UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event Reported): January 12, 2026

Scholar Rock Holding Corporation

(Exact Name of Registrant as Specified in Charter)

Delaware	**001-38501**	**82-3750435**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(I.R.S. Employer Identification Number)

301 Binney Street, 3rd Floor, Cambridge, MA 02142
(Address of Principal Executive Offices) (Zip Code)

(857) 259-3860
(Registrant's telephone number, including area code)

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.001 per share	SRRK	The Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR §230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR §240.12b-2). Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02 Results of Operations and Financial Condition.

On January 12, 2026, Scholar Rock Holding Corporation (the "Company") issued a press release announcing its preliminary cash balance as of December 31, 2025, a business update and further details on its 2026 strategic priorities.

Although it has not finalized its full financial results for the fourth quarter and fiscal year ended December 31, 2025, the Company announced on January 12, 2026, that it estimates it had approximately $365 million (unaudited) of cash, cash equivalents and marketable securities as of December 31, 2025.

This financial information is unaudited and preliminary and does not present all information necessary for an understanding of the Company's financial condition as of December 31, 2025 and its results of operations for the fourth quarter and fiscal year ended December 31, 2025. The audit of the Company's consolidated financial statements for the year ended December 31, 2025 is ongoing and could result in changes to the information set forth above.

The information contained in Item 2.02 of this Current Report on Form 8-K is being furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act") or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended (the "Securities Act"), or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 7.01. Regulation FD Disclosure.

On January 12, 2026, the Company issued a press release announcing business updates and further details on its 2026 strategic priorities. A copy of the press release is furnished as Exhibit 99.1 to this Current Report on Form 8-K.
The information contained in Item 7.01 and Exhibit 99.1 of this Current Report on Form 8-K is being furnished and shall not be deemed "filed" for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 8.01. Other Events.

The Company will be conducting meetings with participants attending the 44th Annual J.P. Morgan Healthcare Conference (the "Conference") during the week of January 12, 2026. A copy of the slides to be presented by the Company at the Conference is filed as Exhibit 99.2 to this Current Report on Form 8-K. The Company undertakes no obligation to update, supplement or amend the materials attached hereto as Exhibit 99.2.

Forward-Looking Statements

This Current Report on Form 8-K contains "forward-looking statements" within the meaning of the federal securities laws, including Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. In this context, forward-looking statements often address expected future business and financial performance and financial condition, and often contain words such as "anticipate," "believe," "expect," "intend," "plan," "seek," "see," "will," "would," "may," "target," and similar expressions and variations or negatives of these words. Forward-looking statements by their nature address matters that are, to different degrees, uncertain, such as statements regarding the Company'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. These and other forward-looking statements are not guarantees of future results and are subject to risks, uncertainties and assumptions that could cause actual results to differ materially from those expressed in any forward-looking statements. Important risk factors that may cause such a difference include, but are not limited to the risks described in the Company's most recent quarterly report on Form 10-Q for the fiscal quarter ended September 30, 2025. We undertake no obligation to update any forward-looking statements as a result of new information, events or

circumstances or other factors arising or coming to our attention after the date hereof. The provision of the information in this report shall not be deemed an admission as to the materiality of any of the information contained herein.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	**Description**
99.1	Press release issued by Scholar Rock Holding Corporation on January 12, 2026, furnished herewith.
99.2	Corporate Presentation, dated January 12, 2026.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Scholar Rock Holding Corporation

Date: January 12, 2026

By: /s/ Junlin Ho
Junlin Ho
General Counsel & Corporate Secretary

Scholar Rock Highlights 2026 Strategic Priorities

- *Apitegromab Biologics License Application (BLA) resubmission and U.S. launch, following FDA approval, are anticipated in 2026 for the treatment of children and adults with spinal muscular atrophy (SMA)*
- *FDA has scheduled meeting with Catalent Indiana, LLC (part of Novo Nordisk) in early 2026 to discuss progress of remediation efforts*
- *Apitegromab Investigational New Drug (IND) application cleared for facioscapulohumeral muscular dystrophy (FSHD); Phase 2 study initiation and patient dosing expected mid-2026*
- *Subcutaneous apitegromab advancing; Phase 1 study demonstrated favorable bioavailability, with pharmacodynamic profile comparable to IV administration*
- *SRK-439 Phase 1 trial ongoing in healthy volunteers; topline data expected in H2 2026*
- *Approximately $365 million[1] in cash as of December 31, 2025; cash runway expected to support operations into 2027*

CAMBRIDGE, Mass. — January 12, 2026 — Scholar Rock (NASDAQ: SRRK), a global biopharmaceutical company dedicated to dramatically improving the lives of children and adults with spinal muscular atrophy (SMA) and additional rare, severe, and debilitating neuromuscular diseases by applying its leading platform in myostatin biology to advance musculoskeletal health, today provided a corporate update and highlighted its strategic priorities for 2026.

"We believe 2026 will be a transformative year for Scholar Rock. We are executing with urgency, and we have strong momentum across our rare, severe neuromuscular disease programs as we build our foundation to become the next global biotech powerhouse," said David L. Hallal, Chairman and Chief Executive Officer of Scholar Rock. "Our foremost objective remains bringing apitegromab to children and adults with SMA as quickly as possible, and we are confident in the steps we are taking to support a BLA resubmission and a U.S. commercial launch, following approval, this year."

