Scholar Rock Announces Issuance of U.S. Patent Broadly Relevant to Inhibitors of Myostatin Activation

April 26, 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 26, 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 that the United States Patent Office (USPTO) issued U.S. Patent No. 10,981,981 with an expiry of May 2034. This patent broadly covers methods for making inhibitors of myostatin (GDF8) activation based on Scholar Rock's proprietary platform approach of targeting the precursor forms of growth factors.

"This is another important addition to our patent portfolio protecting our proprietary approach to developing antibodies that bind to the pro and latent forms of myostatin, including apitegromab being developed for the treatment of spinal muscular atrophy," said Tony Kingsley, President and CEO of Scholar Rock. "Recently announced positive results from the TOPAZ phase 2 trial demonstrate both proof-of-concept for apitegromab in helping improve motor function for patients with Type 2 and Type 3 SMA as well as the therapeutic potential of our scientific platform of targeting the latent forms of growth factors."

The issued claims are directed to manufacturing methods for developing antibodies that bind selectively to pro/latent myostatin thereby inhibiting myostatin signaling. Scholar Rock's apitegromab is a selective inhibitor of the activation of latent myostatin and is an investigational product candidate being developed towards an aim of improving motor function in patients with spinal muscular atrophy (SMA). Positive 12-month top-line results from the TOPAZ Phase 2 clinical trial were recently announced, demonstrating the transformative potential of apitegromab as a potential first muscle-directed therapy for SMA.

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 the potential of apitegromab, 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, 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 the possibility that data from the TOPAZ final analysis will be inconsistent with the data observed in the interim analysis, 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 is inconsistent with subsequent data, competition from third parties that are developing products for similar uses, Scholar Rock's ability to obtain, maintain and protect its intellectual property, 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 including its TOPAZ clinical trial, as well as those risks more fully discussed in the section entitled "Risk Factors" in Scholar Rock's Annual Report on Form 10-K for the year ended December 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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Scholar Rock Contacts:
Investor Contact:
Catherine Hu
chu@scholarrock.com

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

Source: Scholar Rock, Inc.