# 44th Annual J.P. Morgan Healthcare Conference

Scholar Rock Investor Presentation

**JANUARY 12, 2026**



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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 growth, strategy, progress and timing of its clinical trials for apitegromab, SRK-439 and its preclinical programs, 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 candidates, its ability to address the observations identified in the complete response letter, its cash runway into 2027, expectations regarding commercial launch timing in the US and in Europe, expectations regarding a new fill finish facility 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 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; the results from the Phase 3 SAPPHIRE trial will be sufficient to support regulatory approval; 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 2026; 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, and Quarterly Report on Form 10-Q for the quarter ended September 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.

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 in a position of strength entering 2026

## SHAPING THE FUTURE OF TREATMENT FOR PATIENTS LIVING WITH RARE NEUROMUSCULAR DISEASES (NMDs)

### APITEGROMAB FOR PATIENTS WITH SMA

**1ST**

- Myostatin inhibitor with a successful Phase 3 study
- Only muscle-targeted treatment to demonstrate clinically meaningful benefit in SMA

**2026**
**ON TRACK**

- BLA resubmission and U.S. launch anticipated[1]
- EMA decision expected in mid-2026

**GLOBALLY**

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

**ONGOING**

**SRK-439 DOSING COMMENCED** in Phase 1 healthy volunteer study

**MID-2026**

**INITIATE PHASE 2 STUDY** for apitegromab in facioscapulohumeral muscular dystrophy

**APPROXIMATELY $365M** in cash and cash equivalents as of Dec 31, 2025[2]

**CASH RUNWAY INTO 2027**

1. Subject to U.S. FDA approval. 2. Financial information as of December 31, 2025 has not been audited and has been prepared by, and is the responsibility of management. This information could change as a result of further review. SMA, Spinal Muscular Atrophy; BLA, Biologics License Application; EMA, European Medicines Agency; SMN, survival motor neuron.

# 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**

**NEXT PHASE OF INNOVATION: MUSCLE**

2016 · 2019 · 2020 · 2025 · 2026 · 2030+

SPINRAZA® (nusinersen) injection 12 mg/5 mL

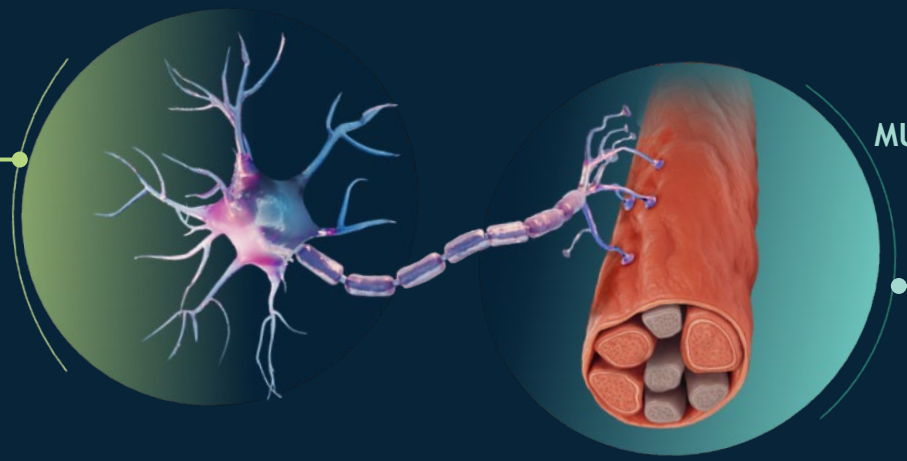
zolgensma® (onasemnogene abeparvovec-xioi) suspension for intravenous infusion

Evrysdi® risdiplam for oral solution

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

**SMN-Targeted Therapies**

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




Motor unit consists of motor neuron *and* muscle

## Advancing Muscle-Targeted Treatments

Addressing progressive muscle atrophy with potential to improve motor function

NOTE: Apitegromab and SRK-439 launch expectations following regulatory approval(s). SMN, survival motor neuron.

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


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 next**

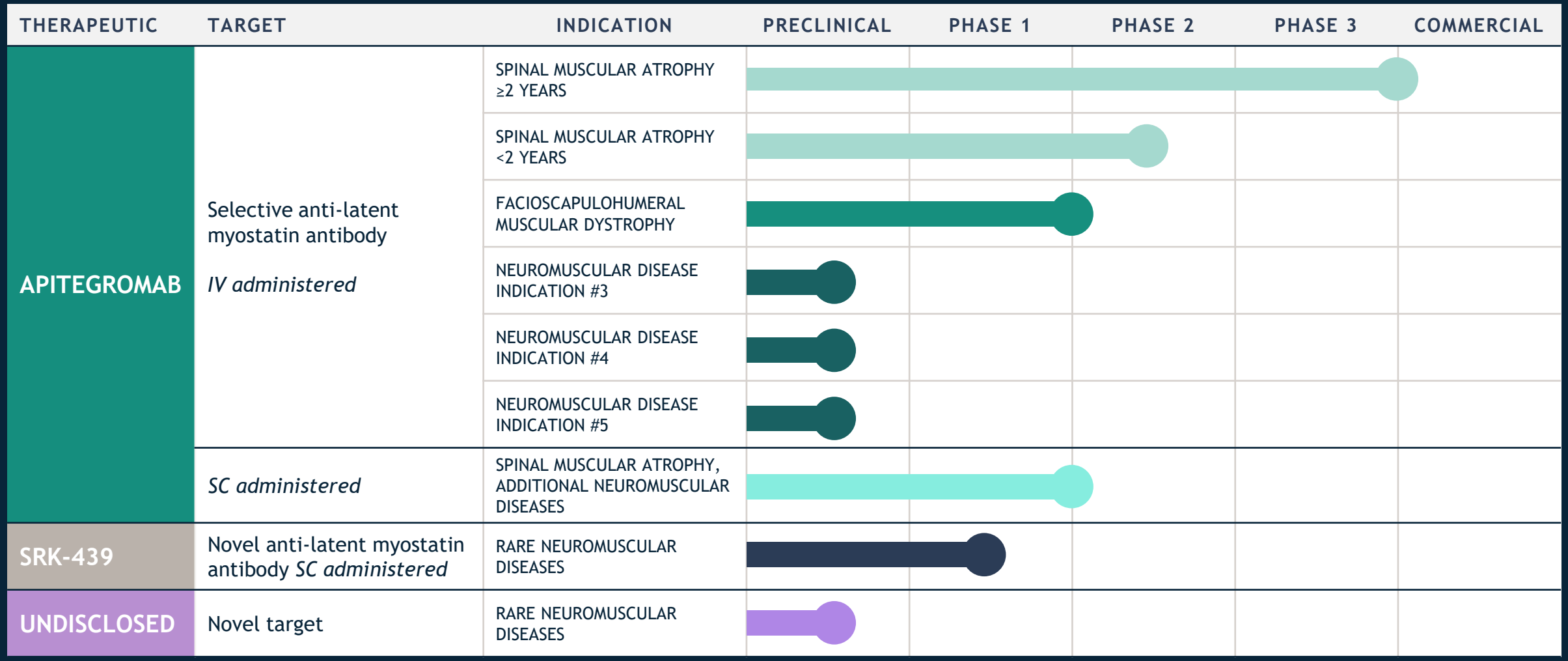
1. Biogen Q4 2023 Report; 2. Roche Q3 2024 report; 3. Novartis Q4 2024 Report. SMN, survival motor neuron.

