

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event Reported): January 14, 2025

**Scholar Rock Holding Corporation**

(Exact Name of Registrant as Specified in Charter)

**Delaware**  
(State or Other Jurisdiction of  
Incorporation)

**001-38501**  
(Commission File Number)

**82-3750435**  
(I.R.S. Employer Identification Number)

**301 Binney Street, 3rd Floor, Cambridge, MA 02142**  
(Address of Principal Executive Offices) (Zip Code)

**(857) 259-3860**  
(Registrant's telephone number, including area code)

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.001 per share	SRRK	The Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR §230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR §240.12b-2). Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

**Item 7.01. Regulation FD Disclosure.**

A copy of the presentation that will be used by management of Scholar Rock Holding Corporation at the 43rd Annual J.P. Morgan Healthcare Conference on January 14, 2025 is being furnished as Exhibit 99.1 to this report on Form 8-K.

The information in this Item 7.01 and Exhibit 99.1 attached hereto is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934 (the "Exchange Act") or otherwise subject to the liabilities of that section. It may only be incorporated by reference in another filing under the Exchange Act or the Securities Act of 1933, as amended, if such subsequent filing specifically references the information furnished pursuant to Item 7.01 and Exhibit 99.1 of this Current Report on Form 8-K.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits

<b>Exhibit No.</b>	<b>Description</b>
99.1	<a href="#">Scholar Rock Holding Corporation Corporate Presentation dated January 14, 2025.</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

**Scholar Rock Holding Corporation**

Date: January 14, 2025

By: /s/ Junlin Ho

Junlin Ho  
General Counsel & Corporate Secretary

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# 43rd Annual J.P. Morgan Healthcare Conference

Jay Backstrom, M.D., MPH  
President and Chief Executive Officer

January 14, 2025

# 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 initiation of and reporting results from its preclinical studies and clinical trials for apitegromab, SRK-439, linavonkibart and other product candidates and indication selection; 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, 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 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 the 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 a 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 the 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 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



# 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



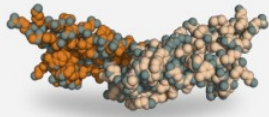
IND submission on track for 3Q 2025 for **SRK-439**, a highly innovative myostatin inhibitor

\*Pending regulatory approval.

# Scholar Rock Has Succeeded Where Others Have Failed

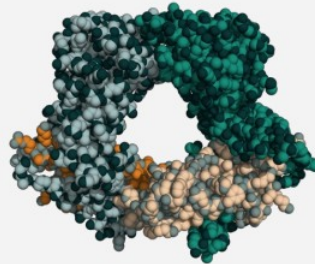
Selectivity Drives Success

## 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	<b>Apitegromab</b> ( <i>latent myostatin</i> )	SPINAL MUSCULAR ATROPHY				
	( <i>undisclosed</i> )	* <i>Indication</i>				
CARDIOMETABOLIC	<b>Apitegromab</b> ( <i>latent myostatin</i> )	OBESITY				
	<b>SRK-439</b> ( <i>novel latent myostatin</i> )	OBESITY				
IMMUNO-ONCOLOGY	<b>Linavonkibart (SRK-181)</b> ( <i>latent TGFβ1</i> )	UC, ccRCC**				
HEMATOLOGY	<b>SRK-256</b> ( <i>RGMc</i> )	ANEMIA				
FIBROSIS	<b>SRK-373</b> ( <i>L TBP1/3</i> )	<i>Fibrotic indications</i>				

