Q1 2026 Earnings Call

MAY 7, 2026

ScholarRock®

Q1 2026 EARNINGS CALL

	TOPIC	SPEAKER
1	BUSINESS UPDATE	David L. Hallal Chairman & Chief Executive Officer
2	R&D PROGRESS	Akshay Vaishnaw, M.D., Ph.D. President of R&D
3	COMMERCIAL READINESS	Keith Woods Chief Operating Officer
4	COMPANY FINANCIALS	Vikas Sinha Chief Financial Officer
5	Q&A SESSION	

ScholarRock.

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 growth, strategy, progress and timing of its clinical trials and development programs for apitegromab, including its subcutaneous formulation, SRK-439 and its preclinical programs, and indication selection and development timing, including the timing of any regulatory submissions, decisions and anticipated approvals, the therapeutic potential, clinical benefits and safety of any product candidates, its ability to address the observations identified in the complete response letter, expectations regarding actions by the FDA after its reinspection of the Catalent Indiana facility; the expected timing and outcome of FDA review of the accepted BLA for apitegromab; expectations regarding the availability and timing of commercial supply of apitegromab from third-party U.S.-based fill-finish facility, including expected supply from the second fill-finish facility; expectations regarding commercial launch timing, and 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,” “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 2 or Phase 3 clinical trial of apitegromab, data from any ongoing or future trials of apitegromab or data for SRK-439, 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; whether the FDA will accept the remediations to the Catalent Indiana fill finish facility in response to the FDA Observations, whether the updated BLA will be sufficient to support regulatory approval, 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; 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 obtain additional funding when needed to support its business activities; 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 2026; 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, 2026, 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 presentation is as of the date of this presentation, 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-439 are 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 and SRK-439 have not been established.

Business Update

David L. Hallal
Chairman and Chief Executive Officer

Scholar Rock is positioned for a pivotal year ahead

Our top priority: Bringing apitegromab to children and adults living with SMA

Apitegromab BLA Accepted by FDA

Steady and rapid progress continues in collaboration with the FDA

PDUFA Action Date of September 30th

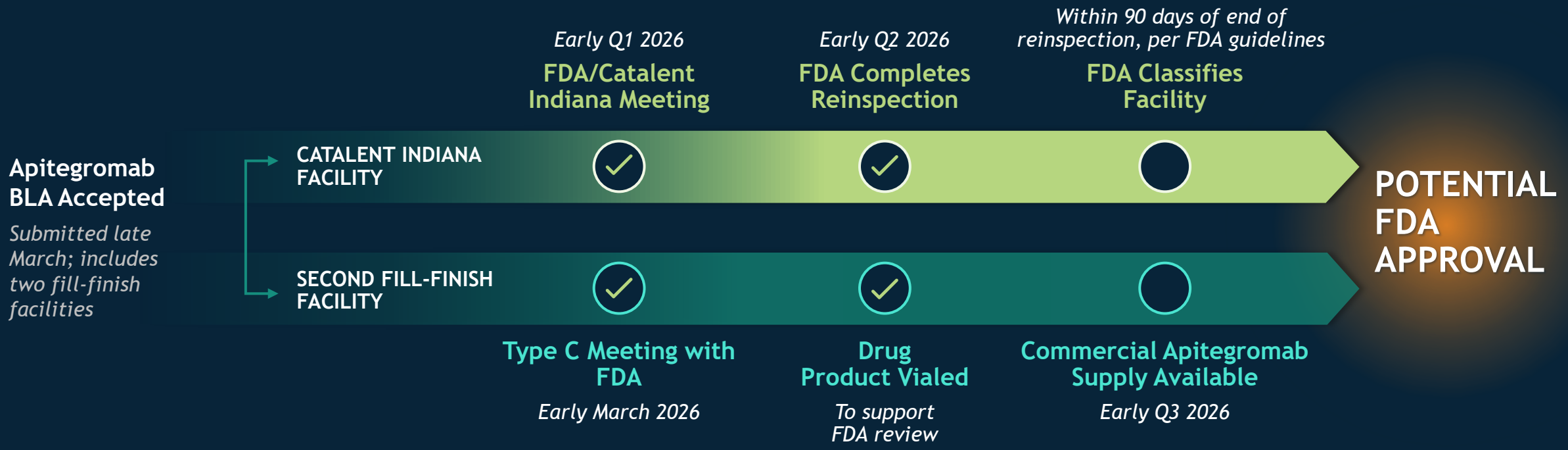
U.S. Commercial team is ready for launch immediately upon approval, which may be granted at any time through September 30th