2026 Strategic Priorities

Scholar Rock is focused on three core strategic priorities in 2026 to create sustainable long-term value and maximize the potential of its innovative anti-myostatin platform to serve patients:

- FDA and European Medicines Agency (EMA) regulatory approvals and commercialization of apitegromab for children and adults with SMA.
- Develop apitegromab for patients with SMA (<2 years old) and for additional rare, severe neuromuscular diseases.

---

[1] Unaudited, estimated cash, cash equivalents and marketable securities as of December 31, 2025.

- Advance world-leading anti-myostatin pipeline with SRK-439 in addition to apitegromab.

FDA and EMA regulatory approvals and commercialization of apitegromab for children and adults with SMA

Scholar Rock remains focused on bringing apitegromab, the world's first and only muscle-targeted treatment candidate to improve motor function in patients with SMA, to patients as quickly as possible.

- **BLA resubmission and U.S. launch, following FDA approval, are anticipated in 2026.** Scholar Rock continues to work closely with the FDA and Catalent Indiana LLC, part of Novo Nordisk, to progress remediation activities at the Bloomington, Indiana fill-finish facility to support BLA resubmission as rapidly as possible.
- **EMA regulatory review ongoing**. The Company continues to expect a decision on its apitegromab Marketing Authorisation Application (MAA) in mid-2026.
- **U.S. and European launch preparations ongoing**. U.S. and European customer-facing teams are in the field and continue to engage with key stakeholders on SMA disease awareness and education initiatives, including the unmet need in SMA and the importance of targeting muscle.
- **Second U.S. fill-finish facility commercial capacity reserved beginning Q1 2026.** Scholar Rock is advancing activities at a second U.S.-based fill-finish facility to strengthen supply continuity and support future commercial demand. This facility manufactures commercially available, FDA-approved products and has a strong regulatory inspection track record. The Company expects to submit a supplemental BLA (sBLA) with this second site later in 2026.

Develop apitegromab for patients with SMA (<2 years old) and for additional rare, severe neuromuscular diseases

As part of Scholar Rock's long-term strategy, the Company is developing apitegromab for patients with SMA under two years of age and for additional rare, severe, and debilitating neuromuscular diseases.

- **Phase 2 OPAL clinical trial underway**. The Phase 2 OPAL trial is designed to evaluate apitegromab in infants and toddlers with SMA under two years of age who have received an approved SMN1-targeted gene therapy or who are receiving ongoing treatment with an approved SMN2-targeted therapy. The trial is enrolling participants and patient dosing is underway.
- **Phase 2 FORGE trial on track to initiate in mid-2026**. Scholar Rock is developing apitegromab for the treatment of people living with facioscapulohumeral muscular dystrophy (FSHD). FSHD is a rare, progressive neuromuscular disease characterized by muscle atrophy and functional decline, affecting approximately 30,000 individuals across the U.S. and Europe. The IND application is cleared, and the Company plans to initiate a Phase 2 randomized, double-blind, placebo-controlled trial, called FORGE, in mid-2026.

Advance world-leading anti-myostatin pipeline with SRK-439 in addition to apitegromab

As the first and only company to deliver a successful Phase 3 study with a myostatin inhibitor, Scholar Rock is building a robust pipeline of anti-myostatin product candidates to serve patients by addressing a broad range of rare, severe, and debilitating neuromuscular diseases.

- **Subcutaneous apitegromab Phase 1 study complete**. Scholar Rock is advancing a subcutaneous presentation of apitegromab. This formulation is intended to provide optionality for patients as a small volume, self- or caregiver-administered anti-myostatin antibody suitable for an autoinjector. A Phase 1 study in healthy volunteers has been completed, and further development activities are ongoing, including planned FDA and EMA regulatory engagements.
- **Dosing underway in SRK-439 Phase 1 healthy volunteer study**. SRK-439 is a novel, investigational, subcutaneously administered myostatin inhibitor that binds to pro- and latent myostatin with high affinity and selectivity (i.e., no GDF11 or Activin A binding). SRK-439 has demonstrated the potential to potently inhibit myostatin and increase muscle mass in preclinical studies. A Phase 1 study in healthy volunteers is underway, and topline data are expected in the second half of 2026.

Corporate Updates

As of December 31, 2025, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $365 million. This cash is expected to fund operations into 2027.

2025 Highlights and Accomplishments

- **Progressed U.S. and European regulatory activities for apitegromab for the treatment of children and adults with SMA.** Scholar Rock submitted a BLA to the FDA in January 2025, received Priority Review designation in March 2025, and achieved acceptance of the EMA MAA in March 2025, representing significant regulatory milestones toward commercialization. The Company completed a constructive and collaborative in-person Type A meeting with the FDA in November 2025, following the receipt of a Complete Response Letter (CRL) from FDA on September 22, 2025.
- **Transformed leadership team.** Appointed 8-year Board Chairman, David Hallal, as Chief Executive Officer; Akshay Vaishnaw, M.D., Ph.D. as President of R&D; R. Keith Woods as Chief Operating Officer; Vikas Sinha as Chief Financial Officer; and Rebecca McLeod as Chief Brand Officer and U.S. General Manager, strengthening the Company's leadership as it transitions toward a global commercial-stage organization.
- **Initiated U.S. and European build-out of commercial organization.** Established a lean, experienced U.S. customer-facing team of approximately 50 professionals with deep expertise in neurology and rare disease. Initiated commercial build-out in Europe to support the planned European launch, starting with Germany.
- **Advanced industry-leading anti-myostatin pipeline.** Advanced Scholar Rock's strategic plan to build a robust pipeline of therapies for the treatment of people living with rare, severe, and debilitating neuromuscular diseases.

