Dedicated to Dramatically Improving the Lives of Patients with SMA



Q1 2025 Business Update
May 14, 2025

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Q1 Earnings Call

TOPIC	SPEAKER
Scholar Rock Next Phase of Growth	**David L. Hallal** 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	

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Forward-Looking Statements

This presentations 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, the therapeutic potential, clinical benefits and safety of any product candidates, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, its cash runway, expectations relating to 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, that preclinical and clinical data, including whether 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, or Part A or Part B of the Phase 1 clinical trial of SRK-181, 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 March 31, 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.

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Scholar Rock
Next Phase of Growth

David L. Hallal
Chief Executive Officer



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Strong Q1: Advances Scholar Rock Toward Apitegromab US Commercial Launch & Series of Country Launches in Years Ahead

SPINAL MUSCULAR ATROPHY



- **Detailed Positive Phase 3 SAPPHIRE data**
  Presented at MDA
- **BLA Granted Priority Review**
  PDUFA date September 22
- **MAA Validated**
  anticipated 2026 approval

PIPELINE



- **Apitegromab in DMD**
  Preclinical data presented at MDA
- **Exploratory Ph 2 EMBRAZE POC Study in Obesity**
  Readout on track – June

CORPORATE

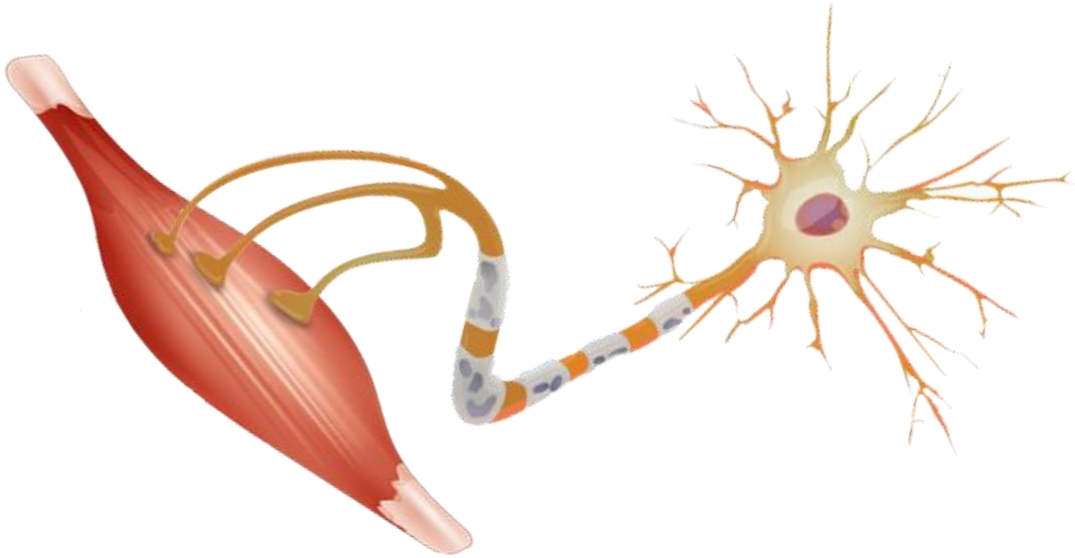


- **Seasoned leadership team**
  for next phase of growth
- **Commercial buildout**
  continues to progress
- **$364.4 million cash**
  as of March 31, 2025

Pending regulatory approval

Advancing Apitegromab for Patients and Families with SMA

Apitegromab in SMA alone is opportunity
to build sustainable growth through the end of this decade and into the next



Commercial launch anticipated in SMA

Global expansion, starting w/ Europe, LATAM, APAC & beyond

SMA expansion with Ph 2 OPAL trial for patients <2 years old

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R&D Progress

Akshay Vaishnaw, M.D., Ph.D
President of R&D



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Positive Phase 3 Trial with Gold Standard Hammersmith Motor Function Scale in SMA

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%

of apitegromab patients **ACHIEVED ≥3PT IMPROVEMENT IN HFMSE†** compared to placebo

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) + 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.

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FDA Accepted BLA Under Priority Review (PDUFA 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 either:

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

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Growing Our Innovative Anti-myostatin Platform: SRK-439



1 | Optimized for Subcutaneous Formulation

Novel, highly selective myostatin inhibitor



2 | Strong Scientific Validation

Preclinical data demonstrated favorable muscle mass preservation



3 | Advancing to Clinic

On track to file the IND application for SRK-439 in Q3

SRK-439 is a novel, investigational, preclinical myostatin inhibitor

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Goals of the Exploratory EMBRAZE Proof-of-Concept Study



PRESERVE LEAN MASS



COMPARABLE WEIGHT LOSS

REDUCE FAT REGAIN



COMPARABLE SAFETY
(no added toxicities)

