Fourth Quarter 2024 Business Update

February 27, 2025



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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 and other product candidates and indication selection and development timing, its cash runway, its ability to use its expanded credit facility, 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, 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, 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 is an investigational drug candidates under evaluation. Apitegromab 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 SRK-439 have not been established.

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Agenda

TOPIC	SPEAKER
Apitegromab in SMA SAPPHIRE Phase 3 Pivotal Trial Results and Regulatory Milestones	**Jay Backstrom,** President & Chief Executive Officer
Apitegromab in SMA Planning for a 2025 Launch to Serve Patients	**Tracey Sacco,** Chief Commercial Officer
Scholar Rock Innovative Myostatin Platform Ambition to Transform Current GLP-1 Treatment Paradigm	**Jay Backstrom,** President & Chief Executive Officer
Q&A Session	

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Strong Momentum in 2024 Sets the Stage for a Transformative Year Ahead

RECENT ACCOMPLISHMENTS



- **Positive Phase 3** SAPPHIRE data
- **BLA Submitted** for apitegromab in SMA
- **SAPPHIRE data and DMD poster** accepted for presentation at MDA

2025 ANTICIPATED MILESTONES

2025

	Q1	Q2	Q3	Q3	Q4
SMA	**MAA submission** apitegromab		**OPAL** study initiation		**US launch** apitegromab*
Cardiometabolic		**EMBRAZE Ph2 POC** data readout		**SRK-439 IND filing**	

* Assuming priority review granted and regulatory approval received.

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Positive Phase 3 Trial Using HFMSE, Gold Standard SMA Scale

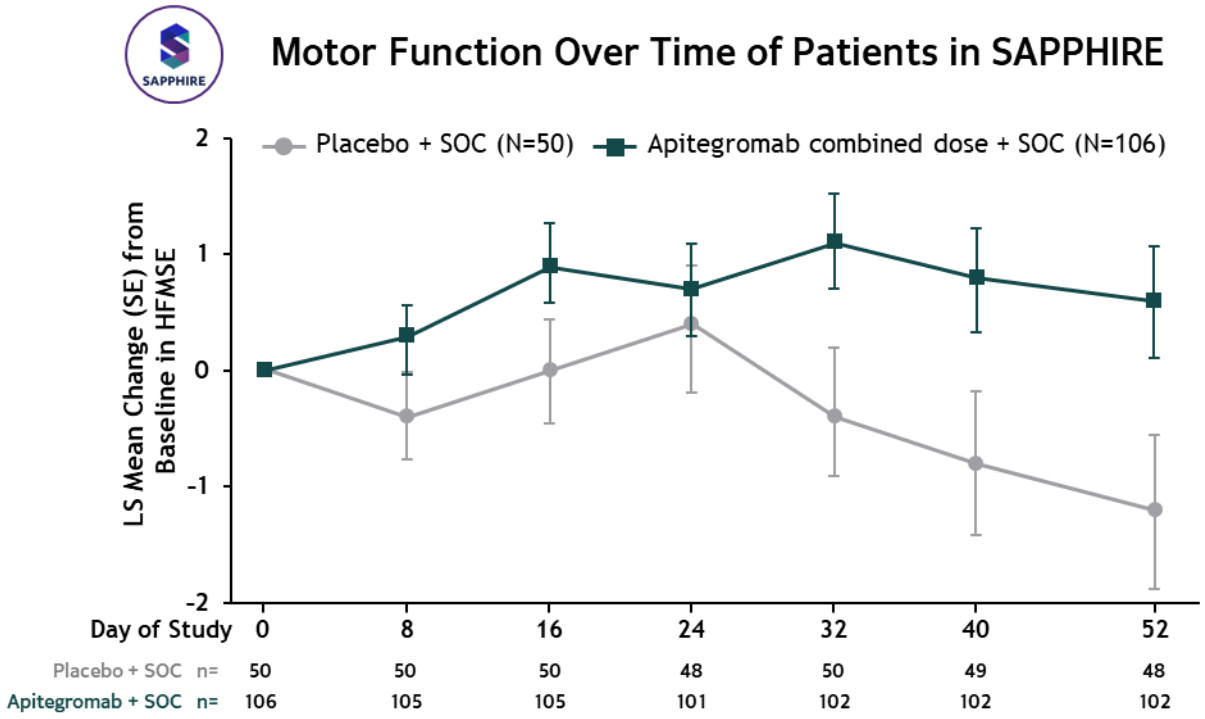
The Only Muscle-Targeted Therapy with Over 4 Years of Clinical Experience in >200 Patients