Two Fill-Finish Facilities

Included in BLA resubmission, providing two independent paths to FDA approval

BLA, Biologics License Application; PDUFA, Prescription Drug User Fee Act.

Two independent paths to apitegromab FDA approval by September 30th PDUFA date



U.S. Commercial team is ready to launch apitegromab immediately upon FDA approval

BLA, Biologics License Application; PDUFA, Prescription Drug User Fee Act.

Scholar Rock is poised for a transformative year

SHAPING THE FUTURE OF TREATMENT FOR PATIENTS LIVING WITH RARE NEUROMUSCULAR DISEASES

APITEGROMAB FOR PATIENTS WITH SMA

1ST

- Myostatin inhibitor with a successful Phase 3 study
- Only muscle-targeted treatment to show a statistically significant, clinically meaningful benefit in SMA

2026
ON TRACK

- BLA resubmission accepted by FDA; PDUFA action date of September 30th
- CHMP opinion anticipated near mid-2026

GLOBALLY

- ~35,000 have received an SMN-targeted therapy[1-3]
- $2B+ opportunity to serve patients with SMA alone

UNDERWAY

PHASE 2 OPAL STUDY for apitegromab in infants and toddlers with SMA

ONGOING

SC APITEGROMAB development activities

MID-2026

INITIATE PHASE 2 STUDY for apitegromab in facioscapulohumeral muscular dystrophy

H2 2026

SRK-439 PHASE 1 STUDY topline data in healthy volunteers

$480M in cash and cash equivalents as of March 31, 2026

SMA, Spinal Muscular Atrophy; PDUFA, Prescription Drug User Fee Act; CHMP, Committee for Medicinal Products for Human Use; SC, Subcutaneous. 1. Biogen Q4 2023 Report; 2. Roche Q3 2024 report; 3. Novartis Q4 2024 Report.

R&D Progress

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

Advancing Scholar Rock's world-leading anti-myostatin pipeline

Program	Indication	Milestone
Apitegromab	Spinal Muscular Atrophy ≥2 YoA	PDUFA action date of September 30; CHMP opinion mid-2026
	Spinal Muscular Atrophy <2 YoA	Phase 2 OPAL study in infants and toddlers ongoing
	Facioscapulohumeral muscular dystrophy	Phase 2 FORGE study initiation in mid-2026
Subcutaneous Apitegromab	Spinal Muscular Atrophy	Development activities ongoing, with anticipated FDA and EMA regulatory engagements on clinical path forward*
SRK-439	Healthy Volunteers	Phase 1 study topline results expected in H2 2026

*Following FDA and EMA regulatory approval of IV apitegromab; YoA, Years of Age; PDUFA, Prescription Drug User Fee Act; CHMP, Committee for Medicinal Products for Human Use.

Furthering our commitment to broad SMA community

Ongoing Phase 2 OPAL study evaluating apitegromab in infants and toddlers with SMA

- Evaluating PK, PD, efficacy, safety, and tolerability of apitegromab over 48 weeks

Focused on addressing the needs of children <2 years of age with SMA
to reach patients earlier

Expanding our potential impact
including evaluation of apitegromab in patients who received SMN1-targeted gene therapy

Time is muscle
seeking to address the motor neuron and muscle in youngest patients

Patient enrollment and dosing underway in Phase 2 OPAL study

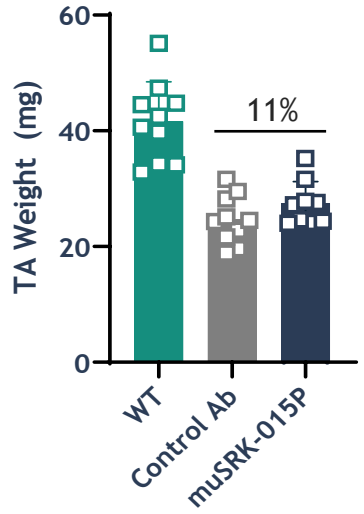
PK, Pharmacokinetic; PD, Pharmacodynamic.

