Scholar Rock Reports Third Quarter 2024 Financial Results and Highlights Business Progress

November 12, 2024

- Reported positive topline data from pivotal Phase 3 SAPPHIRE trial evaluating apitegromab in patients with Spinal Muscular Atrophy (SMA), achieving primary endpoint

- On track to submit a U.S. Biologics License Application (BLA) and European Union marketing authorisation application (MAA) in 1Q 2025

- Phase 2 EMBRAZE trial enrollment completed for apitegromab in obesity with topline data expected in 2Q 2025

- Successful completion of upsized $345 million public offering to fund planned commercial launch in SMA and continue to advance priority programs

- Management to host update call today at 8:15 a.m. ET

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 12, 2024-- Scholar Rock (NASDAQ: SRRK), a late-stage biopharmaceutical company focused on advancing innovative treatments for spinal muscular atrophy (SMA), cardiometabolic disorders, and other serious diseases where protein growth factors play a fundamental role, today reported financial results and corporate updates for the third quarter ended September 30, 2024.

“We were thrilled to announce positive and statistically significant topline data from the pivotal Phase 3 SAPPHIRE trial, a major achievement that underscores apitegromab’s potential to redefine the standard of care for patients with SMA,” said Jay Backstrom, M.D., MPH, President and Chief Executive Officer of Scholar Rock. “The positive SAPPHIRE trial validates our approach to targeting pro-and latent myostatin and demonstrates apitegromab’s potentially transformative benefit. Based on these results, we plan to submit a BLA and MAA in the first quarter of 2025 and are working diligently to prepare for the commercial launch of apitegromab, and if approved, to deliver the potentially transformative benefits of apitegromab to children and adults living with SMA.”

Dr. Backstrom continued, “The robust results from SAPPHIRE also reinforce the broader potential of our selective myostatin inhibition strategy across our pipeline, with potential readthrough to our cardiometabolic program as we evaluate apitegromab in obesity in our Phase 2 EMBRAZE proof-of-concept trial. We were pleased to announce in September that the EMBRAZE trial is fully enrolled, and we expect to report initial data in the second quarter of next year.”

Company Highlights and Upcoming Milestones

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. Apitegromab is the first muscle-targeted therapy to show clinical proof-of-concept in SMA.

- **Reported positive topline data from Phase 3 SAPPHIRE clinical trial**. The study achieved its primary endpoint demonstrating a statistically significant and clinically meaningful improvement for apitegromab versus placebo in motor function as measured by the gold standard HFMSE in SMA patients on chronic dosing of standard of care therapies (either nusinersen or risdiplam).
    - In the main efficacy population (ages 2-12), the mean difference in change from baseline in HFMSE was 1.8 points (p=0.0192) for all patients receiving apitegromab 10 mg/kg and 20 mg/kg (n=106) compared to placebo (n=50). Patients receiving 20 mg/kg of apitegromab (n=53) showed a 1.4 point mean difference compared to placebo (p=0.1149).
    - The prespecified analysis of the 10 mg/kg dose showed that patients receiving 10 mg/kg of apitegromab (n=53) showed a mean difference in change from baseline in HFMSE of 2.2 points compared to placebo (nominal p=0.0121).
    - Based upon PK/PD data from the SAPPHIRE trial, similar levels of target engagement were observed for the 10 mg/kg and 20 mg/kg dose groups.
    - Scholar Rock plans to submit a U.S. BLA and a European Union MAA in 1Q 2025.
- **Presented preliminary baseline characteristics from SAPPHIRE during a poster presentation at the 29th Annual Congress of the World Muscle Society in October in Prague, Czech Republic.** Analyses of the full Phase 3

SAPPHIRE data are ongoing, and Scholar Rock plans to present detailed results at an upcoming medical conference in early 2025.
- **The ONYX open-label, multi-center extension study remains ongoing.** The study is evaluating the long-term safety and efficacy of apitegromab in patients who completed the TOPAZ or SAPPHIRE trials. More than 90 percent of patients on combination therapy in the TOPAZ trial have completed 4 years of apitegromab treatment and enrolled into ONYX. Following trial completion, 98 percent of SAPPHIRE patients (185/188) enrolled in the ongoing ONYX open-label expansion study.
- **On track to initiate OPAL clinical trial in SMA patients under two years old in mid-2025.**

Cardiometabolic Program

SRK-439 is a novel, preclinical, investigational myostatin inhibitor that binds to pro- and latent myostatin with high affinity and is selective for myostatin (i.e., no GDF11 or Activin A binding), and is initially being developed for the treatment of obesity. Based on preclinical data, SRK-439 has the potential to support healthier weight management by preserving lean mass during weight loss.