- Initiated dosing in the Phase 2 OPAL study evaluating apitegromab in infants and toddlers under 2 years of age with SMA
- Advanced preclinical development of apitegromab for FSHD and filed an IND application to support the initiation of a Phase 2 study
- Completed a subcutaneous apitegromab Phase 1 study in healthy volunteers
- Initiated dosing in a Phase 1 study evaluating SRK-439 study in healthy volunteers
- Continued the ongoing ONYX open-label extension study evaluating the long-term safety and efficacy of apitegromab in patients with SMA who participated in the TOPAZ and SAPPHIRE clinical programs
- Completed the Phase 2 EMBRAZE study, demonstrating proof-of-concept in the ability of apitegromab to drive statistically significant preservation of lean mass during tirzepatide-induced weight loss

J.P. Morgan Healthcare Conference Presentation and Webcast

Scholar Rock management will discuss these updates in a corporate presentation at the 44th Annual J.P. Morgan Healthcare Conference on Monday, January 12, 2026 at 7:30 a.m. PST (10:30 a.m. EST). A live webcast of the presentation can be accessed by visiting the Investors & Media section of the Scholar Rock website at http://investors.scholarrock.com. An archived replay of the webcast will be available on the Company's website for approximately 90 days following the presentation.

About Scholar Rock

Scholar Rock is a late-stage biopharmaceutical company focused on developing and commercializing apitegromab for children and adults with spinal muscular atrophy (SMA) and other rare, severe, and debilitating neuromuscular diseases. As a global leader in myostatin biology, a field focused on proteins that regulate muscle mass, the biopharmaceutical company is named for the visual resemblance of a scholar rock to protein structures. Our commitment to unlock fundamentally different treatment approaches is powered by broad application of a proprietary platform, which has developed novel monoclonal antibodies to modulate protein growth factors with extraordinary selectivity. Scholar Rock works every day to create new possibilities for patients through its highly innovative anti-myostatin program, including opportunities in additional rare neuromuscular diseases. Learn more at ScholarRock.com and follow @ScholarRock on X and on LinkedIn.

Scholar Rock® is a registered trademark of Scholar Rock, Inc.

Availability of Other Information About Scholar Rock

Investors and others should note that we communicate with our investors and the public using our company website www.scholarrock.com, including, but not limited to, company disclosures, investor presentations and FAQs, Securities and Exchange Commission filings, press releases, public conference call transcripts and webcast transcripts, as well as on X (formerly known as

Twitter) and LinkedIn. The information that we post on our website or on X (formerly known as Twitter) or LinkedIn could be deemed to be material information. As a result, we encourage investors, the media and others interested to review the information that we post there on a regular basis. The contents of our website or social media shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

Forward-Looking Statements

This press release 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, 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," "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 preclinical and clinical data, including the results from the Phase 2 or Phase 3 clinical trial of apitegromab, or the preclinical 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 Novo Nordisk Bloomington Indiana fill finish facility in response to the FDA Observations, whether Scholar Rock will be able to resubmit its BLA in a timely manner and 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; 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 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.

Scholar Rock Contacts

Investors

Laura Ekas, Ph.D.

ir@scholarrock.com

917-439-0374

Media

Molly MacLeod, Ph.D.

media@scholarrock.com

802-579-5995

Exhi

44th Annual J.P. Morgan Healthcare Conference

Scholar Rock Investor Presentation

JANUARY 12, 2026

© 2026 Scholar Rock, Inc. All rights reserved.

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 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.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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 apitegron in facioscapulohume muscular dystroph

APPROXIMATELY $365M

in cash and cash equivalents as o 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.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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

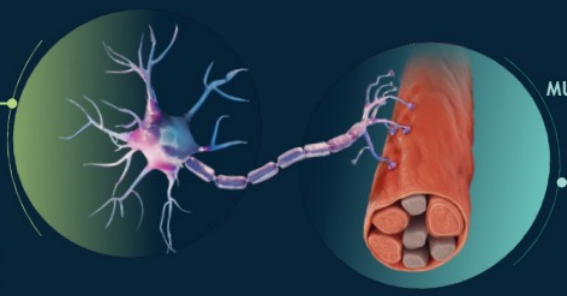
NEXT PHASE OF INNOVATION: MUSCLE

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

SPINRAZA (nusinersen) · zolgensma (onasemnogene abeparvovec-xioi) · Evrysdi (risdiplam) · itvisma (onasemnogene abeparvovec-brve)

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.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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 o 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.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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