1.8
POINT IMPROVEMENT (p=0.0192)
in HFMSE* vs. placebo

CONSISTENT
clinically meaningful benefit observed across **all age groups (2–21)**

30% vs. 12.5%
ACHIEVED ≥3
POINT IMPROVEMENT IN HFMSE†
apitegromab + SOC patients vs. placebo + SOC

FAVORABLE SAFETY
profile consistent with >48 months experience in Phase 2 TOPAZ trial



Apitegromab has the potential to transform the standard of care in Spinal Muscular Atrophy (SMA)

* 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
Apitegromab is subject to regulatory approval.
SOC=Standard of care (i.e., nusinersen or risdiplam); HFMSE=Hammersmith Functional Motor Scale-Expanded.

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Apitegromab in SMA: Planning for a 2025 Launch to Serve Patients



Tracey Sacco, MBA
Chief Commercial Officer

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Progressive, Debilitating Muscle Atrophy Remains a Critical Unmet Need in SMA Despite SMN-Targeted Therapies



97%
of patients

seek improving muscle strength from a new treatment in SMA[1]



>80% of neurologists agree

efforts to preserve muscle should start as early as possible in SMA[2]

> *Personal hygiene, using the toilet and the shower on my own would be huge. My four-year-old can do it on her own. It's degrading.*
>
> US Patient

> *Muscle atrophy and loss of strength is a key issue in these patients. Increasing a patients' HFMSE score is really important. It's measurable and meaningful.*
>
> Pediatric Neurologist (UK)

The SMA community is calling for
a treatment to address progressive muscle weakness

1. Cure SMA State of SMA 2023 Report. https://www.curesma.org/wp-content/uploads/2024/06/9042024_State-of-SMA_vWeb.pdf; 2. Scholar Rock Internal Market Research (US Neurologists), 2024.

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The Future of SMA is in Treating the Muscle and the Motor Neuron

SMA Today

IN THE US



~10,000
living with SMA[1]



2/3
SMA patients received an SMN therapy[1]

WORLDWIDE



~35,000
patients have received an **approved SMN-targeted therapy**[2,3,4]

74% of neurologists
agree that in the future, **multiple modalities are necessary** to treat SMA[5]

> *"Many researchers believe that it will take a combination of SMN-dependent and SMN-independent treatments to provide the most benefit for those with SMA"*
> – Cure SMA[6]

1. Cure SMA State of SMA 2023 Report. https://www.curesma.org/wp-content/uploads/2024/06/9042024_State-of-SMA_vWeb.pdf; 2. Biogen Q4 2023 Report; 3. Roche Q3 2024 report; 4. Novartis Q4 2024 Report;5. Scholar Rock internal market research (US Neurologists), 2024; 6. Cure SMA, 2025. Approaches to Drug Development. https://www.curesma.org/approaches-to-drug-development/

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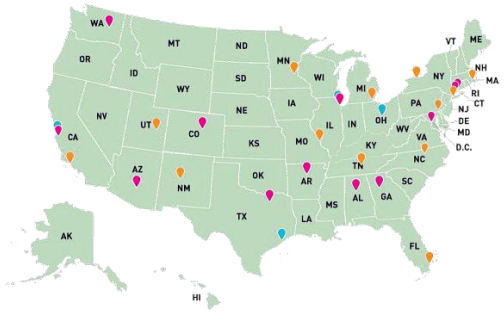
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Preparing the Market for a Potential New Treatment Paradigm in SMA



Disease Education

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



HCP Engagement

- MSL team engaged 100% of Cure SMA-identified sites
- Key HCPs, including doctors, nurses, PTs and OTs engaged since SAPPHIRE topline



Patient Advocacy Partnership

- Long-tenured relationship and strong partnerships across initiatives on education and access

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Significant Rare Disease Experience to Drive Successful Apitegromab Launch

INTERNAL COMMERCIAL EXPERTISE

Across market access, marketing, sales, and operations

SEASONED COMMERCIAL LEADERSHIP

Commercial leadership team averaging 20+ years of industry experience

RARE DISEASE LAUNCH EXPERIENCE

Among Commercial, Medical Affairs, and Patient Advocacy teams

ATTRACTING TOP TALENT

To build world-class commercial organization



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Scholar Rock is Positioned for a Successful Launch in SMA

Preparing **patient services** and **home infusion** offering for a Q4 launch*

Building a customer-facing team of sales, reimbursement, and patient support personnel in Q2/Q3

Initiating payer **outreach** to top US commercial and federal payers in Q1

Ongoing disease **education** and SMA stakeholder **engagement**

* Assuming priority review granted and regulatory approval received.