\*undisclosed indication

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

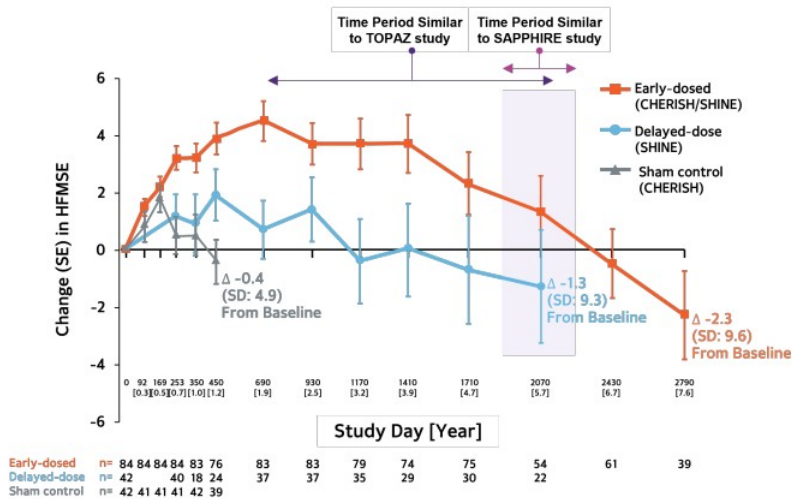


# Transforming the Treatment of SMA

Commercializing Apitegromab

# Progressive Muscle Weakness Remains Core Unmet Need in SMA

Motor Function Over Time of Patients Treated with Nusinersen<sup>1</sup>



What do patients seek most from a new treatment in SMA?<sup>2</sup>

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 SAPHIRE clinical trial results.  
 HF MSE=Hammersmith Functional Motor Scale Expanded; SMA=Spinal Muscular Atrophy.

# 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**

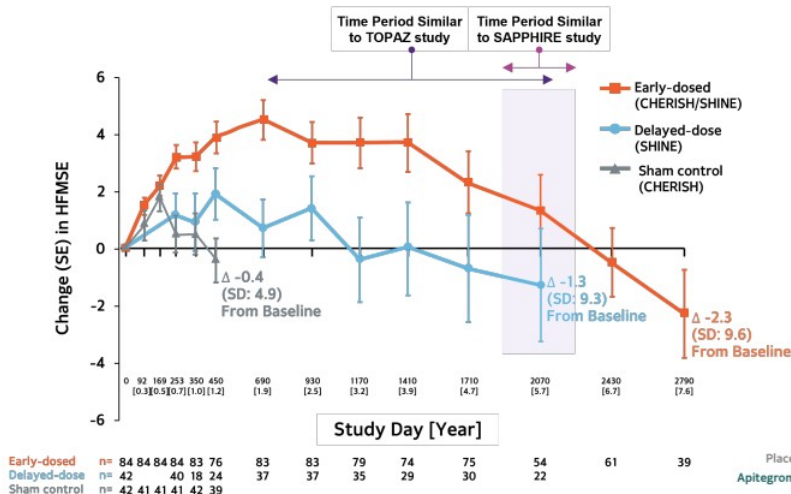
\* Based on apitegromab combined dose (10 mg/kg and 20 mg/kg) + SOC versus placebo + SOC (Hochberg multiplicity adjustment).

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

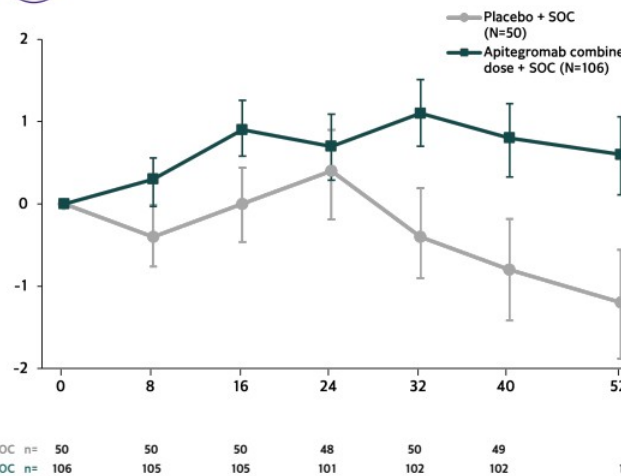
SOC=Standard of care (i.e., nusinersen or risdiplam); HFMSE=Hammersmith Functional Motor Scale-Expanded.

# Apitegromab: Potential to Transform the Standard of Care in SMA

Motor Function Over Time of Patients Treated with Nusinersen



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; HFMSE=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

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## EXPANDING Our Impact

Including patients who received gene therapy

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## 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

