Dedicated to Dramatically Improving the Lives of Children and Adults with SMA

Q2 2025 BUSINESS UPDATE

August 6, 2025

Q2 EARNINGS CALL

TOPIC	SPEAKER
SCHOLAR ROCK NEXT PHASE OF GROWTH	**David L. Hallal** Chairman and Chief Executive Officer
R&D PROGRESS	**Akshay Vaishnaw**, M.D., Ph.D President of R&D
COMMERCIAL READINESS	**Keith Woods** Chief Operating Officer
COMPANY FINANCIALS	**Vikas Sinha** Chief Financial Officer
Q&A SESSION	

Forward-Looking Statements

 This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding Scholar Rock's future expectations, plans and prospects, including without limitation, Scholar Rock's expectations regarding its growth, strategy, progress and timing of its clinical trials for apitegromab and its preclinical programs, including SRK- 439, and indication selection and development timing, including the timing of any regulatory submissions and anticipated approvals, the therapeutic potential, clinical benefits and safety of any product candidatesits cash runway, expectations regarding commercial launch timing in the US and in Europe, expectations regarding the achievement of important milestones, 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," "might," "could," "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. 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, whether preclinical and clinical data, including the results from the Phase 3 SAPPHIRE trial will be sufficient to support regulatory approval, that the full results from the Phase 3 SAPPHIRE trial may differ from the topline data, that preclinical and clinical data, including the results from the Phase 2 or Phase 3 clinical 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 candidates; Scholar Rock's ability to manage expenses or provide the financial support, resources and expertise necessary to identify and develop product candidates on the expected timeline; 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; and Scholar Rock's dependence on third parties for development and manufacture of product candidates including, without limitation, to supply any clinical trials as well as those risks more fully discussed in the section entitled "Risk Factors" in Scholar Rock's Quarterly Report on Form 10-Q for the quarter ended June 30, 2025, 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.

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 and SRK-439 have not been established.

SCHOLAR ROCK
NEXT PHASE OF GROWTH

David L. Hallal

Chairman and
Chief Executive Officer

2025 Priorities Support Long-Term Growth

Apitegromab in SMA Regulatory Approvals & Commercialization

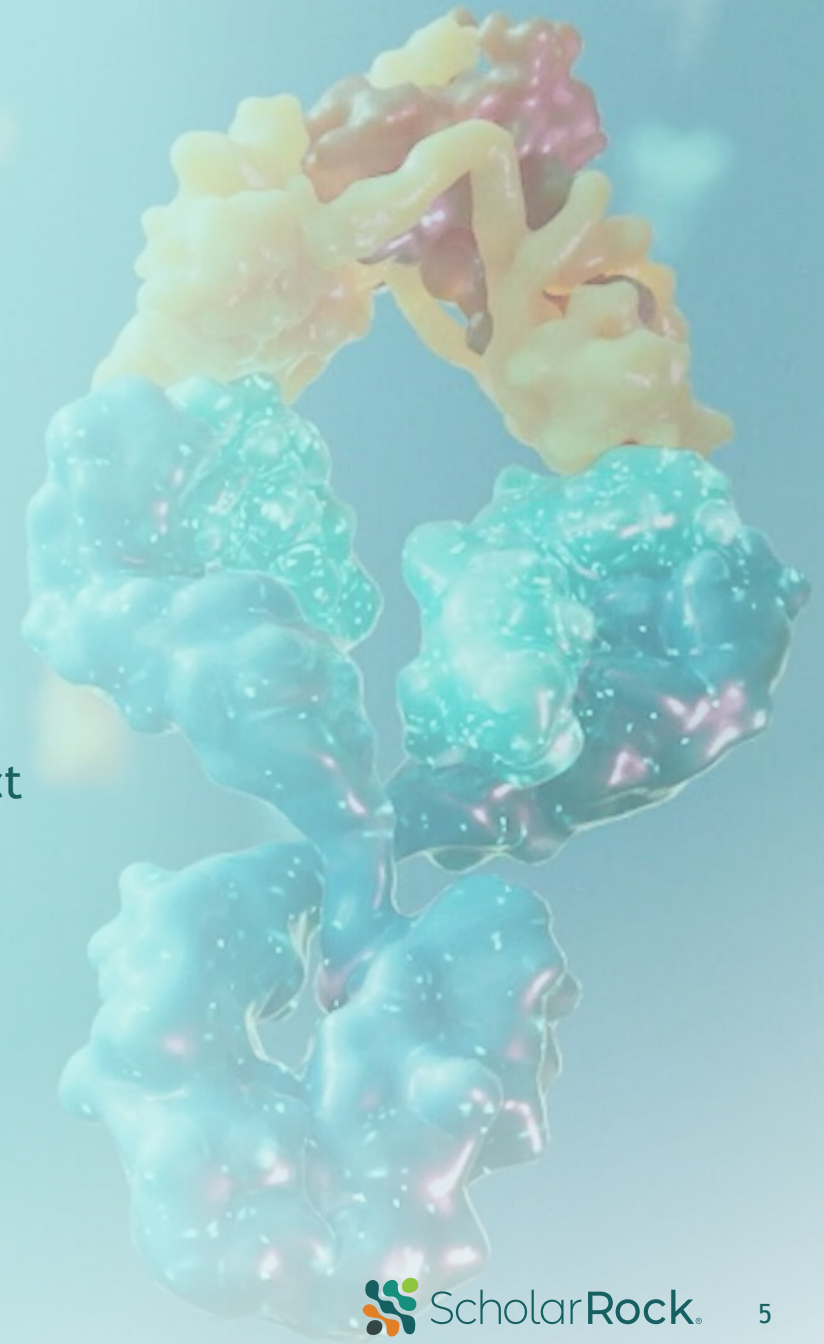
- Execute Successful US Commercial Launch*
- Advance EU Launch Preparedness