EMBRAZE

Exploratory program to generate insights

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Remain Focused & On Track to Deliver R&D Priorities

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

Commercial Readiness

Keith Woods
Chief Operating Officer

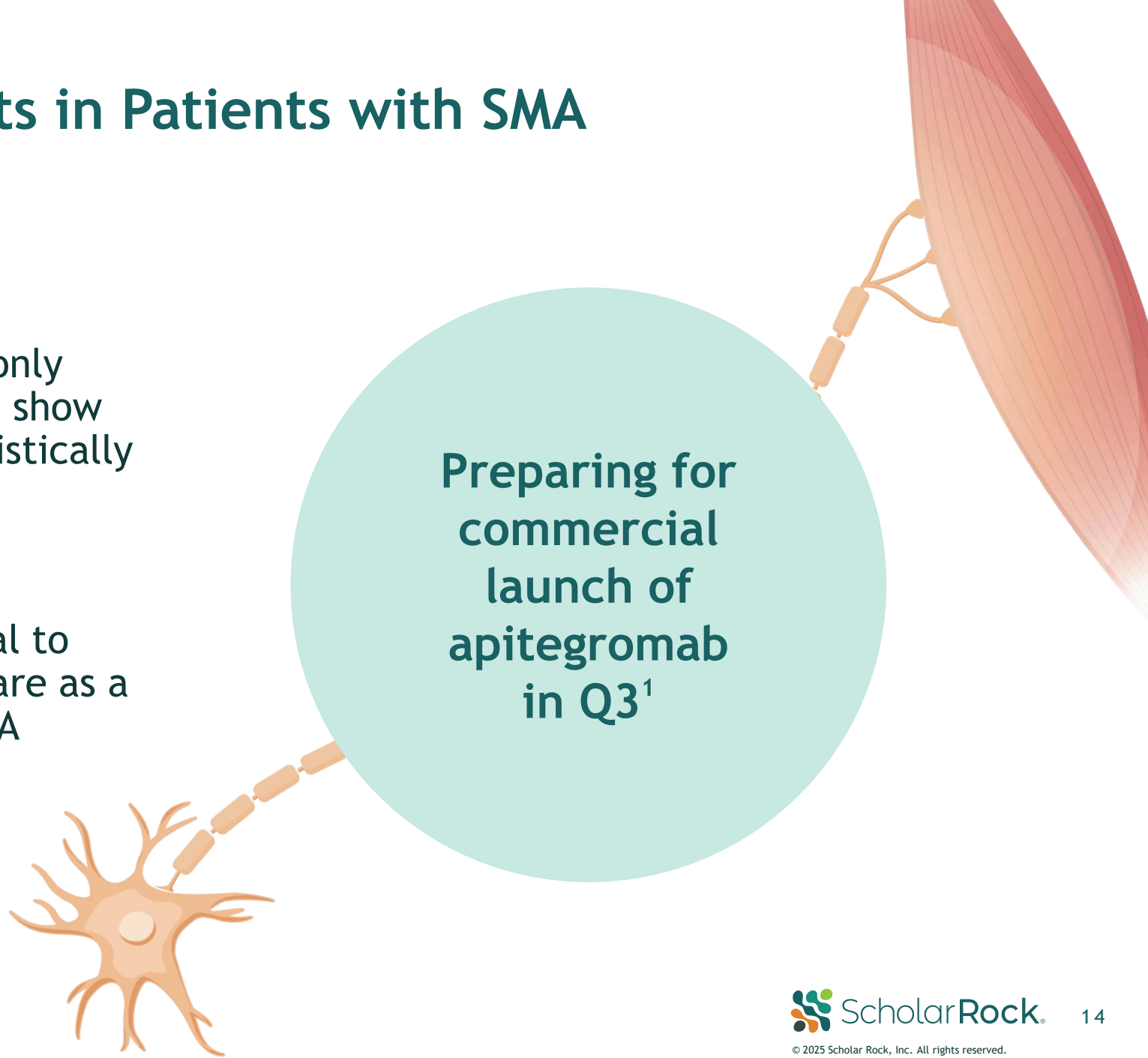


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Compelling Phase 3 Results in Patients with SMA

- **Apitegromab** is the first and only muscle-targeted treatment to show clinically meaningful and statistically significant motor function improvement in SMA.
- **Apitegromab** has the potential to establish a new standard of care as a first-in-class, best-in-class SMA therapy.

