Scholar Rock Presents Additional Data Analyses from the Apitegromab TOPAZ Phase 2 Trial at the World Muscle Society 2021 Virtual Congress

September 23, 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 23, 2021-- Scholar Rock (NASDAQ: SRRK), a clinical-stage biopharmaceutical company focused on the treatment of serious diseases in which protein growth factors play a fundamental role, today announced two poster presentations as part of the World Muscle Society (WMS) Virtual Congress taking place September 20-24, 2021.

Details for the virtual posters at the WMS meeting are as follows:

- **Title (late breaker):** Apitegromab in Spinal Muscular Atrophy (SMA): An Analysis of Multiple Efficacy Endpoints in the TOPAZ Trial (LBP.10)
  - This late breaking poster presentation will feature multiple efficacy endpoints, including Hammersmith scales and Revised Upper Limb Module (RULM) function, from the 12-month TOPAZ Phase 2 trial evaluating apitegromab in patients with Type 2 and 3 Spinal Muscular Atrophy (SMA).
  - An additional exploratory analysis evaluating time to achieving various thresholds of improvement in Hammersmith Functional Motor Scale Expanded (HFMSE) scores further support the dose response in clinical efficacy.
  - Increases in RULM were also observed in both non-ambulatory cohorts.
  - Greater increases in HFMSE (non-ambulatory) and Revised Hammersmith Scale (ambulatory) scores were seen in patients who were not limited by scoliosis or joint contractures.
- **Title**: Insights into the Potential Pharmacological Effects of Apitegromab in Health and Disease: Data from Preclinical and Clinical Studies (EP.277)
  - The poster will showcase insights into the target engagement and exposure levels of apitegromab in the TOPAZ trial as well as from Phase 1 healthy volunteer and preclinical studies.
- Virtual poster presentations (LBP.10 and EP.277) on September 23, 2021, at 16:30-18:30 BST
- E-Posters available to view on demand and exhibition area open starting Monday, September 20, 2021.

“These additional analyses from the TOPAZ Phase 2 trial further reinforce our enthusiasm for the potential of apitegromab to improve motor function for patients with SMA, and provide exploratory insights in both the ambulatory and non-ambulatory populations,” said Yung Chyung, M.D., Chief Medical Officer of Scholar Rock. “We anticipate initiating a Phase 3 trial to evaluate apitegromab in patients with non-ambulatory Type 2 and Type 3 SMA by the end of 2021 as we work towards our aim of establishing apitegromab as the potential first muscle-directed therapy to treat SMA.”

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 promote a clinically meaningful improvement in motor function in patients with SMA. The U.S. Food and Drug Administration (FDA) has granted Fast Track (FTD), Orphan Drug (ODD) and Rare Pediatric Disease (RPD) 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/).

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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, progress and timing of its clinical trials for apitegromab, SRK-181, 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," "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 preclinical and clinical data, including the 12-month top-line results from the Phase 2 trial of apitegromab, are not predictive of, are inconsistent with, or more favorable than, data generated from future clinical trials of the same product candidate, including the planned Phase 3 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, the success of Scholar Rock's current and potential future collaborations, including its collaboration with Gilead, Scholar Rock's dependence on third parties for development and manufacture of product candidates including 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, 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
Catherine Hu
chu@scholarrock.com

Media
Ariane Lovell
Finn Partners
ariane.lovell@finnpartners.com
917-565-2204

Source: Scholar Rock