Expand into Additional Rare, Severe & Debilitating Neuromuscular Diseases

- **Apitegromab Development Program:** Building a Pipeline in a Product
- Leverage Highly Innovative Anti-myostatin Platform

Disciplined Capital Allocation

- Efficient Commercial Build
- Phase Investments to Support Future High-value Commercial & Pipeline Initiatives



*Pending regulatory approval

Progress at a Glance: Our Key Milestones

SPINAL MUSCULAR ATROPHY

- **FDA Target Action Date**
  Sept 22, 2025
- **MAA Validated**
  Anticipated 2026 approval
- **Positive SAPPHIRE Data** Presented at Cure SMA Research Meeting

PIPELINE

- **EMBRAZE Achieved Primary Endpoint**
  Positive Ph 2 proof-of-concept trial in obesity
- **File IND for SRK-439 in 2H 2025**
- **Initiate OPAL Trial in Q3**
  In infants & toddlers with SMA

CORPORATE

- **Experienced Team**
  Powering growth & accelerating transformation
- **Commercial Buildout**
  Customer-facing team deployed in the U.S.
- **$295 Million Cash**
  as of June 30, 2025

Pending regulatory approval

Apitegromab in SMA

Fueling Scholar Rock’s long-term sustainable growth

R&D PROGRESS

Akshay Vaishnaw, M.D., Ph.D

President of R&D

Positive Phase 3 Trial with Gold Standard Hammersmith Functional Motor Scale-Expanded in SMA

1.8
POINT HFMSE IMPROVEMENT vs placebo*; (n=156)

30% vs 12.5%
of apitegromab patients **ACHIEVED ≥3PT IMPROVEMENT IN HFMSE†** compared to placebo

CONSISTENT
clinically meaningful benefit (1.8 points) observed across **ALL AGE GROUPS, 2-21; (n=188)**

FAVORABLE SAFETY
profile consistent with >48 months experience in **PHASE 2 TOPAZ TRIAL**

Apitegromab has the potential to transform the lives of children and adults with SMA

*Based on apitegromab combined dose (10 mg/kg and 20 mg/kg; n=106) + SOC versus placebo + SOC (n=50) (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.

FDA Accepted BLA Under Priority Review – Target Action Date September 22

Potential clinical benefits of apitegromab, as demonstrated by our Phase 3 trial, are underscored by the FDA's priority review designation

By definition, a priority review designation by the FDA conveys the capacity of apitegromab to potentially impact unmet need in SMA by:

- Being a treatment for a serious or life-threatening condition.
- Providing a significant improvement in safety or effectiveness over existing treatments.