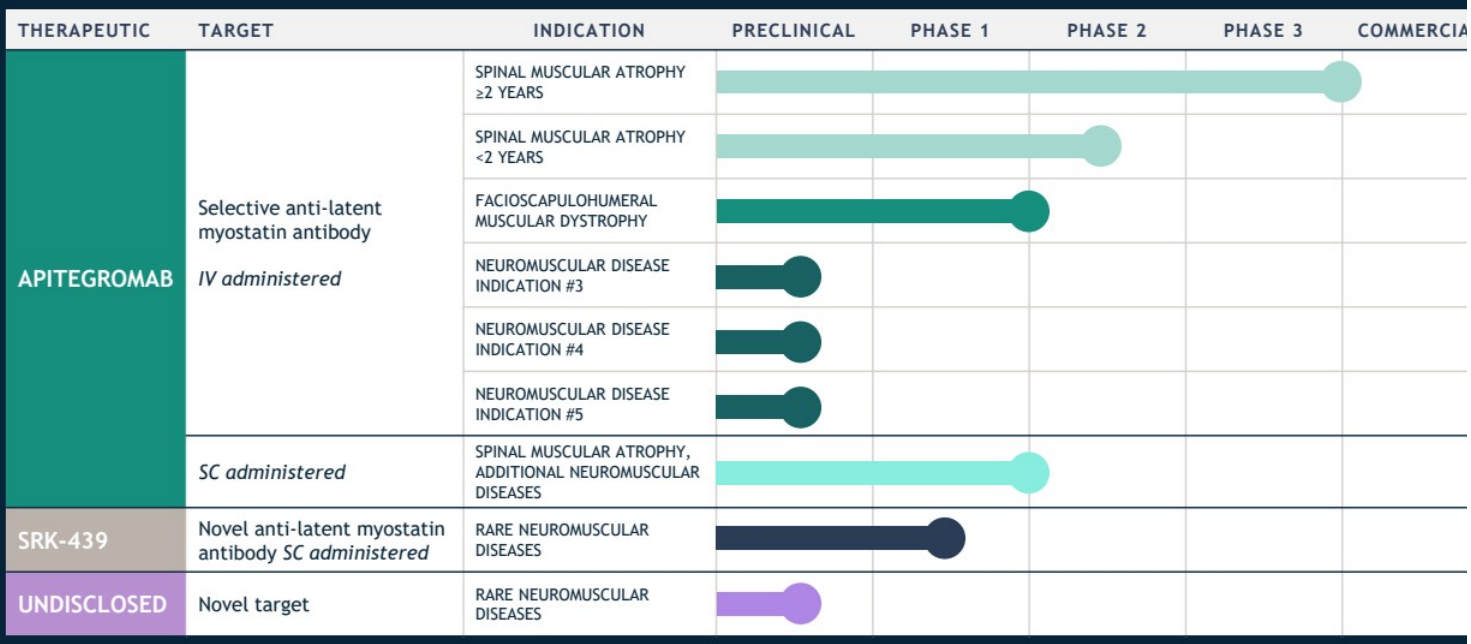
Building a 50-country platform to serve patients with rare, severe neuromuscular diseases

NOTE: Following regulatory approval(s).

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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



IV, Intravenously; SC, Subcutaneously.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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-myostat pipeline

Progress subcutaneous apitegromal 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.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

Commercialize Apitegromab for Treatment of Patients with SMA

Transforming the treatment of SMA with muscle-targeted therapy

© 2026 Scholar Rock, Inc. All rights reserved.

ScholarRock

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**

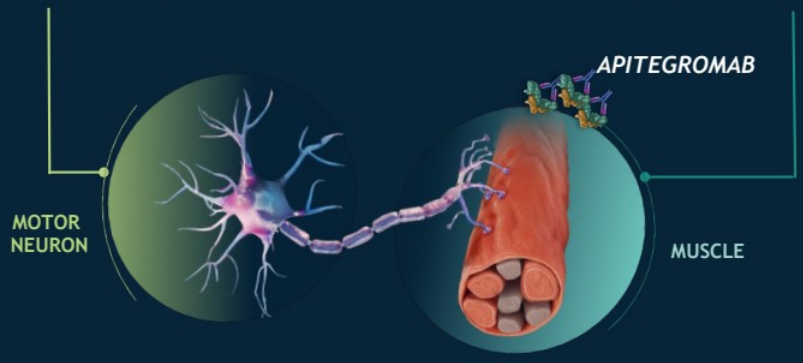
SMN-Targeted Therapies

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.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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 moto
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.

