Scholar Rock Reports Third Quarter 2021 Financial Results and Highlights Business Progress

November 9, 2021

- Phase 3 pivotal trial evaluating apitegromab in patients with non-ambulatory Type 2 and Type 3 SMA on track to initiate by year-end 2021

- Update from Part A of the DRAGON Phase 1 trial evaluating SRK-181's ability to overcome primary resistance to checkpoint inhibitors being presented at the Society for Immunotherapy of Cancer (SITC) 36th Annual Meeting

- Initiated Part B dose expansion portion of the DRAGON trial

- U.S. patent issued providing additional product protection for SRK-181 to May 2040

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 9, 2021-- 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 reported financial results for the third quarter ended September 30, 2021, and highlighted recent progress and upcoming milestones for its pipeline programs.

"The DRAGON trial addresses two key questions; first, can the selectivity of SRK-181 for TGFβ1 increase the therapeutic window for inhibition of this signaling pathway thereby enabling higher doses safely, and second, does SRK-181 exhibit anti-tumor activity effective in overcoming resistance to checkpoint inhibitors in solid tumors. We are pleased with the results of Part A, as will be provided this week at the Society for Immunotherapy of Cancer (SITC) Annual Meeting, and we look forward to exploring the efficacy dimension of our therapeutic hypothesis in Part B," said Nagesh Mahanthappa, Ph.D., Interim CEO of Scholar Rock. "Building on the momentum from the first half of this year, we are also excited about the progress with apitegromab and are on track to initiate by year-end the Phase 3 pivotal trial evaluating its efficacy in patients with non-ambulatory Type 2 and Type 3 spinal muscular atrophy (SMA)."

Company Updates and Upcoming Milestones

Apitegromab *is a selective inhibitor of myostatin activation being developed as the potential first muscle-directed therapy for the treatment of spinal muscular atrophy (SMA).*

- **Additional Exploratory Responder Analyses and Pharmacologic Data from the TOPAZ Phase 2 Trial were Presented at Various Medical Congresses.** In September 2021, two posters were presented at the World Muscle Society Virtual Congress, including a late-breaker poster featuring an exploratory analysis evaluating time to achieving various thresholds of improvement in Hammersmith Functional Motor Scale Expanded (HFMSE) scores, which are consistent with the observed dose response in clinical efficacy. In October 2021, a poster presented at the 25th World Congress of Neurology (WCN) described the positive correlation between the magnitude of target engagement and motor function improvements following apitegromab treatment. At the 50th Child Neurology Society Annual Meeting, additional exploratory responder analyses on Hammersmith scale scores were presented, including time to achieve different thresholds of improvement in HFMSE scores.
- **Phase 3 Trial Evaluating Apitegromab in Patients with Non-Ambulatory Type 2 and 3 Patients Remains on Track to Initiate by Year-End 2021.** Scholar Rock is preparing to announce the design of the Phase 3 pivotal study later this month. The company is on track to initiate by the end of 2021 the randomized, double-blind, placebo-controlled Phase 3 trial evaluating apitegromab as add-on therapy for patients on either nusinersen or risdiplam with non-ambulatory Type 2 and Type 3 SMA. This patient population comprises an estimated two-thirds of the overall prevalence of SMA, and the greatest improvements in motor function (as measured by HFMSE) observed in the TOPAZ Phase 2 trial were in patients with non-ambulatory Type 2 and Type 3 SMA receiving apitegromab as add-on therapy to background nusinersen.

SRK-181 *is a selective inhibitor of latent TGFβ1 activation being developed with the aim of overcoming primary resistance to and increasing the number of patients who may benefit from checkpoint inhibitor therapy.*

- **Update from Part A of the DRAGON Phase 1 Trial and Part B Dose to be Presented at the Society for Immunotherapy of Cancer (SITC) 36th Annual Meeting.** A poster titled, "First-in-Human Phase 1 Trial of SRK-181: A Latent TGFβ1 inhibitor, Alone or in Combination with Anti-PD-(L)1 Treatment in Patients with Advanced Solid Tumors (DRAGON trial)" will be presented at the SITC meeting on November 12, 2021. The poster will include initial clinical data from Part A of the DRAGON trial as well as the rationale for the identified Part B dose.

  The Company has initiated the Part B dose expansion portion of the trial, which consists of multiple cohorts, each enrolling up to 40 patients with various solid tumors who have demonstrated primary resistance to anti-PD-(L)1 therapy.

- **U.S. Patent Issued Providing Composition of Matter Product Protection for SRK-181.** In September 2021, the United States Patent and Trademark Office (USPTO) issued U.S. Patent No. 11,130,803 with an expiry of May 2040, including 313 days of Patent Term Adjustment (PTA). The European counterpart has also been granted.

