



SCHOLAR ROCK

Scholar Rock Announces \$47 Million Series C Financing to Advance Innovative Pipeline for SMA, Immuno-Oncology, Fibrosis, and Iron-Restricted Anemias

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SRK-015 to move into clinical development for Spinal Muscular Atrophy (SMA) in first half of 2018; development in additional neuromuscular disorders to follow

Novel TGF beta 1 inhibitors demonstrate unprecedented selectivity that highlight potential for best-in-class therapeutics in immuno-oncology and fibrosis

Unique inhibitors of hepatic BMP6 signaling open transformative opportunities aimed at treating iron-restricted anemias

CAMBRIDGE, Mass., January 3, 2018 – Scholar Rock, a biotechnology company focused on discovering and developing drugs that selectively modulate growth factor activation in the disease microenvironment, today announced the closing of a \$47 million Series C financing. The financing was led by new investor Invus, with the participation of new investor Redmile Group. All of the existing investors in Scholar Rock also participated in this financing round, including Polaris Partners, Timothy Springer, ARCH Venture Partners, EcoR1 Capital, The Kraft Group, Fidelity Management and Research Company, and Cormorant Asset Management.

Proceeds from the financing will be used to advance SRK-015, the company's lead clinical candidate for Spinal Muscular Atrophy (SMA) and other neuromuscular disorders, into clinical development in the first half of 2018. Scholar Rock plans to evaluate the potential of SRK-015 to improve muscle strength and motor function, both in SMA patients who are on therapies aimed at upregulating the production of the deficient SMN protein and as monotherapy in certain subpopulations of SMA patients. In addition, the efficient and disciplined application of this funding enables Scholar Rock to further progress additional development candidates in disease areas such as fibrosis, immuno-oncology, and anemias associated with iron restriction.

"We are delighted by the strong support and recognition we received in this Series C financing and the expansion of our group of world-class life sciences investors, which now includes Invus and Redmile," said Nagesh Mahanthappa, PhD, President and Chief Executive Officer of Scholar Rock. "We expect 2018 to be a critical year of growth for Scholar Rock, as we advance the first clinical candidate from our groundbreaking platform into the clinic to address an important unmet need in SMA and continue to build out our pipeline."

SRK-015 exemplifies Scholar Rock's proprietary approach to selectively target growth factors in the disease microenvironment through modulating the supracellular activation of latent growth factors. SRK-015 is a highly selective inhibitor of the supracellular activation of myostatin, a member of the TGF-beta superfamily of growth factors. This novel mechanism of action provides for highly specific and localized effects, seeking to overcome the challenges that have limited the traditional approach of inhibiting growth factors systemically. Scholar Rock is the first company to exploit this mechanism to discover and develop a pipeline of potential best-in-class therapeutic candidates.

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 SMN, 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 SRK-015

SRK-015 is a selective and local inhibitor of the supracellular activation of latent myostatin. Myostatin, a member of the TGF-beta 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 that will evaluate the potential to improve muscle strength and motor function in patients with Spinal Muscular Atrophy (SMA). Scholar Rock plans to develop SRK-015 both in SMA patients who are on therapies aimed at upregulating the expression of SMN 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 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 modulating 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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