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

August 10, 2021

- Phase 3 trial to evaluate apitegromab in patients with non-ambulatory Type 2 and Type 3 spinal muscular atrophy (SMA) anticipated to initiate by end of 2021

- DRAGON Phase 1 trial evaluating SRK-181's ability to overcome resistance to checkpoint inhibitors continues to progress and on-track to advance to Part B dose expansion mid-year 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 10, 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 second quarter ended June 30, 2021, and highlighted recent progress and upcoming milestones for its pipeline programs.

"Scholar Rock has had significant momentum in the first half of the year with further demonstration of apitegromab's transformative potential to improve motor function for patients with SMA, as well as continued progression of dose escalation in the DRAGON trial evaluating SRK-181's potential to overcome resistance to checkpoint inhibitors in solid tumors," said Nagesh Mahanthappa, Ph.D., Interim CEO of Scholar Rock. "I look forward to working closely with this exceptional team as we continue to advance our research and clinical programs to further elucidate the potential of our scientific platform and help make a difference in the lives of patients suffering from serious diseases."

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).*

- **Positive Top-line Results and Additional Supportive Exploratory Analyses from the TOPAZ Trial were Presented at the Cure SMA Annual Conference.** In June 2021, positive top-line data from the TOPAZ Phase 2 trial (NCT03921528) were presented by the lead principal investigator, Thomas Crawford, M.D. of Johns Hopkins Medicine. Treatment with apitegromab in conjunction with nusinersen in patients with Type 2 and 3 SMA led to meaningful motor function improvements of up to 20 points as measured by Hammersmith Functional Motor Scale Expanded (HFMSE). The majority (74%, 23/31) of non-ambulatory patients showed a clinical improvement, as defined by at least a 1-point increase in HFMSE. Further evidence that improvements in motor function may be attributed to apitegromab was supported by a post-hoc analysis that showed no correlation between change in HFMSE and duration of prior maintenance nusinersen therapy. In addition, 7/35 non-ambulatory patients also showed major functional achievements as measured by World Health Organization (WHO) Motor Development Milestones, with one patient achieving two and one patient achieving three new WHO motor milestones.
- **Phase 3 Trial Evaluating Apitegromab in Patients with Non-Ambulatory Type 2 and 3 Patients Anticipated to Initiate by Year-End 2021.** Subject to feedback from regulatory agencies, Scholar Rock intends to conduct a randomized, double-blind, placebo-controlled Phase 3 trial to evaluate apitegromab as an add-on therapy to nusinersen or risdiplam in patients with non-ambulatory Type 2 and Type 3 SMA. In the TOPAZ Phase 2 trial, non-ambulatory patients experienced the largest increases in motor function (HFMSE scores) following treatment with apitegromab as add-on to chronic maintenance nusinersen. Patients with non-ambulatory Type 2 and Type 3 SMA are estimated to represent approximately two-thirds of the overall prevalent SMA patient population.
- **Apitegromab Granted Fast Track Designation by the FDA for the Treatment of Patients with Spinal Muscular Atrophy.** In May 2021, the U.S. Food and Drug Administration (FDA) granted Fast Track designation for apitegromab, offering Scholar Rock eligibility to submit a rolling Biologic License Application (BLA) for apitegromab if relevant criteria are met. Fast Track designation is intended to facilitate the development and expedite the review of drugs to treat serious conditions and get new drugs to patients earlier. In addition to Fast Track designation, apitegromab had previously received Orphan Drug and Rare Pediatric Disease designations from the FDA as well as PRIME and Orphan Medicine Product designations from the European Medicines Agency for the treatment of SMA, all recognizing the unmet medical needs of patients with SMA.
- **Additional Potential Indications Identified for Apitegromab.** Scholar Rock has identified multiple diseases for which selectively inhibiting the activation of myostatin may offer therapeutic benefit, including additional patient populations in SMA (such as Type 1 SMA and ambulatory Type 3 SMA) and potential indications outside of SMA.