- **Completed enrollment of Phase 2 EMBRAZE proof-of-concept trial with apitegromab in combination with a GLP-1 receptor agonist (GLP-1 RA) in obesity in September.** The Phase 2 trial is a randomized, double-blind, placebo-controlled, multi-center study evaluating the safety and efficacy of apitegromab, a highly selective investigational myostatin inhibitor, to preserve muscle mass as an adjunctive therapy in overweight and obese adults who are taking a GLP-1 RA. Data are expected in the second quarter of 2025 and will be used to guide clinical development of SRK-439. The Company plans to file an IND for SRK-439 for the treatment of obesity in 2025.

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, such as anti-PD-(L)1 antibodies, in patients with advanced cancer.

- **New SRK-181 data from the Phase 1 DRAGON proof-of-concept trial presented at the SITC 39th Annual Meeting in November.** The presentation, “DRAGON Trial: Durable remission rate with the latent TGFβ1 inhibitor linavonkibart (SRK-181) and pembrolizumab in patients with immune checkpoint inhibitor resistant advanced cancers,” highlighted new clinical and biomarker results. Highlights since the Company’s last update on the DRAGON trial include one additional complete response, seen in the clear cell renal cell (ccRCC) cohort (ORR=23.3%) and durable responses in multiple cohorts. Additionally, biomarker results continue to support proof of mechanism; new data shows suppressed granulocytic myeloid-derived suppressor cells (gMDSC) levels in the tumor microenvironment of responders. Notably new biomarker data in the ccRCC cohort supports a potential patient selection strategy with positive correlations between clinical response and baseline CD8+ infiltration status, elevated baseline Treg levels, and elevated baseline TGFβ1 levels in the tumor compartment. Enrollment of the DRAGON trial was completed in December 2023, and patients who remain on the study continue to be treated.

Other Corporate Updates

- **Appointed Beth Shafer, Ph.D., to the newly created role of Chief Business Officer.** Ms. Shafer brings over 20 years of biopharmaceutical industry leadership and expertise to Scholar Rock, where she will drive the Company’s long-term corporate and business development strategy.
- **Raised $345 million in upsized follow-on offering.** In October, the Company completed an upsized follow-on offering raising aggregate gross proceeds of $345 million.

Third Quarter 2024 Financial Results

For the quarter ended September 30, 2024, net loss was $64.5 million or $0.66 per share compared to a net loss of $42.4 million or $0.53 per share for the quarter ended September 30, 2023.

- The Company did not record any revenue for the quarter ended September 30, 2024, or for the quarter ended September 30, 2023.
- Research and development expense was $48.7 million for the quarter ended September 30, 2024, compared to $30.3 million for the quarter ended September 30, 2023. The increase was primarily attributable to costs associated with the ONYX and EMBRAZE trials, and the development of SRK-439.
- General and administrative expenses were $16.1 million for the quarter ended September 30, 2024, compared to $13.3 million for the quarter ended September 30, 2023. The increase was due to employee-related costs.
- As of September 30, 2024, Scholar Rock had cash, cash equivalents and marketable securities of approximately $139.1 million ($463.5 million, on a pro forma basis, including the approximately $324.4 million raised in the October 2024 equity offering). Along with cash available to the Company, runway is extended into 4Q 2026.