# 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).

# 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**

**BLA resubmission and U.S. launch, following FDA approval, for children and adults with SMA**

**EMA decision 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 patients 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). NMDs, neuromuscular diseases.

# 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**

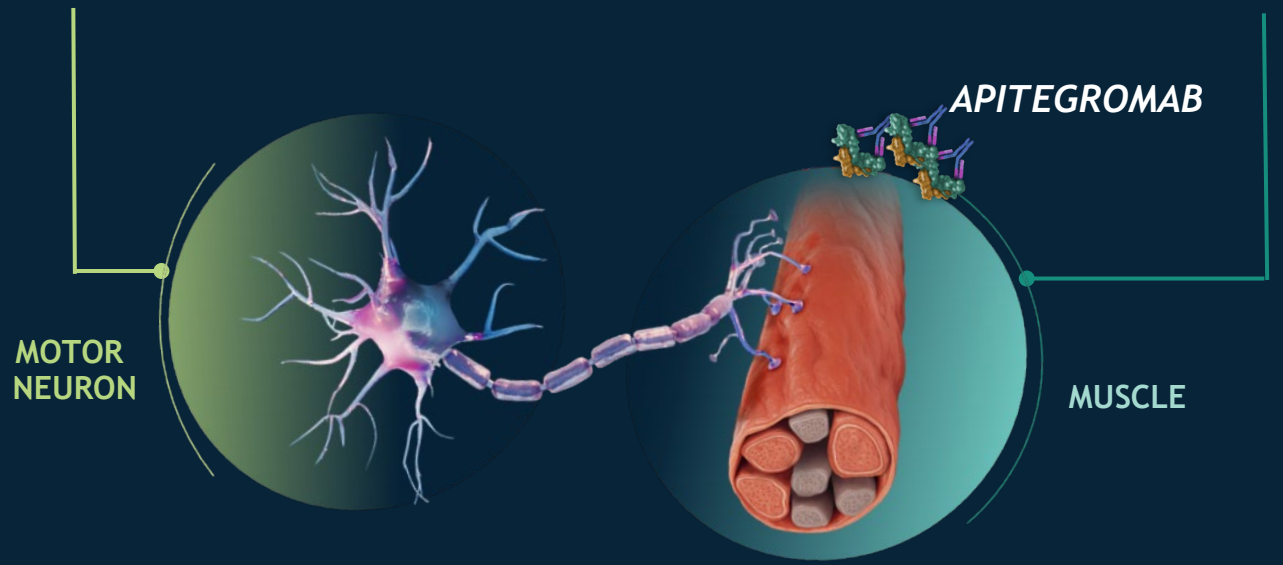
### SMN-Targeted Therapies

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**

SMA, Spinal muscular atrophy; SMN, Survival motor neuron.

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; Accessed April 18, 2021.

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# 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]

