Scholar Rock Corporate Presentation

July 2026

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, cash runway, 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.

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.

Apitegromab poised to usher in next phase of innovation in SMA

Targeting muscle—the principal organ affected in SMA

INITIAL PHASE OF INNOVATION: MOTOR NEURON

2016 — SPINRAZA® (nusinersen) injection 12 mg/5 mL

2019 — zolgensma® (onasemnogene abeparvovec-xioi) suspension for intravenous infusion

2020 — Evrysdi® risdiplam

2025 — itvisma® (onasemnogene abeparvovec-brve) suspension for intrathecal injection

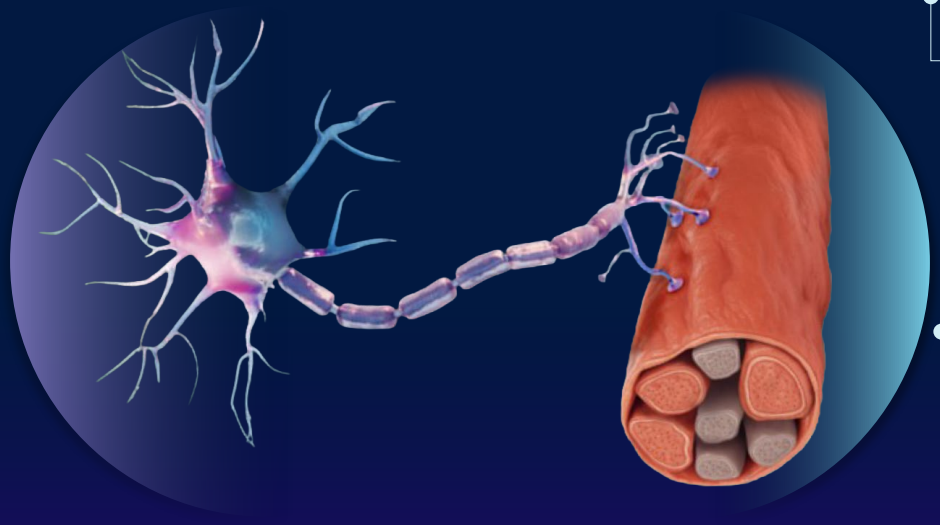
SMN-Targeted Therapies

NEXT PHASE OF INNOVATION: MUSCLE

2026 — 2030+

- **Apitegromab U.S. launch**
- **Apitegromab European launch**
- **Apitegromab global expansion**
- **Subcutaneous apitegromab global launch**
- **SRK-439 global launch**

MOTOR NEURON



MUSCLE

Advancing Muscle-Targeted Treatments

Addressing progressive muscle atrophy with potential to improve motor function

Motor unit consists of motor neuron *and* muscle

NOTE: Apitegromab and SRK-439 launch expectations following regulatory approval(s).
SMA, Spinal Muscular Atrophy; SMN, Survival Motor Neuron.

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

~35,000

SMA patients have received an approved SMN-targeted therapy[1-3]

1. High SMA diagnosis rates and accelerated time to treatment
2. Increasing number of patients receiving SMN-targeted therapies for >10 years
3. Apitegromab has potential to be world's first and only muscle-targeted treatment for patients with SMA

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. SMA, Spinal Muscular Atrophy; SMN, Survival Motor Neuron.

Poised for 2026 U.S. launch of apitegromab, followed by EMA approval and launch in Europe, beginning with Germany

Building a 50-country platform
to serve patients with rare, severe neuromuscular diseases

NOTE: Following regulatory approval(s).

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 potential 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.

Shaping the future of treatment for patients living with rare neuromuscular disease

IV, Intravenously; SC, Subcutaneously.

Scholar Rock 2026 priorities

Focused execution and financial discipline

1 COMMERCIALIZE

Commercialize apitegromab for treatment of patients with SMA[1]

- Advance launch readiness in U.S. and Europe
- Potential FDA approval and U.S. launch for children and adults with SMA by Sept 30, 2026 PDUFA
- CHMP opinion expected mid-2026, initial launch planned in Germany

2 EXPAND

Develop apitegromab for patients with SMA <2 years and for additional rare, severe NMDs

- Progress Phase 2 OPAL study for patients with SMA <2 years of age
- Initiate apitegromab Phase 2 FORGE study in people living with FSHD in mid-2026

3 ADVANCE

Advance world-leading anti-myostatin pipeline

- Progress subcutaneous apitegromab; Phase 1 study complete
- Advance Phase 1 study for SRK-439, a novel, subcutaneously administered myostatin inhibitor

1. Subject to regulatory approval(s). SMA, Spinal Muscular Atrophy; PDUFA, Prescription Drug User Fee Act; CHMP, Committee for Medicinal Products for Human Use; ; NMDs, Neuromuscular Diseases; FSHD, Facioscapulohumeral Muscular Dystrophy.

