REGENXBIO Reports Third Quarter 2026 Financial Results and Operational Highlights

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- *RGX-202 BLA submission for Duchenne muscular dystrophy on track for Q1 2027, with potential accelerated approval in 2H 2027*
- *Topline data from pivotal subretinal wet AMD studies, ATMOSPHERE® and ASCENT®, expected in Q4 2026*
- *Phase IIb/III NAAVIGATE trial for diabetic retinopathy ongoing*
- *Webcast today at 8:00 a.m. ET*

ROCKVILLE, Md., Nov. 5, 2026 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today reported financial results and operational highlights for the third quarter ended September 30, 2026.

"We are continuing to build momentum as we prepare to launch potential first- or best-in-class gene therapies in 2027 and 2028," said Curran Simpson, President and Chief Executive Officer of REGENXBIO. "With topline pivotal data expected this quarter, sura-vec has the potential to transform the treatment paradigm for a broad spectrum of patients with wet AMD. We also remain on track for the RGX-202 BLA submission early next year as we work toward delivering this important therapy to Duchenne patients as quickly as possible. We remain focused on executing these near-term milestones and leveraging our fully integrated capabilities to deliver meaningful new treatment options for patients."

PROGRAM HIGHLIGHTS AND MILESTONES

Neuromuscular Disease: RGX-202 is a potential best-in-class gene therapy for Duchenne muscular dystrophy (Duchenne). RGX-202 is designed to address the underlying cause of Duchenne by enabling targeted expression of a novel microdystrophin that is closest to naturally occurring dystrophin. The differentiated therapeutic approach behind RGX-202 includes a novel construct, with the C-Terminal domain, a proactive immune suppression regimen, and a suspension-based manufacturing process that delivers industry-leading product purity levels. RGX-202 is designed for improved muscle function, durability and positive safety outcomes and is being evaluated in the Phase I/II/III AFFINITY DUCHENNE® trial and confirmatory trial in ambulatory patients aged 1+.

- The Company expects to have a pre-Biologics License Application (BLA) meeting with the FDA in Q4 2026 and is on track to submit a BLA in Q1 2027 under the accelerated approval pathway, supporting potential approval in 2H 2027.
- REGENXBIO continues to manufacture intended commercial supply of RGX-202 at its in-house Manufacturing Innovation Center.
- REGENXBIO expects to initiate AFFINITY® RISE, an ex-U.S. randomized, placebo-controlled study to support RGX-202 global regulatory submissions, in 1H 2027.

Retinal Disease: Surabgene lomparvovec (sura-vec, ABBV-RGX-314), developed in collaboration with AbbVie, is potentially the first-in-class gene therapy treatment for wet age-related macular degeneration (wet AMD) and diabetic retinopathy (DR).

Sura-vec for the Treatment of Wet AMD (Subretinal Delivery)

- REGENXBIO expects to announce topline data with AbbVie from the ATMOSPHERE® and ASCENT® pivotal trials of sura-vec using subretinal delivery in Q4 2026.
- Global regulatory submissions are expected in 2027.

Sura-vec for the Treatment of DR (Suprachoroidal Delivery)

- Long-term follow-up data from the ALTITUDE® trial, presented at the Retina Society 59th Annual Scientific Meeting in September 2026, showed a durable safety and efficacy profile through 3 years at the dose being evaluated in the Phase IIb/III NAAVIGATE study with short-course prophylactic topical steroids.

- NAAVIGATE, a multicenter, randomized, masked, sham-controlled study enrolling subjects with non-proliferative DR (NPDR) without center-involved diabetic macular edema (CI-DME) to evaluate the safety and efficacy of a one-time, in-office administration of sura-vec, is enrolling.

Neurodegenerative Disease: RGX-121 (clemidsogene lanparvovec) is a potential first-in-class treatment for MPS II, also known as Hunter syndrome, being developed and potentially commercialized in partnership with Nippon Shinyaku.

- In August 2026, REGENXBIO announced the FDA placed a clinical hold on RGX-121 following the discovery of asymptomatic spine MRI findings in five participants in the CAMPSIITE® study. REGENXBIO continues to collect and evaluate additional patient imaging and long-term follow-up data.

FINANCIAL RESULTS
Cash Position: Cash, cash equivalents and marketable securities were $251.0 million as of September 30, 2026, compared to $240.9 million as of December 31, 2025. The increase was primarily attributable to a $100.0 million milestone payment received from AbbVie in July 2026 for the dosing of the first patient in the Phase IIb/III NAAVIGATE study and net proceeds of $107.8 million from an underwritten public offering of common stock and pre-funded warrants completed in July 2026, and was partially offset by cash used to fund operating activities during the nine months ended September 30, 2026. Cash, cash equivalents and marketable securities as of September 30, 2026 excludes a $39.0 million payment received in October 2026 pursuant to a settlement agreement with Sarepta Therapeutics.

