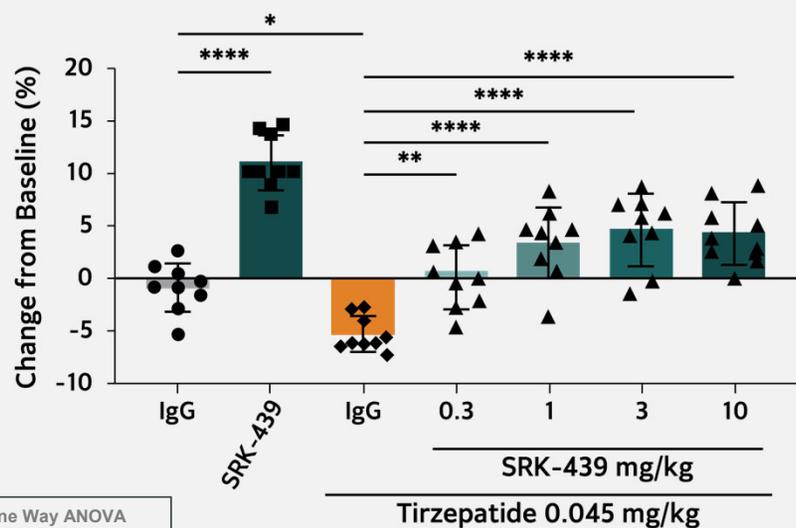
- ✓ Preservation of lean mass
- ✓ Improvement in metabolic parameters
- ✓ Increase in lean mass and attenuation of fat mass regain following GLP-1 RA withdrawal
- ✓ Greater potency compared to an anti-ACR11 antibody
- ✓ Works across the class of GLP-1 RAs

KEYSTONE SYMPOSIA



SRK-439 Protects from Tirzepatide-Induced Muscle Loss in DIO Mice

qNMR % Lean Mass Change from Baseline

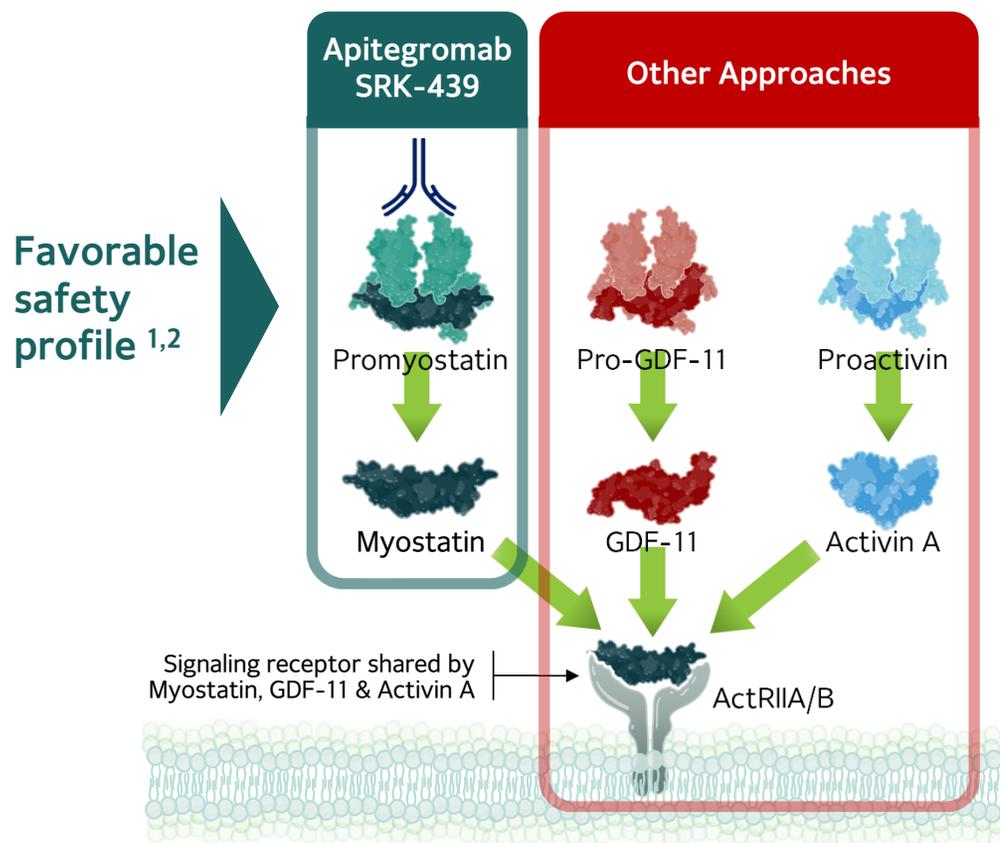


One Way ANOVA
* p < 0.05
** p < 0.01
*** p < 0.001
**** p < 0.0001

Key Observations

- Considerable lean mass loss with tirzepatide treatment
- Combination with SRK-439 led to dose-dependent lean mass preservation
- Lean mass preservation seen with doses as low as 0.3 mg/kg and lean mass gain at higher doses
- Dose dependent enhancement of fat mass loss also observed, improving overall body composition