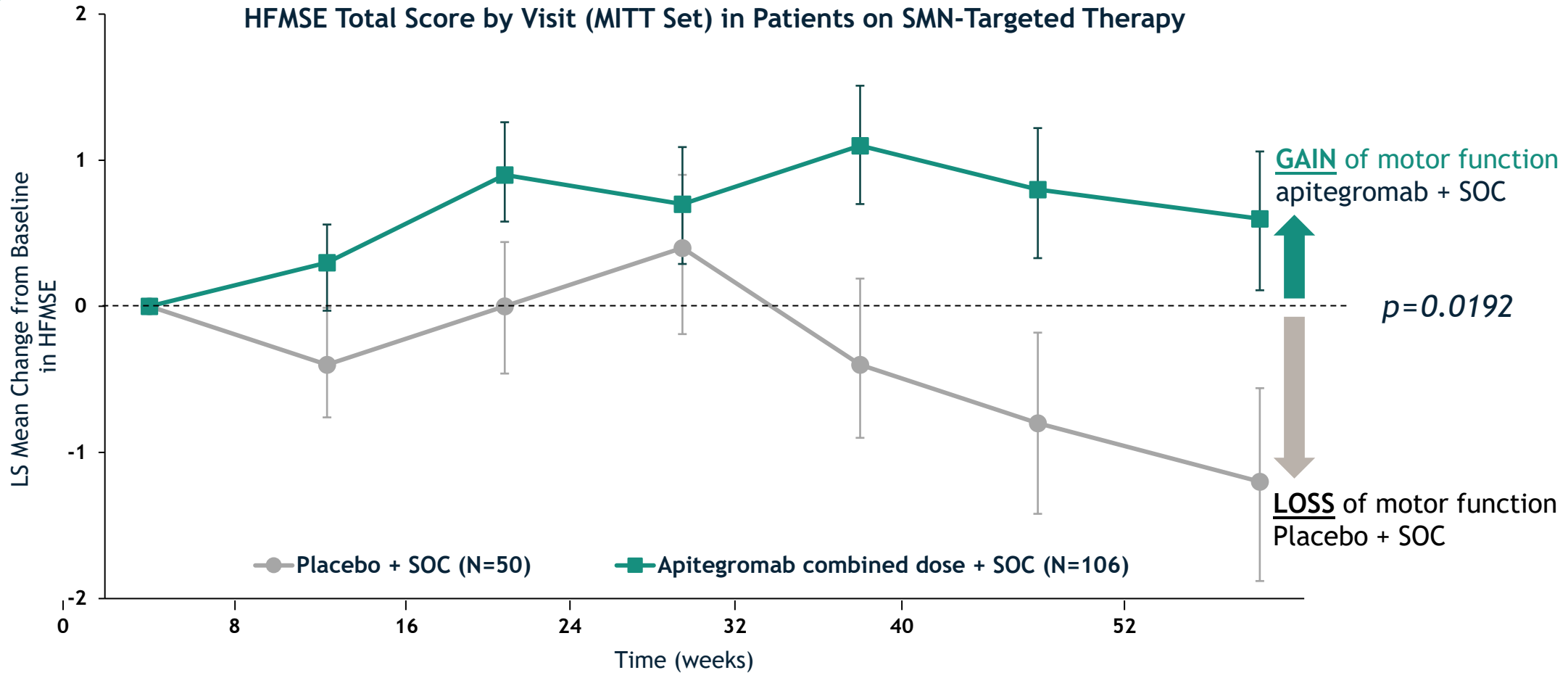
1. Biogen Q4 2023 Report; 2. Roche Q3 2024 report; 3. Novartis Q4 2024 Report; 4. Cure SMA State of SMA 2023 Report; 5. Cure SMA State of SMA 2024 Report. https://www.curesma.org/wp-content/uploads/2025/04/State-of-SMA-Report2024_vWeb.pdf; 6. Scholar Rock internal market research (US Neurologists), 2024. Approaches to Drug Development. SMN, survival motor neuron.

# Apitegromab successful in Phase 3 SMA study

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

SAPPHIRE

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. HFMSE, Hammersmith Functional Motor Scale—Expanded
P-value reflects data from Primary Endpoint for SMA treatment population ages 2–12. 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).

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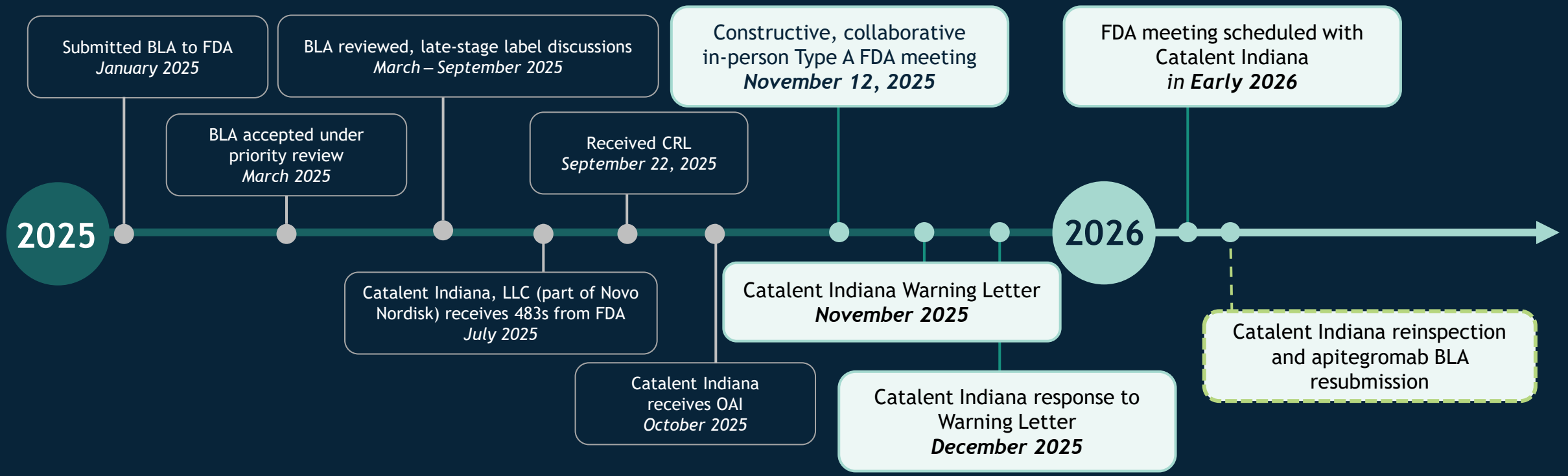
# Apitegromab BLA resubmission and U.S. launch, following approval, anticipated in 2026