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Scholar Rock Innovative Myostatin Platform: Ambition to Transform Current GLP-1 Treatment Paradigm

Jay Backstrom, M.D., MPH



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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

novo nordisk®

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

Lilly

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 patients after stopping GLP-1 RA treatment, with fat regain > lean mass

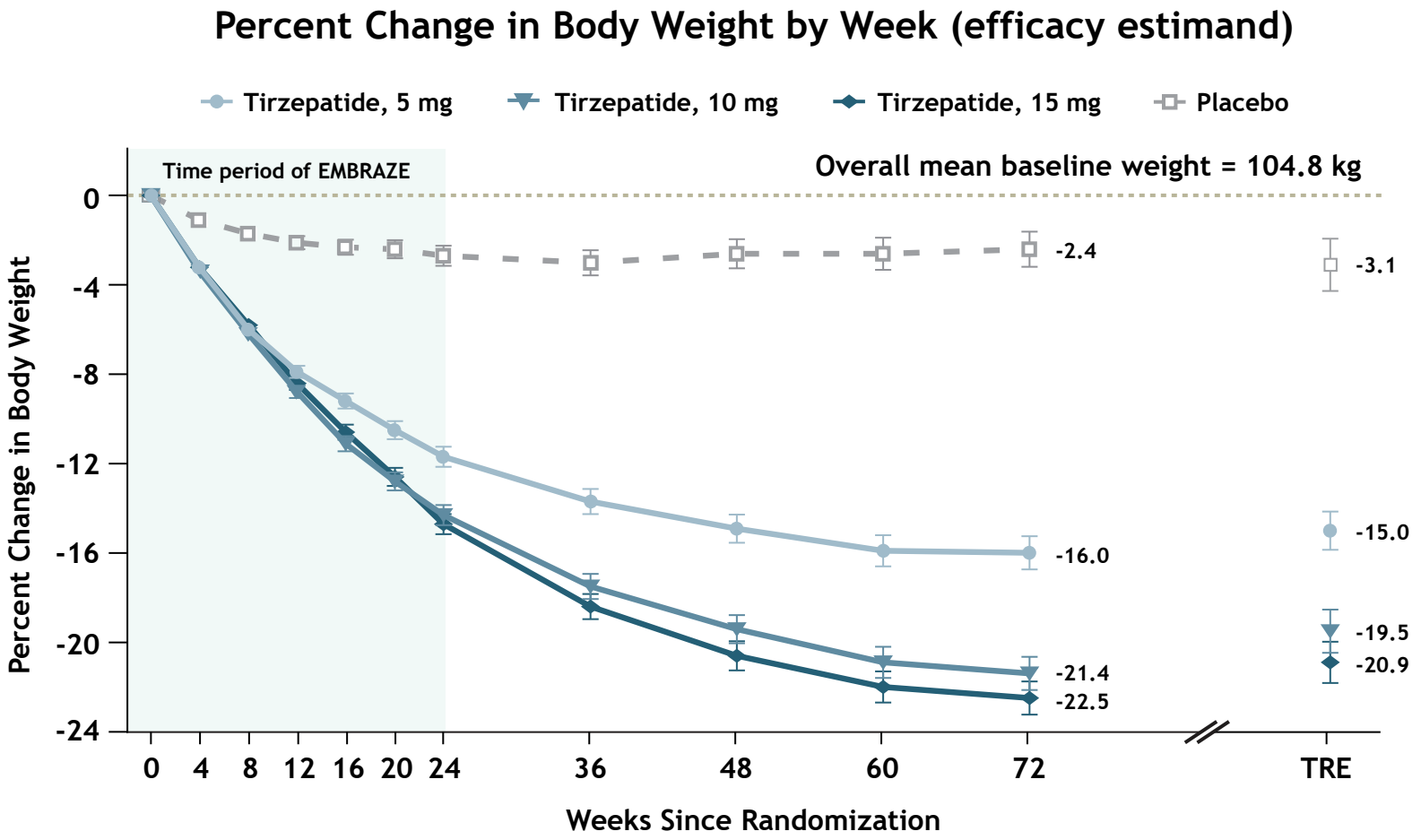
> *Patients feel good about the number on the scale, but there are issues with muscle loss*
>
> *… they complain of weakness or reduced strength.*
>
> Obesity Clinician

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.

