Scholar Rock Announces Issuance of U.S. Patent Providing Composition of Matter Protection for SRK-015

August 27, 2020

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 27, 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 that the United States Patent Office (USPTO) issued U.S. Patent No. 10,751,413 providing product protection for SRK-015, including composition of matter and methods of use, with an expiry of January 2037.

“We are at the forefront in the development of antibodies that target the latent form of many growth factors. This composition of matter patent further protects SRK-015, which targets the latent form of myostatin and is being evaluated in the TOPAZ Phase 2 trial for patients with Spinal Muscular Atrophy,” said Tony Kingsley, President and CEO of Scholar Rock. “We continue to build and strengthen our patent portfolio with patents that specifically protect our product candidates, SRK-015 and SRK-181, as well as a broader family of platform patents directed to our unique approach to targeting the TGFβ superfamily of growth factors more generally.”

The composition of matter patent is directed to the full-length amino acid sequence of SRK-015 that specifically binds to pro/latent myostatin while avoiding binding to mature myostatin and related growth factors, such as GDF11 and Activin A. The patent also covers methods of treating myopathy, muscle atrophy and metabolic disorders. The ongoing TOPAZ Phase 2 trial is evaluating SRK-015’s potential in improving motor function in patients with Type 2 and Type 3 Spinal Muscular Atrophy. A 6-month interim readout is anticipated in the fourth quarter of 2020 with top-line data for the 12-month treatment period expected in the first half of 2021.

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 mass and strength in multiple animal species. Scholar Rock believes the inhibition of the activation of myostatin with SRK-015 may promote a clinically meaningful increase in muscle strength. A Phase 2 clinical trial in patients with Type 2 and Type 3 SMA is ongoing. The U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) and Rare Pediatric Disease (RPD) designation, 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 and SRK-015 has not been approved for any use by the FDA or any other regulatory agency.

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/).

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 SRK-015, SRK-181, and other product candidates and development timing, the potential of its proprietary platform, and its intellectual property protection. 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, 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, 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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