FSHD: Rare, devastating NMD with significant unmet need

>30,000 patients diagnosed in U.S. and Europe[1-3]; no approved therapies

FSHD FLExDUX4 Mouse Model

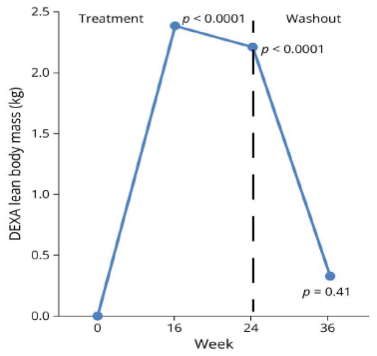
Robust Increase in Muscle Mass (28 Days)[4]



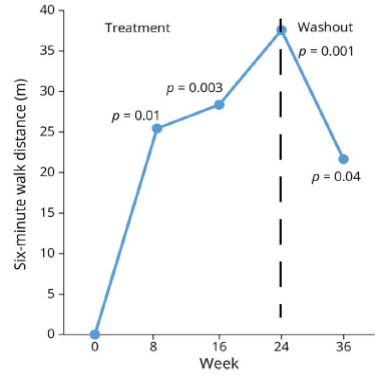
Support for apitegromab therapeutic hypothesis in FSHD

- Randomized studies of exercise programs suggest muscle has capacity to show functional benefit[5,6]
- Study of anabolic agents suggests increase in lean mass and muscle function[7]

Increase in Lean Body Mass



Improvement in 6MWD



Phase 2 FORGE study on track to initiate mid-2026

FLExD, FLExDUX4.Cre; FSHD, facioscapulohumeral muscular dystrophy; NMD, Neuromuscular Disease; WT, wild type; Ab, antibody; 6MWD, 6-minute walk distance. 1.Scholar Rock. Data on file. 2. Tihaya MS, et al. *Nat Rev Neurol*. 2023;19(2):91-108. 3. Attarian S, et al. *J Neurol*. 2024;271(9):5778-5803. 4. Fogel A. Presented at: FSHD Society International Research Congress; Jun 12-13, 2025; Amsterdam, Netherlands. 5. Bankole, LC, et al. *Medicine*. 2016; 95(31). 6. Andersen, G, et al. *Neurology*. 2015; 85:396-403. 7. Heatwole, CR, et al. *Neurol Genet*. 2025;11:e200292.