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Significant Muscle Loss Accompanies Tirzepatide-Induced Weight Loss



Tirzepatide

DECREASE 14-16% **body weight** (week 24)

~25% of weight lost is lean mass

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.

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Every Kilogram Matters: Significant Cost of Losing Lean Mass

Loss of ~1 kg lean mass resulted in
decreased leg strength and increased insulin resistance*

- **~7% decrease** in leg strength
- **~6% reduction** in exercise capacity
- **~30% decrease** in insulin sensitivity

LEAN MASS
Loss of ~1% per year^

Adults >60 (M>F)

STRENGTH
Loss of 10–15% per decade^

*Marlou L Dirks, Benjamin T Wall, et al; Diabetes 2016; 65:2862-2875

^Mitchell W.K, Williams J, et al Front. Physiol. 2012; 3: 260 | Gustafsson T and Ulfhake B; Int. J. Molecular Sciences. 2024; 25: 1-16 | Siparsky P.N., Kirkendall D.T et al. Sports Health 2024; 6 (1): 36-40 | Macaluso, A. and De Vito, G. Eur. J. Appl. Physiol. 2004; 91(4):450-472.

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Goals of the EMBRAZE Proof-of-Concept Study

EMBRAZE



PRESERVE LEAN MASS



COMPARABLE WEIGHT LOSS

REDUCE FAT REGAIN



COMPARABLE SAFETY
(no added toxicities)

Exploratory: Potential to improve
METABOLIC PROFILE (e.g., HbA1c)
and PHYSICAL FUNCTION

INFORMING THE DEVELOPMENT OF SRK-439

Highly selective muscle-targeted therapy in obesity

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SRK-439: Best in Class Potential

