Scholar Rock Presents Clinical and Preclinical Data for SRK-015 at the 2020 Virtual Cure SMA Research and Clinical Care Meeting

June 12, 2020

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 12, 2020-- 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 presentations as part of the 2020 Virtual Cure SMA Research and Clinical Care Meeting. The two presentations will review previously presented data from preclinical studies, the Phase 1 healthy volunteer trial, as well as preliminary pharmacokinetic and biomarker data and baseline demographics from the TOPAZ Phase 2 proof-of-concept trial evaluating SRK-015 for the treatment of patients with Type 2 and Type 3 Spinal Muscular Atrophy (SMA).

Details for the two virtual presentations at the meeting are as follows:

- **Title:** Clinical Development of SRK-015, a Fully Human Anti-proMyostatin Monoclonal Antibody, for the Treatment of Later-Onset Spinal Muscular Atrophy
- **Title:** A Phase 2 Study to Evaluate the Efficacy and Safety of SRK-015 in Patients with Later-Onset Spinal Muscular Atrophy (TOPAZ): An Introduction
- **Clinical Drug Development Session:** Virtual presentations on June 12, 2020 at 12:00-3:15pm CST.

About SRK-015

SRK-015 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 strength in multiple animal species[(1)]. Preclinical SMA mouse model studies have demonstrated that selective inhibition of myostatin may have therapeutic potential in improving muscle strength[(2)]. Scholar Rock believes by targeting the precursor forms of myostatin, and preventing the release of the mature growth factor, SRK-015 may promote a clinically meaningful improvement in motor function. A Phase 2 clinical trial in patients with Type 2 and Type 3 SMA is ongoing (NCT03921528). The U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD), and the European Commission (EC) has granted Orphan Medicinal Product Designation, to SRK-015 for the treatment of SMA. The effectiveness and safety of SRK-015 have not been established. SRK-015 has not been approved for any use by the FDA or any other regulatory agency.

(1) Han, H.Q., Zhou, Xiaolan, Goldberg, A. Myostatin/activin pathway antagonism: Molecular basis and therapeutic potential. *International Journal of Biochemistry & Cell Biology,* 2013, 45(10):2119-2348

(2) Long, K., O'Shea, K., Khairallah, R., et al. Specific Inhibition of Myostatin Activation is Beneficial in Mouse Models of SMA Therapy. 2018. Human Molecular Genetics, ddy382

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/).

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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 the timing of its clinical trials for SRK-015; the potential of SRK-015 to address

certain patient unmet needs; and the ability of any product candidate to perform in humans in a manner consistent with nonclinical or preclinical study 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; preclinical data and results may not be predictive of clinical results; Scholar Rock's dependence on third parties for development and manufacture of product candidates including to supply any clinical trials; and 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, 2020, 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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