Scholar Rock Reports Full Year 2022 Financial Results and Highlights Business Progress

March 7, 2023

- Enrollment of pivotal Phase 3 SAPPHIRE trial evaluating apitegromab on track for completion in 2023, and 36-month extension data from Phase 2 TOPAZ trial expected in mid-2023

- Phase 1 DRAGON trial of SRK-181 continues to advance with biomarker and clinical data updates planned in 2023

- Company expanded commercial experience with recent appointment of Chief Commercial Officer

- Ended 2022 with approximately $315 million in cash, cash equivalents, and marketable securities, which is expected to fund operations into 2025

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 7, 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 full year ended December 31, 2022.

“2023 is off to a great start as we continue to build upon our momentum from last year. Importantly, we are on track to complete the enrollment of our pivotal SAPPHIRE trial of apitegromab and are looking forward to providing 36-month extension data from the Phase 2 TOPAZ trial of apitegromab in the coming months. Additionally, we look forward to sharing biomarker and clinical updates throughout the year from the SRK-181 Phase 1 DRAGON trial in patients with relapsed and/or refractory cancers resistant to checkpoint inhibitor therapy,” said Jay Backstrom, M.D., M.P.H., President & Chief Executive Officer of Scholar Rock.

Company Highlights and Upcoming Milestones

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

- **Continue to progress and complete enrollment of Phase 3 SAPPHIRE clinical trial.** The last patient is expected to be enrolled in SAPPHIRE 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. SAPPHIRE is a randomized, double-blind, placebo-controlled Phase 3 clinical trial evaluating apitegromab in patients with nonambulatory Types 2 and 3 SMA on either nusinersen or risdiplam. The U.S. Food and Drug Administration (FDA) has granted Fast Track, Orphan Drug, and Rare Pediatric Disease designations, and the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) and Orphan designations to apitegromab for the treatment of SMA.
- **36-month extension data from Phase 2 TOPAZ trial to be presented in mid-2023.** As of December 31, 2022, approximately 90 percent of patients who entered the trial’s long-term extension study (51/57) remained on study. At the Cure SMA Research & Clinical Care Meeting in June 2022, the company presented 24-month extension data from the TOPAZ trial which demonstrated sustained gains in Hammersmith Functional Motor Scale Expanded (HFMSE), increased Revised Upper Limb Module (RULM) scores, and positive trends in quality-of-life data for nonambulatory patients with Types 2 and 3 SMA receiving an SMN therapy.

SRK-181 is an investigational selective inhibitor of latent TGFβ-1 activation and is being developed with the aim of overcoming resistance to checkpoint therapy in patients with advanced cancer.

- **Continue to progress Phase 1 DRAGON trial.** The company expects to provide biomarker and clinical updates from the DRAGON proof-of-concept trial throughout 2023. In November 2022, Scholar Rock presented data from DRAGON at the Society for Immunotherapy of Cancer’s (SITC) Annual Meeting that showed SRK-181 continued to be generally well tolerated with early indications of efficacy (as of the data cut-off date of August 29, 2022).
- **Encore data from DRAGON will be presented at the European Society for Medical Oncology Targeted Anticancer Therapies (ESMO TAT) Congress** in Paris, France on March 7 at 11:05-12:30 p.m. EST. The presentation is titled “Safety and efficacy results of SRK-181 (a latent TGFβ1 inhibitor) from a phase 1 trial (DRAGON trial).”

Corporate

- **Appointed Tracey M. Sacco as Chief Commercial Officer.** In February 2023, Ms. Sacco joined Scholar Rock to build and lead all commercial functions, including sales, marketing, market access, and commercial operations. She will help the company prepare for the potential commercial launch of apitegromab, if approved. Ms. Sacco brings extensive biopharmaceutical industry experience, including commercial strategy and global launch experience of rare disease products.

Full Year 2022 Financial Results

For the full year ended December 31, 2022, net loss was $134.5 million or $2.26 per share compared to a net loss of $131.8 million or $3.59 per share for the full year ended December 31, 2021.

- Revenue was $33.2 million for the year ended December 31, 2022, compared to $18.8 million for the year ended December 31, 2021. Revenue recognized during both periods was associated with the Gilead collaboration which ended in 2022.
- Research and development expense was $124.4 million for the year ended December 31, 2022, compared to $108.5 million for the year ended December 31, 2021. The increase was primarily attributable to costs associated with running the company's Phase 3 SAPPHIRE pivotal trial for apitegromab in SMA, including clinical trials costs as well as costs associated with employee compensation and benefits.
- General and administrative expense was $43.1 million for the year ended December 31, 2022, compared to $40.3 million for the year ended December 31, 2021. The increase was due to an increase in employee compensation and benefits, primarily associated with equity-based compensation and professional fees.
- As of December 31, 2022, Scholar Rock had cash, cash equivalents, and marketable securities of approximately $315 million, which is expected to fund the Company's anticipated operating and capital expenditure requirements into 2025.

"As Scholar Rock works to develop transformative therapies for patients with our highly differentiated platform, we are entering 2023 with an experienced team, a strong balance sheet, and a clear line of sight to important anticipated milestones, all of which are expected to help advance the next phase of growth for the company," said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock.

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/). 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.

Scholar Rock® is a registered trademark of Scholar Rock, Inc.

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, and 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 Annual Report on Form 10-K for the year ended December 31, 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)

	Years Ended December 31	
	2022	**2021**
Revenue	$ 33,193	$ 18,816
Operating expenses		
Research and development	124,444	108,468
General and administrative	43,119	40,269
Total operating expenses	167,563	148,737
Loss from operations	(134,370)	(129,921)
Other income (expense), net	(132)	(1,878)
Net loss	$ (134,502)	$ (131,799)
Net loss per share, basic and diluted	$ (2.26)	$ (3.59)
Weighted average common shares outstanding, basic and diluted	59,611,656	36,711,833

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

	December 31, 2022	**December 31, 2021**
Assets		
Cash, cash equivalents and marketable securities	$ 315,361	$ 252,994
Other current assets	12,663	12,325
Total current assets	328,024	265,319
Other assets	30,144	39,126
Total assets	$ 358,168	$ 304,445
Liabilities and Stockholders' Equity		
Current liabilities	$ 36,389	$ 64,297
Long-term liabilities	61,544	68,074
Total liabilities	97,933	132,371
Total stockholders' equity	260,235	172,074
Total liabilities and stockholders' equity	$ 358,168	$ 304,445

View source version on businesswire.com: https://www.businesswire.com/news/home/20230307005330/en/

Scholar Rock:

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

Media
Ariane Lovell
Finn Partners
ariane.lovell@finnpartners.com
media@scholarrock.com
917-565-2204

Source: Scholar Rock