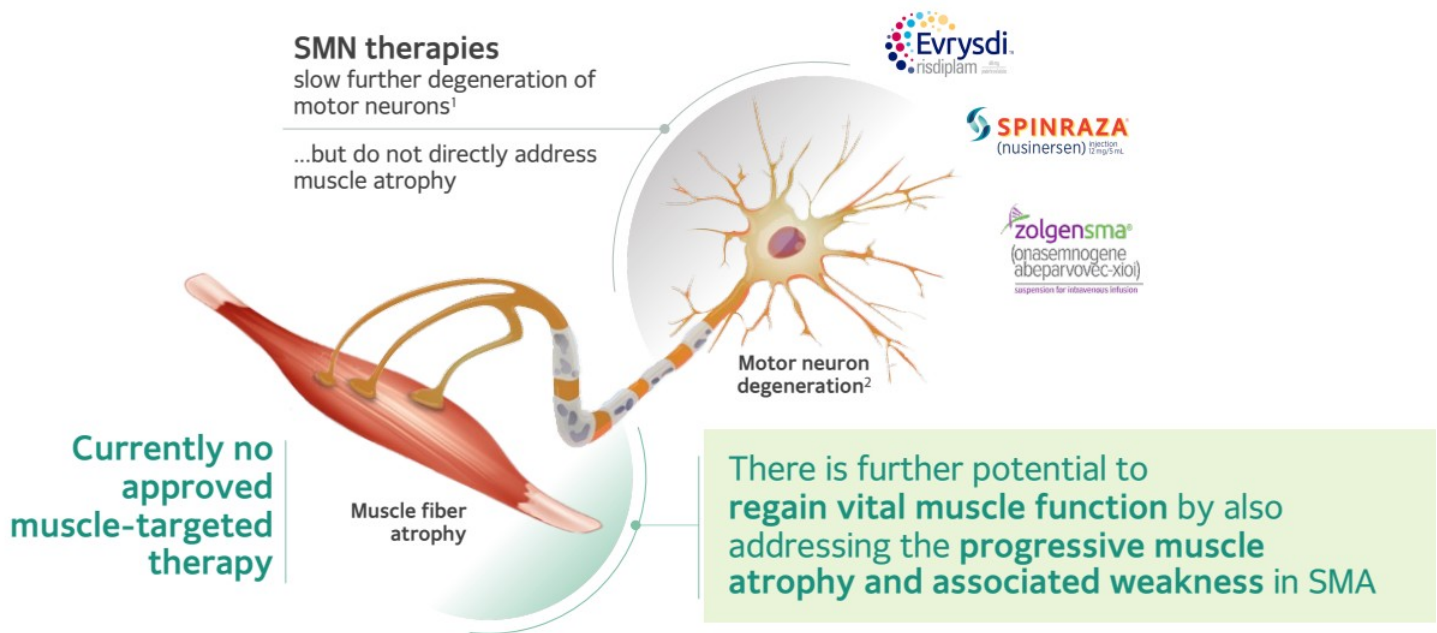


### PLAN

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

~\$4.5B<sup>1</sup>  
Global revenue for  
three SMN-targeted  
therapies

**SPINRAZA**  
(nusinersen)

**Evrysdi**  
(risdiplam)

**zolgensma**  
(onasemnogene  
abeparvovec-xioi)  
approved by FDA and EMA

**1st**  
And only  
muscle-targeted  
treatment to  
show clinical  
benefit in SMA

Apitegromab global  
revenue potential

**\$2B+**<sup>2</sup>



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

<sup>1</sup> Revenue as of Biogen 4Q23 financial update, Roche 4Q23 financial update, and Novartis 4Q23 financial update.

<sup>2</sup> 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**



# Partnering With the SMA Community and Engaging Top HCPs

## Disease EDUCATION



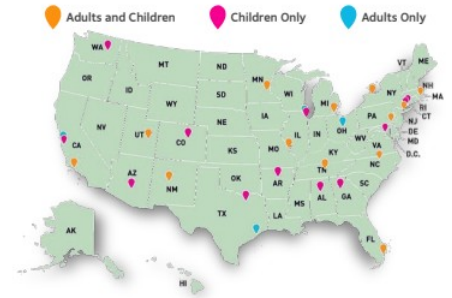
Amplifying the patient voice in first muscle-focused SMA disease education campaign