Scholar Rock®

Commercialize Apitegromab for Treatment of Patients with SMA

Transforming the treatment of SMA with muscle-targeted therapy

SMA causes motor neuron loss leading to muscle atrophy and progressive weakness

HALLMARKS OF SMA

Spinal Muscular Atrophy

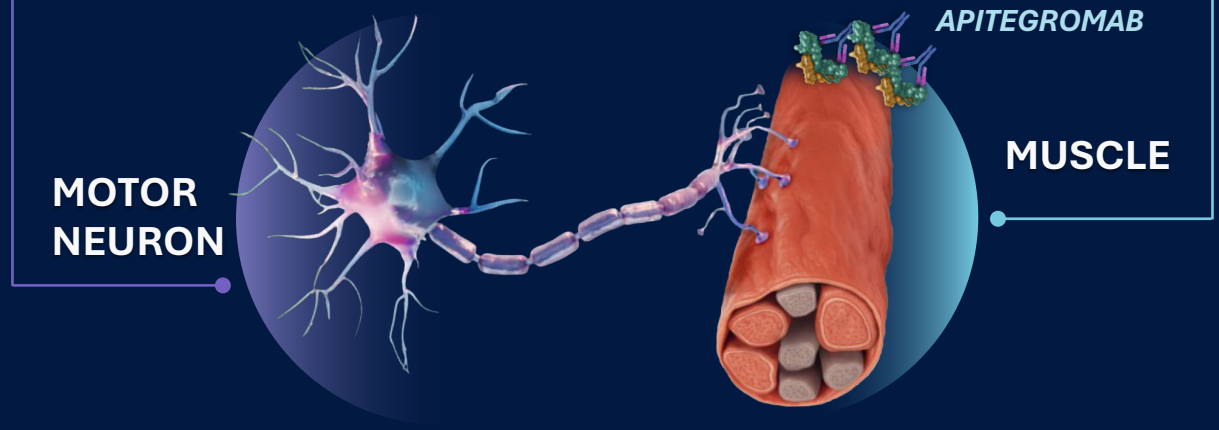
Muscle atrophy and weakness can lead to deterioration in **mobility, swallowing, and breathing,** and can cause **debilitating fatigue**

SMN-Targeted Therapies

Slow further degeneration of motor neurons[1]
...but do not target muscle

Muscle-Targeted Treatment

Apitegromab has the potential to directly **target progressive muscle atrophy and weakness**



Despite significant advancements, progressive muscle weakness remains #1 unmet need in SMA

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;
SMA, Spinal Muscular Atrophy; SMN, Survival Motor Neuron.

Addressing progressive muscle weakness: Apitegromab positioned to be future standard-of-care with ongoing SMN-targeted therapy

GLOBAL SMA OPPORTUNITY

WORLDWIDE

~35,000

patients have received an approved SMN-targeted therapy[1-3]

IN THE U.S.

~7,000

patients have received an approved SMN-targeted therapy[4]

90%

of patients with SMA

rate muscle strength and motor function as top unmet needs[5]

74%

of neurologists

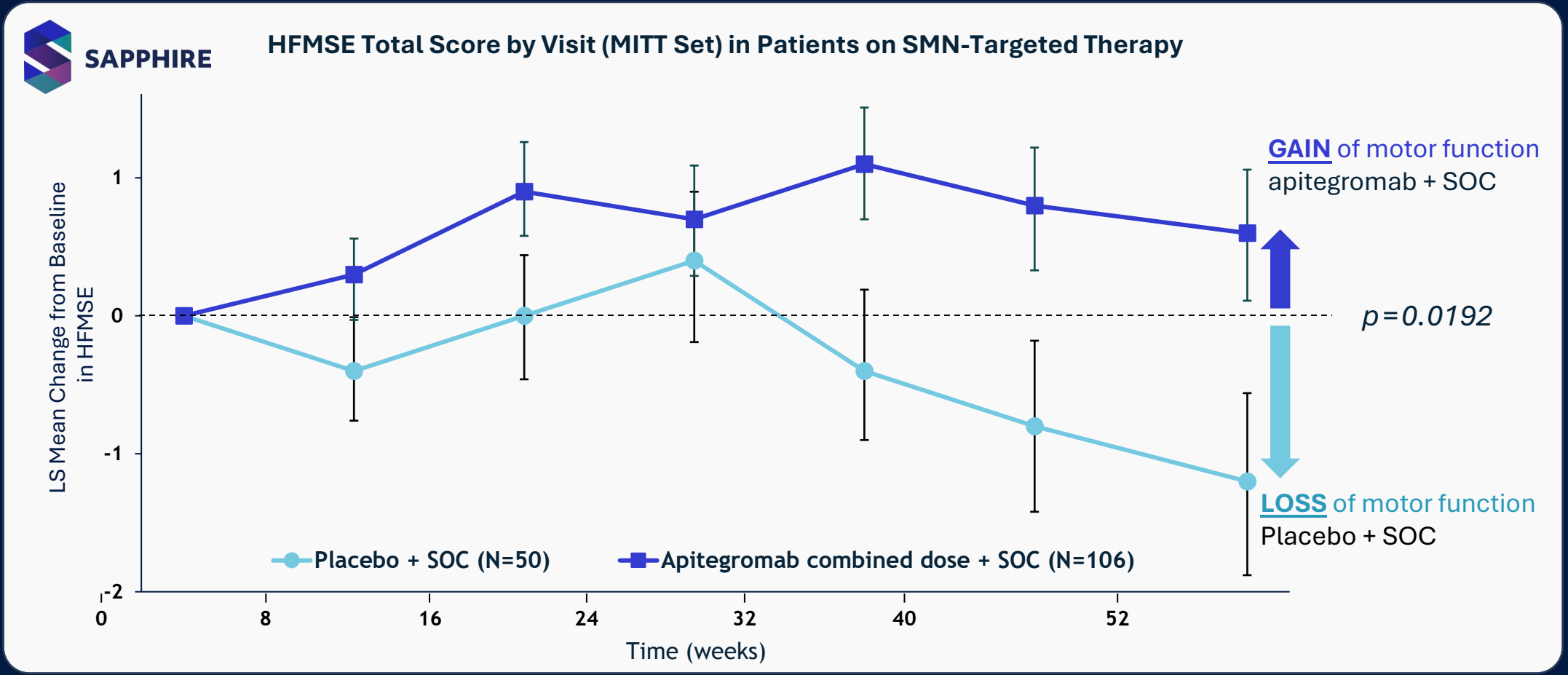
agree that multiple modalities are necessary to treat SMA[6]

