Scholar Rock to Present New 24-Month Data from Phase 2 TOPAZ Study at Two International Conferences: World Muscle Society Congress and International Scientific Congress on Spinal Muscular Atrophy

October 4, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 4, 2022-- 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, today announced that it will be presenting additional data from the Phase 2 TOPAZ trial extension period evaluating patient outcomes after 24-months of treatment with apitegromab, an investigational selective inhibitor of the activation of myostatin for spinal muscular atrophy (SMA). An analysis of multiple efficacy endpoints, including patient reported outcomes, from the study will be presented as a poster at the World Muscle Society (WMS) Hybrid Congress in Halifax, Canada on October 11-15, and a podium presentation at the International Scientific Congress on Spinal Muscular Atrophy in Barcelona, Spain on October 21-23.

“These findings provide further evidence of the potential of apitegromab, which is the first and only muscle-directed myostatin therapy to show positive proof of concept in SMA,” said Nagesh Mahanthappa, Founding and Interim Chief Executive Officer of Scholar Rock. “We look forward to sharing the latest information from our clinical development program with the medical community, in particular new data on multiple efficacy endpoints from our TOPAZ study.”

Details of the presentations are as follows:

WMS Hybrid Congress:
Title: Apitegromab in SMA: An analysis of multiple efficacy endpoints in the TOPAZ extension study (P.102)
Presentation Type: Poster Session 2
Presenter: Thomas Crawford, M.D., Professor of Neurology and Pediatrics, Johns Hopkins University
Date and Time: Wednesday, October 12, 4:00-5:30 p.m. AST

International Scientific Congress on SMA:
Title: Apitegromab in SMA: Efficacy, Safety and PK/PD Assessments in 24 Months of TOPAZ
Presentation Type: Oral
Presenter: Basil Darras, M.D., Associate Neurologist-in-Chief, Boston Children’s Hospital; and Professor of Neurology, Harvard Medical School
Date and Time: Saturday, October 22, Session 4 – Therapy Data Update, 11:20-12:50 p.m. CEST

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

For conference information, visit https://www.wms2022.com/page/programme and https://barcelona2022.sma-europe.eu/.

About Apitegromab
Apitegromab is a selective inhibitor of the activation of myostatin and is an investigational product candidate for the treatment of patients with 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 inhibiting myostatin activation with apitegromab may result in 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. The underlying pathology of SMA is caused by insufficient production of the SMN (survival of motor neuron) protein, essential for the survival of motor neurons, and is encoded by two genes, SMN1 and SMN2. While there has been progress in the development of therapeutics that address the underlying SMA genetic defect, via SMN-dependent pathways, there continues to be a high unmet need for therapeutics that directly address muscle function.

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/). 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, Scholar Rock's expectations regarding its growth, strategy, and progress and indication selection and development timing, 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, 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, and Scholar Rock's ability to manage expenses and to obtain additional funding when needed to support its business activities, 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 June 30, 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.

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