Potential to Optimize Benefit-Risk with Myostatin Selectivity



Health Risks Observed with Non-Selective Inhibition of ActRII Pathway:

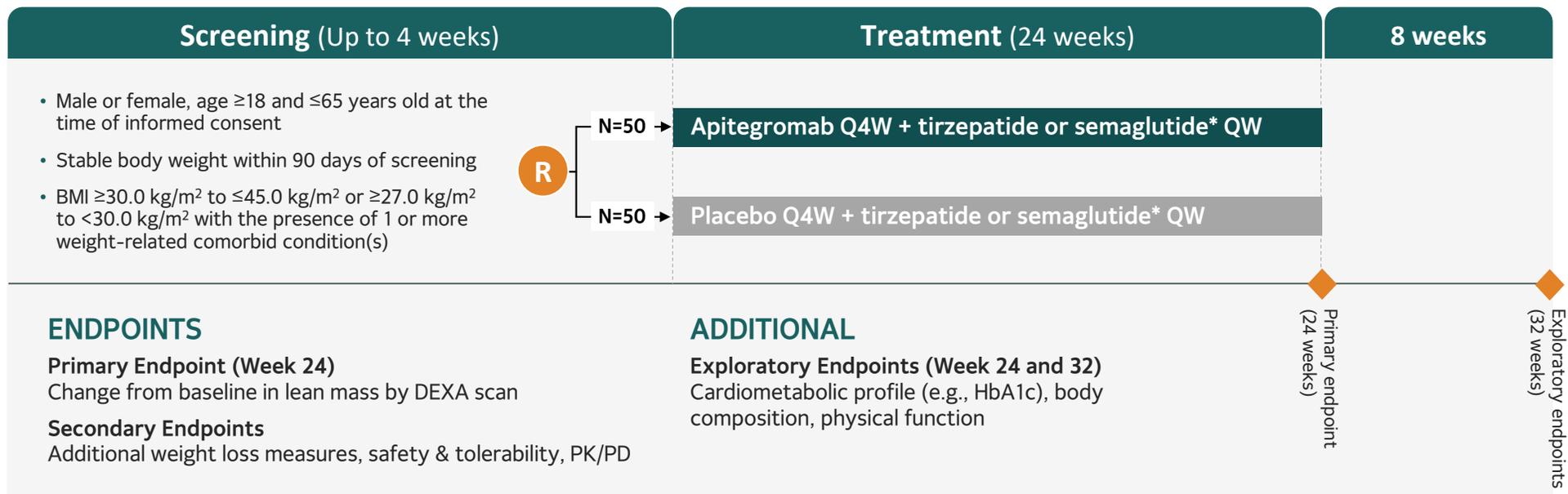
- GI problems, e.g., diarrhea, pancreatitis³⁻⁶
- Nose bleeds (epistaxis), low platelet count, telangiectasias⁷⁻¹⁰
- Reduction in reproductive hormones in males and females^{3, 7, 11, 12}
- Acne, rash, skin abscesses^{5, 13, 14}
- Madarosis (loss of eyebrows or eyelashes)¹⁴

1. Barrett et al. *Adv Therapy*. 2021; 2. Crawford T et al. *Neurology*. 2024; 3. Garito T et al. *Clin Endocrinol (Oxf)*. 2018; 4. Amato AA et al. *Neurology*. 2021; 5. Heymsfield SB et al. *JAMA*. 2021; 6. Vanhoutte F et al. *J Clin Pharmacol*. 2020; 7. Attie KM et al. *Muscle Nerve*. 2013; 8. Attie KM et al. *Am J Hematol*. 2014; 9. Campbell C et al. *Muscle Nerve*. 2017; 10. Hooper MM et al. *N Engl J Med*. 2023; 11. Ruckle J et al. *J Bone Miner Res*. 2009; 12. Sherman ML et al. *J Clin Pharmacol*. 2013; 13. Muntoni F et al. *Neurol Ther*. 2024. 14. Di Rocco M et al. *Nat Med*. 2023.

Data from Phase 2 Proof-of-Concept Study of Apitegromab in Obesity Expected Q2 2025



Randomized, double-blind, placebo-controlled (n=102 enrolled)
 Enrolled patients who are overweight or obese
 Enrollment completed ahead of schedule; topline data expected in Q2 2025



*Due to expedited enrollment and timing of semaglutide clinical supply, all enrolled patients received tirzepatide. Apitegromab dose regimen will be 10 mg/kg Q4W, based on projected exposure in the obese population comparable to that of 20 mg/kg Q4W in SMA. Tirzepatide and semaglutide dose regimen will follow the United States Prescribing Information.