1. Biogen Q4 2023 Report; 2. Roche Q3 2024 report; 3. Novartis Q4 2024 Report; 4. Cure SMA State of SMA 2023 Report; 5. Cure SMA State of SMA 2024 Report. 6. Scholar Rock internal market research (US Neurologists), 2024. Approaches to Drug Development. SMA, Spinal Muscular Atrophy; SMN, Survival Motor Neuron.

Apitegromab successful in Phase 3 SMA study

First and only myostatin inhibitor with a positive, statistically significant Phase 3 outcome

Phase 3 randomized, placebo-controlled study in SMA patients (N=188) on standard-of-care (nusinersen or risdiplam)



Sources: Long KK, et al. *Hum Mol Genet*. 2019;28:1076-1089; Pirruccello-Straub M, et al. *Sci Rep*. 2018;8:2292. P-value reflects data from Primary Endpoint for SMA treatment population ages 2–12.
HFMSE, Hammersmith Functional Motor Scale—Expanded; SMA, Spinal Muscular Atrophy; SMN, Survival Motor Neuron; SOC, Standard-of-care.

Statistically significant, clinically meaningful benefits underscore apitegromab's potential to impact broad SMA patient population

30.0% vs 12.5%

30.0% of apitegromab patients

ACHIEVED ≥3PT IMPROVEMENT IN HFMSE[1]

compared to 12.5% on SMN2-targeted treatment alone

- **+1.8 HFMSE points**[2] (p=0.0192) vs. SMN2-targeted treatment alone
- **Consistent clinically meaningful benefit** across all age groups (2-21 yrs)
- **Encouraging safety profile** consistent with >48 months experience in Phase 2 TOPAZ trial

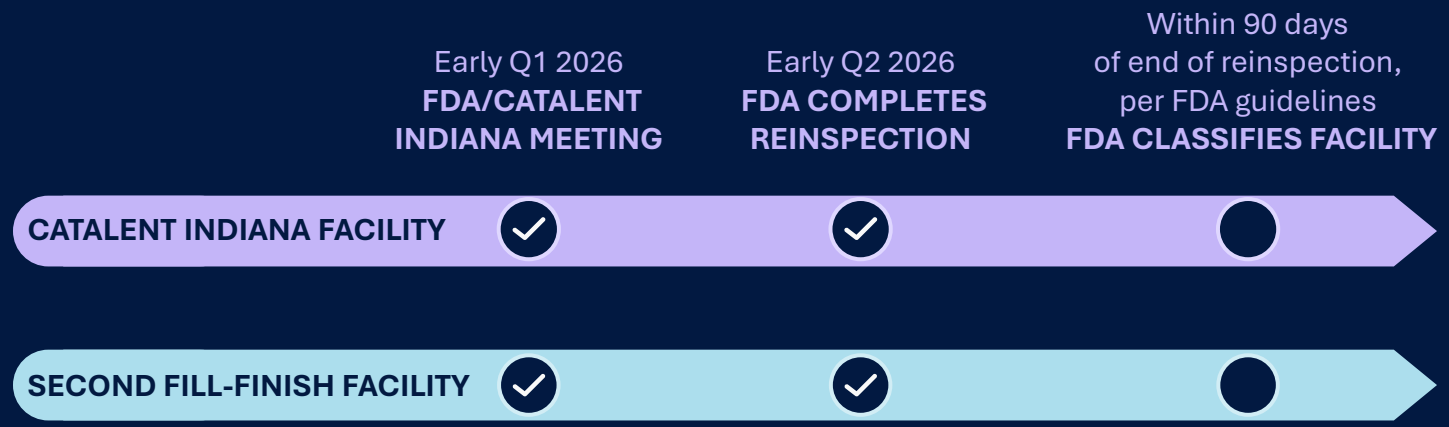
Apitegromab has potential to be the first and only muscle-targeted treatment to improve motor function in SMA

1. 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; 2. Based on apitegromab combined dose (10 mg/kg and 20 mg/kg; n=106) + SOC versus placebo + SOC (n=50) (Hochberg multiplicity adjustment).