**Tech transfer ongoing at second fill-finish facility; commercial capacity reserved beginning in Q1 2026**

BLA, Biologics License Application; OAI, Official Action Indicated

# U.S. commercial field team focused on engagement, disease education across SMA stakeholder landscape

## Prescribers and SMA Treatment Centers

**>2,600**
SMA Prescribers

**140**
SMA Centers

Broaden and deepen engagement

Understand patient journey and roles of treatment team

## Patient Advocacy

Strong collaboration with advocacy groups

Building lasting relationships
one patient, one caregiver, one family at a time

## National & Regional Payers

Market access team engaging with national and regional payers

Educating on unmet need and potential benefit of apitegromab

# Strong momentum with apitegromab launch readiness in Europe in advance of mid-2026 EMA decision

*Large opportunity to serve SMA patients in Europe*

## Building World-Class Team

- Key leadership hires made and additional offers extended
- Expanded physical presence in Zug and Dublin
- Distribution model in place

## Engaging SMA Community

- Global Medical Affairs team focused on SMA disease education
- Expanded engagement with KOLs and SMA Centers of Excellence
- Deepening relationships with multiple country patient advocacy groups

## Establishing Access

- Multiple reimbursement dossiers on track for submission
- Compassionate use program enrolling in Germany
- Finalizing plans with international partner markets

EMA, European Medicines Agency; KOL, Key Opinion Leader.

# Apitegromab in SMA represents a large global opportunity to serve patients

**~$5B**[1]

Estimated 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 demanding the first and only muscle-targeted therapy**

1. Revenue as of Biogen 3Q25 financial update, Roche 3Q25 financial update, and Novartis 3Q25 financial update; 2. Scholar Rock internal estimates as of December 2025. SMN, survival motor neuron; SMA, spinal muscular atrophy.

# 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*


- 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.

# FSHD: Rare, devastating NMD with significant unmet need

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

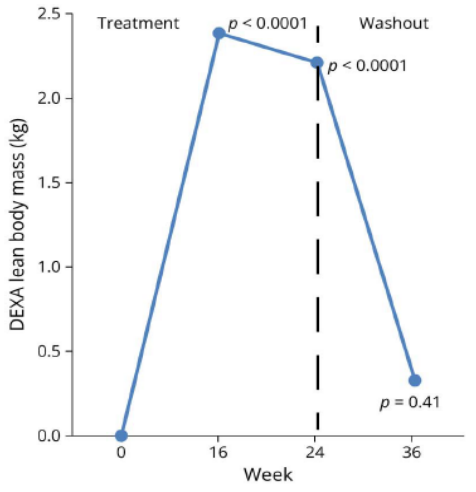
## FSHD results in progressive muscle atrophy leading to cumulative loss of function and loss of independence

- 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

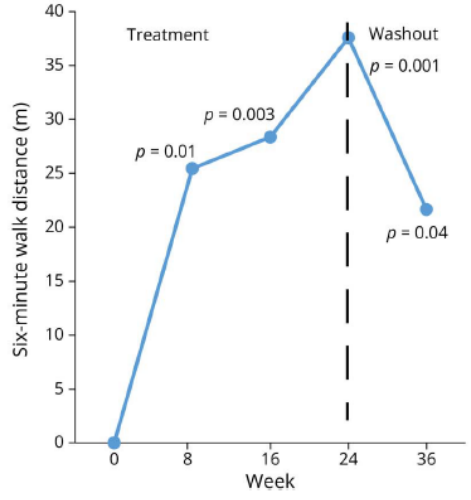
## Support for apitegromab therapeutic hypothesis in FSHD

- 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



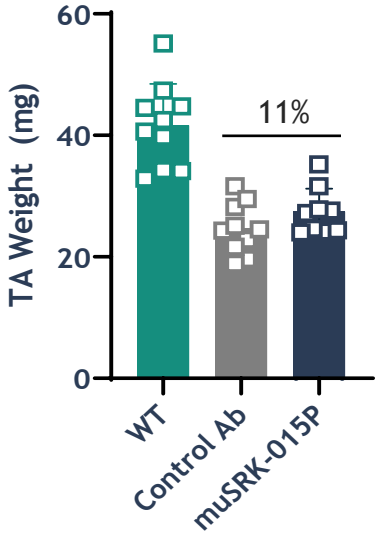

1. Tawil R, et al. *Skelet Muscle*. 2014;4:12; 2. Hubregtse L, et al. *Neuromuscul Disord*. 2024;36:6-15; 3. Tihaya MS, et al. *Nat Rev Neurol*. 2023;19(2):91-108; 4. FSHD Society. Accessed Oct 7, 2025. https://www.fshdsociety.org/wp-content/uploads/2020/11/Voice-of-the-Patient-Report-FINAL.pdf; 5. Bankole, 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.

# Preclinical studies provide mechanistic rationale for apitegromab in FSHD

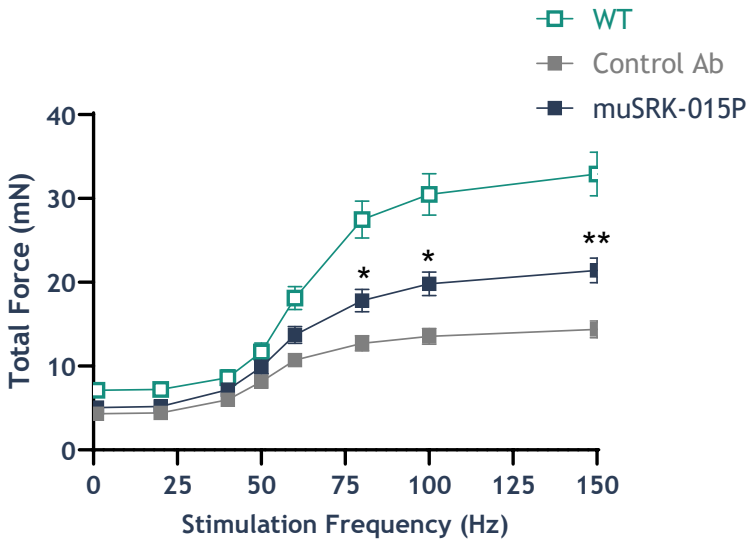
*Increased muscle mass, strength, and endurance in gold standard FLExDUX4 mouse model of FSHD*

