



Scholar Rock Announces Issuance of U.S. Patent Protecting Add-On or Combination Therapy with a Myostatin Inhibitor for the Treatment of Spinal Muscular Atrophy

March 17, 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 17, 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. 10,946,036 with an expiry of June 2037. The claims of the patent broadly cover both add-on and combination therapy with a myostatin inhibitor and a neuronal corrector therapy (such as SMN upregulator therapy) for the treatment of Spinal Muscular Atrophy (SMA).

"We are delighted to have been granted this unique patent that further protects our myostatin approach to improving motor function for patients with SMA and we look forward to the top-line read-out from the apitegromab TOPAZ Phase 2 trial next quarter, which will provide additional insights on its potential to help patients with Type 2 and Type 3 SMA," said Tony Kingsley, President and CEO of Scholar Rock. "In parallel with preclinical and clinical advancements, we will continue to build and strengthen our family of patents protecting our product candidates and our scientific platform."

The broad claims of this therapeutic method patent are not limited to apitegromab or to add-on or combination treatment with any specific SMN upregulator therapy. Similar claims have also been granted in Japan (JP Patent No. 6823167) and in Europe (EP 3368069B1).

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, there continues to be a high unmet need for therapeutics that directly address muscle atrophy.

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 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, 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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