Expanding Our Impact: Initiating Phase 2 OPAL Trial in Q3

Studying apitegromab in patients under 2 years old

ADDRESSING THE NEEDS OF INFANTS & TODDLERS WITH SMA

Potential to alter the course of SMA in children under 2

EXPANDING OUR IMPACT

Including evaluation of apitegromab in patients who received gene therapy

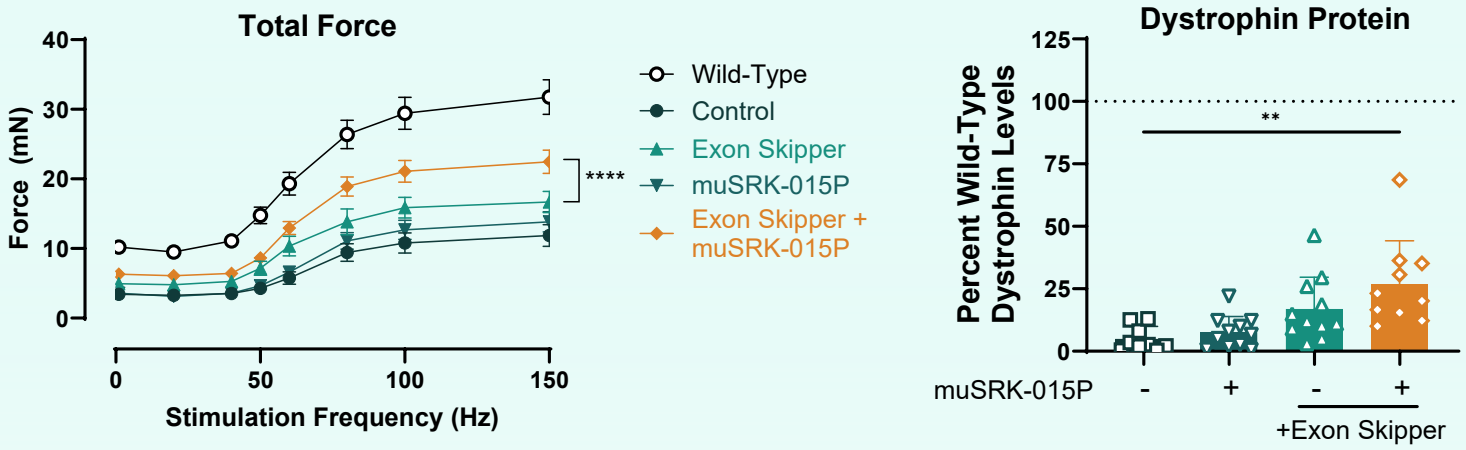
TIME IS MUSCLE

Reaching patients earlier

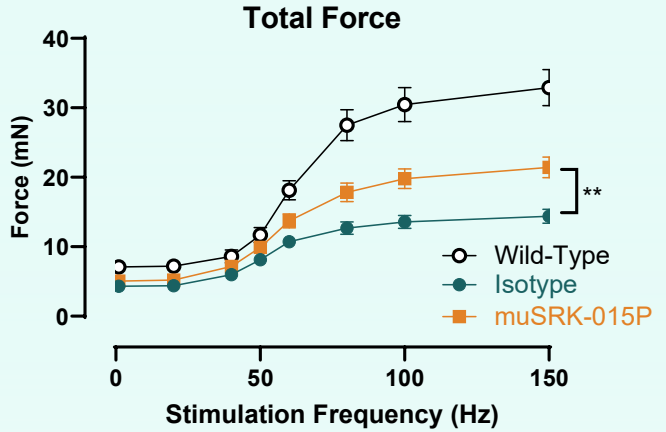
Apitegromab: Pipeline in a Product

Potential to address additional rare, severe and debilitating neuromuscular disorders

MuSRK-015P Increased Muscle Force and Dystrophin Protein in Combination With an Exon Skipper in a Mouse Model of DMD[1]



MuSRK-015P Increased Muscle Force in a Model of FSHD as Stand-Alone Therapy[2]



Preclinical data presented at Muscular Dystrophy Association (MDA) Clinical and Scientific Conference 2025

- muSRK-015P treatment increased muscle size as a single agent and in combination with exon skipper (data not shown)
- Combined with an exon skipper, muSRK-015P further enhanced total muscle force and augmented dystrophin expression induced by the exon skipper