**FSHD FLExDUX4 Mouse Model**

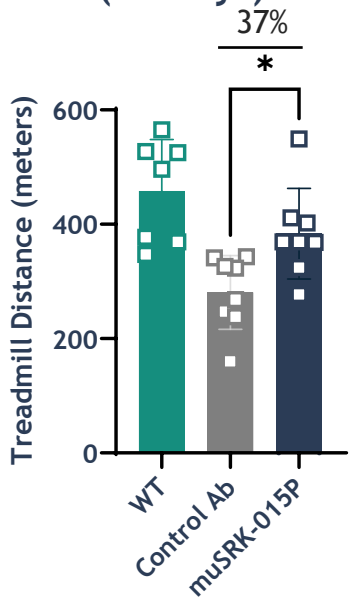
## Robust Increase in Muscle Mass (28 Days)




## Significant Improvements in Muscle Force (28 Days)




## Consistent Gains in Endurance (28 Days)




**Data from 8-month-old mice; similar results observed in younger mice.*

*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.

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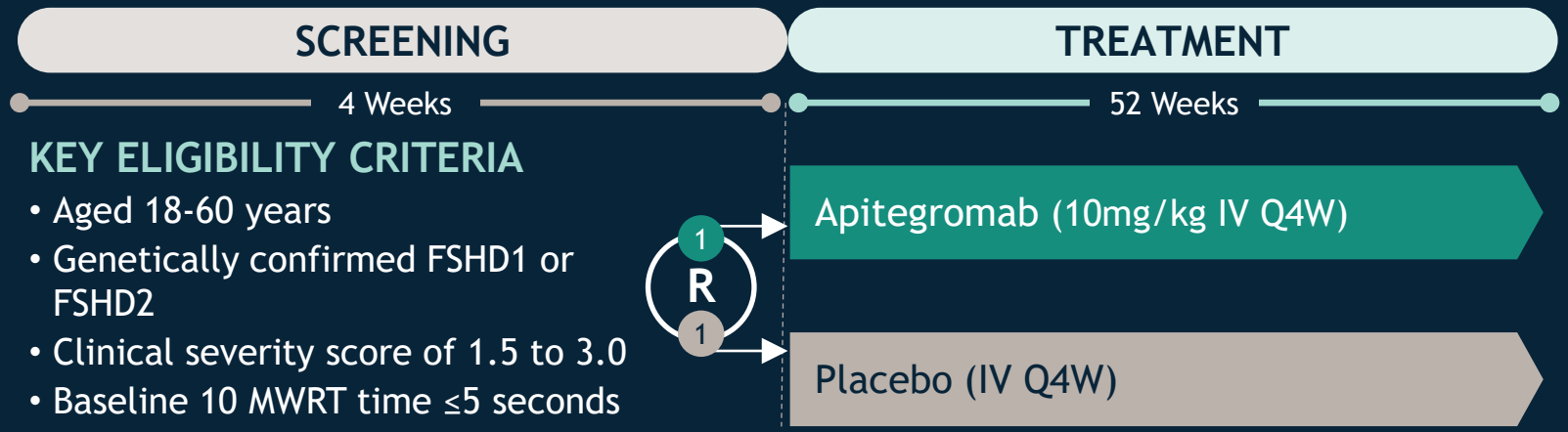
# FORGE Phase 2 trial evaluating apitegromab in patients with FSHD

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.

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# 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

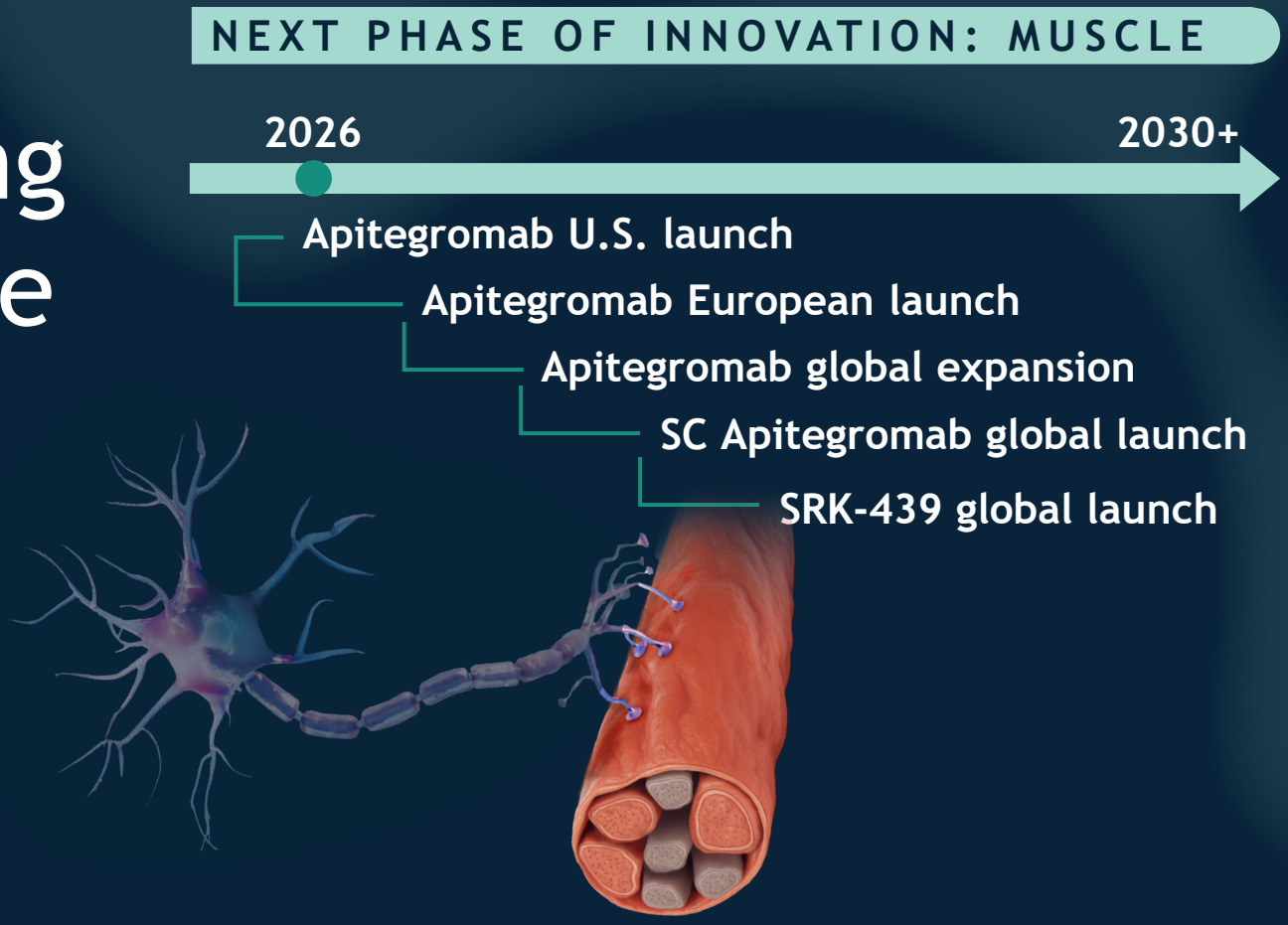
- Amyotrophic Lateral Sclerosis (ALS)
- Myasthenia gravis (MG)
- Guillain-Barré syndrome
- Inflammatory neuropathies

