Scholar Rock Announces Issuance of U.S. Patent Protecting SRK-181, an Inhibitor of TGFβ1 Activation

September 28, 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 28, 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 and Trademark Office (USPTO) issued U.S. Patent No. 11,130,803, with an expiry of May 2040, including 313 days of Patent Term Adjustment (PTA). This US patent includes composition-of-matter claims, providing product protection for SRK-181, a selective inhibitor of TGFβ1 activation that avoids binding to latent TGFβ2, latent TGFβ3, or any of the three active TGFβ growth factors. SRK-181 is an investigational clinical candidate being developed to overcome primary resistance to and increase the number of patients who could benefit from checkpoint inhibitors, such as anti-PD-(L)1 therapies.

“This is another important addition to our patent portfolio providing product protection for SRK-181 as we continue to progress the DRAGON Phase 1 trial in patients with locally advanced or metastatic solid tumors,” said Nagesh Mahanthappa, Ph.D., Interim CEO of Scholar Rock. “As we observed in preclinical studies, our hypothesis remains that a selective approach to targeting the TGFβ1 isoform could allow for an improved safety and efficacy profile compared to non-selective approaches and we’re very excited to continue pursuing our unique approach to provide potential clinical benefit for patients who are resistant to treatment with PD-(L)1 inhibitors.”

SRK-181 is being evaluated in the two-part DRAGON trial (NCT04291079) in patients with locally advanced or metastatic solid tumors exhibiting primary resistance to anti-PD-(L)1 therapy. Part A dose escalation is evaluating the safety and pharmacokinetics of SRK-181 as a single-agent and in combination with anti-PD-(L)1 therapy to identify the recommended dose for Part B of the trial. Part B dose expansion will consist of multiple cohorts, including urothelial carcinoma, cutaneous melanoma, non-small cell lung cancer, and other solid tumors and patients will be treated with SRK-181 in combination with an anti-PD-(L)1 therapy. An update on dose escalation and initial clinical data from Part A of the DRAGON trial is anticipated by year-end.

About SRK-181

SRK-181 is a selective inhibitor of TGFβ1 activation and is an investigational product candidate being developed to overcome primary resistance to checkpoint inhibitor therapy, such as anti-PD-(L)1 antibodies. TGFβ1 is the predominant TGFβ isoform expressed in many human tumor types. Based on analyses of various human tumors that are resistant to anti-PD-(L)1 therapy, data suggest TGFβ1 is a key contributor to the immunosuppressive tumor microenvironment, excluding and preventing entry of cytotoxic T cells into the tumor, thereby inhibiting anti-tumor immunity [(1)]. Scholar Rock believes SRK-181, which specifically targets the latent TGFβ1 isoform, has the potential to overcome this immune cell exclusion and induce tumor regression when administered in combination with anti-PD-(L)1 therapy while potentially avoiding toxicities associated with non-selective TGFβ inhibition. The DRAGON Phase 1 proof-of-concept clinical trial (NCT04291079) in patients with locally advanced or metastatic solid tumors is ongoing. The efficacy and safety of SRK-181 have not been established. SRK-181 has not been approved for any use by the FDA nor any other regulatory agency.

(1) Martin et al., Sci. Transl. Med. 12: 25 March 2020

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 https://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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