Scholar Rock to Present Preclinical and Phase 1 Clinical Data on SRK-015 at the World Muscle Society Congress

October 2, 2019

Preclinical and Phase 1 data support the evaluation of SRK-015 in ongoing Phase 2 trial for the treatment of patients with Type 2 and Type 3 spinal muscular trophy

CAMBRIDGE, Mass., Oct. 02, 2019 (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 a poster presentation of preclinical and Phase 1 healthy volunteer data for SRK-015 will be presented at the World Muscle Society Congress being held October 1-5, 2019 in Copenhagen, Denmark. SRK-015 is a highly selective inhibitor of the activation of myostatin, a negative regulator of muscle mass, and is being evaluated in the TOPAZ Phase 2 clinical trial for the treatment of patients with Type 2 and Type 3 spinal muscular atrophy (SMA).

“Collectively, these preclinical and Phase 1 healthy volunteer data provided the basis for advancing SRK-015 to our TOPAZ Phase 2 clinical trial,” said Nagesh Mahanthappa, Ph.D, President and CEO of Scholar Rock. “We are excited for the potential role of SRK-015 as the first muscle-directed therapy to address the continued functional deficits experienced by some patients with SMA. We look forward to data from the upcoming clinical read-outs of the TOPAZ trial, starting with the preliminary PK/PD results at the end of 2019 and the interim efficacy and safety data in the first half of 2020.”

Consistent with previously reported results, the preclinical and Phase 1[(1)] clinical data to be presented at World Muscle Society show:

Preclinical studies:

- Improved muscle strength following administration of muSRK-015P (mouse analog of SRK-015) in mouse models of early and late SMN restoration. Treatment with muSRK-015P resulted in 20%-51% increases in maximal torque (at ≥ 40 Hz) of the plantar flexor muscle group and a greater percentage of muscle fibers compared to treatment with placebo (vehicle).
- Multi-fold increase in serum latent myostatin levels following treatment with muSRK-015P in both early and late SMN restoration mouse models confirm the presence of the latent myostatin target in a modeled diseased setting.
- Relative dose-proportional accumulation of serum latent myostatin, demonstrating target engagement, in rats and cynomolgus monkeys following administration of SRK-015 compared to no meaningful change with placebo.
- Well-behaved pharmacokinetic (PK) profile displayed across animal species (adult rats and cynomolgus monkeys).

Phase 1 healthy volunteer trial:

- SRK-015 was well-tolerated with no apparent safety signals and no dose-limiting toxicities were identified up to the highest evaluated dose of 30 mg/kg.
- Robust and sustained target engagement, along with durable saturation, following administration of SRK-015 in both the single-ascending and multiple-ascending dose portions of the Phase 1 trial.
- Well-behaved PK profile, supporting dosing once every 4-weeks, which is being evaluated in the ongoing Phase 2 trial.

The details for the SRK-015 poster at the World Muscle Society Congress are as follows:

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- Poster Title: SRK-015, a Fully Human Monoclonal Antibody Inhibiting Myostatin Activation, Offers Sustained Target Engagement Across Multiple Species, Including Humans
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- Poster Number: P126
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- Poster Presentation: Wednesday, October 2, 2019, 4:45 – 6:15pm (local time)
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- Poster Hall: Andersen Hall, Lumbye Hall and Gemyse
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(1) Preclinical and Phase 1 clinical data are not predictive of clinical outcomes in Phase 2 and Phase 3 clinical trials

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 [(1)] and Europe [(2)]. 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[(3)]. 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.

(1) Lally, C. et al. Indirect estimation of the prevalence of spinal muscular atrophy Type I, II, and III in the United States. *Orphanet Journal of Rare Diseases*. (2017) 12:175.
(2) Briefing Document to the Clinical Trial Readiness in Spinal Muscular Atrophy (SMA) SMA Europe, TREAT-NMD and European Medicines Agency meeting. Prepared by SMA Europe and TREAT-NMD. November 11, 2016.
(3) Parente, V. and Corti, S. Advances in spinal muscular atrophy therapeutics. *Therapeutic Advances in Neurological Disorders.* (2018) 11:1.

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) 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 future expectations, plans and prospects, including without limitation, 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 the risks that earlier preclinical and clinical data and testing of SRK-015 may not be predictive of the results or success of additional clinical trials, the development of SRK-015 will take longer and/or cost more than planned, SRK-015 will not receive regulatory approval 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 June 30, 2019, which is on file with the Securities and Exchange Commission, 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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Source: Scholar Rock