Scholar Rock Reports Third Quarter 2026 Financial Results and Recent Business Highlights

November 10, 2026

- *U.S. launch of ISEMBYLD™ (apitegromab-mstn), the first and only muscle-targeted therapy for children and adults living with spinal muscular atrophy (SMA), underway following September 11, 2026 FDA approval*
- *ISEMBYLD shipped to specialty network prior to quarter-end, reflecting Q3 2026 ISEMBYLD revenue of $0.6 million*
- *Strong early patient and physician demand for ISEMBYLD, with initial patients infused in October 2026*
- *Apitegromab Marketing Authorisation Application (MAA) for treatment of children and adults living with SMA resubmitted to European Medicines Agency (EMA) and validated in October 2026; on track for Committee for Medicinal Products for Human Use (CHMP) opinion in H1 2027*
- *Apitegromab Japanese New Drug Application (JNDA) for treatment of children and adults living with SMA submitted to Japan's Pharmaceuticals and Medical Devices Agency (PMDA) and accepted under priority review*
- *Cash, cash equivalents, and marketable securities of $413.6 million as of September 30, 2026*
- *In November, reached definitive agreement to sell Priority Review Voucher (PRV) for $205 million; cash to be received upon closing*
- *Management to host a conference call today at 8:00 a.m. ET*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 10, 2026-- Scholar Rock (NASDAQ: SRRK), a global biopharmaceutical company dedicated to improving the lives of patients with rare, severe, and debilitating neuromuscular diseases by applying its leading platform in myostatin biology, today reported financial results for the third quarter ended September 30, 2026, and provided an update on recent company developments.

"The third quarter was a defining period for Scholar Rock, marked by the FDA approval and U.S. launch of ISEMBYLD, the world's first and only muscle-targeted therapy for children and adults living with SMA," said David L. Hallal, Chairman and Chief Executive Officer of Scholar Rock. "We are very encouraged by the early ISEMBYLD launch dynamics in the U.S., where the breadth of early demand across SMA treatment centers, physicians, and patients reinforces the significant unmet need in the SMA community. With the U.S. launch underway, our regulatory applications advancing in Europe and Japan, important progress across our world-leading anti-myostatin pipeline, and our strengthened financial position, we are building a foundation to redefine the treatment of neuromuscular disease globally and drive sustainable growth for years to come."

Business Highlights and Upcoming Milestones

Apitegromab Spinal Muscular Atrophy (SMA) Program

- **ISEMBYLD (apitegromab-mstn) approved by FDA on September 11, 2026.** ISEMBYLD is the first and only muscle-targeted therapy for SMA and is indicated for adults and children two years of age and older who are currently receiving a survival motor neuron 2 (SMN2)-targeted treatment. In the Phase 3 pivotal, randomized, double-blind, placebo-controlled SAPPHIRE study, individuals receiving ISEMBYLD with an SMN2-targeted treatment demonstrated a robust, clinically meaningful improvement in motor function at one year while those on an SMN2-targeted therapy alone experienced a loss of motor function.
- **U.S. launch of ISEMBYLD underway with strong early patient and physician demand**. The U.S. team is in the field engaging with physicians, SMA care teams, and patients as well as national and regional payers. ISEMBYLD vials were labeled, packaged, and shipped to the specialty network within one week of FDA approval. A J-code application was submitted to the Centers for Medicare & Medicaid Services (CMS) in September for an ISEMBYLD-specific J-code on April 1, 2027. Encouraging early, broad-based demand for ISEMBYLD is reflected in the breadth of prescribing physicians and enrolling patients. Early launch dynamics are reinforcing the significant opportunity to serve children and adults with SMA in the U.S.
- **Apitegromab application in Europe on track for H1 2027 Committee for Medicinal Products for Human Use (CHMP) opinion**. In alignment with guidance from the European Medicines Agency (EMA), Scholar Rock resubmitted the apitegromab Marketing Authorisation Application (MAA) for the treatment of children and adults living with SMA in October 2026. Following validation of the submission, review of the MAA is underway, and the Company expects a CHMP opinion in the first half of 2027. The resubmitted MAA includes Scholar Rock's alternate fill-finish facility in place of the Catalent Indiana LLC fill-finish facility. The alternate fill-finish facility is the same site that supported the U.S. FDA approval of ISEMBYLD in September 2026. As the regulatory review progresses, the Scholar Rock team in Europe continues to engage with key stakeholders on SMA disease awareness and education initiatives.
- **Apitegromab Japanese New Drug Application (JNDA) submitted in Japan and accepted under priority review**. Scholar Rock submitted the apitegromab JNDA in Japan, which was accepted under priority review. For priority review applications, Japan's Pharmaceuticals and Medical Devices Agency (PMDA) has typically targeted a review cycle of approximately nine months. In addition, apitegromab for the treatment of people living with SMA was granted an Orphan

