Scholar Rock Reports Third Quarter 2022 Financial Results and Highlights Business Progress

November 14, 2022

- Presented 24-month apitegromab data on quality-of life-measures from the TOPAZ trial and SRK-181 data from DRAGON trial at major medical/scientific congresses

- Announced key leadership appointments

- Amended existing debt facility with Oxford finance and Silicon Valley Bank, providing an additional $25 million of capacity and extending interest-only period and loan maturity date

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 14, 2022-- 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 third quarter ended September 30, 2022.

"We continue to build conviction in our clinical programs with recent TOPAZ data indicating apitegromab's potential for sustained improvements of quality-of-life measures over 24 months, and with the most recently available SRK-181 data from DRAGON that we presented at SITC," said Jay Backstrom, M.D., M.P.H., Chief Executive Officer & President of Scholar Rock. "I am excited to strengthen our experienced executive team with the addition of Scholar Rock's new CMO, Dr. Jing Marantz. I am confident the continued momentum of the Phase 3 SAPPHIRE trial and the DRAGON trial will be further bolstered under her leadership."

Company Highlights and Upcoming Milestones

Pipeline Updates

- ***Apitegromab** is a selective inhibitor of myostatin activation being developed as the potential first muscle-targeted therapy for the treatment of spinal muscular atrophy (SMA).*
    - **Presented new Phase 2 TOPAZ trial data indicating positive trends in quality-of-life measures over 24 months with apitegromab for nonambulatory patients with Types 2 and 3 SMA.** Scholar Rock presented these data, which indicate the potential for sustained improvement in quality-of-life measures for patients with symptomatic SMA and offer further signs of possible durable effects of apitegromab, at the World Muscle Society (WMS) Hybrid Congress and the International Scientific Congress on Spinal Muscular Atrophy in October.
    - **Continued enrollment of the Phase 3 SAPPHIRE clinical trial.** SAPPHIRE, a randomized, double-blind, placebo-controlled clinical trial evaluating apitegromab for patients with non-ambulatory Types 2 and 3 SMA on either nusinersen or risdiplam, is actively enrolling SMA patients across 55 sites in the U.S. and Europe. The anticipated enrollment is approximately 156 patients aged 2-12 in the main efficacy population.
- ***SRK-181** is an investigational selective inhibitor of latent TGFβ1 activation and is being developed with the aim of overcoming resistance in patients with advanced cancer.*
    - **Data from the Phase 1 DRAGON trial presented at the Society for Immunotherapy of Cancer's Annual Meeting show that SRK-181 continues to be generally well tolerated with early indications of efficacy (as of the data cut-off date of August 29, 2022).**
        - No dose-limiting toxicities were observed in patients participating in Part A, and all dose levels were generally well tolerated, including the recommended SRK-181 dose of 1500 mg q3w or 1000 mg q2w in combination with anti-PD-(L)1 for Part B.
        - In Part A2 of the trial (n=15), there was one confirmed partial response ("PR") in a patient with anti-PD-1 resistant clear cell renal cell carcinoma at 800mg who remained in the study for 30 weeks. Response was assessed based on RECIST 1.1 by principal investigators. One ongoing patient in the 2400 mg dose group of Part A2 with head and neck cancer experienced a 29.4% tumor reduction.
        - Enrollment in DRAGON Part B is ongoing. Across the cohorts, 14 patients have been dosed as of the data cut-off date. The recommended dose of 1500mg q3w or 1000mg q2w SRK-181 in combination with anti-PD-(L)1 was generally well tolerated. There was a confirmed PR in one ongoing patient with anti-PD-1 resistant clear cell renal cell carcinoma. Scholar Rock will continue to provide updates at future scientific meetings.

