Scholar Rock to Present Comprehensive Update at 2025 Annual Cure SMA Research and Clinical Care Meeting, Including Positive Results from Pivotal Phase 3 SAPPHIRE Trial

June 23, 2025

- *Oral presentation on the positive Phase 3 SAPPHIRE trial of apitegromab for people living with spinal muscular atrophy (SMA), which demonstrated statistically significant improvement and clinically meaningful benefit as measured by the Hammersmith Functional Motor Scale Expanded (HFMSE)*
- *Apitegromab Biologics License Application (BLA) accepted under priority review for patients with SMA, and U.S. Food and Drug Administration (FDA) Prescription Drug User Fee Act (PDUFA) date set for September 22*
- *European Medicines Agency (EMA) validated Marketing Authorisation Application (MAA) for apitegromab for patients with SMA*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 23, 2025-- Scholar Rock (NASDAQ: SRRK), a late-stage biopharmaceutical company focused on developing and commercializing apitegromab for patients with spinal muscular atrophy (SMA) and other severe and debilitating neuromuscular diseases, announced today that it will present data from its Phase 3 SAPPHIRE clinical trial **(NCT05156320)** in an oral presentation at Cure SMA's Annual SMA Research & Clinical Care Meeting being held June 25-27, 2025, in Anaheim, California.

SAPPHIRE evaluated the safety and efficacy of apitegromab, an investigational muscle-targeted treatment that is being developed to provide clinically meaningful improvement in motor function for people living with SMA who are receiving SMN-targeted treatments. Scholar Rock **previously shared positive topline data** from the trial in October 2024 and **announced** in March 2025 that the U.S. FDA accepted its BLA for apitegromab. The application has a priority review designation, and the FDA has assigned a PDUFA target action date of September 22, 2025.

Details of the oral presentation are as follows:

Title: Efficacy and Safety of Apitegromab in Individuals with Type 2 and Type 3 Spinal Muscular Atrophy Evaluated in the Phase 3 SAPPHIRE Trial
Presentation type: Oral presentation
Presenter: Basil T. Darras, M.D., Associate Neurologist-in-Chief, Boston Children's Hospital and Professor of Neurology, Harvard Medical School
Location: Sequoia Ballroom, Disney's Grand Californian Hotel
Date and time: Friday, June 27, 11:20 a.m. PDT

Scholar Rock will also present a poster on a systematic literature review of characterization of the longer-term effectiveness of SMN-targeted therapies for SMA. The details of that poster presentation are as follows:

Title: Characterization of the Longer-Term Effectiveness of SMN-Targeted Treatments for Spinal Muscular Atrophy: A Systematic Literature Review
Presentation type: Poster presentation
Presenter: Jena M. Krueger, M.D., Helen DeVos Children's Hospital Grand Rapids, Michigan
Location: Frontier Tower Magic Kingdom Rooms 2 & 3, Disneyland Hotel
Date and time: Wednesday, June 25, 4:30-6:30 p.m. PDT

The presentations will be made available in the **Publications & Posters section** of Scholar Rock's website following the conference.

For conference information, visit **https://www.curesma.org/annual-sma-conference**.

About Apitegromab

Apitegromab is an investigational fully human monoclonal antibody inhibiting myostatin activation by selectively binding the pro- and latent forms of myostatin in the skeletal muscle. It is the first muscle-targeted treatment candidate in spinal muscular atrophy (SMA) to demonstrate clinical success in a pivotal phase 3 clinical trial. Additionally, in the EMBRAZE Phase 2 proof-of-concept trial in obesity, patients receiving apitegromab dosed at 10mg/kg with tirzepatide over 24 weeks showed a statistically significant preservation of lean mass relative to tirzepatide alone. Myostatin, a member of the TGFβ superfamily of growth factors, is expressed primarily by skeletal muscle cells, and the absence of its gene is associated with an increase in muscle mass and

strength in multiple animal species, including humans. Scholar Rock believes that its highly selective targeting of pro- and latent forms of myostatin with apitegromab may lead to a clinically meaningful improvement in motor function in patients with SMA. The U.S. Food and Drug Administration (FDA) has granted Fast Track, Orphan Drug and Rare Pediatric Disease designations, and the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) and Orphan Medicinal Product designations, to apitegromab for the treatment of SMA. Apitegromab has not been approved for any use by the FDA or any other regulatory agency.

About the Phase 3 SAPPHIRE Trial

SAPPHIRE was a randomized, double-blind, placebo-controlled Phase 3 clinical trial that evaluated the safety and efficacy of apitegromab in nonambulatory patients with Types 2 and 3 SMA who were receiving current standard of care (either nusinersen or risdiplam). SAPPHIRE enrolled 156 patients aged 2-12 years old in the main efficacy population. These patients were randomized 1:1:1 to receive either apitegromab 10 mg/kg, apitegromab 20 mg/kg, or placebo by intravenous (IV) infusion every 4 weeks for 12 months. An exploratory population including 32 patients aged 13-21 years old was also evaluated. These patients were randomized 2:1 to receive either apitegromab 20 mg/kg or placebo every 4 weeks for 12 months.

The SAPPHIRE trial met its primary endpoint for the main efficacy population with a statistically significant 1.8-point improvement (p=0.0192) based on apitegromab combined dose (10 mg/kg and 20 mg/kg) and standard of care (SOC) versus placebo and SOC as measured by the Hammersmith Functional Motor Scale-Expanded at week 52 (**additional details in the topline data release**).

About SMA

Spinal muscular atrophy (SMA) is a rare, genetic neuromuscular disease that afflicts an estimated 30,000 to 35,000 people in the United States and Europe. The disease is characterized by the loss of motor neurons, atrophy of the voluntary muscles of the limbs and trunk, and progressive muscle weakness. While there has been progress in the development of therapeutics that address the loss of motor neurons, there continues to be a high unmet need for therapies that directly address the progressive muscle weakness that leads to loss of motor function in SMA.

About Scholar Rock

Scholar Rock is a biopharmaceutical company that discovers, develops, and delivers life-changing therapies for people with serious diseases that have high unmet need. As a global leader in the biology of the transforming growth factor beta (TGFβ) superfamily, the company is named for the visual resemblance of a scholar rock to protein structures. Over the past decade, Scholar Rock has created a pipeline with the potential to advance the standard of care for neuromuscular disease, cardiometabolic disorders, cancer, and other conditions where growth factor-targeted drugs can play a transformational role.

This commitment to unlocking fundamentally different therapeutic approaches is powered by broad application of a proprietary platform, which has developed novel monoclonal antibodies to modulate protein growth factors with extraordinary selectivity. By harnessing cutting-edge science in disease spaces that are historically under-addressed through traditional therapies, Scholar Rock works every day to create new possibilities for patients. Learn more about our approach at ScholarRock.com and follow @ScholarRock and on LinkedIn.

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 plans for apitegromab. 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 the results from the Phase 3 clinical trial of apitegromab, 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, and may not be sufficient for 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 studies and clinical trials; 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; Scholar Rock's dependence on third parties for development and manufacture of product

candidates including, without limitation, supply for apitegromab; and Scholar Rock's ability to manage expenses and to obtain additional funding when needed to support its business activities and establish and maintain strategic business alliances; its ability to obtain regulatory approval of apitegromab; and the anticipated commercial launch in the United States of apitegromab in the fourth quarter of 2025 and new business initiatives; as well as those risks more fully discussed in the section entitled "Risk Factors" in Scholar Rock's Quarterly Report on Form 10-Q for the quarter ended March 31, 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.

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