



SCHOLAR ROCK

Scholar Rock Granted Orphan Drug Designation by the FDA for SRK-015 for the Treatment of Patients with Spinal Muscular Atrophy

March 29, 2018

CAMBRIDGE, Mass., March 29, 2018 – Scholar Rock, a biopharmaceutical company focused on developing medicines for the treatment of serious diseases in which signaling by protein growth factors plays a fundamental role, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to its lead antibody product candidate, SRK-015, for the treatment of spinal muscular atrophy (SMA). SRK-015 is an inhibitor of the activation of myostatin, which Scholar Rock believes has the potential to be the first muscle-directed therapy to reverse or prevent muscle atrophy in SMA patients and could be used both as a monotherapy or in conjunction with the current standard of care.

“We are very pleased that the FDA granted Orphan Drug Designation to SRK-015 for the treatment of patients suffering from SMA, and we appreciate that the agency’s decision came much earlier than anticipated,” said Nagesh Mahanthappa, PhD, President and Chief Executive Officer of Scholar Rock. “This designation is an important milestone in the development of our lead product candidate along the path towards a first-in-human Phase 1 clinical trial in the second quarter of 2018.”

Under the U.S. Orphan Drug Act, FDA’s Office of Orphan Products Development (OOPD) grants orphan drug designation to drugs and biologic products that are intended for the treatment of rare diseases or disorders that affect fewer than 200,000 people in the U.S. Orphan drug status is intended to facilitate drug development for rare diseases and may provide several benefits and incentives to drug developers, including assistance with clinical study design and drug development, tax credits for qualified clinical trials costs, and seven years of market exclusivity upon regulatory product approval.

About SRK-015

SRK-015 is a selective first-in-class inhibitor of the activation of myostatin. Myostatin, a member of the TGF-beta superfamily of growth factors, is expressed primarily in 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 mass and strength. Scholar Rock is advancing SRK-015 into clinical development for the treatment of patients with SMA. SRK-015 is an investigational drug candidate. The effectiveness and safety of SRK-015 have not been established and SRK-015 has not been approved by the FDA or any other regulatory agency.

About SMA

Spinal muscular atrophy is a rare, and often fatal, genetic disorder that typically manifests in young children. An estimated 30,000 to 35,000 patients suffer from 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 protein, essential for the survival of motor neurons and is encoded by two genes, SMN1 and SMN2. While there has been meaningful 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 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 safely and effectively inhibiting growth factors for therapeutic effect. Scholar Rock is focused on biologically validated growth factors to aim for a more efficient development path. Scholar Rock is led by a highly-experienced management team of leaders who have built successful biopharmaceutical companies.

Safe Harbor Statement

Various statements in this release concerning Scholar Rock’s future expectations, plans and prospects, including without limitation, Scholar Rock’s expectations regarding SRK-015 for the treatment of patients suffering from SMA, and its expectations with respect to the receipt of orphan drug designation for SRK-015 for the treatment of SMA, constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Scholar Rock’s ability to successfully demonstrate the efficacy and safety of SRK-015, the preclinical and clinical results for SRK-015 as a treatment for SMA, which may not support further development of SMA for the treatment of SMA, actions of regulatory agencies, which may affect the initiation, timing and progress of preclinical and clinical development,

competition from others developing products for similar uses, and Scholar Rock's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, 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. Scholar Rock explicitly disclaims any obligation to update any forward-looking statements.

Scholar Rock Contact:

Catherine Hu, 917-601-1649
chu@scholarrock.com

Media Contact:

The Yates Network
Barbara Yates, 781-258-6153
barbara@theyatesnetwork.com