Scholar Rock Achieves First Milestone in Strategic Fibrosis Collaboration with Gilead Sciences

January 9, 2020

Successful demonstration of preclinical in vivo proof-of-concept studies advances collaboration to develop highly specific inhibitors of TGFβ activation for the potential treatment of patients suffering from fibrotic diseases

Discovery efforts have yielded potent and selective inhibitors of latent TGFβ activation for fibrosis indications

Scholar Rock earns $25 million with achievement of initial milestone; eligible to receive up to an additional $1,425 million in potential payments from Gilead

CAMBRIDGE, Mass., Jan. 09, 2020 (GLOBE NEWSWIRE) -- Scholar Rock (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 Company has earned a $25 million milestone payment from Gilead Sciences, Inc. (NASDAQ: GILD) for the successful demonstration of efficacy in preclinical *in vivo* proof-of-concept studies. This is the most advanced program of the collaboration focused on the discovery and development of potent and selective inhibitors of latent transforming growth factor beta (TGFβ) activation for the treatment of fibrotic diseases.

“Achieving this important first milestone at the one year mark of the collaboration is indicative of the progress we are making together towards unlocking the potential of this novel approach to TGFβ inhibition in the treatment of patients suffering from fibrotic diseases,” said Nagesh Mahanthappa, Ph.D., President and CEO of Scholar Rock. “This accomplishment further validates the broad applicability of our platform by targeting the activation of latent growth factors to overcome historic challenges with selectivity and to discover and develop safe and effective therapeutics to address a wide range of serious diseases, including neuromuscular disorders, cancers, fibrosis, and anemia.”

The collaboration between Gilead and Scholar Rock was established in December 2018 to discover and develop therapeutics that target TGFβ-driven signaling, a central regulator of fibrosis. Scholar Rock retains exclusive worldwide rights to discover, develop, and commercialize certain TGFβ inhibitors for oncology and cancer immunotherapy.

Scholar Rock received $80 million in upfront payments in connection with the collaboration, comprised of $50 million cash and $30 million purchase of Scholar Rock Holding Corporation common stock. In addition to this one-time $25 million preclinical milestone, Scholar Rock is eligible to receive up to an additional $1,425 million in potential payments from Gilead aggregated across all three programs based on the successful achievement of certain research, development, regulatory and commercialization milestones. Scholar Rock would also receive high single-digit to low double-digit tiered royalties on sales of potential future products originating from the collaboration.

Under the collaboration, Gilead has exclusive options to license worldwide rights to product candidates that emerge from three Scholar Rock TGFβ programs: inhibitors that target activation of latent TGFβ1 with high affinity and specificity, inhibitors that selectively target activation of latent TGFβ1 localized to extracellular matrix, and a third TGFβ discovery program. Scholar Rock is responsible for antibody discovery and preclinical research through product candidate nomination, after which, upon exercising the option for a program, Gilead will be responsible for the program’s preclinical and clinical development and commercialization.

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. The Company’s two lead product candidates include SRK-015, a selective inhibitor of the activation of myostatin, for the treatment of patients with Spinal Muscular Atrophy and SRK-181, an isoform-selective inhibitor of TGFβ1 activation as a cancer immunotherapy in combination with anti-PD(L)1 antibodies. 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 and LinkedIn.

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Forward-Looking Statement

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, expectations regarding the potential of Scholar Rock's platform, TGFβ programs and molecules and progress made under Scholar Rock's collaboration with Gilead. 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 the risks that preclinical data and results may not be predictive of clinical results or the success of Scholar Rock's platform or of Scholar Rock's collaboration with Gilead and 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, 2019, 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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