## Patient ADVOCACY



Partnering with US and European patient advocacy groups

## HCP ENGAGEMENT



Engaging with 100% of Cure SMA-identified centers

# Preparing for a Successful Launch



## Delivering Apitegromab

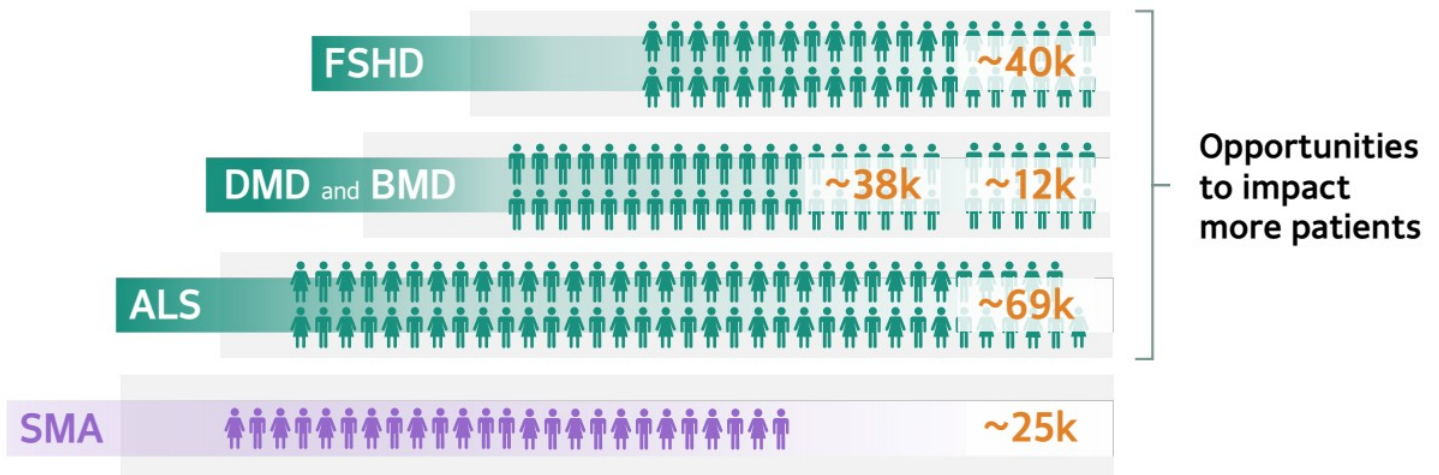
- ✓ Commercial launch supply secured
- ✓ Rare disease distribution partners selected
- ✓ Home infusion at launch



## 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



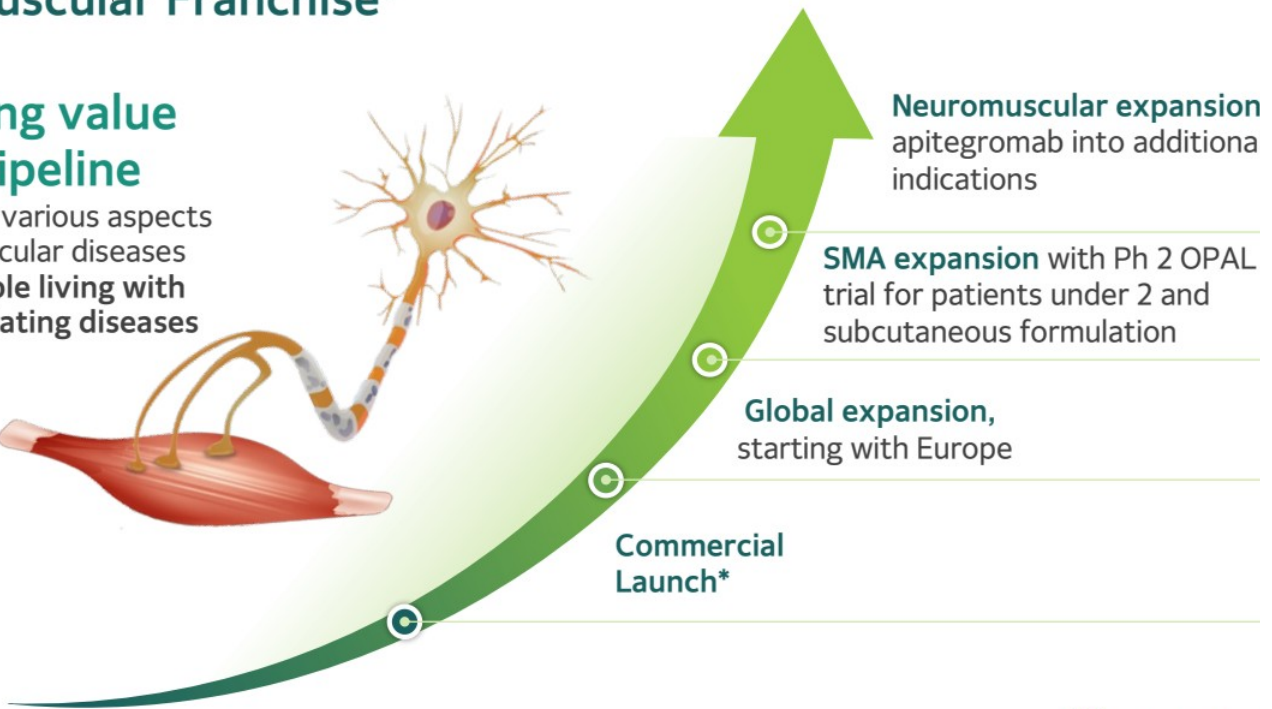
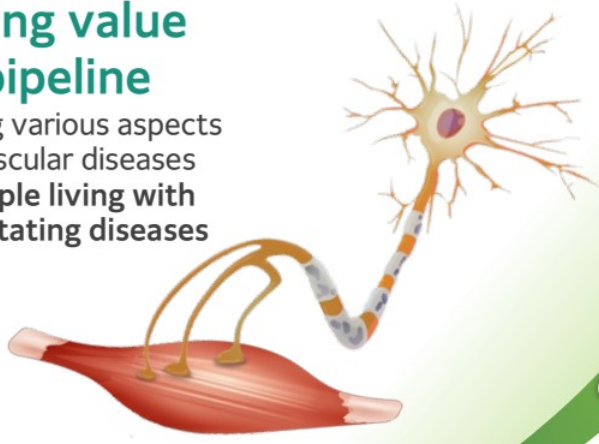
Building a neuromuscular franchise is a key driver towards future growth