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

APITEGROMAB BLA ACCEPTED

Submitted late March; includes two fill-finish facilities



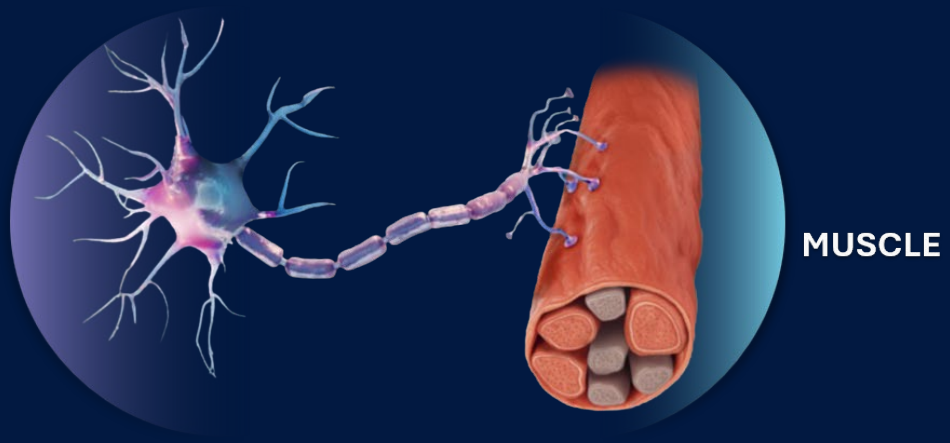
POTENTIAL FDA APPROVAL

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

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

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

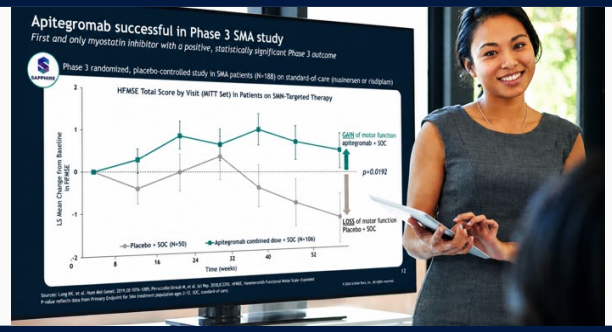
TARGETING 140 SMA Centers

TARGETING >2,600 SMA Prescribers

Patient Engagement & Community Activation

MDA CLINICAL & SCIENTIFIC CONFERENCE 2026

National & Regional Payers Medicare & Medicaid



SMA, Spinal Muscular Atrophy.

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

5th International Scientific Congress on **Spinal Muscular Atrophy**

1. **Building world-class team**
2. **Engaging the SMA community**
3. **Establishing access**

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

Apitegromab in SMA represents a large global opportunity to serve patients

>$5B[1]

2025 global revenue for SMN-targeted therapies

1st & Only

muscle-targeted treatment to show clinical benefit in SMA

$2B+[2]

Apitegromab global revenue potential

SMA community is demanding the

first and only muscle-targeted therapy

1. Revenue as of Biogen 4Q25 financial update, Roche 4Q25 financial update, and Novartis 4Q25 financial update; 2. Scholar Rock internal estimates as of December 2025.
SMA, Spinal Muscular Atrophy; SMN, Survival Motor Neuron.

Scholar Rock®

Develop Apitegromab for Patients with SMA Under 2 and for Additional Neuromuscular Diseases (NMDs)

Building a pipeline-in-a-product to reach more patients with SMA and with additional rare, severe NMDs

Furthering our commitment to broad SMA community

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

OPAL

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

Addressing the needs of children under 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; SMA, Spinal Muscular Atrophy; SMN, Survival Motor Neuron.

FSHD: Rare, devastating NMD with significant unmet need

>30,000 patients diagnosed in U.S. and Europe; no approved therapies

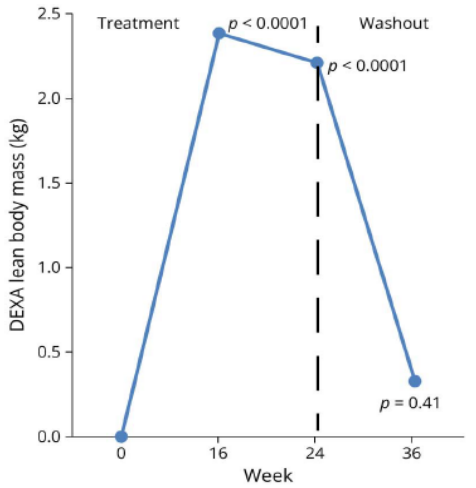
FSHD results in progressive muscle atrophy leading to cumulative loss of function and loss of independence

- Caused by myotoxic effects from abnormal expression of DUX4[1,2]
- Symptoms often begin age 15 – 30[3]
- >80% of patients report moderate/severe impact on activities involving arms, core, and/or legs; ~20% will become wheelchair dependent[3,4]
- Standard-of-care (e.g., physical therapy) only addresses symptoms, not underlying disease

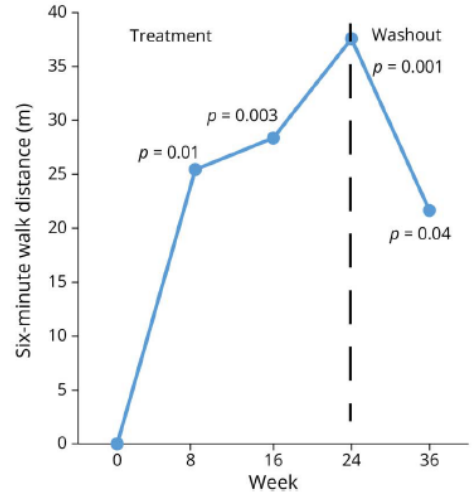
Support for apitegromab therapeutic hypothesis in FSHD