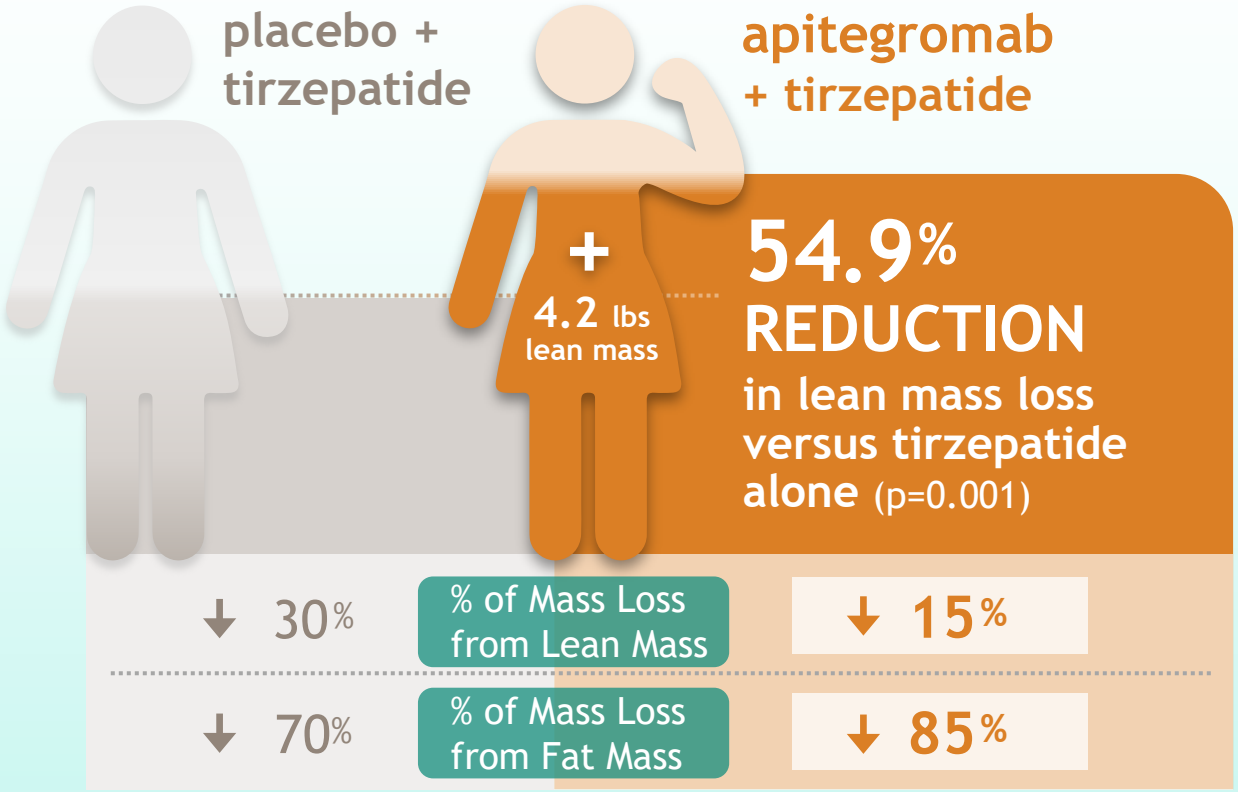
Preclinical data presented at FSHD Society International Research Congress 2025

- muSRK-015P treatment increased muscle size (data not shown)
- Treatment led to increase in total force and enhanced endurance (data not shown)

muSRK-015P used in these studies is a murine version of apitegromab.

1. Fogel, A., et al. "muSRK-015 builds muscle mass and strength in combination with dystrophin upregulation in a mouse model of DMD." Presented at Muscular Dystrophy Association (MDA) Clinical & Scientific Conference, March 2025. 2. Fogel, A., et al. "SRK-015 improves muscle mass, strength, and endurance in the FLExDUX4.Cre mouse model of FSHD." Presented at FSHD Society International Research Congress, June 2025.