\*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



**Neuromuscular expansion**  
apitegromab into additional indications

**SMA expansion** with Ph 2 OPAL trial for patients under 2 and subcutaneous formulation

**Global expansion,**  
starting with Europe

**Commercial Launch\***

\*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**<sup>®</sup>  
semaglutide injection 2.4 mg

*Lilly*

once weekly  
**zepbound**<sup>™</sup>  
(tirzepatide) injection 0.5 mL  
2.5 mg | 5 mg | 7.5 mg | 10 mg | 12.5 mg | 15 mg

novo nordisk<sup>®</sup>

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.

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# 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**.*

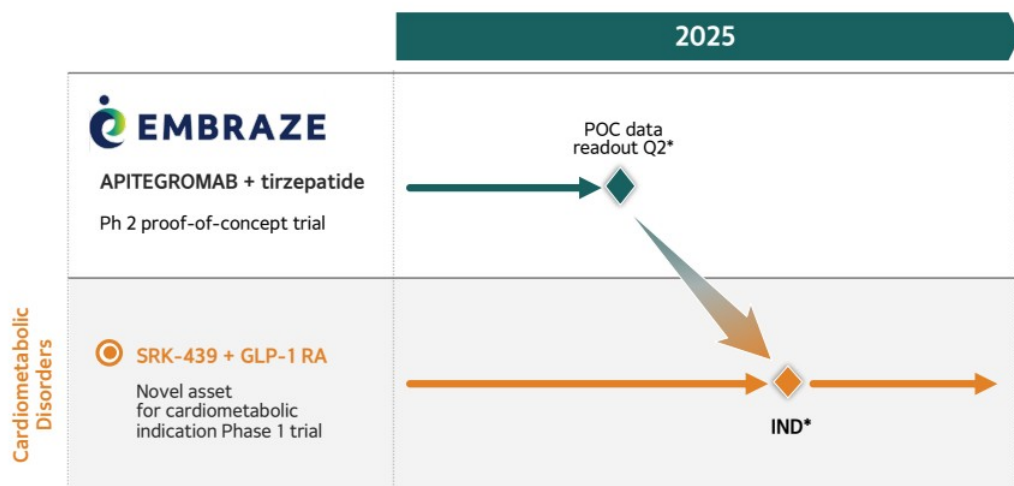
Obesity Clinician

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.