- 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



1. Tawil R, et al. *Skelet Muscle*. 2014;4:12; 2. Hubregtse L, et al. *Neuromuscul Disord*. 2024;36:6-15; 3. Tihaya MS, et al. *Nat Rev Neurol*. 2023;19(2):91-108; 4. FSHD Society. Accessed Oct 7, 2025. https://www.fshdsociety.org/wp-content/uploads/2020/11/Voice-of-the-Patient-Report-FINAL.pdf; 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. 6MWD, 6-minute Walk Distance; FSHD, Facioscapulohumeral Muscular Dystrophy; NMD, Neuromuscular Disease.

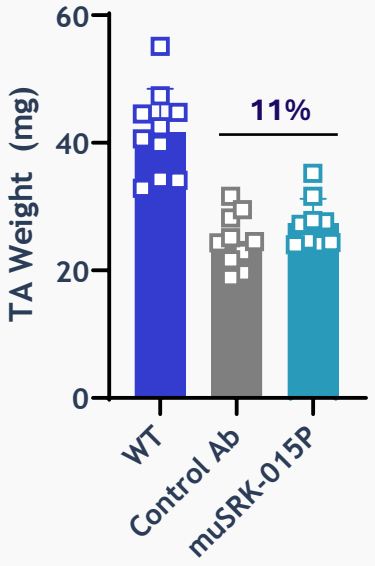
Preclinical studies provide mechanistic rationale for apitegromab in FSHD

Increased muscle mass, strength, and endurance in gold standard FLExDUX4 mouse model of FSHD

FSHD FLEXDUX4 MOUSE MODEL

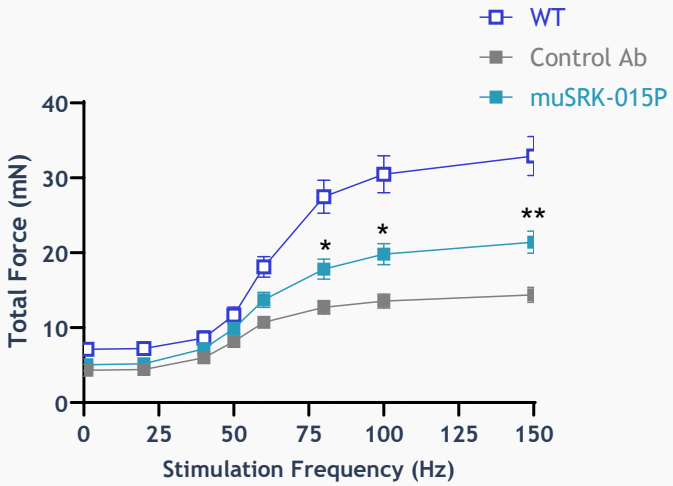
Robust Increase in MUSCLE MASS

(28 Days)



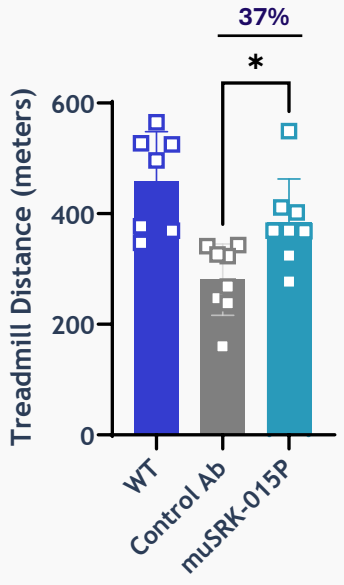
Significant Improvements in MUSCLE FORCE

(28 Days)



Consistent Gains in ENDURANCE

(28 Days)

