Scholar Rock to Present New Data Evaluating Apitegromab at 36 Months from the Phase 2 TOPAZ trial at the 2023 Annual Cure SMA Conference

May 8, 2023

- *Apitegromab is the only muscle-targeted therapy to demonstrate proof of concept in spinal muscular atrophy*
- *Two oral presentations to cover long-term efficacy, safety, and patient-reported outcome data*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 8, 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 it will present extension data from the Phase 2 TOPAZ trial evaluating patient outcomes after 36 months of treatment with apitegromab. Apitegromab is an investigational antibody inhibiting myostatin activation by selectively binding the pro- and latent forms of myostatin and has demonstrated clinical proof of concept in spinal muscular atrophy (SMA). In two back-to-back oral presentations, Thomas O. Crawford, M.D., will present 36-month safety and efficacy data, and an analysis of patient-reported outcome measures such as fatigue, mobility, and activities of daily living at the Cure SMA Research & Clinical Care Meeting, taking place June 28-30 in Orlando, Florida.

“As we progress enrollment of our Phase 3 SAPPHIRE trial toward completion this year, we are excited to share the 36-month data from the Phase 2 TOPAZ study, the longest efficacy and safety data on an anti-myostatin in SMA to date,” said Jay Backstrom, M.D., M.P.H., President and Chief Executive Officer of Scholar Rock. “These data continue to deepen our understanding of the essential role a selective muscle-targeted treatment could play in transforming care in spinal muscular atrophy, and we look forward to sharing the 36-month TOPAZ data with the community at the Cure SMA Annual Conference.”

Details of the presentations are as follows:

Title: Effect of apitegromab on PEDI-CAT and PROMIS-fatigue questionnaire at 36 months in patients with Type 2 and nonambulatory Type 3 spinal muscular atrophy
Presentation type: Oral presentation
Presenter: Thomas O. Crawford, M.D., Professor of Neurology and Pediatrics, Johns Hopkins University
Date and time: Friday, June 30, 2023, 10:40 AM EST
Location: Disney Swan and Dolphin Hotels, Orlando, FL

Title: Effect of apitegromab on motor function at 36 months in patients with Type 2 and nonambulatory Type 3 spinal muscular atrophy
Presentation type: Oral presentation
Presenter: Thomas O. Crawford, M.D., Professor of Neurology and Pediatrics, Johns Hopkins University
Date and time: Friday, June 30, 2023, 11:00 AM EST
Location: Disney Swan and Dolphin Hotels, Orlando, Florida

For conference information, visit https://www.researchandclinicalcaremeeting.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 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 non-ambulatory patients with Types 2 and 3 SMA who are receiving SMN therapy (either nusinersen or risdiplam). Approximately 156 patients aged 2-12 years old are anticipated to be enrolled in the main efficacy population. These patients will be 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 of approximately 48 patients aged 13-21 years old will also separately be

evaluated. These patients will be randomized 2:1 to receive either apitegromab 20 mg/kg or placebo. In this subpopulation of older individuals with SMA, the safety and tolerability of apitegromab will be characterized, and efficacy will also be evaluated in an exploratory, nonpowered manner. For more information about SAPPHIRE, visit www.clinicaltrials.gov.

About Apitegromab

Apitegromab is an investigational antibody inhibiting myostatin activation by selectively binding the pro- and latent forms of myostatin and has demonstrated 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 promote 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, and often fatal, genetic disorder that typically manifests in young children. An estimated 30,000 to 35,000 patients are afflicted with SMA in the United States and Europe. It 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 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.

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

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, and other product candidates and indication selection and development timing, its cash runway, 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 2 clinical trial of apitegromab, and 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 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 any clinical trials, 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, and the impacts of public health pandemics such as COVID-19 on business operations and expectations, as well as those risks more fully discussed in the section entitled "Risk Factors" in Scholar Rock's Annual Report on Form 10-K for the year ended December 31, 2022, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20230508005121/en/

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

Source: Scholar Rock