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



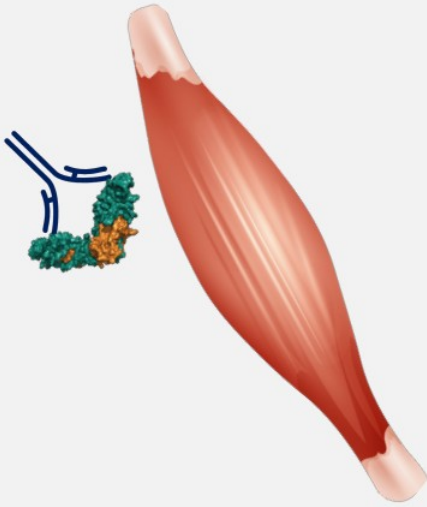
Testing hypothesis of selective anti-myostatin antibody in obese population

- 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

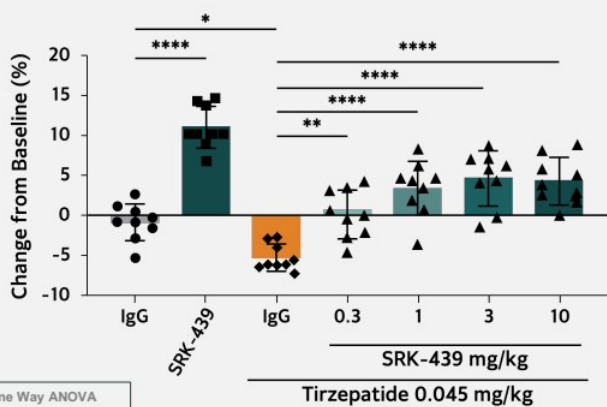
American Diabetes Association

obesityweek

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

## qNMR % Lean Mass Change from Baseline

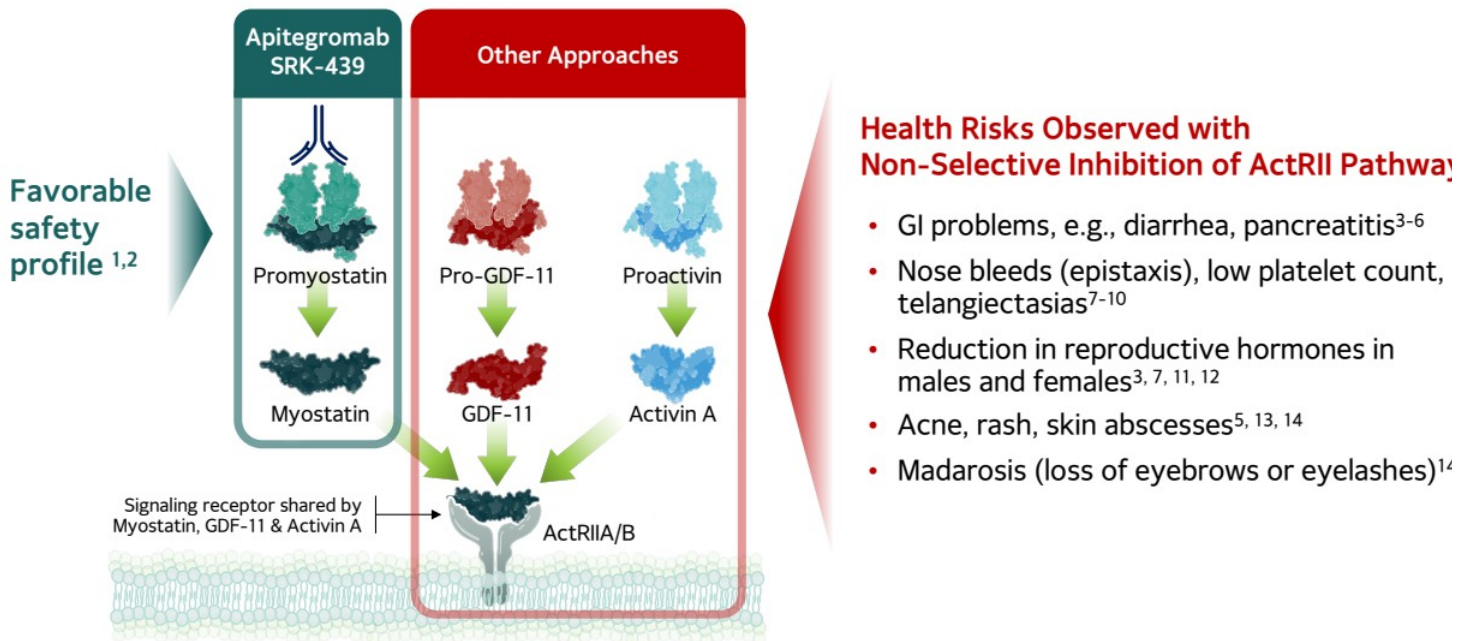


## Key Observations

- 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

DIO=Diet-induced obesity.