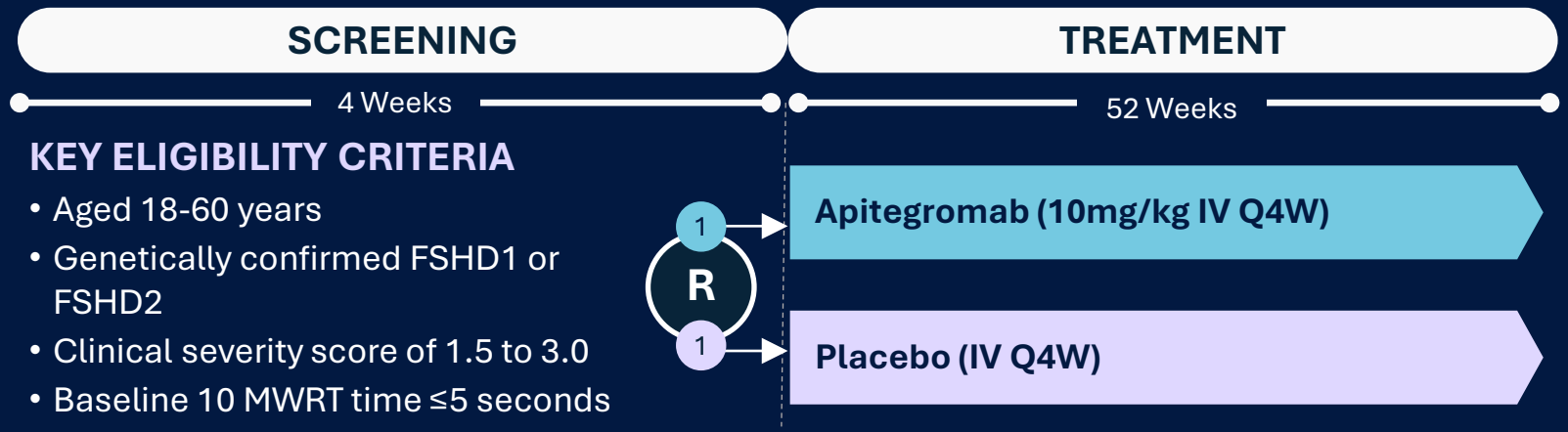


*Muscle mass=weight of tibialis anterior muscle; muSRK-015P is a murine version of apitegromab. FLExD, FLExDUX4.Cre; FSHD, facioscapulohumeral muscular dystrophy; WT, wild type; Ab, antibody. Fogel A. Presented at: FSHD Society International Research Congress; Jun 12-13, 2025; Amsterdam, Netherlands. Oral presentation.

Phase 2 trial evaluating apitegromab in patients with FSHD

FORGE

Randomized, double-blind, placebo-controlled, multicenter study (N≈60)



KEY ELIGIBILITY CRITERIA

- Aged 18-60 years
- Genetically confirmed FSHD1 or FSHD2
- Clinical severity score of 1.5 to 3.0
- Baseline 10 MWRT time ≤5 seconds

PRIMARY ENDPOINT:

Mean lean muscle volume (LMV) change from baseline at 12 months

SECONDARY & OTHER ENDPOINTS:

- Mean LMV change from baseline at 6 months
- Mean change from baseline in additional muscle parameters (6 and 12 months)
- Quantitative myometry testing (QMT)
- Safety, PK/PD, ADA

IND application cleared; on track to initiate dosing in Phase 2 FORGE study in mid-2026

10 MWRT, 10-Meter Walk/Run Test; ADA, Antidrug Antibody; FSHD, Facioscapulohumeral Muscular Dystrophy; IV, Intravenous; LMV, Lean Muscle Volume; N, Total Number of Participants; PD, Pharmacodynamics; PK, Pharmacokinetics; Q4W, Every 4 Weeks; QMT, Quantitative Muscle Testing; R, Randomization.

Unlocking pipeline-in-a-product with apitegromab in additional rare NMDs, beginning with FSHD

Broad landscape of potential indications supports significant opportunity for muscle-targeted therapies

GENETIC NEUROPATHIC DISEASES

Spinal muscular atrophy (SMA)

Amyotrophic Lateral Sclerosis (ALS)

Peripheral neuropathies

GENETIC MYOPATHIES

Muscular Dystrophies:
- Facioscapulohumeral dystrophy (FSHD)
- Duchenne muscular dystrophy (DMD)
- limb girdle muscular dystrophy (LGMD)

Congenital Myopathies, e.g., Myotubular

Metabolic Myopathies, e.g., Pompe disease

ACQUIRED MYOPATHIES

Inflammatory Myopathies:
- Inclusion myositis

Toxic Myopathies:
- Drug-induced myopathy

Endocrine Myopathies, e.g., Cushing's disease

ACQUIRED NEUROPATHIC DISEASES

Amyotrophic Lateral Sclerosis (ALS)

Myasthenia gravis (MG)

Guillain-Barré syndrome

Inflammatory neuropathies

Scholar Rock deploying innovative, world-leading anti-myostatin pipeline to potentially address a range of rare, severe neuromuscular diseases

NMDs, neuromuscular diseases.

Advance World-Leading Anti-Myostatin Pipeline

Driving continued innovation for treatment of patients living with rare, severe NMDs