Drug Designation.

- **Regulatory engagement for subcutaneous apitegromab program to progress**. Scholar Rock has developed a high concentration subcutaneous formulation of apitegromab. Following the September 11, 2026 FDA approval of ISEMBYLD, the Company is engaging with regulators on the development path for this formulation. Subcutaneous apitegromab is designed to provide optionality for the SMA community as a small volume, self- or caregiver-administered anti-myostatin antibody.
- **Phase 2 OPAL trial advancing**. Enrollment is well advanced and dosing is ongoing (NCT07047144). The OPAL study is evaluating apitegromab in infants and toddlers with SMA under two years of age who have received an approved SMN1-targeted gene therapy or who are receiving ongoing treatment with an approved SMN2-targeted therapy.

Apitegromab Facioscapulohumeral Muscular Dystrophy (FSHD) Program

- **Enrollment and dosing in Phase 2 FORGE trial ongoing**. The Phase 2 randomized, double-blind, placebo-controlled trial, called FORGE, is expected to enroll approximately 60 patients with FSHD who will be randomized 1:1 to receive either apitegromab 10mg/kg IV or placebo every 4 weeks for 52 weeks. The primary endpoint is mean lean muscle volume (LMV) change from baseline at 12 months. Other endpoints include safety, pharmacokinetics/ pharmacodynamics, mean LMV change from baseline at 6 months, as well as several exploratory functional endpoints. In September 2026, the FDA granted Fast Track and Orphan Drug designations to apitegromab for the treatment of people living with FSHD.

SRK-439 Program

- **Phase 1 study in healthy volunteers ongoing**. A Phase 1 study evaluating SRK-439 in healthy volunteers is progressing well. Scholar Rock expects to have topline data by year end 2026 and plans to share data from this study in early 2027. SRK-439 is a novel, investigational, subcutaneously administered myostatin inhibitor that binds to pro- and latent myostatin with high affinity and selectivity. Based on non-clinical data, SRK-439 has the potential to potently inhibit myostatin and increase muscle mass.

Financial Update

- **Definitive agreement to sell Rare Pediatric Disease Priority Review Voucher (PRV) for $205 million**. In November, Scholar Rock entered into a definitive agreement to sell its PRV for $205 million. The closing of the PRV transaction is subject to customary closing conditions, including the expiration of the applicable waiting period under the Hart-Scott-Rodino (HSR) Antitrust Improvements Act. The cash will further strengthen the Company's balance sheet with non-dilutive capital. The PRV was awarded following the September 2026 FDA approval of ISEMBYLD.

Third Quarter 2026 Financial Results

Scholar Rock reported a net loss of $107.2 million, including stock-based compensation of $17.9 million, for the quarter ended September 30, 2026, compared to a net loss of $102.2 million, including stock-based compensation of $18.3 million, for the quarter ended September 30, 2025. Net loss per common share was $0.81 for the quarter ended September 30, 2026, compared to $0.90 per common share for the quarter ended September 30, 2025.

