Scholar Rock Reports Second Quarter 2023 Financial Results and Highlights Business Progress

August 9, 2023

- Recently presented 36-month apitegromab extension data from Phase 2 TOPAZ trial demonstrate long-term substantial and sustained improvement of motor function and further strengthen conviction in pivotal Phase 3 SAPPHIRE trial

- Pivotal Phase 3 SAPPHIRE trial evaluating apitegromab on track to complete enrollment in Q3 2023 with top-line data expected in 2H 2024

- Clinical and biomarker update from the Part B portion of the Phase 1 DRAGON trial of SRK-181 in solid tumors anticipated in 2H 2023

- Approximately $249 million in cash, cash equivalents, and marketable securities as of June 30, 2023, expected to fund operations into 2025

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 9, 2023-- Scholar Rock (NASDAQ: SRRK), a Phase 3 clinical-stage biopharmaceutical company focused on the treatment of serious diseases in which protein growth factors play a fundamental role, today reported financial results and corporate updates for the second quarter ended June 30, 2023.

“We continue to advance our apitegromab program, notably with the recent presentation of 36-month data from the Phase 2 TOPAZ trial which supports apitegromab’s therapeutic potential to meaningfully improve motor function for patients with SMA,” said Jay Backstrom, M.D., M.P.H., President and Chief Executive Officer of Scholar Rock. “The continued success of TOPAZ increases our conviction in SAPPHIRE and we look forward to completing enrollment this quarter and sharing a topline data readout in 2024.”

Recent Company Highlights and Upcoming Milestones

Spinal Muscular Atrophy (SMA) Program

Apitegromab is an investigational fully human monoclonal antibody that inhibits myostatin activation by selectively binding the pro- and latent forms of myostatin in skeletal muscle and is being developed as a potential first muscle-targeted therapy for the treatment of SMA.

- **Presented 36-month extension data from Phase 2 TOPAZ trial at Cure SMA Research & Clinical Care Meeting in June.** The company shared new data evaluating outcomes after 36 months of treatment with apitegromab, which showed substantial and sustained improvement in motor function, as well as improvements in patient-reported outcome measures in patients with nonambulatory Types 2 and 3 SMA receiving survival motor neuron (SMN) therapy. The company also hosted a virtual investor day event in July featuring key opinion leaders to discuss the current SMA treatment landscape and apitegromab’s potential to advance the standard of care.

- **Continued progress towards completion of enrollment for Phase 3 SAPPHIRE clinical trial.** The randomized, double-blind, placebo-controlled Phase 3 clinical trial evaluating the safety and efficacy of apitegromab in patients with nonambulatory Types 2 and 3 SMA receiving either nusinersen or risdiplam is actively enrolling SMA patients across sites in the U.S. and Europe. Enrollment completion is expected in the third quarter of 2023, with top-line data expected in 2024. If successful and if approved, the company expects to initiate a commercial product launch in 2025.

- **Initiated the ONYX trial, the long-term extension study for patients from both the TOPAZ and SAPPHIRE studies.** As of July 2023, more than 90 percent of patients from TOPAZ receiving apitegromab and a SMN therapy remained in the extension study.

Immuno-Oncology Program

SRK-181 is an investigational selective inhibitor of latent TGFβ1 activation and is being developed with the aim of overcoming resistance to checkpoint therapy in patients with advanced cancer.

- **Advancing Phase 1 DRAGON proof-of-concept trial.** DRAGON is evaluating SRK-181 in patients with locally advanced or metastatic solid tumors that exhibit resistance to anti-PD-(L)1 antibodies. The Company will provide biomarker and clinical updates from Part B of the DRAGON trial in the second half of 2023.

Second Quarter 2023 Financial Results

For the quarter ended June 30, 2023, net loss was $37.9 million compared to a net loss of $44.0 million for the quarter ended June 30, 2022.

- The Company did not record any revenue for either the quarter ended June 30, 2023 or June 30, 2022.

