Scholar Rock Announces Completion of Enrollment for the Phase 3 SAPPHIRE Trial

September 19, 2023

- *Topline data from Phase 3 trial expected in Q4 2024*
- *Company to present encore 36-month efficacy, safety, and patient-reported outcomes data from the Phase 2 TOPAZ trial extension at the 2023 World Muscle Society (WMS) Annual Congress*
- *Apitegromab is the only muscle-targeted therapy candidate with clinical data showing proof of concept in spinal muscular atrophy*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 19, 2023-- Scholar Rock (NASDAQ: SRRK), a Phase 3 clinical-stage biopharmaceutical company focused on the treatment of serious diseases in which protein growth factors play a fundamental role, announced today that it has completed enrollment of the SAPPHIRE Phase 3 trial evaluating the safety and efficacy of apitegromab in nonambulatory patients with Types 2 and 3 spinal muscular atrophy (SMA) who are receiving SMN therapy (either nusinersen or risdiplam). The company also announced that it plans to present encore data from its Phase 2 TOPAZ trial extension period evaluating patient outcomes in SMA after 36 months of treatment with apitegromab. Efficacy and safety data at 36 months, and an analysis of patient-reported outcome measures such as fatigue, mobility, and activities of daily living, will be presented during two poster presentations at the 28th Annual Congress of the World Muscle Society from October 3 – 7 in Charleston, South Carolina, USA.

“Completing SAPPHIRE enrollment is a critical step on the path to bringing apitegromab, a potentially transformative therapy, to individuals living with SMA. SAPPHIRE was designed to build on the positive TOPAZ results, and we look forward to reporting the topline results from this registrational study in Q4 next year,” said Jay Backstrom, M.D., MPH, President and Chief Executive Officer of Scholar Rock. “The 36-month data from TOPAZ, which reinforced long-term sustained and clinically meaningful improvement in motor function on top of SMN therapy, gives us strong conviction that apitegromab could provide substantial benefit beyond SMN therapy alone.”

Details of the poster presentations are as follows:

Title: Effect of apitegromab on PEDI-CAT and PROMIS-fatigue questionnaire at 36 months in patients with spinal muscular atrophy
Presentation type: Poster presentation
Presenter: Thomas O. Crawford, M.D., Professor of Neurology and Pediatrics, Johns Hopkins University
Date and time: Wednesday, October 4, 2023, 2:30 – 3:30 PM ET
Location: Charleston Area Convention Center, North Charleston, SC; Ballroom A – C

Title: Effect of apitegromab on motor function at 36 months in patients with nonambulatory spinal muscular atrophy aged 2 – 12 years old
Presentation type: Poster presentation
Presenter: Thomas O. Crawford, M.D., Professor of Neurology and Pediatrics, Johns Hopkins University
Date and time: Wednesday, October 4, 2023, 2:30 – 3:30 PM ET
Location: Charleston Area Convention Center, North Charleston, SC; Ballroom A – C

For conference information, visit https://www.wms2023.com/.

The presentations will be made available in the Publications & Posters section of Scholar Rock’s website following the presentation.

About the Phase 2 TOPAZ Trial

The TOPAZ trial is an ongoing proof-of-concept, open-label Phase 2 trial evaluating the safety and efficacy of apitegromab in patients with Types 2 and 3 SMA. In the main treatment period, patients were dosed intravenously every four weeks as monotherapy or with nusinersen, an approved SMN-targeted therapy. The trial enrolled 58 patients in the U.S. and Europe. The primary efficacy endpoints were mean change from baseline in Revised Hammersmith Scale (RHS) score at 12 months for the ambulatory population (Cohort 1), and mean change from baseline in HFMSE score at 12 months for the nonambulatory population (Cohorts 2 and 3). The trial also includes multiple 12-month extension periods designed to evaluate longer-term patient outcomes.

About the Phase 3 SAPPHIRE Trial

SAPPHIRE is an ongoing randomized, double-blind, placebo-controlled, phase 3 clinical trial evaluating the safety and efficacy of apitegromab in nonambulatory patients with Types 2 and 3 SMA who are receiving SMN-targeted therapy (either nusinersen or risdiplam). SAPPHIRE targeted enrolling approximately 156 patients aged 2-12 years old in the main efficacy population. These patients were randomized 1:1:1 to receive for 12 months either apitegromab 10 mg/kg, apitegromab 20 mg/kg, or placebo by intravenous (IV) infusion every 4 weeks. An exploratory population that targeted enrolling up to 48 patients aged 13-21 years old will also separately be evaluated. These patients were randomized 2:1 to receive either apitegromab 20 mg/kg or placebo. For more information about SAPPHIRE, visit www.clinicaltrials.gov.

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 to demonstrate clinical proof of concept in spinal muscular atrophy

(SMA). 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 our 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. The efficacy and safety of apitegromab have not been established and apitegromab has not been approved for any use by the FDA or any other regulatory agency.

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 clinical-stage biopharmaceutical company focused on the discovery and development of innovative medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role. Scholar Rock is creating a pipeline of novel product candidates with the potential to transform the lives of patients suffering from a wide range of serious diseases, including neuromuscular disorders, cancer, and fibrosis. Scholar Rock's approach to targeting the molecular mechanisms of growth factor activation enabled it to develop a proprietary platform for the discovery and development of monoclonal antibodies that locally and selectively target these signaling proteins at the cellular level. By developing product candidates that act in the disease microenvironment, the Company intends to avoid the historical challenges associated with inhibiting growth factors for therapeutic effect. Scholar Rock believes its focus on biologically validated growth factors may facilitate a more efficient development path. For more information, please visit www.ScholarRock.com or follow Scholar Rock on Twitter (@ScholarRock) and LinkedIn (https://www.linkedin.com/company/scholar-rock/).

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.

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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, any development plans, strategy and progress for apitegromab, the timing of its clinical trials, anticipated clinical data, and therapeutic benefits for apitegromab, and other product candidates and indication selection and development timing, the ability of any product candidate to perf,orm in humans in a manner consistent with earlier nonclinical, preclinical or clinical trial data, and the potential of apitegromab and its other product candidates and its 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 2 clinical trial of apitegromab, are not predictive of, may be inconsistent with, or more favorable than, data generated from future clinical trials of the same product candidates, including, without limitation, the Phase 3 clinical trial of apitegromab in SMA, 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 SAPPHIRE Phase 3 clinical trial, information provided or decisions made by regulatory authorities, 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 Annual Report on Form 10-Q for the quarter ended June 30, 2023, 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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Scholar Rock:

Investors
Rushmie Nofsinger
Scholar Rock
rnofsinger@scholarrock.com
ir@scholarrock.com
857-259-5573

Media
Teagan White

Finn Partners
teagan.white@finnpartners.com
media@scholarrock.com
650-766-3955

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