Scholar Rock to Present Apitegromab TOPAZ Phase 2 Trial Results Highlighting Pharmacokinetic (PK) and Pharmacodynamic (PD) Data at the 2021 European Academy of Neurology Congress

June 18, 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 18, 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 an oral e-presentation of clinical results from the TOPAZ Phase 2 trial (NCT03921528) that evaluated apitegromab in patients with Type 2 and Type 3 spinal muscular atrophy (SMA) at the Virtual 7th Congress of the European Academy of Neurology (EAN) being held June 19-22, 2021.

Details for the virtual EAN oral e-presentation are as follows:

- **Title**: Apitegromab, an Investigational Anti-proMyostatin Monoclonal Antibody for Spinal Muscular Atrophy: Phase 2 Results
- **Muscle and Neuromuscular Junction Disease 2 Session**: Virtual e-presentation #EPR-184 on Saturday, June 19 from 14:00 to 14:45 CEST (8:00-8:45am ET) in Room Paris
- **Presenter**: Amy Place, PhD, MBA, MS, RD, CLT on behalf of the apitegromab development team

The e-presentation will highlight the 12-month top-line TOPAZ clinical results, including PK/PD data as these relate to the efficacy of apitegromab, an investigational selective inhibitor of myostatin activation. In the TOPAZ trial, apitegromab showed therapeutic potential to further improve motor function in patients with non-ambulatory Type 2 and 3 SMA who were already receiving chronic maintenance therapy with a background SMN upregulator (nusinersen). Two doses of apitegromab (20 mg/kg and 2 mg/kg) were evaluated in the TOPAZ trial and a dose response was observed based upon clinical efficacy (improvements in Hammersmith Functional Motor Scale Expanded scores) and pharmacodynamics (target engagement). Scholar Rock plans to initiate a Phase 3 trial to further evaluate apitegromab in patients with non-ambulatory Type 2 and Type 3 SMA by the end of 2021.

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, fibrosis and anemia. 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/).

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 expectations regarding its growth, strategy, progress and timing of its clinical trials and the ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical, preclinical or clinical trial data. 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 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 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 March 31, 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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