



SCHOLAR ROCK

Scholar Rock Announces Positive Opinion by the European Medicines Agency on Orphan Drug Designation for SRK-015 for the Treatment of Spinal Muscular Atrophy

December 3, 2018

CAMBRIDGE, Mass., Dec. 03, 2018 (GLOBE NEWSWIRE) -- Scholar Rock Holding Corporation (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 European Medicines Agency's (EMA's) Committee for Orphan Medicinal Products (COMP) has adopted a positive opinion recommending SRK-015 (human anti-promyostatin monoclonal antibody) for designation as an orphan medicinal product for the treatment of Spinal Muscular Atrophy (SMA). SRK-015 was previously granted orphan drug designation by the U.S. Food and Drug Administration (FDA) in March 2018.

"We are delighted with the EMA COMP's adoption of a positive opinion for SRK-015 orphan drug designation, which represents another important milestone for this clinical program," said Nagesh Mahanthappa, PhD, President and CEO of Scholar Rock. "We believe SRK-015 has the potential to be the first muscle-directed therapy to improve muscle function in patients with SMA and look forward to working with the EMA as we progress the program, including our intention to initiate a Phase 2 proof-of-concept study in the first quarter of 2019."

Orphan Medicinal Product Designation by the European Commission is available to companies developing products intended to treat a life-threatening or chronically debilitating condition that affects fewer than five in 10,000 people in the EU, and has the potential to be of significant benefit. This designation could allow for a number of incentives, including protocol assistance, access to the centralized authorization procedure, reduced regulatory fees, and a 10-year period of marketing exclusivity in the EU after product approval.

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-beta 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 mass and strength. A Phase 1 clinical trial in healthy volunteers is ongoing. 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 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 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](https://twitter.com/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 the potential of SRK-015 as a therapy in SMA and the timeline for and progress in developing SRK-015. 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 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 September 30, 2018, 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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