Scholar Rock Receives Fast Track Designation from the U.S. FDA for Apitegromab for the Treatment of Patients with Spinal Muscular Atrophy

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- Builds on Priority Medicines (PRIME) designation recently granted by the European Medicines Agency (EMA) recognizing the unmet medical needs of patients with SMA

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 24, 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 U.S. Food and Drug Administration (FDA) has granted Fast Track designation for apitegromab, a selective inhibitor of myostatin activation, for the treatment of patients with Spinal Muscular Atrophy (SMA). Fast Track designation is intended to facilitate the development and expedite the review of drugs to treat serious conditions and get new drugs to patients earlier. Through Fast Track, Scholar Rock is eligible to submit a rolling Biologic License Application (BLA) for apitegromab if relevant criteria are met.

“We are delighted to receive Fast Track designation and look forward to working closely with the FDA towards our aim of establishing apitegromab as the potential first muscle-directed therapy for patients with SMA,” said Tony Kingsley, President and CEO of Scholar Rock. “We believe the recently announced top-line data from the TOPAZ Phase 2 trial showed the transformative potential of apitegromab for patients with SMA.”

Fast Track is intended to fill an unmet medical need defined as providing a therapy where none exists or improving upon available therapies and is based on whether a drug will impact factors such as survival, day-to-day functioning, or if left untreated, progression to a more serious condition. In addition to Fast Track designation, apitegromab had previously received Orphan Drug and Rare Pediatric Disease designations from the FDA and PRIME and Orphan Medicinal Product designations from the EMA for the treatment of SMA.

“Recent Fast Track and PRIME designations granted by the regulatory agencies underscore the continuing unmet medical needs of patients with SMA,” said Yung Chyung, M.D., Chief Medical Officer of Scholar Rock. “The majority of non-ambulatory patients in our TOPAZ trial experienced increases in Hammersmith scores, highlighting the therapeutic potential of apitegromab to address motor function impairments in this patient population.”

About Apitegromab

Apitegromab 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, including humans. Scholar Rock believes that inhibiting myostatin activation with apitegromab may promote a clinically meaningful improvement in motor function in patients with SMA. The U.S. Food and Drug Administration (FDA) has granted Fast Track designation (FTD), Orphan Drug designation (ODD) and Rare Pediatric Disease (RPD) designation, and the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) designation and Orphan Medicinal Product designation, to apitegromab for the treatment of SMA. The efficacy and safety of apitegromab have not been established and apitegromab has not been approved for any use by the FDA or any other regulatory agency.

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, via SMN-dependent pathways, 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 newly elucidated understanding of 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/).

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 SRK-181, 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, 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 clinical trial will be inconsistent with the data observed in subsequent 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 clinical trials, 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 March 31, 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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