Scholar Rock Highlights 2026 Strategic Priorities

January 12, 2026

- *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.--(BUSINESS WIRE)--Jan. 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.
- 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 trial 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.

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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.

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

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