***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



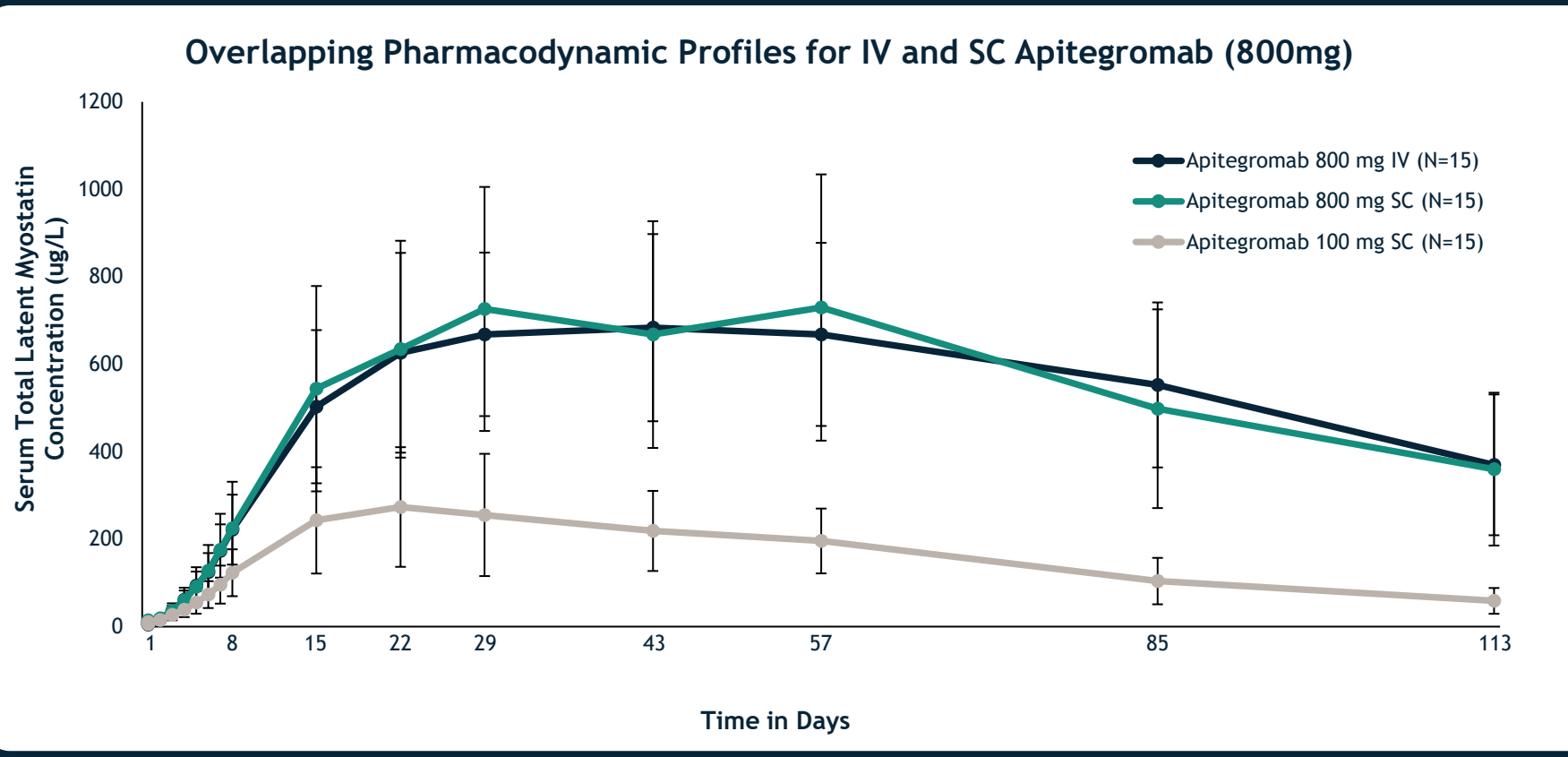



Note: Apitegromab and SRK-439 launch expectations following regulatory approval(s). SC, subcutaneous.