# Potential to Optimize Benefit-Risk with Myostatin Selectivity

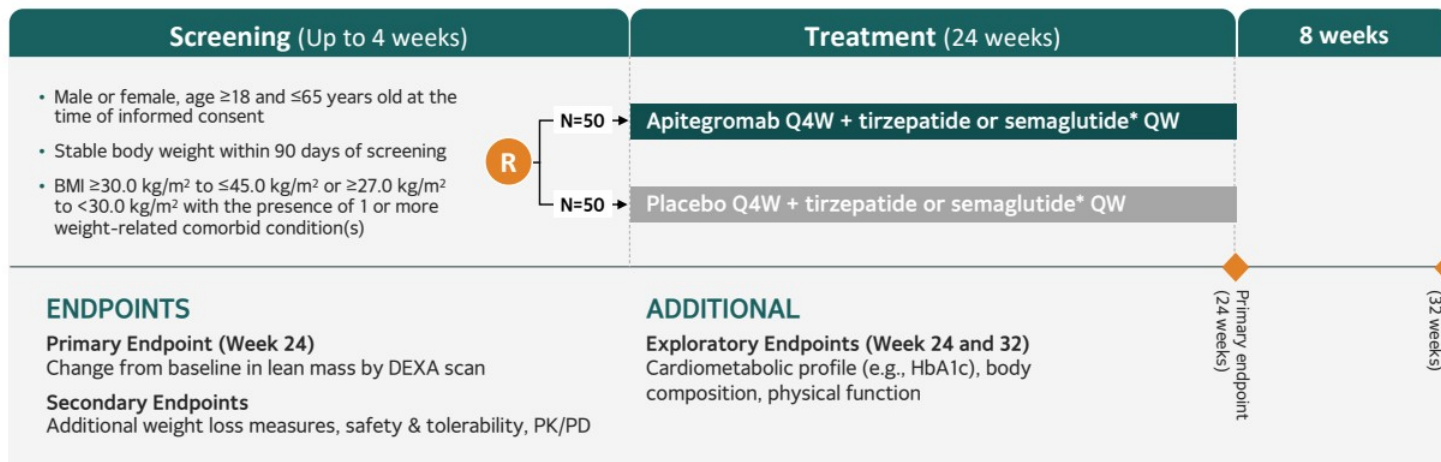


1. Barrett et al. *Adv Therapy*. 2021; 2. Crawford T et al. *Neurology*. 2024; 3. Garito T et al. *Clin Endocrinol (Oxf)*. 2018; 4. Amato AA et al. *Neurology*. 2021; 5. Heymsfield SB et al. *JAMA*. 2021; 6. Vanhoutte F et al. *J Clin Pharmacol*. 2020; 7. Attie KM et al. *Muscle Nerve*. 2013; 8. Attie KM et al. *Am J Hematol*. 2014; 9. Campbell C et al. *Muscle Nerve*. 2017; 10. Hoepfer 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