EMBRAZE Proof-of-Concept Study Achieved Goals



Combining **apitegromab 10 mg/kg** with **tirzepatide** over 24 weeks

HIGHER QUALITY OF WEIGHT LOSS OBSERVED

Apitegromab was generally well tolerated and consistent with safety profile observed in other clinical trials

Growing Our Innovative Anti-myostatin Platform: SRK-439

OPTIMIZED FOR SUBCUTANEOUS ADMINISTRATION

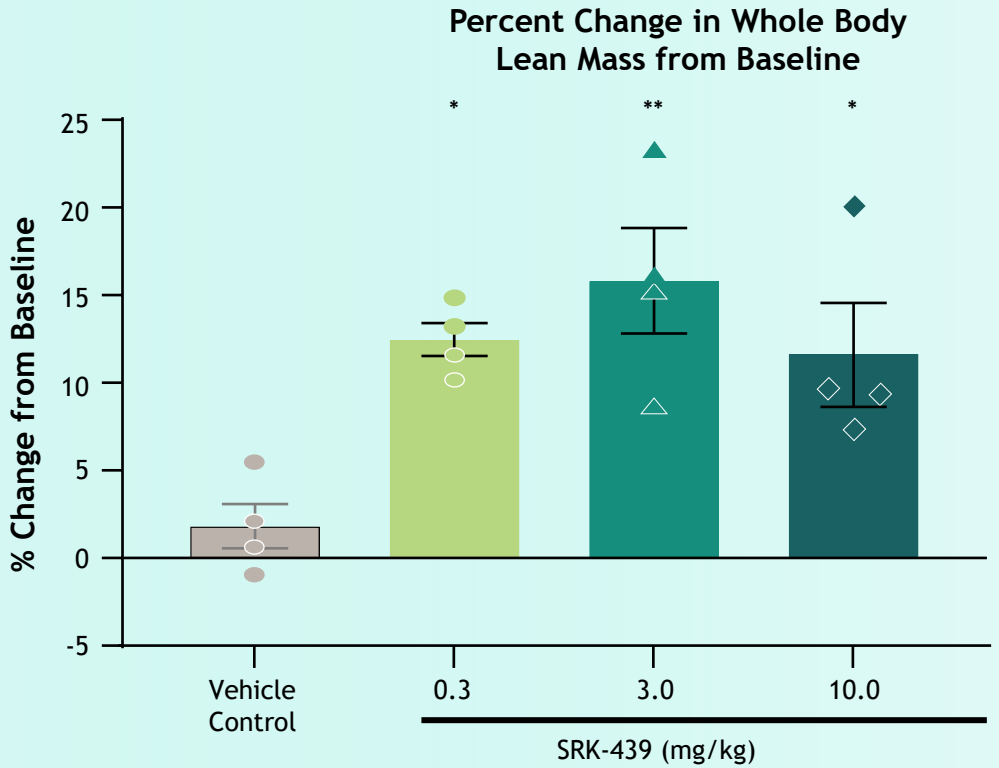
Novel, highly selective myostatin inhibitor

STRONG SCIENTIFIC VALIDATION

Preclinical data demonstrated favorable muscle mass preservation

ADVANCING TO CLINIC

On track to file the IND application for SRK-439 in 2H 2025



Remain Focused On Delivering R&D Priorities

1. **Advance toward anticipated US approval of apitegromab in Q3 2025; anticipated EU approval in 2026**
2. **Initiate Ph 2 OPAL Trial in Q3 2025**
3. **File IND for SRK-439 in 2H 2025**
4. **Complete clinical development plans:** apitegromab in additional rare, severe, and debilitating neuromuscular disorders

COMMERCIAL READINESS

Keith Woods

Chief Operating Officer

Ushering in a New Treatment Era for SMA

Preparing for a global launch of apitegromab in SMA*

SMA is a progressive and devastating disease that leads to loss of mobility, limited activities of daily living, and lack of independence [1]

Apitegromab is the first and only muscle-targeted treatment to show clinically meaningful and statistically significant motor function improvement in SMA.

*Pending regulatory approvals 1. Cure SMA State of SMA 2024 Report. https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_vWeb.pdf
Apitegromab is an investigational drug candidate under evaluation and has not been approved by any regulatory agency.

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

90% **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]

The SMA community is calling for **a treatment to address progressive muscle degeneration** and motor function loss

"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 patient's HFMSE score is really important. It's measurable and meaningful.
– PEDIATRIC NEUROLOGIST (UK)

1. Cure SMA State of SMA 2024 Report. https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_vWeb.pdf; 2. Scholar Rock Internal Market Research (US Neurologists), 2024.