# 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



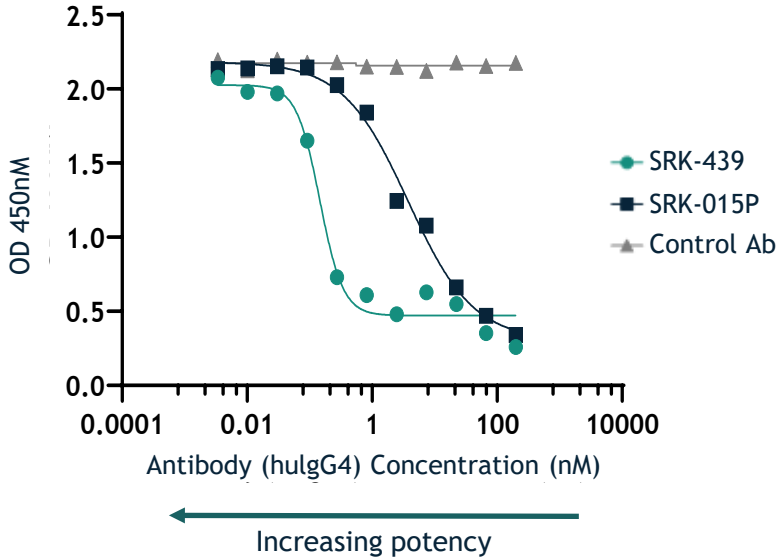

Source: Scholar Rock internal data. PD, pharmacodynamic.

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

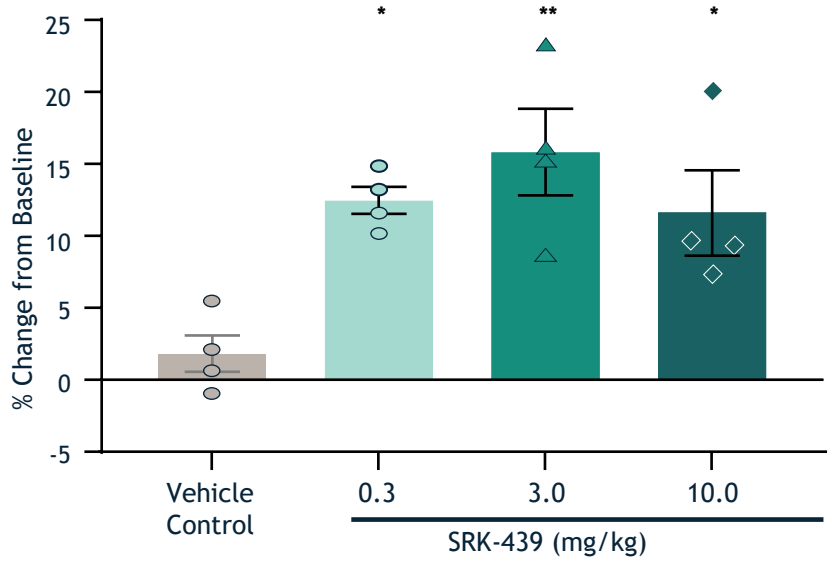
*Leveraging world-leading expertise to drive continued innovation*