- Research and development expense was $26.9 million for the quarter ended June 30, 2023, compared to $32.1 million for the quarter ended June 30, 2022. The decrease was primarily attributable to a decrease in external research and

development costs, including costs for the apitegromab clinical trials and associated clinical trial material manufacturing costs. In addition, there was a decrease in employee compensation and benefits costs, resulting from the restructuring in May 2022.

- General and administrative expense was $12.2 million for the quarter ended June 30, 2023, compared to $11.1 million for the quarter ended June 30, 2022.

- As of June 30, 2023, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $249 million, which is expected to fund the company's anticipated operating and capital expenditure requirements into 2025.

"We are relentlessly focused on execution, and we are encouraged by the continued strong data from TOPAZ and momentum we're seeing in the enrollment of SAPPHIRE. Our overall execution, along with our spend for the first six months of 2023, is aligned with our strategic plan," said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock. "We ended the quarter with $249 million in cash, which we expect to provide runway through a number of anticipated important milestones."

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 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/).

Availability of Other Information About Scholar Rock

Investors and others should note that we communicate with our investors and the public using our company website www.scholarrock.com, including, but not limited to, company disclosures, investor presentations and FAQs, Securities and Exchange Commission filings, press releases, public conference call transcripts and webcast transcripts, as well as on Twitter and LinkedIn. The information that we post on our website or on Twitter or LinkedIn could be deemed to be material information. As a result, we encourage investors, the media and others interested to review the information that we post there on a regular basis. The contents of our website or social media shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

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 and SRK-181 and its preclinical programs and indication selection and development timing, including the therapeutic potential, clinical benefits and safety thereof, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, its cash runway, expectations regarding the achievement of important milestones, 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," "could," "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, without limitation, that preclinical and clinical data, including the results from the Phase 2 clinical trial of apitegromab, or Part A of the Phase 1 clinical trial of SRK-181, are not predictive of, may be inconsistent with, or more favorable than, data generated from future clinical trials of the same product candidates, including, without limitation, the Phase 3 clinical trial of apitegromab in SMA or Part B of the Phase 1 clinical trial of SRK-181; 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; Scholar Rock's dependence on third parties for development and manufacture of product candidates including, without limitation, 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, 2023, 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	
	2023	2022	2023	2022
Revenue	$ —	$ —	$ —	$ 33,193
Operating expenses				
Research and development	26,867	32,073	56,602	61,439
General and administrative	12,215	11,074	22,989	21,834
Total operating expenses	39,082	43,147	79,591	83,273
Loss from operations	(39,082)	(43,147)	(79,591)	(50,080)
Other income (expense), net	1,157	(853)	2,287	(1,870)
Net loss	$ (37,925)	$ (44,000)	$ (77,304)	$ (51,950)
Net loss per share, basic and diluted	$ (0.47)	$ (1.06)	$ (0.97)	$ (1.31)
Weighted average common shares outstanding, basic and diluted	80,117,983	41,622,392	79,865,424	39,550,991

Condensed Consolidated Balance Sheets
(unaudited)
(in thousands)

	June 30, 2023	December 31, 2022
Assets		
Cash, cash equivalents and marketable securities	$ 248,734	$ 315,361
Other current assets	11,076	12,663
Total current assets	259,810	328,024
Other assets	25,090	30,144
Total assets	$ 284,900	$ 358,168
Liabilities and Stockholders' Equity		
Current liabilities	$ 24,270	$ 36,389
Long-term liabilities	58,129	61,544
Total liabilities	82,399	97,933
Total stockholders' equity	202,501	260,235
Total liabilities and stockholders' equity	$ 284,900	$ 358,168

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Scholar Rock:

Investors & Media
Rushmie Nofsinger
Scholar Rock
rnofsinger@scholarrock.com
ir@scholarrock.com
857-259-5573

Source: Scholar Rock