Corporate Updates

- **Announced key leadership appointments:**
  - Jay Backstrom, M.D., M.P.H. was appointed President & CEO in October, bringing an exceptional range of research and development, regulatory, and leadership experience spanning several decades in the biopharmaceutical industry. He has been instrumental in organizing and executing development strategies and has led programs in a broad range of therapeutic areas through regulatory approvals. Dr. Backstrom most recently served as Executive Vice President, Research and Development at Acceleron Pharma, which was acquired by Merck in 2021.
  - Jing L. Marantz, M.D., Ph.D., M.B.A., was appointed Chief Medical Officer in November. Dr. Marantz is an accomplished biopharmaceutical executive with over 20 years of industry experience spanning multiple specialties, including neurology, hematology/oncology, and rare diseases. Most recently, she served as the Executive Vice President and Chief Business Officer at Krystal Biotech. Previously, she was Senior Vice President and Head of Medical Affairs at Acceleron Pharma, where she also was a member of the R&D leadership team.

- **Amended debt facility with Oxford Finance and Silicon Valley Bank.** Scholar Rock amended the existing debt facility with Oxford Finance and Silicon Valley Bank. Under the agreement, an additional $25 million tranche is available at the company's discretion and subject to meeting certain criteria. The amendment also extends both the interest-only period for an additional 24 months to November 2024, and the loan maturity date to November 2027.

Third Quarter 2022 Financial Results

For the quarter ended September 30, 2022, net loss was $43.3 million or $0.55 per share compared to a net loss of $37.5 million or $1.02 per share for the quarter ended September 30, 2021.

- Revenue was $0 for the quarter ended September 30, 2022, compared to $5.5 million for the quarter ended September 30, 2021.

- Research and development expense was $33.4 million for the quarter ended September 30, 2022, compared to $31.3 million for the quarter ended September 30, 2021. The increase was primarily attributable to expenses related to non-cash equity-based compensation expense, a component of employee compensation and benefits costs.

- General and administrative expense was $10.5 million for the quarter ended September 30, 2022, compared to $11.3 million for the quarter ended September 30, 2021. The decrease was attributable to the reduction of employee severance and benefits expenses that were recognized during the same period in the prior year.

- As of September 30, 2022, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $343.7 million, which is expected to fund the Company's anticipated operating and capital expenditure requirements into 2025.

"With the successful equity financing that we completed in the second quarter and the added flexibility provided by the amendment of our debt facility, we are well funded to advance our pipeline through key milestones," said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock. "We believe the company is well positioned to deliver value to patients with unmet medical needs. With sufficient capital to complete the topline data readout from the SAPPHIRE study, progress Part B of the DRAGON trial, and continue advancing select preclinical programs, we are laser focused on the successful execution of these priorities."

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/).

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.

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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," "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 candidate, 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 September 30, 2022, 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	
	2022	2021	2022	2021
Revenue	$ —	$ 5,464	$ 33,193	$ 14,767
Operating expenses				
Research and development	33,392	31,265	94,831	79,417
General and administrative	10,470	11,276	32,304	29,907
Total operating expenses	43,862	42,541	127,135	109,324
Loss from operations	(43,862)	(37,077)	(93,942)	(94,557)
Other income (expense), net	565	(430)	(1,305)	(1,328)
Net loss	$ (43,297)	$ (37,507)	$ (95,247)	$ (95,885)
Net loss per share, basic and diluted	$ (0.55)	$ (1.02)	$ (1.80)	$ (2.62)
Weighted average common shares outstanding, basic and diluted	79,336,161	36,683,026	52,958,447	36,549,833

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

	September 30, 2022	December 31, 2021

Assets		
Cash, cash equivalents and marketable securities	$ 343,655	$ 252,994
Other current assets	13,957	12,325
Total current assets	357,612	265,319
Other assets	32,691	39,126
Total assets	$ 390,303	$ 304,445
Liabilities and Stockholders' Equity		
Current liabilities	$ 33,736	$ 64,297
Long-term liabilities	63,302	68,074
Total liabilities	97,038	132,371
Total stockholders' equity	293,265	172,074
Total liabilities and stockholders' equity	$ 390,303	$ 304,445

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