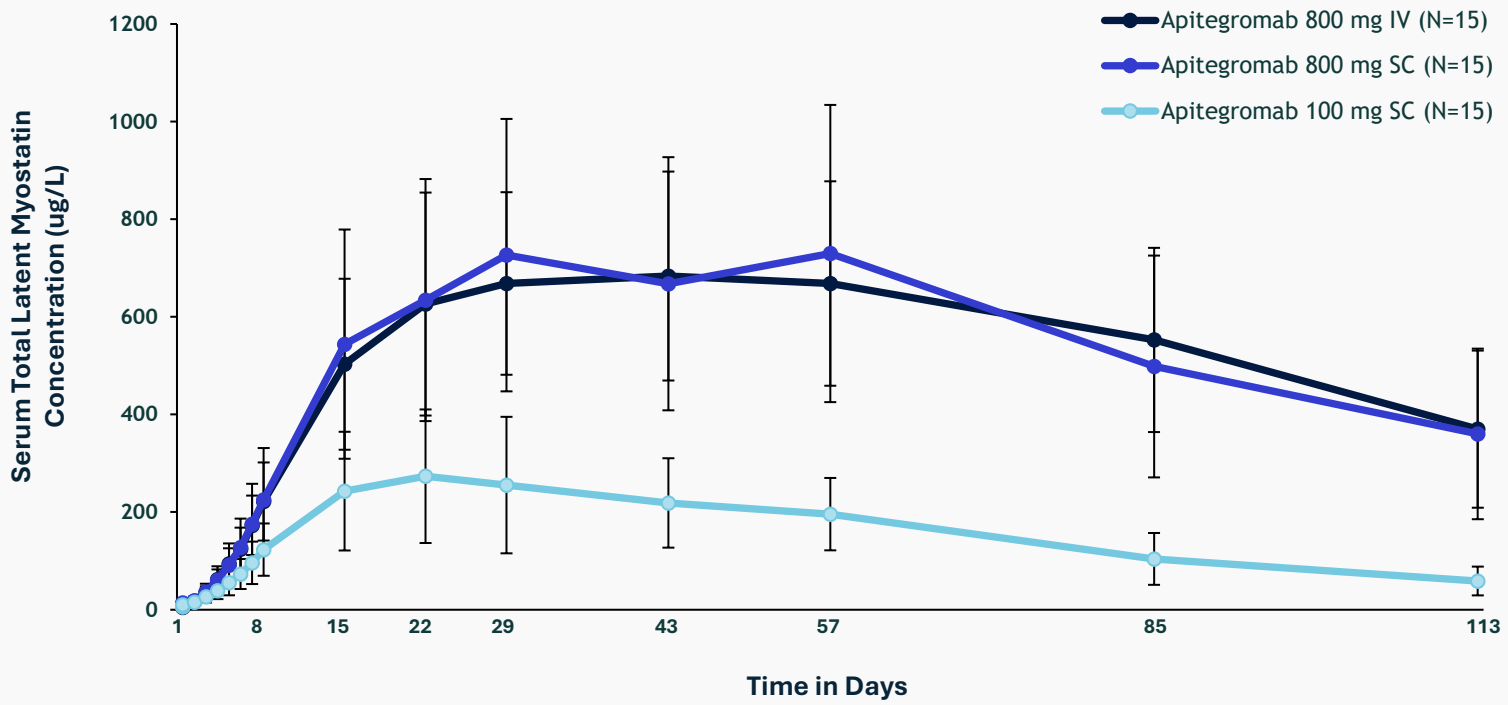
NMDs, neuromuscular diseases.

Subcutaneous apitegromab demonstrated favorable bioavailability, with pharmacodynamic profile comparable to IV administration

Phase 1 study

- 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 planned FDA and EMA regulatory engagements following apitegromab approvals

Overlapping Pharmacodynamic Profiles for IV and SC Apitegromab (800mg)



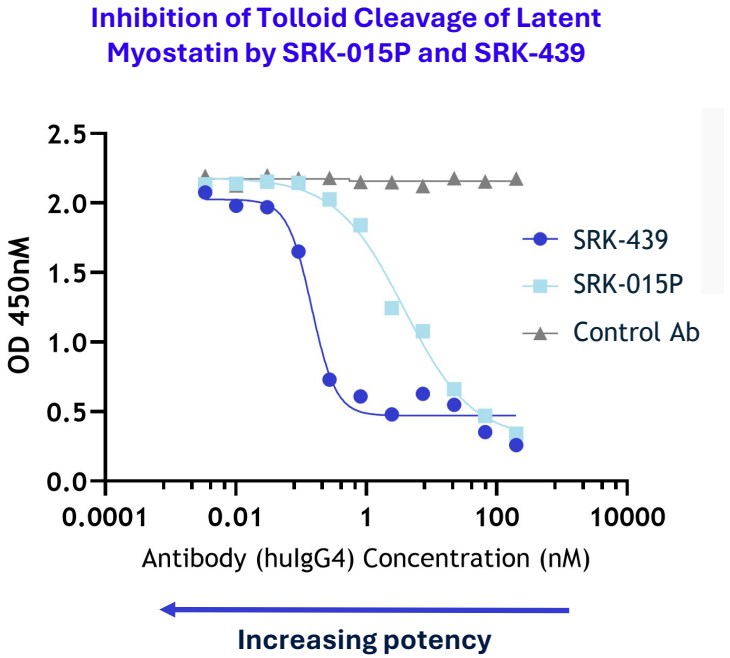
Source: Scholar Rock internal data. PD, Pharmacodynamic.

Advancing Scholar Rock's innovative anti-myostatin platform with SRK-439

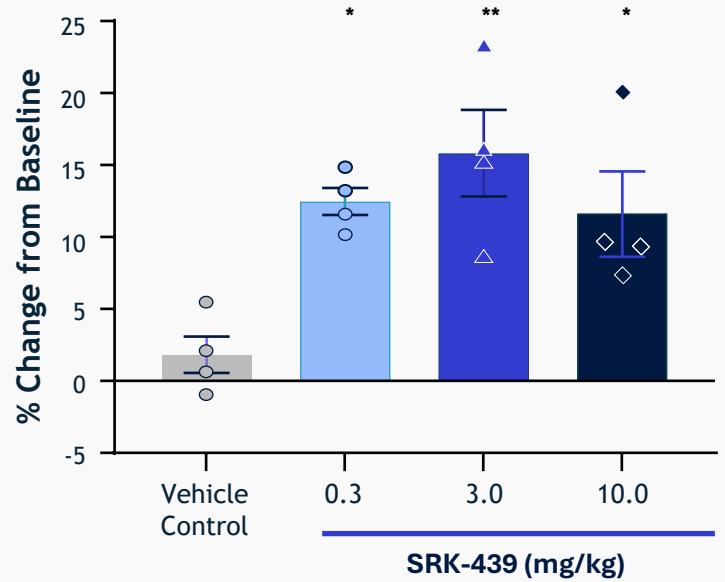
Leveraging world-leading expertise to drive continued innovation

