Scholar Rock Announces Completion of Dosing of First Cohort in Phase 1 Clinical Trial of SRK-015

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CAMBRIDGE, Mass., May 31, 2018 (GLOBE NEWSWIRE) -- Scholar Rock (NASDAQ:SRRK), 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, today announced that it has completed dosing of the first cohort and is continuing enrollment of healthy volunteers in its Phase 1 clinical trial of SRK-015, the Company's lead antibody product candidate. SRK-015 is a selective inhibitor of the activation of myostatin, which Scholar Rock believes has the potential to be the first muscle-directed therapy to reverse or prevent further muscle atrophy in patients with spinal muscular atrophy (SMA) and could be used as a monotherapy or in conjunction with the current standard of care.

"The initiation of this Phase 1 clinical trial of SRK-015 is a momentous milestone for Scholar Rock and we are one step closer to potentially bringing the first muscle-directed therapy to patients suffering from SMA," said Nagesh Mahanthappa, PhD, President and Chief Executive Officer of Scholar Rock. "We are also excited to be building and progressing a pipeline of novel product candidates focused on addressing other neuromuscular disorders, cancer, fibrosis, and anemia."

The placebo-controlled, double-blind Phase 1 clinical trial will evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of single- and multiple-ascending doses of intravenous SRK-015 in healthy adult volunteers. Assuming the successful completion of the Phase 1 trial, the Company plans to initiate a Phase 2 proof-of-concept trial in the first quarter of 2019 to evaluate the efficacy and safety of SRK-015 in patients with later-onset SMA as a monotherapy or in conjunction with an approved SMN upregulator therapy as background standard of care.

About SRK-015
SRK-015 is a selective inhibitor of the activation of myostatin and is an investigational therapy for the treatment of patients with spinal muscular atrophy (SMA). 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. A Phase 1 clinical trial in healthy volunteers is ongoing. The U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) for 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 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 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 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.

Safe Harbor Statement
Various statements in this presentation concerning Scholar Rock's future expectations, plans and prospects, including without limitation, Scholar Rock's expectations regarding its strategy, its product candidate selection and development timing, including timing for the initiation of and reporting results from its clinical trials for its product candidates, its disease indication selection timing, its management team capabilities, and the ability of SRK-015 to affect the treatment of patients suffering from SMA either as a monotherapy or in conjunction with the current standard of care, constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. 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. 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 provide the financial support and resources necessary to identify and develop product candidates on the expected timeline, competition from others 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, and 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 as well as those risks more fully discussed in the section entitled "Risk Factors" in the final prospectus related to Scholar Rock's initial public offering filed with the Securities and Exchange Commission pursuant to Rule 424(b) of the Securities Act, 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. Scholar Rock explicitly disclaims any obligation to update any forward-looking statements unless required by law.

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