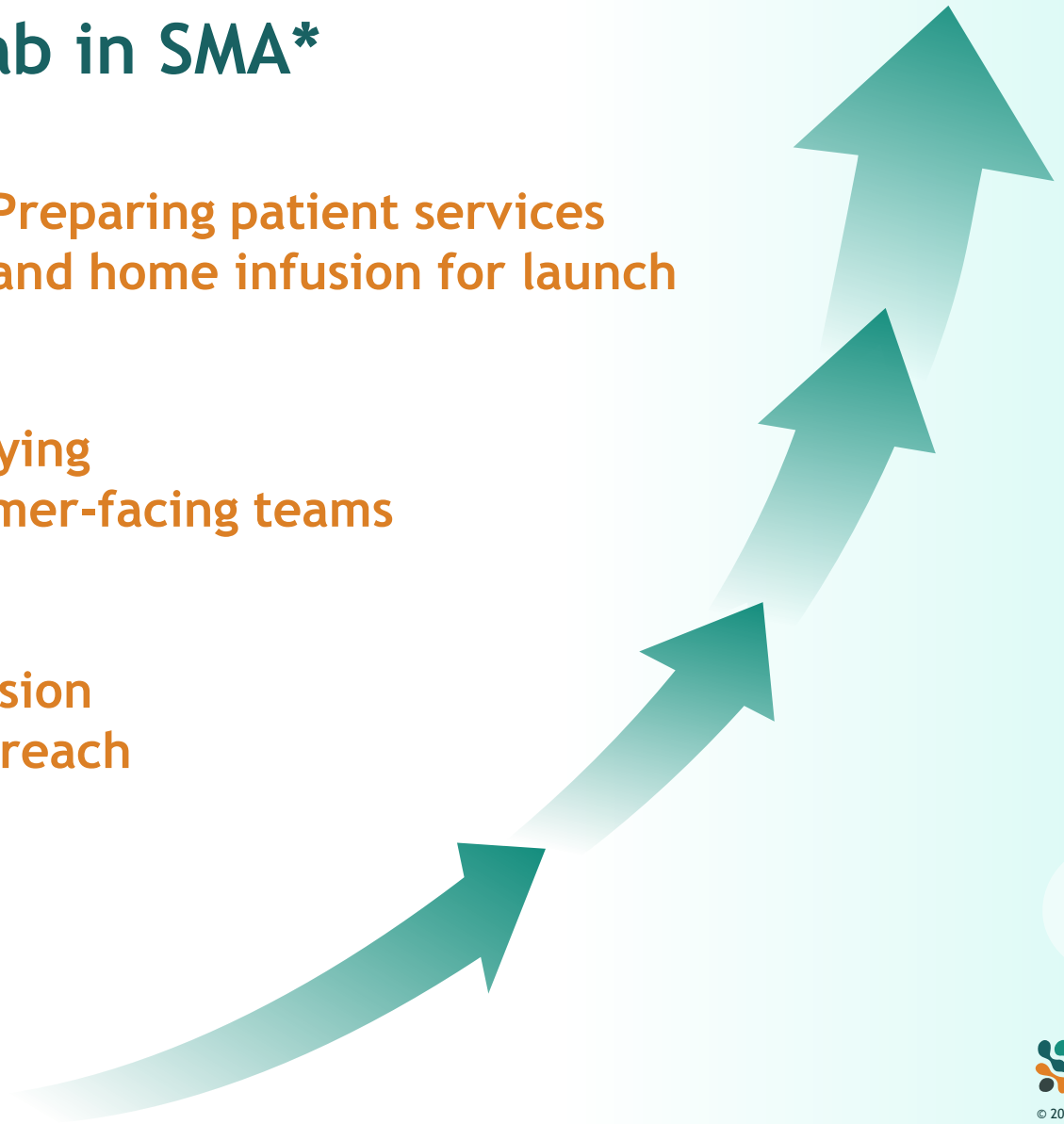
Scholar Rock is Positioned for a Successful U.S. Launch of Apitegromab in SMA*

Preparing patient services and home infusion for launch

Deploying customer-facing teams

Continued expansion of U.S. payer outreach in Q2/Q3

Ongoing disease education and SMA stakeholder engagement



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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/