- Novel, highly potent myostatin inhibitor
- Optimized for subcutaneous administration
- Strong scientific validation
  Preclinical data demonstrated favorable muscle mass preservation

Latent Myostatin Potency Assay Comparing SRK-015P and SRK-439



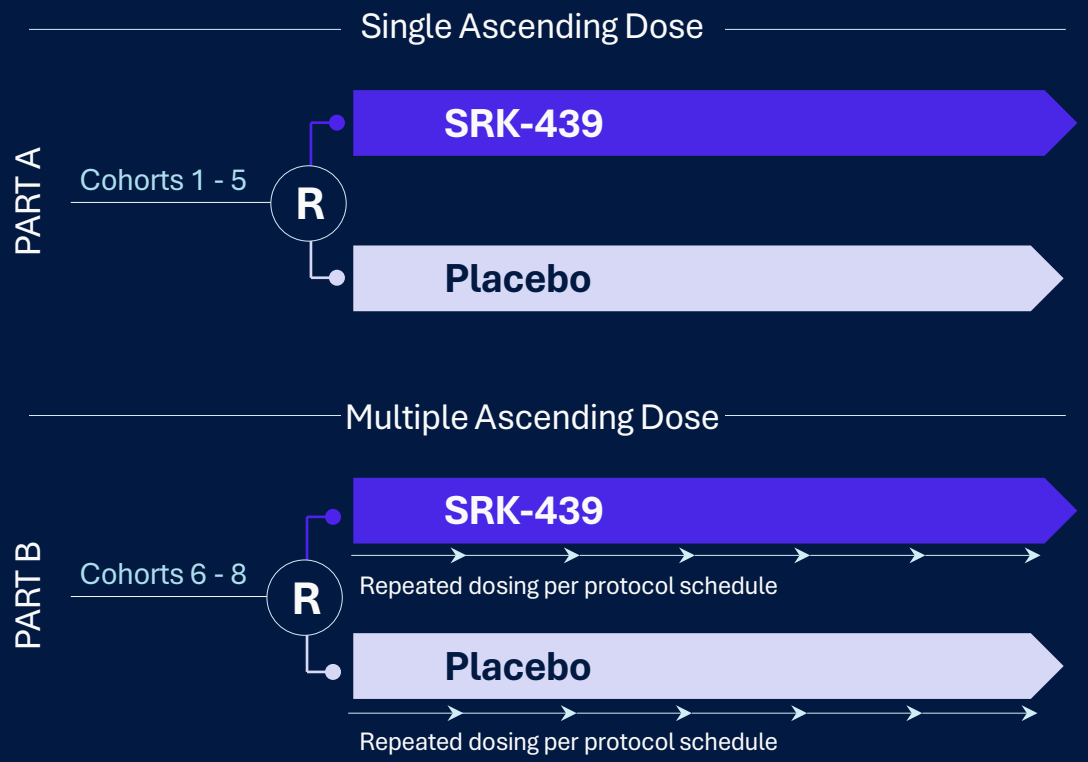
Change in Whole Body Lean Mass from Baseline (NHP)



SRK-439 is a novel, investigational myostatin inhibitor. muSRK-015P is a murine version of apitegromab.

SRK-439 Phase 1 study assessing safety, tolerability, and PK/PD profile

N≈76 Healthy Adult Participants



SRK-439 Program Key Milestones

- IND cleared in **November 2025**
- Dosing commenced in Phase 1 study in **December 2025**
- On track to report topline Phase 1 data in **H2 2026**

PK/PD, Pharmacokinetic/Pharmacodynamic.

Financials and Upcoming Milestones

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

Scholar Rock 2026 priorities

Poised to be next global biotech powerhouse

1

COMMERCIALIZE

Commercialize apitegromab for treatment of patients with SMA[1]

- Advance launch readiness in U.S. and Europe
- Potential FDA approval and U.S. launch for children and adults with SMA by Sept 30, 2026 PDUFA
- CHMP opinion expected mid-2026, initial launch planned in Germany

2

EXPAND

Develop apitegromab for patients with SMA <2 years and for additional rare, severe NMDs

- Progress Phase 2 OPAL study for patients with SMA <2 years of age
- Initiate apitegromab Phase 2 FORGE study in people living with FSHD in mid-2026

3

ADVANCE

Advance world-leading anti-myostatin pipeline

- Progress subcutaneous apitegromab; Phase 1 study complete
- Advance Phase 1 study for SRK-439, a novel, subcutaneously administered myostatin inhibitor

Driving value through focused execution and financial discipline

1. Subject to regulatory approval(s). SMA, Spinal Muscular Atrophy; PDUFA, Prescription Drug User Fee Act; CHMP, Committee for Medicinal Products for Human Use; NMDs, Neuromuscular Diseases; FSHD, Facioscapulohumeral Muscular Dystrophy.

Scholar Rock®

FOR MORE INFORMATION
PLEASE CONTACT


ir@scholarrock.com


media@scholarrock.com


www.scholarrock.com