Preparing for commercial launch of apitegromab in Q3[1]



1 Pending regulatory approval

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

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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]

> *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 degeneration and motor function loss

1. Cure SMA State of SMA 2024 Reporthttps://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_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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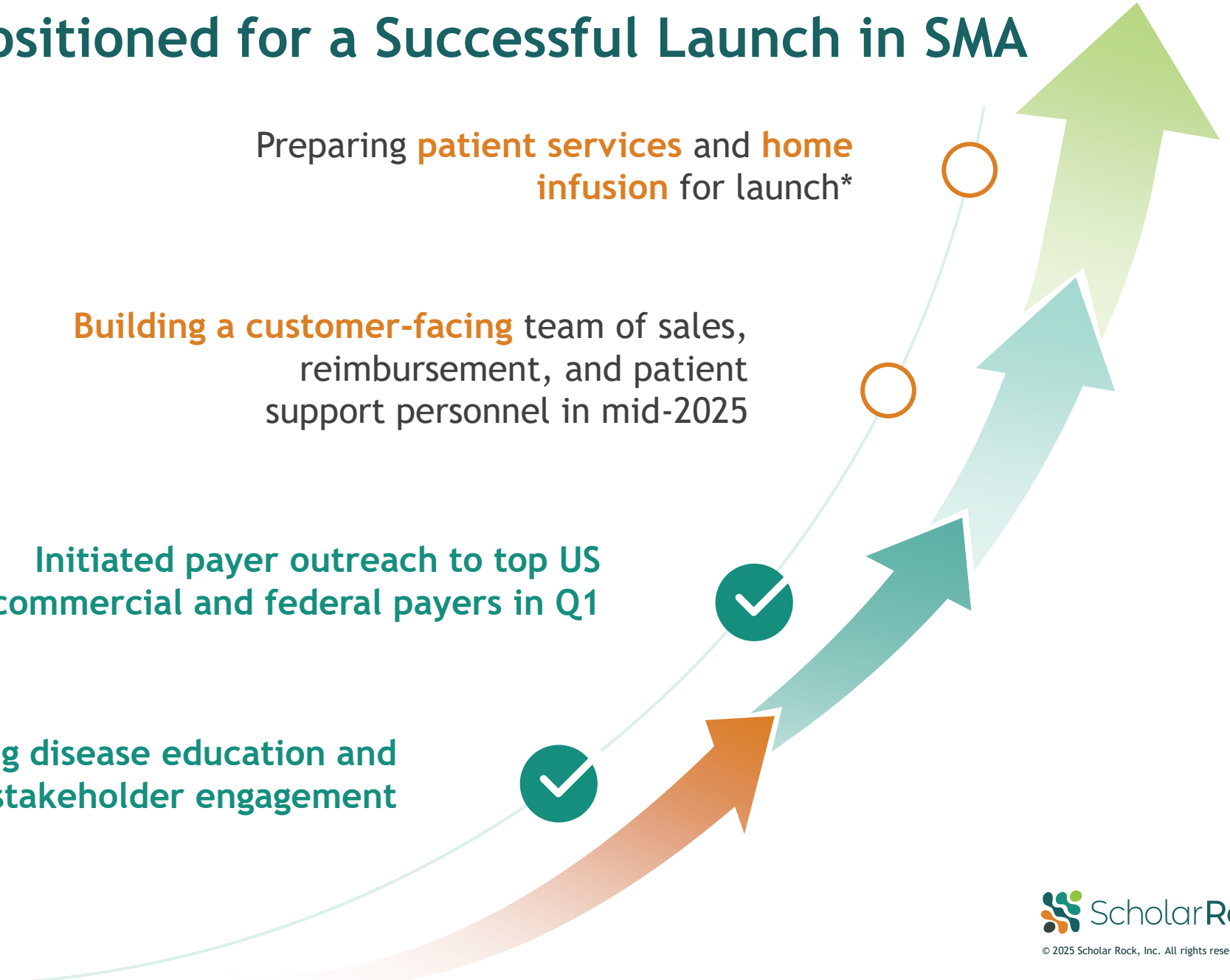
Scholar Rock is Positioned for a Successful Launch in SMA

Preparing **patient services** and **home infusion** for launch*

Building a customer-facing team of sales, reimbursement, and patient support personnel in mid-2025

Initiated payer outreach to top US commercial and federal payers in Q1

Ongoing disease education and SMA stakeholder engagement



* Pending regulatory approval.

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Scholar Rock Financials

Vikas Sinha
Chief Financial Officer



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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
- Exploring additional neuromuscular indications with apitegromab
- SRK-439 IND filing planned for Q3 2025

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Delivering on 2025 Priorities Supports Long-Term Growth

1	2	3
Regulatory Approvals & Commercialization	**Expand in Additional Rare, Severe & Debilitating Neuromuscular Diseases**	**Disciplined Capital Allocation**
Execute Successful US Commercial Launch*	Apitegromab Development Program: Building a Pipeline in a Product	Efficient Commercial Build
Advance EU Launch Preparedness	Leverage Highly Innovative Anti-myostatin Platform	Phase Investments to Support Future High-value Commercial & Pipeline Initiatives

*Pending regulatory approval

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Q&A

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