Advancing innovation with subcutaneous apitegromab and SRK-439

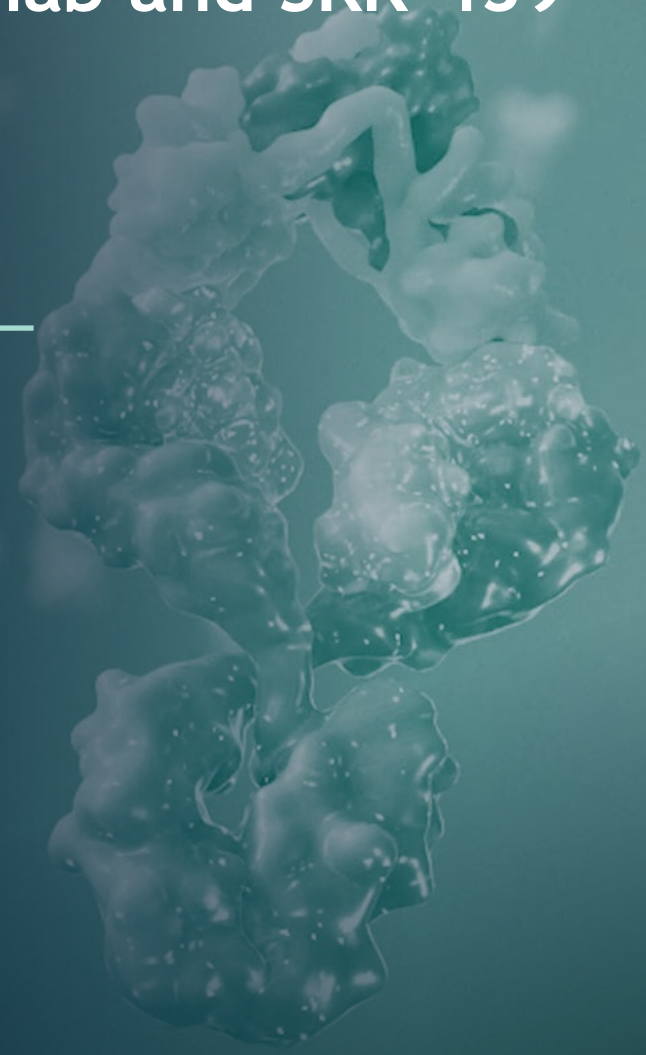
Leveraging world-leading expertise to drive continued innovation

Subcutaneous Apitegromab

- Phase 1 study comparing intravenous (IV) and subcutaneous (SC) apitegromab in healthy volunteers
- At 800mg, SC and IV apitegromab produced overlapping PD responses (total latent myostatin)
- Further development activities ongoing, including anticipated FDA and EMA regulatory engagements following apitegromab approvals

SRK-439

- Novel, highly potent myostatin inhibitor
- Optimized for subcutaneous administration
- Strong preclinical data demonstrating favorable muscle mass preservation
- Phase 1 study in healthy volunteers in progress; topline data expected in H2 2026



SRK-439 is a novel, investigational myostatin inhibitor.

Commercial Readiness

Keith Woods
Chief Operating Officer

Muscle strength and motor function remain the top unmet need in children and adults living with SMA

Scholar Rock's disease education program continues to focus on a broader understanding of SMA as a disease of the ***motor unit***—which consists of both the motor neuron ***and*** the muscle

95%

OF PATIENTS

continue to experience persistent and progressive muscle atrophy that limits function and independence

~1/3

OF PEOPLE LIVING WITH SMA IN THE U.S.

have received two or more FDA approved SMA treatments, either sequentially or in combination

SMA, Spinal Muscular Atrophy.

U.S commercial team operating with urgency to prepare for launch

SMA Treatment Centers, SMA Prescribing Physicians, Multi-Disciplinary Care Teams

TARGETING **140** SMA Centers

TARGETING **>2,600** SMA Prescribers

Patient Engagement & Community Activation

National & Regional Payers Medicare & Medicaid

SMA, Spinal Muscular Atrophy.

Strong momentum with apitegromab launch readiness in Europe in advance of mid-2026 CHMP opinion

1 Building world-class team

2 Engaging the SMA community

3 Establishing access

5th International Scientific Congress on Spinal Muscular Atrophy

SMA, Spinal Muscular Atrophy; CHMP, Committee for Medicinal Products for Human Use.

Global apitegromab opportunity in SMA alone offers potential for many years of sustainable growth

Powering Scholar Rock through the end of this decade and into the next

1. Biogen Q4 2023 Report; 2. Roche Q3 2024 report; 3. Novartis Q4 2024 Report. SMN, survival motor neuron.

Company Financials

Vikas Sinha

Chief Financial Officer

Operating with financial discipline to achieve our ambitions

$480M

in cash and equivalents

as of March 31, 2026

Prioritized investments focused on:

1. Apitegromab commercial launch readiness in the U.S. and Europe
2. Strengthening supply chain to support expanding pipeline and anticipated growing global commercial demand for apitegromab over time
3. Advancing highly innovative clinical programs

Closing Remarks

David L. Hallal
Chairman and Chief Executive Officer

Q&A

ScholarRock®

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