Third Quarter 2021 Financial Results

For the quarter ended September 30, 2021, net loss was $37.5 million or $1.02 per share compared to a net loss of $23.6 million or $0.79 per share for the quarter ended September 30, 2020.

- Revenue was $5.5 million for the quarter ended September 30, 2021, compared to $3.0 million for the quarter ended September 30, 2020 and was related to the Gilead Collaboration Agreement that was executed in December 2018.
- Research and development expense was $31.3 million for the quarter ended September 30, 2021, compared to $18.4 million for the quarter ended September 30, 2020. The increase year-over-year primarily reflects manufacturing costs associated with apitegromab, clinical trial costs for SRK-181, and additional personnel and facility-related costs.
- General and administrative expense was $11.3 million for the quarter ended September 30, 2021, compared to $8.3 million for the quarter ended September 30, 2020. The increase year-over-year was primarily attributed to additional personnel, professional fees, and facility-related costs.

“As we approach the end of 2021, I am incredibly proud of the execution by the entire Scholar Rock team this year. Not only have we initiated Part B of the DRAGON trial, but we’re also very close to initiating our Phase 3 pivotal trial for apitegromab,” said Ted Myles, CFO and Head of Business Operations of Scholar Rock. “We ended the third quarter with approximately $246 million in cash and cash equivalents and are well-funded to continue executing on our development programs while continuing to invest in our robust discovery platform.”

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, and fibrosis. Scholar Rock’s approach to targeting 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 Scholar Rock’s future expectations, plans and prospects, including without limitation, Scholar Rock’s expectations regarding its growth, strategy, progress and timing of its clinical trials for apitegromab, SRK-181, and other product candidates and indication selection and development timing, its cash runway, the ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical, preclinical or clinical trial data, and the potential of its product candidates and proprietary platform. 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 preclinical and clinical data, including the results from the Phase 2 trial of apitegromab or Part A of the Phase 1 trial of SRK-181, are not predictive of, are inconsistent with, or more favorable than, data generated from future clinical trials of the same product candidate, including the planned Phase 3 trial of apitegromab in SMA and Part B of the Phase 1 trial of SRK-181, respectively, Scholar Rock’s ability to provide the financial support, resources and expertise necessary to identify and develop product candidates on the expected timeline, the data generated from Scholar Rock’s nonclinical and preclinical studies and clinical trials, information provided or decisions made by regulatory authorities, competition from third parties that are developing products for similar uses, Scholar Rock’s ability to obtain, maintain and protect its intellectual property, the success of Scholar Rock’s current and potential future collaborations, including its collaboration with Gilead, Scholar Rock’s dependence on third parties for development and manufacture of product candidates including to supply any clinical trials, Scholar Rock’s ability to manage expenses and to obtain additional funding when needed to support its business activities and establish and maintain strategic business alliances and new business initiatives, and the impacts of public health pandemics such as COVID-19 on business operations and expectations, as well as 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, 2021, 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.

Scholar Rock Holding Corporation
Condensed Consolidated Statements of Operations
(unaudited)
(in thousands, except share and per share data)

	Three Months Ended September 30		Nine Months Ended September 30	
	2021	2020	2021	2020
Revenue	$ 5,464	$ 3,037	$ 14,767	$ 11,967
Operating expenses				
Research and development	31,265	18,383	79,417	52,282
General and administrative	11,276	8,272	29,907	20,459
Total operating expenses	42,541	26,655	109,324	72,741
Loss from operations	(37,077)	(23,618)	(94,557)	(60,774)
Other income (expense), net	(430)	57	(1,328)	862
Net loss	$ (37,507)	$ (23,561)	$ (95,885)	$ (59,912)
Net loss per share, basic and diluted	$ (1.02)	$ (0.79)	$ (2.62)	$ (2.02)
Weighted average common shares outstanding, basic and diluted	36,683,026	29,779,114	36,549,833	29,665,995

Scholar Rock Holding Corporation
Condensed Consolidated Balance Sheets
(unaudited)
(in thousands)

	September 30, 2021	December 31, 2020
Assets		
Cash, cash equivalents and marketable securities	$ 246,435	$ 341,031
Other current assets	11,721	3,373
Total current assets	258,156	344,404
Other assets	40,233	43,901
Total assets	$ 298,389	$ 388,305
Liabilities and Stockholders' Equity		
Current liabilities	$ 64,601	$ 42,564
Long-term liabilities	46,528	84,971
Total liabilities	111,129	127,535
Total stockholders' equity	187,260	260,770
Total liabilities and stockholders' equity	$ 298,389	$ 388,305

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