ScholarRock

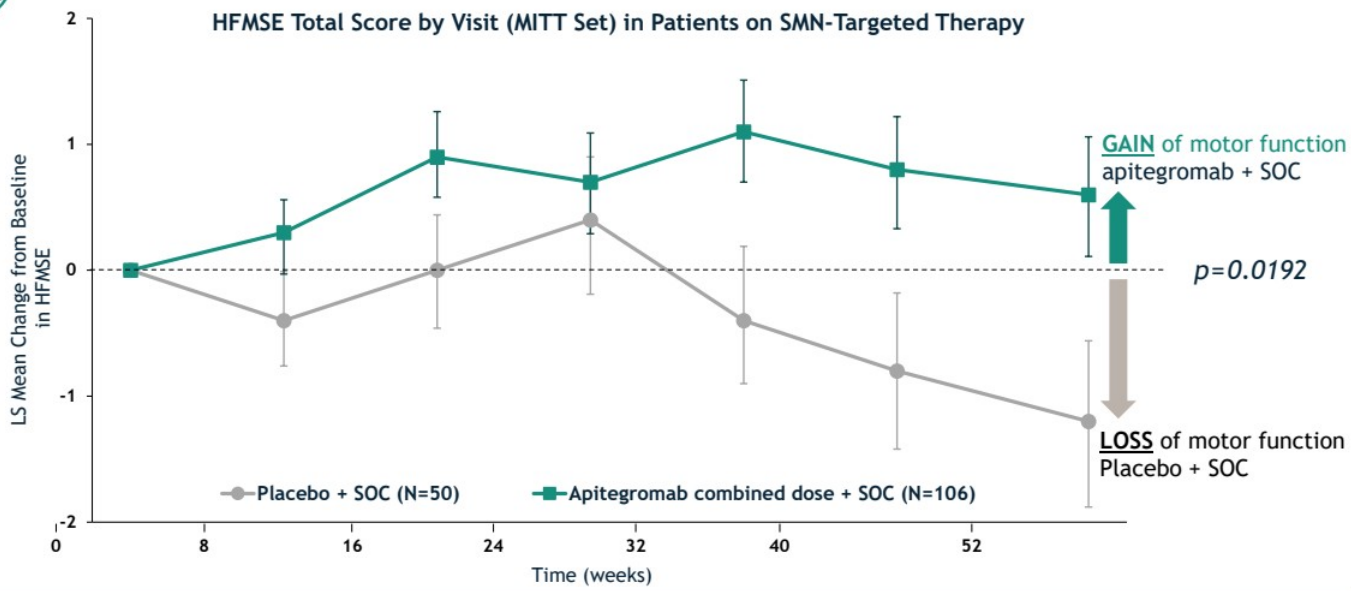
© 2026 Scholar Rock, Inc. All rights reserved

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 risdip



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;

© 2026 Scholar Rock, Inc. All rights reserved

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 benefi** 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).

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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 202

BLA, Biologics License Application; OAI, Official Action Indicated

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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 reimbursemen 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.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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

© 2026 Scholar Rock, Inc. All rights reserved.

ScholarRock

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.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

FSHD: Rare, devastating NMD with significant unmet need

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

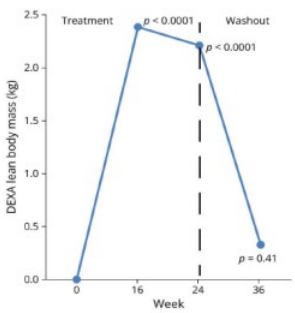
FSHD results in progressive muscle atrophy leading to cumulative loss of function and loss of independence

- 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

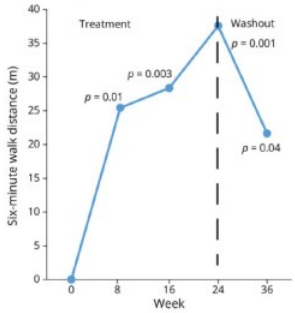
Support for apitegromab therapeutic hypothesis in FSH

- 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.

ScholarRock

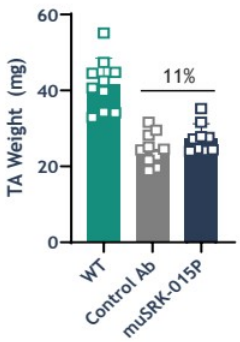
© 2026 Scholar Rock, Inc. All rights reserved

Preclinical studies provide mechanistic rationale for apitegromab in FSH

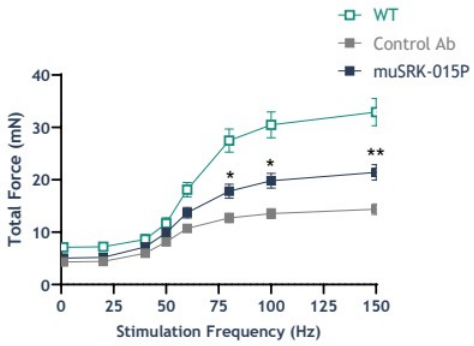
Increased muscle mass, strength, and endurance in gold standard FLExDUX4 mouse model of FSHD

FSHD FLExDUX4 Mouse Model

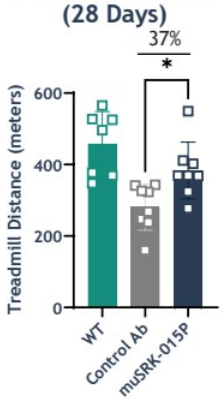
Robust Increase in Muscle Mass (28 Days)



