Scholar Rock Announces Issuance of Broad Patent Covering Therapeutic Approach for Neuromuscular Diseases

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USPTO grants claims covering any monoclonal antibody that binds selectively to latent myostatin and prevents its proteolytic activation

CAMBRIDGE, Mass., September 14, 2017 – Scholar Rock, a biotechnology company focused on discovering and developing drugs that selectively modulate growth factor activation in the disease microenvironment, today announced that the U.S. Patent and Trademark Office (USPTO) has granted to Scholar Rock, U.S. Patent No. 9,758,576, providing coverage of any monoclonal antibody that binds selectively to latent myostatin and prevents its proteolytic activation. The patent provides broad protection for SRK-015, a selective and local inhibitor of the activation of latent myostatin, and an entire class of therapeutic antibodies with the same mechanism of action. These antibodies may offer therapeutic potential for a wide array of neuromuscular and other diseases. SRK-015 is the company's lead clinical candidate, being developed for the improvement of muscle strength and function in patients with Spinal Muscular Atrophy (SMA) and other disorders.

"This patent issuance marks an important milestone for Scholar Rock and the development of SRK-015," said Atsuko Polzin, PhD, Head of Intellectual Property at Scholar Rock. "We are pleased that the USPTO has recognized Scholar Rock's innovation with this patent that provides protection for monoclonal antibodies that selectively inhibit myostatin signaling by blocking the proteolytic activation of the latent precursor. With this broad patent coverage and exclusivity through May 2034, Scholar Rock is strongly positioned to develop SRK-015 as the first and only clinical candidate that works through this unique mechanism of action."

"With five key US patents having issued to Scholar Rock since last year, we are progressively building a robust portfolio of intellectual property that reflects the unique attributes of our drug discovery platform," said Nagesh Mahanthappa, PhD, President and Chief Executive Officer of Scholar Rock. "Targeting the activation of protein growth factors offers a number of important potential benefits that we aim to realize in the clinic in our efforts to build a world-leading pipeline of medicines to treat patients suffering from a range of serious diseases, including SMA."

About SRK-015

SRK-015 is a selective and local inhibitor of the activation of latent myostatin. Myostatin, a member of the TGFβ superfamily of growth factors that is expressed primarily in skeletal muscle cells, is a genetically validated target that regulates muscle mass. Scholar Rock is actively working to advance SRK-015 into clinical trials to improve muscle strength and motor function in patients with Spinal Muscular Atrophy (SMA). Scholar Rock plans to develop SRK-015 both in combination with therapies aimed at correcting the underlying genetic defect and as monotherapy in certain subpopulations of SMA patients. 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 (SMA) is a rare, and often fatal, genetic disorder that affects approximately 1 in every 10,000 births. This disease is due to defects in the SMN1 gene that produces a protein important for the survival and function of lower motor neurons. Deterioration and loss of lower motor neurons that innervate skeletal muscle lead to significant muscle atrophy, particularly in fast-twitch fibers. Muscle weakness is the most common and prominent feature of SMA, leaving many patients suffering from difficulty in performing many basic motor functions. 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. Directly targeting the weakening of skeletal muscle may lead to improvements in muscle strength and motor function that could positively impact patients with SMA.

About Scholar Rock

Scholar Rock is discovering and developing a pipeline of innovative new medicines to treat a range of serious diseases in which growth factors play a fundamental role, including neuromuscular diseases, cancer and fibrosis. By focusing on newly elucidated biology of growth factor activation, Scholar Rock has developed insights which allow us to selectively target growth factors in the disease microenvironment – through the mechanism of supracellular activation. With our proprietary technology, we are developing novel medicines aimed at achieving therapeutic effects specifically at the source of disease to deliver new solutions for patients. Scholar Rock is led by a highly-experienced management team of leaders who have built successful biotechnology companies, and is backed by leading investors.

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