



## Scholar Rock Provides Corporate Update and Highlights Priorities for 2023

January 9, 2023

- Pivotal Phase 3 SAPPHIRE trial enrollment completion expected in 2023

- Phase 1 DRAGON trial of SRK-181 continues to advance with presented data that showed early indications of efficacy; ongoing clinical data updates planned in 2023

- Anticipated cash runway into 2025

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 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 provided recent corporate updates and highlighted upcoming priorities for its pipeline programs in 2023.

"In 2022, Scholar Rock made significant progress in advancing its clinical programs, notably with the 24-month extension data from the Phase 2 TOPAZ trial, which reinforces our conviction behind apitegromab and the Phase 3 SAPPHIRE trial, and with early data readouts from the Phase 1 DRAGON trial. The company also strengthened its financials in June 2022 with a substantial equity raise. We are excited by the potential coming from our highly differentiated platform targeting growth factors like TGF $\beta$ , as we advance our spinal muscular atrophy and oncology programs to address critical unmet needs for patients," said Dr. Jay Backstrom, M.D., M.P.H., President & CEO of Scholar Rock. "In 2023, we see continued momentum for our growing pipeline, including completing enrollment of our pivotal SAPPHIRE trial, disclosing 36-month extension data from the Phase 2 TOPAZ trial and clinical and biomarker updates from the SRK-181 Phase 1 DRAGON trial, and advancing two preclinical assets towards IND-enabling studies in fibrosis and iron-restricted anemia."

### 2023 Priorities:

**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).

- **Complete enrollment of Phase 3 SAPPHIRE clinical trial in 2023.** SAPPHIRE is a randomized, double-blind, placebo-controlled clinical trial evaluating apitegromab for patients with nonambulatory Types 2 and 3 SMA on either nusinersen or risdiplam. The last patient is expected to be enrolled in SAPPHIRE in 2023, with the top-line data readout expected in 2024. If successful, the company expects to initiate a commercial product launch in 2025.
- **Progress TOPAZ long-term extension to 36-month data readout.** The company expects to report 36-month extension data in the first half of 2023. As of December 31, 2022, approximately 90 percent of patients (51/57) remained enrolled in the trial's long-term extension period.

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

- **Advance Progress in DRAGON Phase 1 trial.** Scholar Rock is expecting to provide biomarker and clinical updates from the DRAGON Phase 1 trial in 2023.

### Preclinical fibrosis and iron-restricted anemia assets

- **Advance the fibrosis program towards IND-enabling studies.** Scholar Rock plans to advance a highly potent, anti-latent TGF $\beta$ -1 antibody that selectively inhibits TGF $\beta$ 1 activation within the extracellular matrix by targeting latent TGF $\beta$ -1 associated with latent TGF $\beta$ -binding proteins (LTBPs), thus enabling specific inhibition of TGF $\beta$ -1 in fibrotic tissue.
- **Advance the iron-restricted anemia program towards IND-enabling studies.** Scholar Rock plans to advance a highly selective, RGMc/HJV antibody that targets the signaling of BMP6, a key regulator of iron availability in the body. Utilizing Scholar Rock's unique structural biology insights into BMP6 and its co-receptors and leveraging its novel antibody discovery and optimization platform, the company generated an anti-RGMc antibody that can modulate iron release and has the potential to address anemia.

### 2022 Highlights:

- TOPAZ 24-month extension trial data showed sizeable and sustained gains in Hammersmith Functional Motor Scale Expanded (HFMSSE), increased Revised Upper Limb Module (RULM), and positive trends in quality-of-life data for nonambulatory patients with Types 2 and 3 SMA receiving an SMN-targeted therapy.

- Completed equity financing of \$205 million in June. 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 operations into 2025.
- Phase 1 DRAGON trial data presented at the Society for Immunotherapy of Cancer's Annual Meeting in November showed that SRK-181 continued to be generally well tolerated with early indications of efficacy (as of the data cut-off date of August 29, 2022).
- Presented new data on LTBP showing reduction of TGFβ-1 signaling and fibrosis in relevant in vivo preclinical models. The findings were presented at the 2022 FASEB Science Research Conference in July and the American College of Toxicology Annual Meeting in November.
- Announced 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.
- Announced 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.

"With our strong balance sheet and two well established clinical programs, both of which we expect to generate data in 2023, Scholar Rock is uniquely positioned to bring differentiated therapies to patients suffering from serious diseases in which protein growth factors play a fundamental role," said Ted Myles, Chief Operating Officer and Chief Financial Officer.

## 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](http://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](http://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 current macroeconomic and geopolitical events, including changing conditions from the COVID-19 pandemic, hostilities in Ukraine, increasing rates of inflation and rising interest rates, 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.

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