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

- **Plan to Advance to Part B Dose Expansion Portion of the DRAGON Phase 1 Proof-of-Concept Trial Mid-Year.**

SRK-181 is being evaluated in the two-part DRAGON trial (NCT04291079) in patients with locally advanced or metastatic solid tumors exhibiting primary resistance to anti-PD-(L)1 therapy. Part A dose escalation, which is evaluating the safety and pharmacokinetics of SRK-181 as a single-agent and in combination with anti-PD-(L)1 therapy, continues to progress to identify the recommended dose for Part B of the trial. The Company is on-track to advance to Part B dose expansion, which will consist of multiple cohorts, including urothelial carcinoma, cutaneous melanoma, non-small cell lung cancer, and other solid tumors. Each cohort will enroll up to 40 patients who have demonstrated primary resistance to anti-PD-(L)1 therapy and will be treated with SRK-181 in combination with an approved anti-PD-(L)1 therapy. An update on dose escalation and initial clinical data from Part A of the DRAGON trial is anticipated by year-end.

Second Quarter 2021 Financial Results

For the quarter ended June 30, 2021, net loss was $30.7 million or $0.84 per share compared to a net loss of $19.3 million or $0.65 per share for the quarter ended June 30, 2020.

- Revenue was $4.6 million for the quarter ended June 30, 2021 compared to $3.9 million for the quarter ended June 30, 2020 and was related to the Gilead fibrosis-focused collaboration (the “Gilead Collaboration Agreement”) that was executed in December 2018.
- Research and development expense was $25.6 million for the quarter ended June 30, 2021 compared to $17.0 million for the quarter ended June 30, 2020. The increase year-over-year primarily reflects manufacturing costs associated with apitegromab and higher personnel and facility-related costs, partially offset by lower manufacturing costs associated with SRK-181.
- General and administrative expense was $9.3 million for the quarter ended June 30, 2021 compared to $6.4 million for the quarter ended June 30, 2020. The increase year-over-year was primarily attributed to higher personnel and facility-related costs and professional fees.

“We are executing towards key milestones and continue to work closely with regulatory authorities to finalize the design of the Phase 3 trial for apitegromab and to progress the DRAGON trial to Part B to evaluate SRK-181 across multiple tumor types,” said Ted Myles, CFO and Head of Business Operations of Scholar Rock. “We ended the second quarter with approximately $282 million in cash and cash equivalents, which will allow us to achieve meaningful milestones as we continue to execute against our plan.”

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 12-month top-line results from the Phase 2 trial of apitegromab, 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, 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 June 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 June 30		Six Months Ended June 30	
	2021	2020	2021	2020
Revenue	$ 4,595	$ 3,900	$ 9,303	$ 8,930
Operating expenses				
Research and development	25,603	16,997	48,152	33,899
General and administrative	9,265	6,365	18,631	12,187
Total operating expenses	34,868	23,362	66,783	46,086
Loss from operations	(30,273)	(19,462)	(57,480)	(37,156)
Other income (expense), net	(434)	181	(898)	805
Net loss	$ (30,707)	$ (19,281)	$ (58,378)	$ (36,351)
Net loss per share, basic and diluted	$ (0.84)	$ (0.65)	$ (1.60)	$ (1.23)
Weighted average common shares outstanding, basic and diluted	36,582,708	29,690,280	36,482,132	29,608,814

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

	June 30, 2021	December 31, 2020
Assets		
Cash, cash equivalents and marketable securities	$ 282,217	$ 341,031
Other current assets	8,149	3,373
Total current assets	290,366	344,404
Other assets	41,685	43,901
Total assets	$ 332,051	$ 388,305
Liabilities and Stockholders' Equity		
Current liabilities	$ 67,022	$ 42,564
Long-term liabilities	48,373	84,971
Total liabilities	115,395	127,535
Total stockholders' equity	216,656	260,770
Total liabilities and stockholders' equity	$ 332,051	$ 388,305

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