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

May 9, 2023

- Enrollment of pivotal Phase 3 SAPPHIRE trial evaluating apitegromab on track for completion in 2023

- Upcoming presentations of 36-month apitegromab data from Phase 2 TOPAZ trial at Cure SMA's Annual Research & Clinical Conference

- Continued advancement of Phase 1 DRAGON trial of SRK-181; biomarker and clinical data update planned in the second half of 2023

- Approximately $275 million in cash, cash equivalents, and marketable securities as of March 31, 2023, expected to fund operations into 2025

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 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 first quarter ended March 31, 2023.

"We are executing across our business and leveraging Scholar Rock's differentiated platform, which selectively targets latent forms of growth factors, such as myostatin and TGFβ1, to develop highly selective and potentially transformative therapies for patients who have high unmet medical need," said Jay Backstrom, M.D., M.P.H., President & Chief Executive Officer of Scholar Rock. "We continue to advance our two clinical programs in spinal muscular atrophy and oncology, and we look forward to sharing updates on our progress as we approach several key milestones, including presenting 36-month data on apitegromab from the Phase 2 TOPAZ trial, completing enrollment of our apitegromab Phase 3 SAPPHIRE trial, and providing biomarker and clinical updates from our SRK-181 Phase 1 DRAGON trial."

Recent Company Highlights and Upcoming Milestones

Apitegromab is an investigational antibody inhibiting myostatin activation by selectively binding the pro- and latent forms of myostatin and is being developed as the potential first muscle-targeted therapy for the treatment of spinal muscular atrophy (SMA).

- **Continued progress towards completion of enrollment for Phase 3 SAPPHIRE clinical trial.** The randomized double-blind, placebo-controlled clinical trial evaluating apitegromab for patients with nonambulatory Types 2 and 3 SMA on either nusinersen or risdiplam, is actively enrolling SMA patients across sites in the U.S. and Europe. Enrollment completion is expected in 2023, with the top-line data readout expected in 2024. If successful and if approved, the company expects to initiate a commercial product launch in 2025.
- **36-month data from Phase 2 TOPAZ trial to be presented at Cure SMA conference in June**. The company announced two upcoming oral presentations at the Cure SMA Research & Clinical Conference, taking place June 28-30 in Orlando, Florida.
- **24-month data from the TOPAZ trial presented at the 2023 Muscular Dystrophy Association (MDA) Clinical and Scientific Conference in March.** The company shared data evaluating outcomes after 24 months of treatment with apitegromab, demonstrating sustained improvements in motor function in patients with nonambulatory Types 2 and 3 SMA.

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.** In the second half of 2023, the company expects to provide biomarker and clinical updates from Part B of the DRAGON trial. In March 2023, Scholar Rock presented encore data at the European Society for Medical Oncology Targeted Anticancer Therapies (ESMO TAT) Congress, which showed that SRK-181 continued to be generally well tolerated with early indications of efficacy (as of the data cut-off date of December 2, 2022).

Corporate

Announced the addition of Richard Brudnick to the Board of Directors. In April 2023, Mr. Brudnick joined Scholar Rock's Board of Directors. As an accomplished biotechnology executive, he brings an extensive background in corporate development and strategy, with over 30 years of industry experience across multiple specialties, stages and therapeutic areas.

First Quarter 2023 Financial Results

For the quarter ended March 31, 2023, net loss was $39.4 million or $0.49 per share compared to a net loss of $8.0 million or $0.21 per share for the quarter ended March 31, 2022.

- Revenue was $0 for the quarter ended March 31, 2023, compared to $33.2 million for the quarter ended March 31, 2022. The prior year revenue was related to the Gilead fibrosis-focused research collaboration, which was executed in December 2018 and concluded in December 2021.
- Research and development expense was $29.7 million for the quarter ended March 31, 2023, compared to $29.4 million for the quarter ended March 31, 2022. The increase was primarily attributable to costs associated with clinical trials, including the Phase 3 SAPPHIRE pivotal trial for apitegromab in SMA, and the DRAGON trial for SRK-181. These increases were offset by decreases in employee compensation and benefits costs, resulting from the restructuring in May 2022.
- General and administrative expense was $10.8 million for the quarter ended March 31, 2023, compared to $10.8 million for the quarter ended March 31, 2022.
- As of March 31, 2023, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $275 million, which is expected to fund the company's anticipated operating and capital expenditure requirements into 2025.

"Scholar Rock is in a strong financial position, and we are relentlessly focused on achieving our goal of improving the lives of patients who may potentially benefit from our medicines," said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock. "We continue to execute on our plan by advancing our clinical programs towards key inflection points, while diligently managing our resources."

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.

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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 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 March 31, 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 March 31	
	2023	**2022**
Revenue	$ —	$ 33,193
Operating expenses		
Research and development	29,735	29,366
General and administrative	10,774	10,760
Total operating expenses	40,509	40,126
Loss from operations	(40,509)	(6,933)
Other income (expense), net	1,130	(1,017)
Net loss	$ (39,379)	$ (7,950)
Net loss per share, basic and diluted	$ (0.49)	$ (0.21)
Weighted average common shares outstanding, basic and diluted	79,610,059	37,456,574

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

	March 31, 2023	**December 31, 2022**
Assets		
Cash, cash equivalents and marketable securities	$ 275,284	$ 315,361
Other current assets	11,874	12,663
Total current assets	287,158	328,024
Other assets	27,563	30,144
Total assets	$ 314,721	$ 358,168
Liabilities and Stockholders' Equity		
Current liabilities	$ 26,208	$ 36,389
Long-term liabilities	59,860	61,544
Total liabilities	86,068	97,933
Total stockholders' equity	228,653	260,235
Total liabilities and stockholders' equity	$ 314,721	$ 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, Inc.