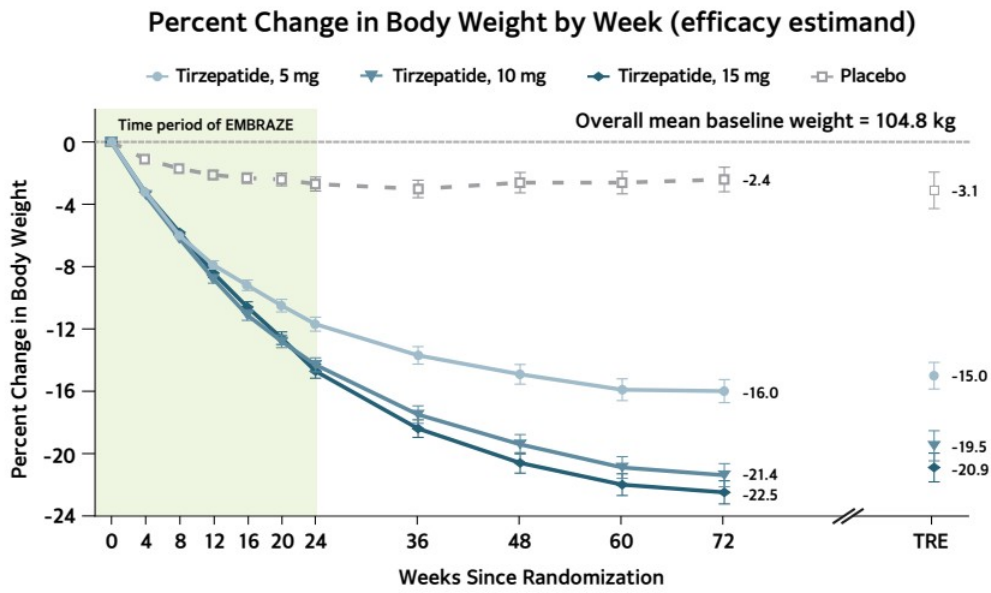
INSIGHTS GAINED  
FROM EMBRAZE STUDY  
to inform SRK-439  
development

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

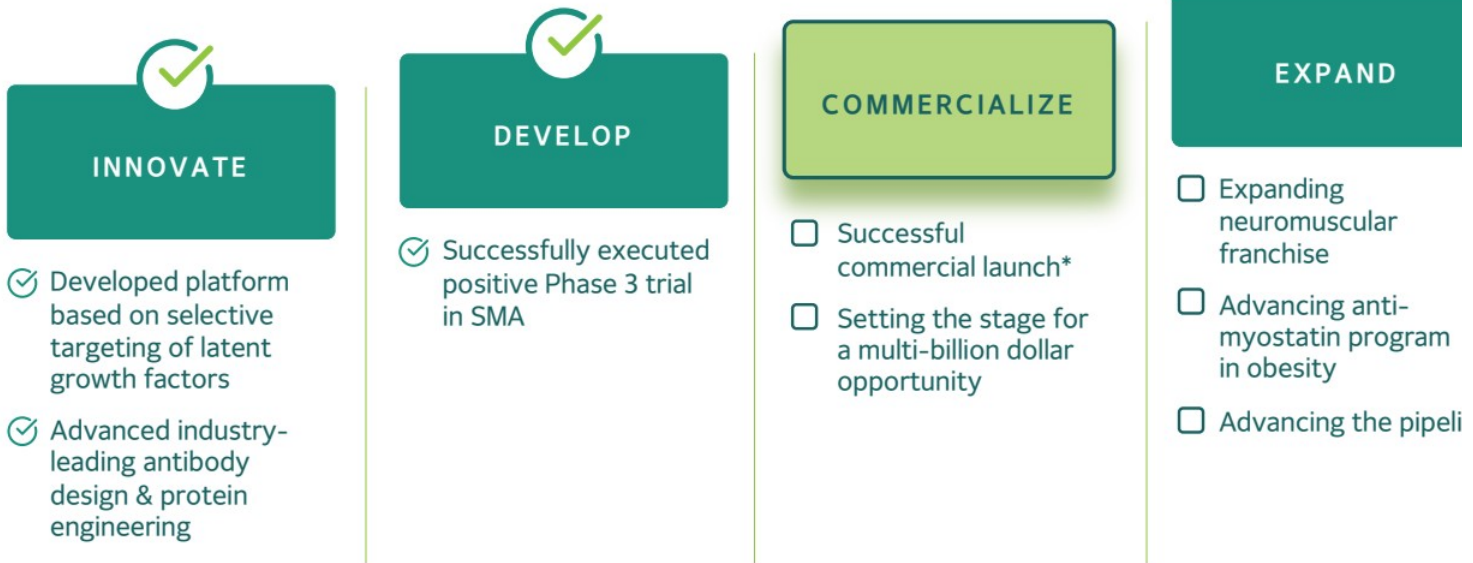
Figure from Jastreboff, A.M. et al. *N Engl J Med.* 2022;387(3):205-216.  
 \* Data from Phase 3 clinical trial of tirzepatide in adults with obesity.



## Conclusion

# Delivering on the Mission of Bringing Transformative Medicines to Patients

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



\* 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!