Goals of the EMBRAZE Proof-of-Concept Study



Study Aims to Demonstrate

- ➔ Preservation of lean mass in obese or overweight patients
- ➔ Safety and tolerability
- ➔ Potential to improve metabolic profile and physical function

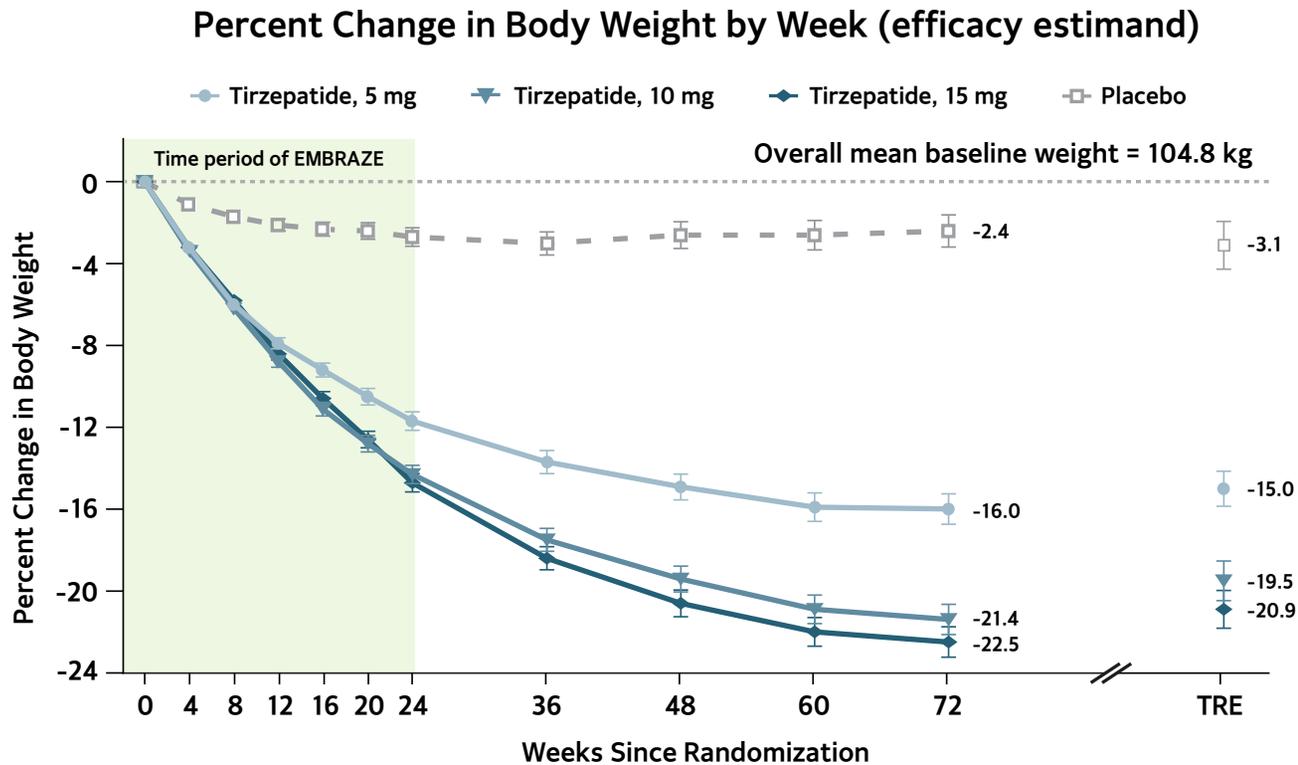
INSIGHTS GAINED
FROM EMBRAZE STUDY
to inform SRK-439
development

Initiated trial in May 2024,
ahead of target timeline

Enrollment completed in
September 2024

Topline data expected in
Q2 2025

Tirzepatide-Induced Weight Loss Accompanies Significant Muscle Loss



Tirzepatide

DECREASE
 in lean mass of
10.9%
 accompanies
 DECREASE
 in **body weight**

Figure from Jastreboff, A.M. et al. *N Engl J Med.* 2022;387(3):205-216.
 * Data from Phase 3 clinical trial of tirzepatide in adults with obesity.



Conclusion

Delivering on the Mission of Bringing Transformative Medicines to Patients

Leveraging R&D success to build a multi-billion dollar biopharmaceutical company



INNOVATE

- ✓ Developed platform based on selective targeting of latent growth factors
- ✓ Advanced industry-leading antibody design & protein engineering



DEVELOP

- ✓ Successfully executed positive Phase 3 trial in SMA

COMMERCIALIZE

- Successful commercial launch*
- Setting the stage for a multi-billion dollar opportunity

EXPAND

- Expanding neuromuscular franchise
- Advancing anti-myostatin program in obesity
- Advancing the pipeline

* Pending approval from regulatory agencies.
Apitegromab is an investigational drug candidate under evaluation and has not been approved by any regulatory agency.

2025 Milestones: A Transformative Year for Scholar Rock

1

COMMERCIALIZE

Apitegromab in
SMA

- Submit FDA and EMA applications in 1Q 2025
- US launch expected in 4Q 2025 and EU launch to follow*

2

EXPAND

Apitegromab Development
Program: Building a Pipeline
in a Product

- SMA: Under 2 study initiation planned for mid-2025
- Exploring additional neuromuscular indications

3

ADVANCE

Anti-myostatin Program
into Cardiometabolic
Indications

- Obesity: EMBRAZE readout expected in 2Q 2025
- SRK-439 IND filing planned for 3Q 2025

* Pending regulatory approval.
Apitegromab is an investigational drug candidate under evaluation and has not been approved by any regulatory agency.

Thank you!