# Scholar Rock to Present Additional Clinical Data from the Phase 3 SAPPHIRE Trial at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference

February 21, 2025

- *Oral presentation to include additional efficacy data from the Phase 3 SAPPHIRE trial of apitegromab in patients with spinal muscular atrophy (SMA)*
- *Poster presentations include preclinical data on a combination treatment approach for building muscle mass and strength in a Duchenne muscular dystrophy (DMD) model*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 21, 2025-- Scholar Rock (NASDAQ: SRRK), a late-stage biopharmaceutical company focused on advancing innovative treatments for spinal muscular atrophy (SMA), cardiometabolic disorders, and other serious diseases where protein growth factors play a fundamental role, announced today that it will present additional data from its Phase 3 SAPPHIRE clinical trial (NCT05156320) at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference being held March 16-19 in Dallas, Texas.

SAPPHIRE evaluated the safety and efficacy of apitegromab, an investigational muscle-targeted therapy designed and 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 the submission of a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for apitegromab in January 2025. A detailed analysis of the study will be presented as an oral presentation by Thomas O. Crawford, M.D., Professor of Neurology and Pediatrics at Johns Hopkins University on March 19th, as well as through an accompanying poster.

In addition, Scholar Rock will present a poster sharing preclinical data on an investigational approach combining a preclinical version of apitegromab with a dystrophin corrector for addressing muscle defects in a Duchenne muscular dystrophy (DMD) model. That poster will be presented by Adam I. Fogel, Ph.D., Director of Discovery Biology at Scholar Rock.

“We look forward to the data from our positive Phase 3 SAPPHIRE trial of apitegromab in patients with spinal muscular atrophy being featured at the upcoming Muscular Dystrophy Association Clinical and Scientific Conference, along with promising preclinical data that show the therapeutic potential for apitegromab in DMD," said Jay Backstrom, M.D., MPH, President and Chief Executive Officer of Scholar Rock. “Building on the success of apitegromab in SMA, we are actively working to enhance our understanding of the role that apitegromab can play in treating other rare neuromuscular disorders such as DMD with the goal to reach more people living with these serious conditions.”

Details of the presentations 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:** Thomas O. Crawford, M.D., Professor of Neurology and Pediatrics, Johns Hopkins University
**Location:** Hilton Anatole Dallas, Coronado ABCD
**Date and time:** Wednesday, March 19, 2025, 10:45-11:00 AM CT

**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:** Poster presentation (Poster #O284)
**Presenter:** Thomas O. Crawford, M.D., Professor of Neurology and Pediatrics, Johns Hopkins University
**Location:** Trinity Exhibit Hall; Hilton Anatole Dallas
**Date and time:** Sunday, March 16 – Tuesday, March 18, 6:00 p.m. - 8:00 p.m. CT

**Title:** muSRK-015 builds muscle mass and strength in combination with dystrophin upregulation in a mouse model of DMD
**Presentation type:** Poster presentation (Poster #P160)
**Presenter:** Adam I. Fogel, Ph.D., Director, Discovery Biology, Scholar Rock
**Location:** Trinity Exhibit Hall; Hilton Anatole Dallas
**Date and time:** Sunday, March 16 – Tuesday, March 18, 6:00 p.m. - 8:00 p.m. CT

The abstracts for these presentations are available on MDA’s website: https://www.mdaconference.org/abstracts/2025-abstract-library/

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.mdaconference.org**.

**About Apitegromab**

Apitegromab is an investigational fully human monoclonal antibody, which inhibits myostatin activation by selectively binding the pro- and latent forms of myostatin in the skeletal muscle. It is the first muscle-targeted treatment candidate to demonstrate clinically meaningful and statistically significant motor function improvement in a pivotal Phase 3 trial in spinal muscular atrophy (SMA). Myostatin, a member of the TGFβ superfamily of growth factors, is expressed primarily by skeletal muscle cells, and its absence is associated with an increase in muscle mass and strength in multiple animal species, including humans. 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 for all patients receiving apitegromab 10 mg/kg and 20 mg/kg (with an SMN-targeted treatment) compared to placebo (with an SMN-targeted treatment), as measured by the Hammersmith Functional Motor Scale-Expanded at week 52.

**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 and named for the visual resemblance of a scholar rock to protein structures, the clinical-stage company is focused on advancing innovative treatments where protein growth factors are fundamental. 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.

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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 Twitter and LinkedIn. The information that we post on our website or on 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 for apitegromab, including the therapeutic potential, clinical benefits and safety thereof, expectations regarding timing, success and data announcements of current ongoing clinical trials, 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, that preclinical and clinical data, including 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 and preclinical 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, to 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 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 September 30, 2024, 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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