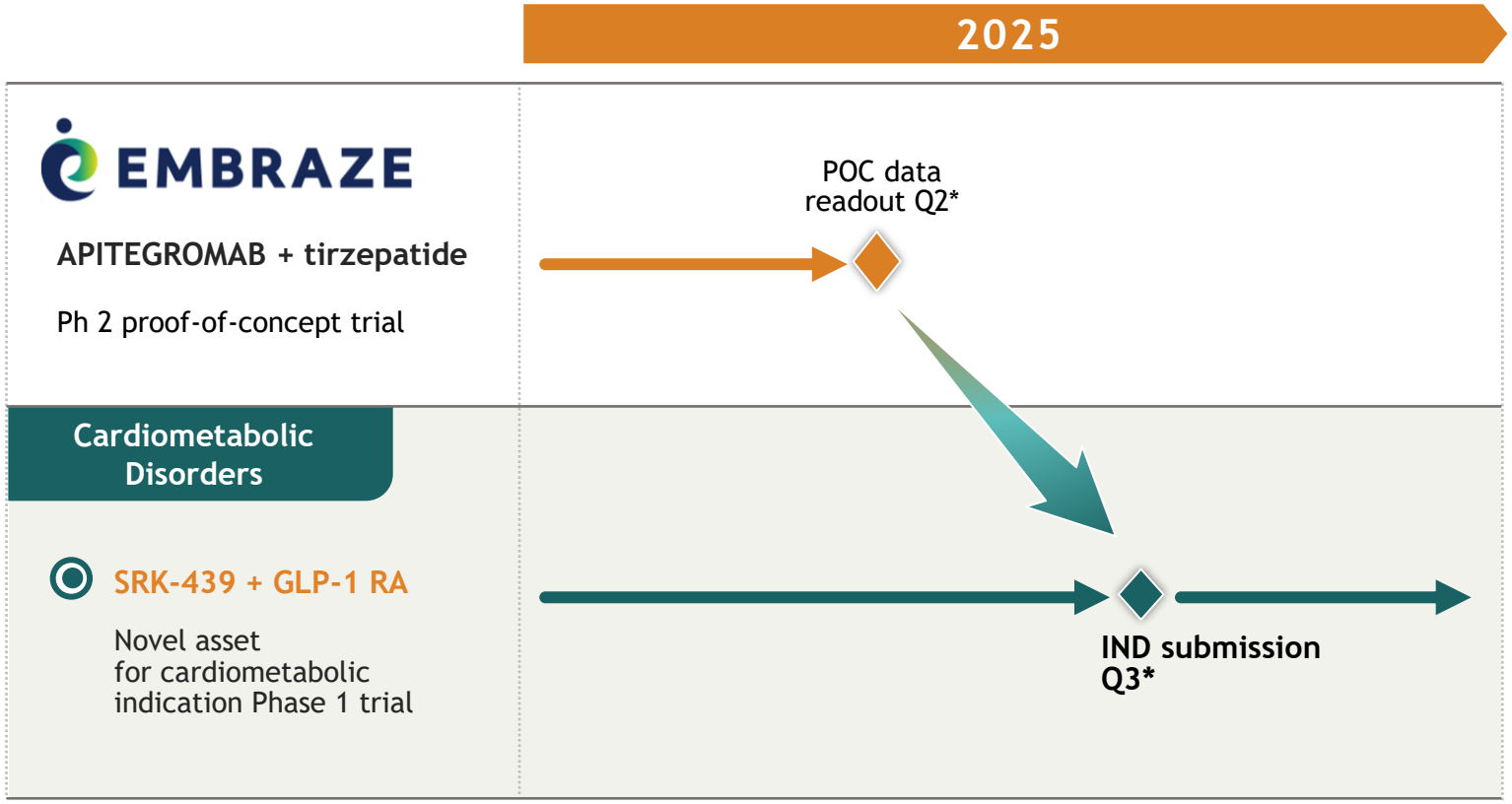
	SRK-439	ActRII Ab	Ligand Trap	Adnectin
Selectivity for myostatin	✓	✗	✗	✗
Action limited to muscle	✓	✗	✗	✗
Combination GLP-1 RA data in obesity preclinical models[1-3]	✓	✓	✓	✓
Low efficacious dose in preclinical obesity models[1-3]	✓	✗	✗	✗
Potential lower risk of undesirable effects in clinic[4-17]	✓	✗	✗	✗

GLP-1 RA: GLP1 receptor agonist

1. Nunn E, et al., Mol Metab 2024; 2. Schang G., et al, J. Endoc Soc 2023; 3. Ackerman, P, et al. Obesity Week 2023 Poster 211;
4. 1. Barrett et al. *Adv Therapy.* 2021; 5. Crawford T et al. *Neurology.* 2024; 6. Garito T et al. *Clin Endocrinol (Oxf).* 2018; 7. Amato AA et al. *Neurology.* 2021; 8. Heymsfield SB et al. *JAMA.* 2021; 9. Vanhoutte F et al. *J Clin Pharmacol.* 2020; 10. Attie KM et al. *Muscle Nerve.* 2013; 11. Attie KM et al. *Am J Hematol.* 2014; 12. Campbell C et al. *Muscle Nerve.* 2017; 13. Hoeper MM et al. *N Engl J Med.* 2023; 14. Ruckle J et al. *J Bone Miner Res.* 2009; 15. Sherman ML et al. *J Clin Pharmacol.* 2013; 16. Muntoni F et al. *Neurol Ther.* 2024. 17. Di Rocco M et al. *Nat Med.* 2023.

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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.

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Conclusions



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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*

2 EXPAND
Apitegromab Development Program: Building a Pipeline in a Product

3 ADVANCE
Anti-myostatin Program into Cardiometabolic Indications



BLA submitted
for apitegromab in SMA and potential US launch in 4Q 2025



MAA on track
for 1Q 2025 submission



$2B+ opportunity in **SMA**



Earlier treatment in SMA
on track to initiate OPAL study for patients under 2 years of age with SMA in 3Q 2025



Reaching more patients
for apitegromab in additional rare neuromuscular diseases



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.

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Q&A

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Q&A: Scholar Rock Management



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



Ted Myles, MBA
Chief Operating Officer and Chief Financial Officer



Mo Qatanani, PhD
Chief Scientific Officer



Tracey Sacco, MBA
Chief Commercial Officer

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