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.*

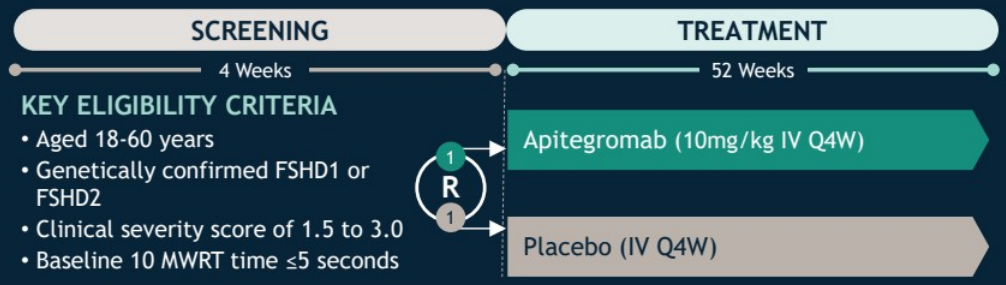
*Muscle mass=weight of tibialis anterior muscle; muSRK-015P is a murine version of apitegromab.
FLExD, FLExDUX4.Cre; FSHD, facioscapulohumeral muscular dystrophy; WT, wild type; Ab, antibody. Fogel A. Presented at: FSHD Society International Research Congress; Jun 12-13, 2025; Amsterdam, Netherlands.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

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

FORGE Phase 2 trial evaluating apitegromab in patients with FSH

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:

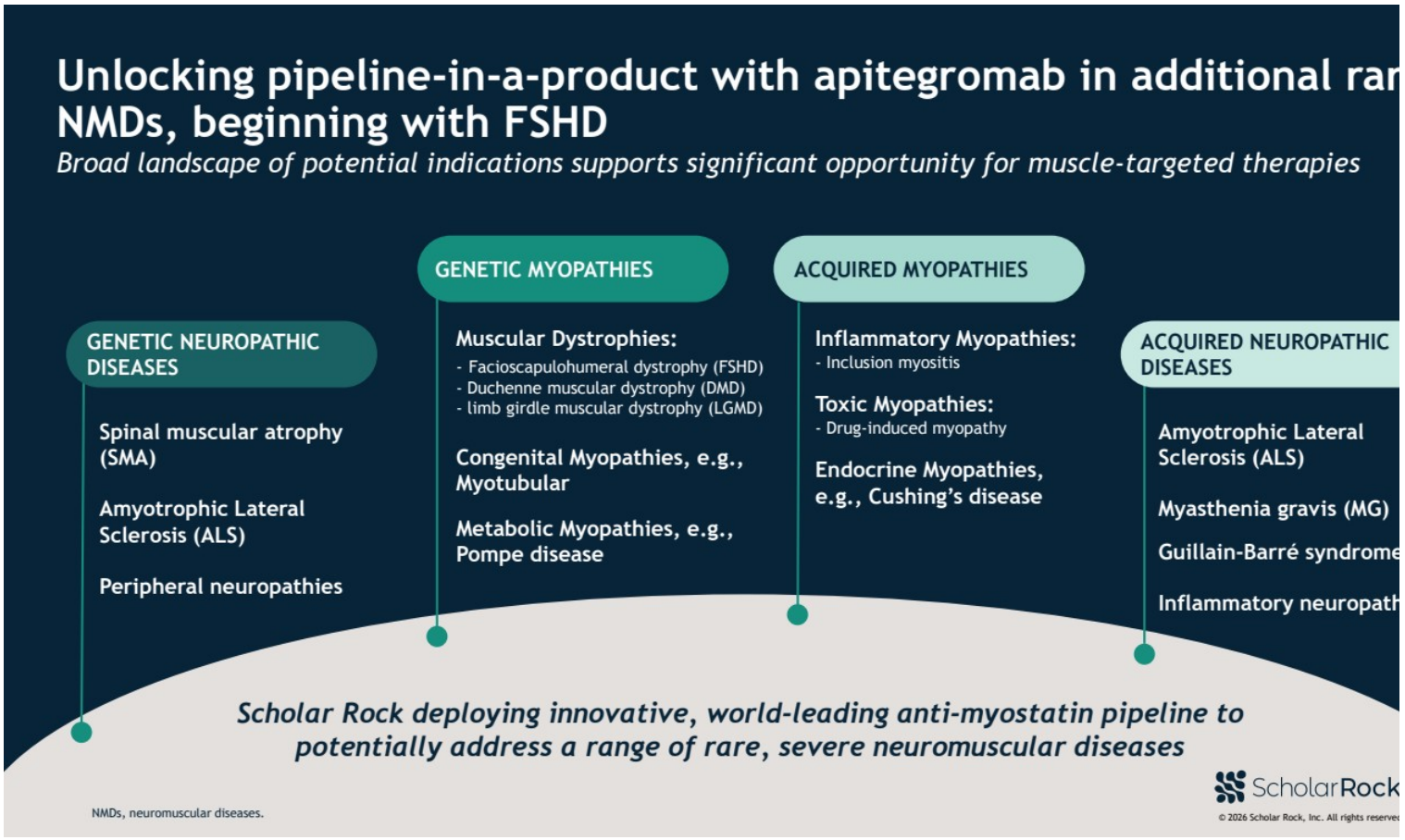
- Mean LMV change from baseline at 6 months
- Mean change from baseline in addition muscle parameters (6 and 12 months)
- Quantitative myometry testing (QMT)
- Safety, PK/PD, ADA

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.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved


Unlocking pipeline-in-a-product with apitegromab in additional rar
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 neuropath
Scholar Rock deploying innovative, world-leading anti-myostatin pipeline to potentially address a range of rare, severe neuromuscular diseases
NMDs, neuromuscular diseases.
ScholarRock
© 2026 Scholar Rock, Inc. All rights reserved

Advance World-Leading Anti-Myostatin Pipeline

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

NEXT PHASE OF INNOVATION: MUSCLE

2026 — 2030

- Apitegromab U.S. launch
- Apitegromab European launch
- Apitegromab global expansion
- SC Apitegromab global launc
- SRK-439 global launch



ScholarRock

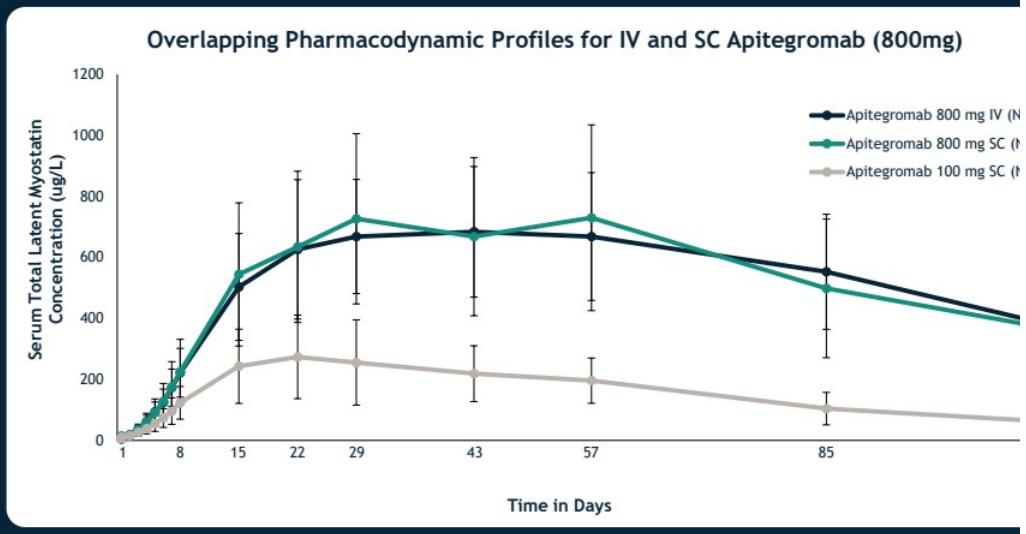
© 2026 Scholar Rock, Inc. All rights reserved.

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.

ScholarRock

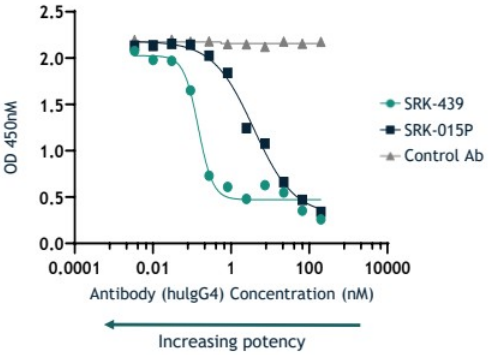
© 2026 Scholar Rock, Inc. All rights reserved

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

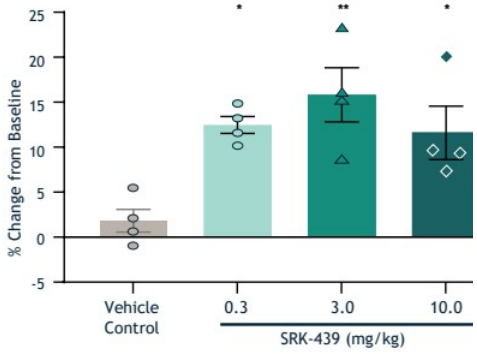
Leveraging world-leading expertise to drive continued innovation

- **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)

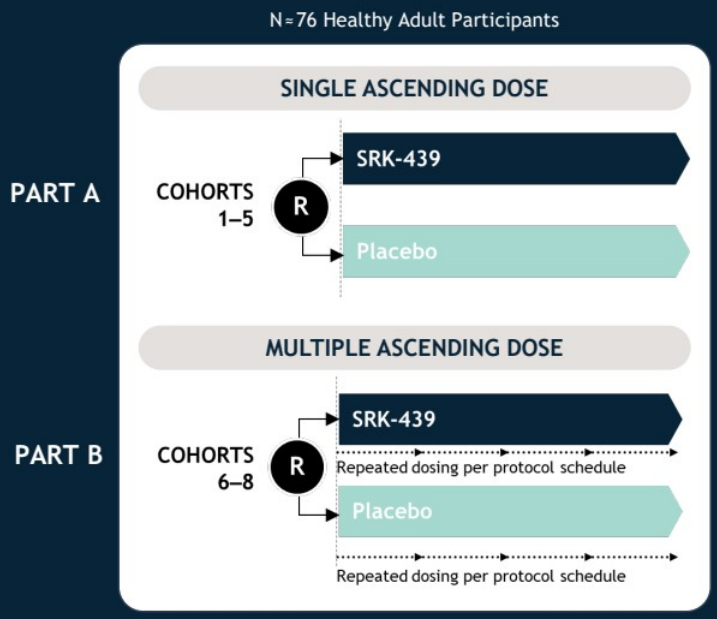


SRK-439 is a novel, investigational myostatin inhibitor. muSRK-015P is a murine version of apitegromab.

ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

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



PK/PD, pharmacokinetic/pharmacodynamic.

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**

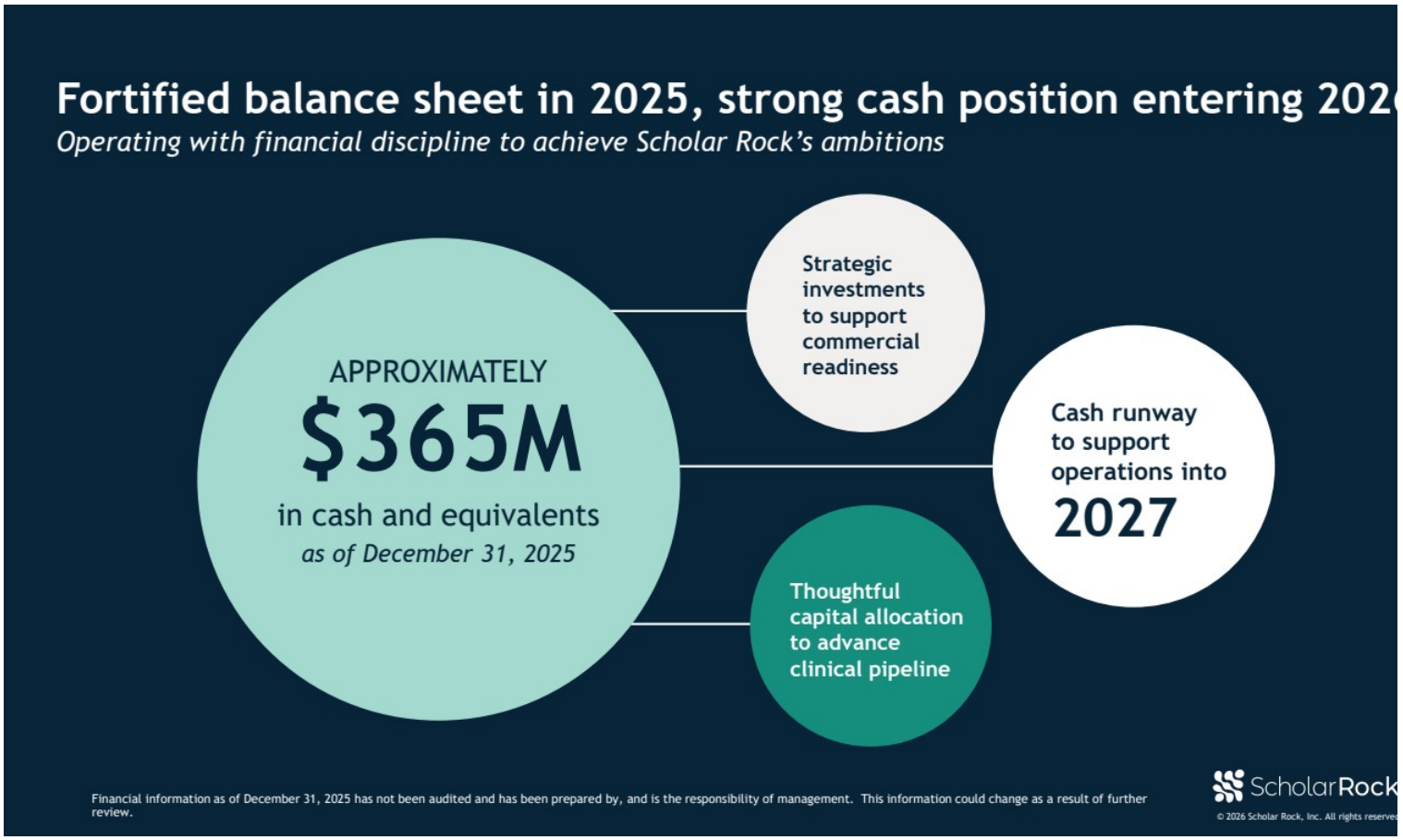
ScholarRock

© 2026 Scholar Rock, Inc. All rights reserved

Financials & Upcoming Milestones

© 2026 Scholar Rock, Inc. All rights reserved.

ScholarRock


Fortified balance sheet in 2025, strong cash position entering 202
Operating with financial discipline to achieve Scholar Rock's ambitions
APPROXIMATELY
$365M
in cash and equivalents
as of December 31, 2025
Strategic investments to support commercial readiness
Cash runway to support operations into 2027
Thoughtful capital allocation to advance clinical pipeline
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.
ScholarRock
© 2026 Scholar Rock, Inc. All rights reserved


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
BLA resubmission and U.S. launch following FDA approval for children and adults with SMA
EMA decision 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-myostat pipeline
Progress subcutaneous apitegromal 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.
ScholarRock
© 2026 Scholar Rock, Inc. All rights reserved

ScholarRock®

For more information, please contact:
ir@scholarrock.com
media@scholarrock.com
Or visit us at www.scholarrock.com

© 2026 Scholar Rock, Inc. All rights r