



Scholar Rock Announces that SRK-015 has Received Rare Pediatric Disease Designation from U.S. FDA for the Treatment of Spinal Muscular Atrophy

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 13, 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 U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease (RPD) designation for SRK-015 for the treatment of Spinal Muscular Atrophy (SMA), a progressive, rare genetic disease that leads to motor function impairments. Scholar Rock is on track to report 6-month interim efficacy and safety data from the ongoing TOPAZ Phase 2 clinical trial of SRK-015 in patients with Type 2 and Type 3 SMA in the fourth quarter of 2020. Top-line data for the 12-month treatment period are expected in the first half of 2021.

"This Rare Pediatric Disease designation along with the previously granted Orphan Drug Designation highlights the FDA's recognition of the unmet medical needs of patients with SMA. We continue to aim to establish SRK-015 as the potential first muscle-directed therapy to address motor function deficits that persist despite the availability of SMN upregulators," said Yung Chyung, M.D., Chief Medical Officer of Scholar Rock. "We are encouraged by the progress of the TOPAZ Phase 2 trial and look forward to the interim read-out next quarter that will provide important insights into the potential of SRK-015 in patients with SMA."

The FDA grants Rare Pediatric Disease designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 individuals in the United States. If a biologics license application (BLA) for SRK-015 for the treatment of SMA is approved by the FDA, Scholar Rock may be eligible to receive a priority review voucher, which may be redeemed to obtain priority review for any subsequent marketing application or be sold or transferred.

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 TGF β 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/>).

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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 SRK-015, SRK-181, and other product candidates and indication selection and development timing, the ability of any product candidate to perform in humans in a manner consistent with nonclinical or preclinical study data, the potential of its proprietary platform, and the impact of COVID-19 on its clinical trials and its business and operations in general. 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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