- **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

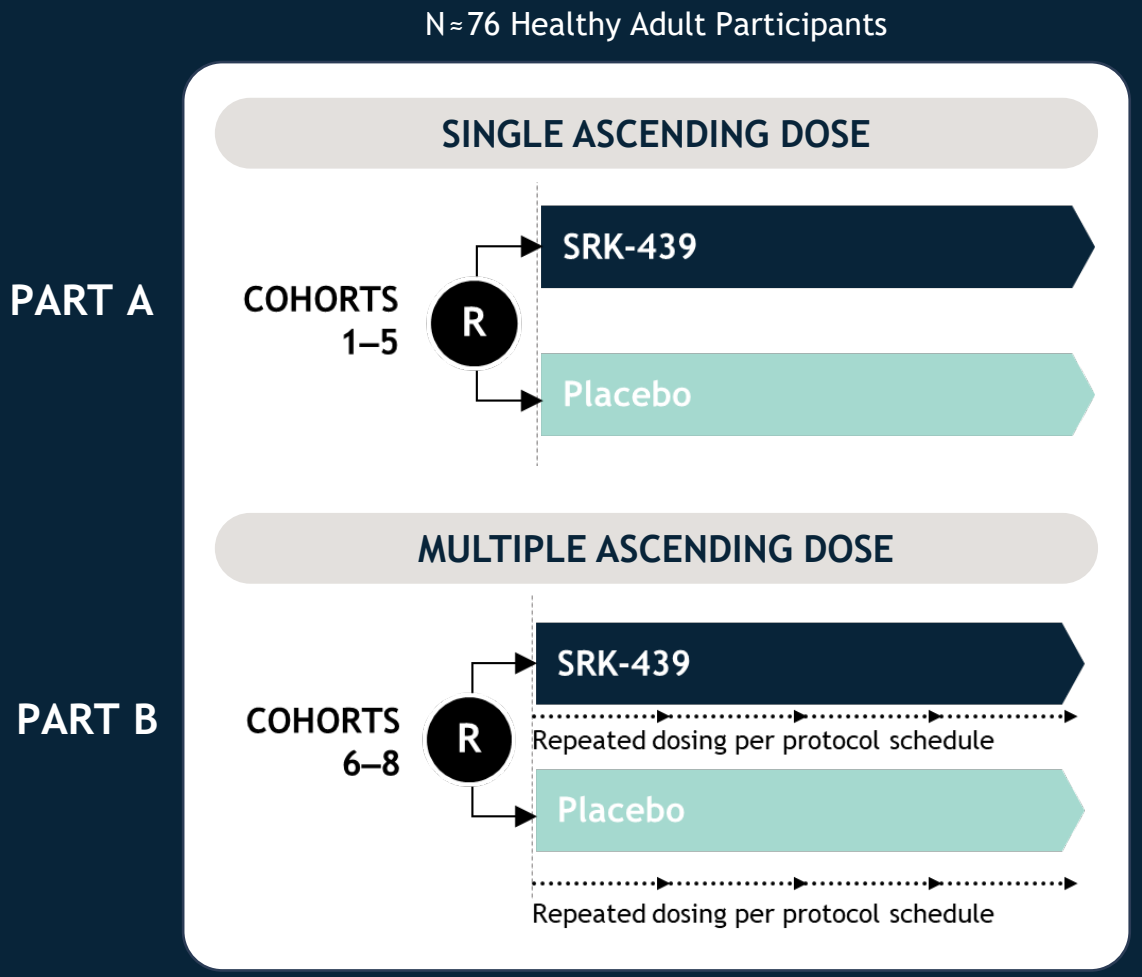



## Change in Whole Body Lean Mass from Baseline (NHP)




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




# SRK-439 program key milestones

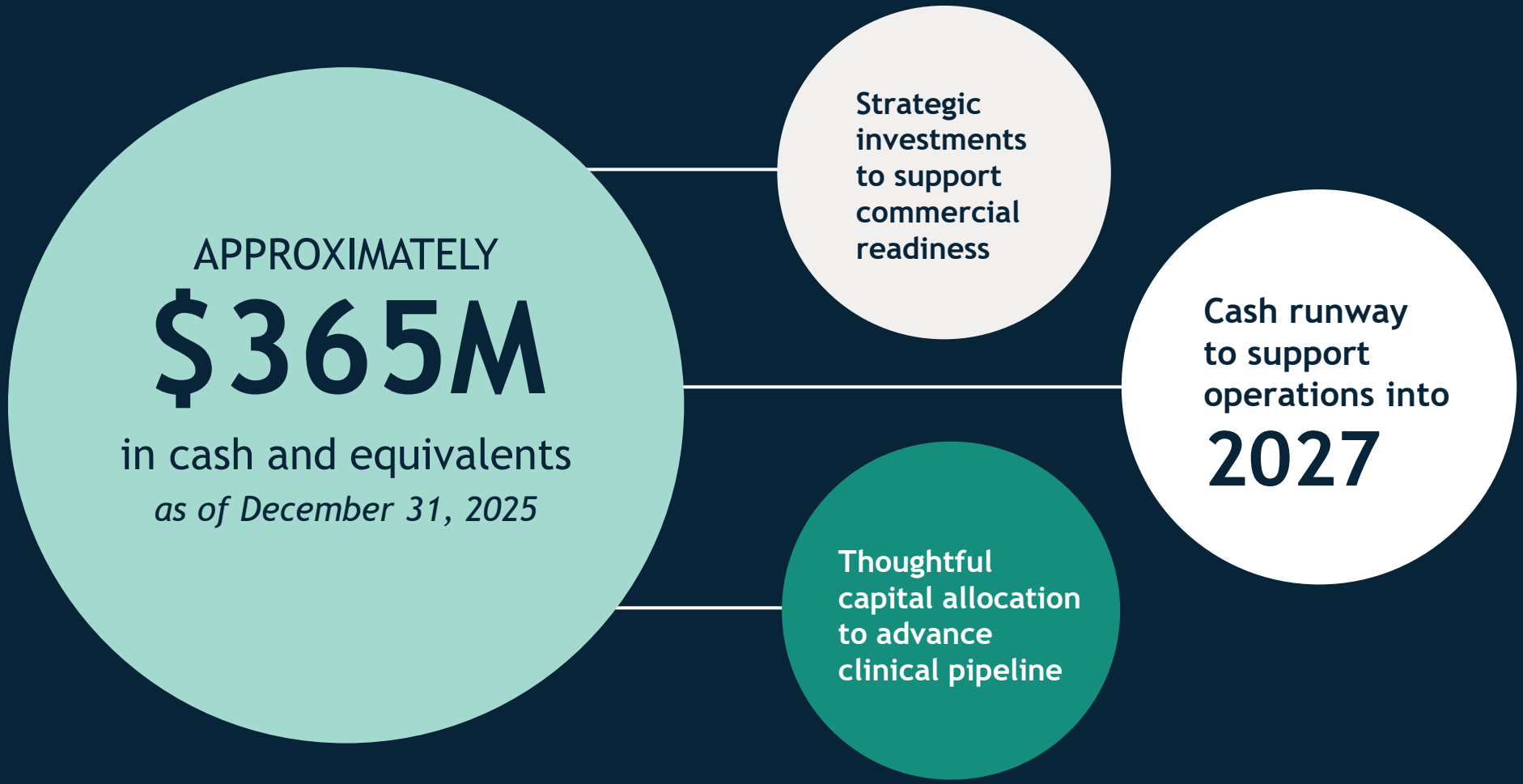
- IND cleared in **November 2025**
- Dosing commenced in Phase 1 study in **December 2025**
- On track to report topline Phase 1 data in **H2 2026**

PK/PD, pharmacokinetic/pharmacodynamic.

# Financials & Upcoming Milestones

# Fortified balance sheet in 2025, strong cash position entering 2026

*Operating with financial discipline to achieve Scholar Rock's ambitions*




Financial information as of December 31, 2025 has not been audited and has been prepared by, and is the responsibility of management. This information could change as a result of further review.

# Scholar Rock 2026 Priorities

*Poised to be next global biotech powerhouse*

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**BLA resubmission and U.S. launch following FDA approval for children and adults with SMA**

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**Initiate apitegromab Phase 2 FORGE study in patients 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, with topline data expected in H2 2026**

**Driving value through focused execution and financial discipline**

1. Upon U.S. FDA approval. NMDs, neuromuscular diseases.

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