- The Company recorded net product revenue of $0.6 million from sales of ISEMBYLD in the U.S. for the quarter ended September 30, 2026. The Company did not record any revenue for the quarter ended September 30, 2025.
- Cost of revenue was immaterial for the quarter ended September 30, 2026. Prior to the FDA approval of ISEMBYLD, product costs were expensed as research and development expense. The Company did not record any cost of revenue for the quarter ended September 30, 2025.
- Research and development expense was $54.1 million, including $6.4 million in stock-based compensation, for the quarter ended September 30, 2026, compared to $50.5 million, including $5.5 million in stock-based compensation, for the quarter ended September 30, 2025.
- Selling, general and administrative expense was $52.5 million, including $11.5 million in stock-based compensation, for the quarter ended September 30, 2026, compared to $53.1 million, including $12.8 million in stock-based compensation, for the quarter ended September 30, 2025.
- As of September 30, 2026, Scholar Rock had cash, cash equivalents, and marketable securities of $413.6 million.

Conference Call Information

Scholar Rock will host a conference call and webcast today, Tuesday, November 10, at 8:00 a.m. ET to review its third quarter 2026 financial results and discuss recent business updates. To access the live audio webcast, please go to "Events and Presentations" in the Investors 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.

A replay of the webcast will be available on the Company's website for approximately 90 days.

About Scholar Rock

Scholar Rock is delivering muscle-targeted breakthroughs to transform the treatment of spinal muscular atrophy (SMA) and other

rare neuromuscular diseases where muscle atrophy remains a critical unmet need. Following FDA approval in September 2026, Scholar Rock is commercializing ISEMBLYD (apitegromab-mstn) in the U.S. for people living with SMA who are 2 years of age and older and currently receiving a survival motor neuron 2 (SMN2)-targeted treatment. The Company plans to expand access to ISEMBYLD globally, in up to 50 countries worldwide, subject to regulatory approvals.

As a global leader in myostatin biology, a field focused on proteins that regulate muscle mass, the biopharmaceutical company is named for the visual resemblance of a scholar rock to protein structures. Our commitment to unlock fundamentally different treatment approaches is powered by broad application of a proprietary platform, which has developed novel monoclonal antibodies to modulate protein growth factors with extraordinary selectivity. Scholar Rock works every day to create new possibilities for patients through its highly innovative anti-myostatin program, including opportunities in additional rare neuromuscular diseases. Learn more about our neuromuscular franchise at **ScholarRock.com** and follow @ScholarRock on X and on LinkedIn.

Scholar Rock® is a registered trademark and ISEMBYLD ™is a trademark of Scholar Rock, Inc.

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 X (formerly known as Twitter) and LinkedIn. The information that we post on our website, X, 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.