“Supported by our recent upsized public offering on the heels of successful SAPPHIRE data, Scholar Rock is advancing our mission from a position of strength as we move forward with preparations for our first commercial launch in the fourth quarter of 2025,” said Ted Myles, Chief Operating Officer and Chief Financial Officer of Scholar Rock. “We are working tirelessly to obtain approval for apitegromab and deliver it to patients with SMA as soon as possible, as well as continuing to advance our portfolio of highly selective myostatin inhibition programs.”

Conference Call Information

Management will provide an update on the Company and discuss third quarter 2024 results via conference call on Tuesday, November 12 at 8:15 am ET. To access the live conference call, participants may register here. The live audio webcast of the call will be available under “Events and Presentations” in the Investor Relations section of the Scholar Rock website at http://investors.scholarrock.com. To participate via telephone, please register in advance here. Upon registration, all telephone participants will receive a confirmation email detailing how to join the conference call, including the dial-in number along with a unique passcode and registrant ID that can be used to access the call. An archived replay of the webcast will be available on the Company’s website for approximately 90 days.

About Scholar Rock

Scholar Rock is a biopharmaceutical company that discovers, develops, and delivers life-changing therapies for people with serious diseases that have high unmet need. As a global leader in the biology of the transforming growth factor beta (TGFβ) superfamily of cell proteins and named for the visual resemblance of a scholar rock to protein structures, the clinical-stage company is focused on advancing innovative treatments where protein growth factors are fundamental. Over the past decade, Scholar Rock has created a pipeline with the potential to advance the standard of care for neuromuscular disease, cardiometabolic disorders, cancer, and other conditions where growth factor-targeted drugs can play a transformational role.

This commitment to unlocking fundamentally different therapeutic approaches is powered by broad application of a proprietary platform, which has developed novel monoclonal antibodies to modulate protein growth factors with extraordinary selectivity. By harnessing cutting-edge science in disease spaces that are historically under-addressed through traditional therapies, Scholar Rock works every day to create new possibilities for patients.

The efficacy and safety of apitegromab, SRK-181, and SRK-439 have not been established and apitegromab, SRK-181, and SRK-439 have not been approved for any use by the FDA or any other regulatory agency.

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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 we post on our website, Twitter or LinkedIn could be deemed material information. As a result, we encourage investors, the media and others interested to review the information we post there regularly. 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, including SRK-439, and indication selection and development timing, including the timing of any regulatory submissions, the therapeutic potential, clinical benefits and safety of any product candidates, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, its cash runway, expectations relating to commercial launch in the US in the fourth quarter of 2025, 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 3 SAPPHIRE trial will be sufficient to support regulatory approval, that the full results from the Phase 3 SAPPHIRE trial may differ from the topline data, that preclinical and clinical data, including the results from the Phase 2 or Phase 3 clinical trial of apitegromab, or Part A or Part B 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 or ongoing clinical trials of the same product candidates; the data generated from Scholar Rock’s nonclinical and preclinical studies and clinical trials; 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; and 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 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, 2024, 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,	
	2024	2023	2024	2023
Operating expenses				
Research and development	$ 48,719	$ 30,337	$ 134,185	$ 86,939
General and administrative	16,061	13,335	48,512	36,324
Total operating expenses	64,780	43,672	182,697	123,263
Loss from operations	(64,780)	(43,672)	(182,697)	(123,263)
Other income (expense), net	301	1,313	2,857	3,600
Net loss	$ (64,479)	$ (42,359)	$ (179,840)	$ (119,663)
Net loss per share, basic and diluted	$ (0,66)	$ (0,53)	$ (1,86)	$ (1,49)
Weighted average common shares outstanding, basic and diluted	97,050,637	80,606,438	96,587,149	80,115,143

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

	September 30, 2024	December 31, 2023
Assets		
Cash, cash equivalents and marketable securities	$ 139,065	$ 279,938
Other current assets	13,862	8,256
Total current assets	152,927	288,194
Other assets	26,206	22,841
Total assets	$ 179,133	$ 311,035
Liabilities and Stockholders' Equity		
Current liabilities	$ 39,372	$ 32,741
Long-term liabilities	60,565	53,076
Total liabilities	99,937	85,817
Total stockholders' equity	79,196	225,218
Total liabilities and stockholders' equity	$ 179,133	$ 311,035

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