Scholar Rock FINANCIALS

Vikas Sinha

Chief Financial Officer

Strong Cash Position Supports Launch & Additional Priorities

1 Financial Discipline

2 Capital Efficient Commercial Build

3 Disciplined Allocation to Advance Pipeline

2025 PRIORITIES

- US launch expected in 2025 and EU launch in 2026
- SMA: <2 years old study initiation planned for Q3 2025
- Expand in additional rare, severe, and debilitating neuromuscular indications with apitegromab
- SRK-439 IND filing planned for 2H 2025

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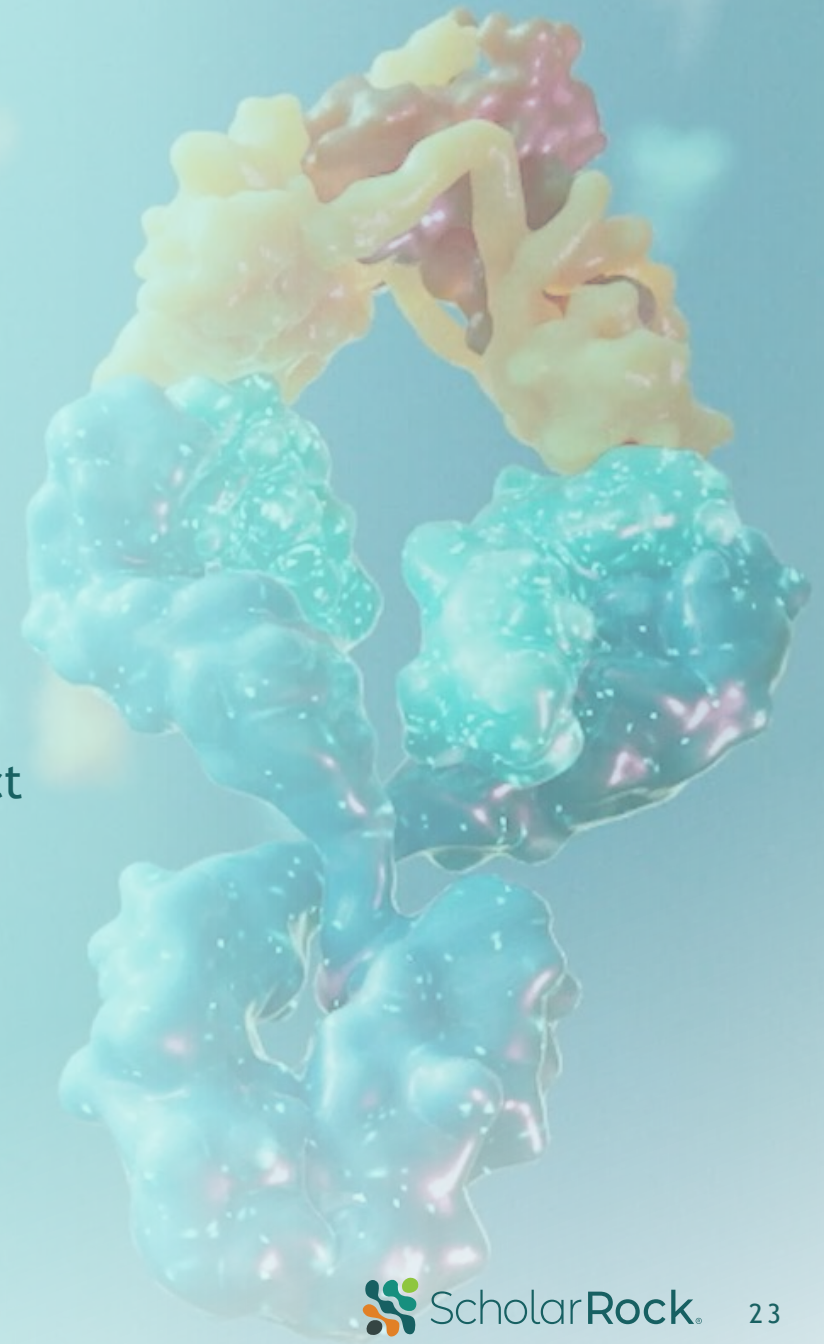

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Q&A