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 the U.S. commercial launch and commercial performance of ISEMBYLD, including patient and physician demand, launch dynamics, patient enrollment trends, market acceptance, coverage and reimbursement, and the timing of an ISEMBYLD-specific J-code; expectations regarding its growth, strategy, progress and timing of its clinical trials and development programs for apitegromab, including its subcutaneous formulation, the potential characteristics and benefits of such formulation, and its engagement with regulators regarding the development path for such formulation, as well as the ongoing Phase 2 OPAL and FORGE trials, and for SRK-439, including the expected timing of topline data from its Phase 1 clinical trial and the timing of public disclosure of such data, the timing of any regulatory submissions, decisions and anticipated approvals, and the therapeutic potential, clinical benefits and safety of any product candidates; expectations regarding the timing and outcome of EMA review of the resubmitted MAA for apitegromab, including the expected timing of a CHMP opinion in the first half of 2027; expectations regarding the timing and outcome of PMDA review of the JNDA for apitegromab under priority review; expectations regarding the availability and timing of commercial supply of ISEMBYLD and apitegromab, including from Scholar Rock's alternate fill-finish facility; expectations regarding the closing of the sale of Scholar Rock's PRV, including the satisfaction of closing conditions, including the expiration of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act, and the anticipated receipt and use of proceeds therefrom; plans to expand access to ISEMBYLD globally, including in up to 50 countries worldwide, and expectations regarding commercial launch timing in additional jurisdictions, and the achievement of important milestones, the ability of any product candidate to perform in humans in a manner consistent with earlier nonclinical or clinical 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, whether nonclinical and clinical data, including the results from the Phase 3 SAPPHIRE trial and any results from ongoing or future clinical trials, including the Phase 2 OPAL clinical trial, the Phase 2 FORGE trial and the Phase 1 clinical trial of SRK-439, will be sufficient to support regulatory approval or further development; that nonclinical and clinical data, including the results from the Phase 2 or Phase 3 clinical trial of apitegromab, data from any ongoing or future trials of apitegromab or data for SRK-439, 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; whether the resubmitted MAA and the JNDA will be sufficient to support regulatory approval of apitegromab in the European Union and Japan, respectively, on the expected timelines or at all; Scholar Rock's ability to successfully commercialize ISEMBYLD, including achieving market acceptance among patients, physicians and payors and obtaining adequate coverage and reimbursement; whether early patient and physician demand for ISEMBYLD translates into sustained patient treatment and product revenue; Scholar Rock's ability to obtain regulatory approval for, and to successfully expand access to, ISEMBYLD in additional countries; Scholar Rock's ability to maintain sufficient commercial supply of ISEMBYLD, including through its alternate fill-finish facility; whether the conditions to the closing of the PRV sale will be satisfied on the expected timeline or at all; Scholar Rock's need for additional capital and its ability to obtain it on acceptable terms; Scholar Rock's ability to manage expenses or provide the financial support, resources and expertise necessary to identify and develop product candidates on the expected timeline; information provided or decisions made by regulatory authorities, including regarding the development path for subcutaneous apitegromab; competition from third parties that are developing products for similar uses; Scholar Rock's ability to obtain, maintain and protect its intellectual property; and Scholar Rock's dependence on third parties for development and manufacture of apitegromab, ISEMBYLD and other product candidates

including, without limitation, to supply commercial demand for ISEMBYLD and any clinical trials 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, 2026, 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,	
	2026	**2025**	**2026**	**2025**
Revenue:				
Product revenue, net	$ 570	$ —	$ 570	$ —
Operating expenses:				
Cost of revenue	8	—	8	-
Research and development	54,128	50,486	164,176	161,565
Selling, general and administrative	52,495	53,064	153,364	131,184
Total operating expenses	106,631	103,550	317,548	292,749
Loss from operations	(106,061)	(103,550)	(316,978)	(292,749)
Other income (expense), net	(1,178)	1,330	(5,667)	5,775
Net loss	$ (107,239)	$ (102,220)	$ (322,645)	$ (286,974)
Net loss per share, basic and diluted	$ (0.81)	$ (0.90)	$ (2.48)	$ (2.54)
Weighted average common shares outstanding, basic and diluted	132,372,289	113,729,850	130,106,052	112,763,974

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

	September 30, 2026	**December 31, 2025**
Assets		
Cash, cash equivalents and marketable securities	$ 413,599	$ 367,563
Other current assets	19,830	17,584
Total current assets	433,429	385,147
Other assets	12,543	19,125
Total assets	$ 445,972	$ 404,272
Liabilities and Stockholders' Equity		
Current liabilities	$ 80,124	$ 55,419
Long-term liabilities	197,172	103,365
Total liabilities	277,296	158,784
Total stockholders' equity	168,676	245,488
Total liabilities and stockholders' equity	$ 445,972	$ 404,272

View source version on businesswire.com: https://www.businesswire.com/news/home/20261110252867/en/

Investors
Laura Ekas, Ph.D.
917-439-0374
ir@scholarrock.com

Media
Jeff Smith
682-401-8428

Molly MacLeod, Ph.D.
802-579-5995
media@scholarrock.com

Source: Scholar Rock