Revenues: Revenues were $9.2 million for the three months ended September 30, 2026, compared to $29.7 million for the three months ended September 30, 2025. The decrease was primarily attributable to ZOLGENSMA® royalty revenues, which decreased by $21.2 million due primarily to the expiration of licensed patents in the U.S. in January 2026. The decrease was partially offset by ITVISMA® royalty revenues of $2.8 million for the third quarter of 2026. Novartis launched U.S. sales of ITVISMA in the first quarter of 2026. ITVISMA is now also approved in the EU, Japan and multiple Gulf countries. REGENXBIO is entitled to royalties on certain net sales of ITVISMA in the U.S. and other jurisdictions with patents extending until 2037.

Research and Development (R&D) Expenses: R&D expenses were $61.0 million for the three months ended September 30, 2026, compared to $56.1 million for the three months ended September 30, 2025. The increase was primarily attributable to clinical trial expenses for RGX-202.

General and Administrative (G&A) Expenses: G&A expenses were $20.6 million for the three months ended September 30, 2026, consistent with $20.3 million for the three months ended September 30, 2025.

Gain on Litigation Settlement: REGENXBIO recorded a gain of $38.0 million in the third quarter of 2026 related to a settlement agreement with Sarepta Therapeutics to resolve all patent litigation matters between the parties. The gain consisted of a $39.0 million settlement payment due from Sarepta, net of proceeds payable to certain licensors as a result of the settlement. The $39.0 million settlement payment was received from Sarepta in October 2026.

Net Loss: Net loss was $40.7 million, or $0.62 basic and diluted net loss per share, for the three months ended September 30, 2026, compared to a net loss of $61.9 million, or $1.20 basic and diluted net loss per share, for the three months ended September 30, 2025.

FINANCIAL GUIDANCE
REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $251.0 million as of September 30, 2026, along with the $39.0 million settlement proceeds received in October 2026, are sufficient to fund operations into late Q4 2027. This cash runway guidance is based on the Company's current operational plans and excludes the impact of any material payments that may potentially be received from partners or licensees upon the achievement of development or regulatory milestones, or upon the approval or commercialization of product candidates, and excludes any additional potential dilutive or non-dilutive funding opportunities.

CONFERENCE CALL
In connection with this announcement, REGENXBIO will host a conference call and webcast at 8:00 a.m. ET today. Listeners can register for the webcast via this link. Analysts wishing to participate in the question and answer session should use this link. A replay of the webcast will be available via the company's investor website approximately two hours after the call's conclusion. Those who plan on participating are advised to join 15 minutes prior to the start time.

ABOUT REGENXBIO Inc.
REGENXBIO is a biotechnology company on a mission to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the field of AAV gene therapy. REGENXBIO is advancing a late-stage pipeline of one-time treatments for rare and retinal diseases, including RGX-202 for the treatment of Duchenne; surabgene lomparvovec (ABBV-RGX-314) for the treatment of wet AMD and diabetic retinopathy, in collaboration with AbbVie, and RGX-121 (clemidsogene lanparvovec) for the treatment of MPS II and RGX-111 for the treatment of MPS I, both in partnership with Nippon Shinyaku. Thousands of patients have been treated with REGENXBIO's AAV platform, including those receiving Novartis' ZOLGENSMA® and ITVISMA®. REGENXBIO's investigational gene therapies have the potential to change the way healthcare is delivered for millions of people. For more information, please visit www.REGENXBIO.com.

FORWARD-LOOKING STATEMENTS
This press release includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "assume," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations, clinical trials, costs and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timing or likelihood of payments from AbbVie or Nippon Shinyaku, the

monetization of any priority review voucher, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2025, filed with the U.S. Securities and Exchange Commission (SEC) and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the SEC and are available on the SEC's website at **WWW.SEC.GOV**. All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. Except as required by law, REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

ZOLGENSMA® and ITVISMA® are registered trademarks of Novartis. All other trademarks referenced herein are registered trademarks of REGENXBIO.

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REGENXBIO INC.
CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands)

	September 30, 2026	December 31, 2025
Assets		
Current assets		
Cash and cash equivalents	$ 95,795	$ 34,466
Marketable securities	155,181	195,604
Accounts receivable	8,536	26,379
Prepaid expenses	13,009	11,927
Restricted cash	225	—
Other current assets	48,146	12,905
Total current assets	320,892	281,281
Marketable securities	—	10,785
Accounts receivable	450	2,312
Property and equipment, net	95,347	104,855
Operating lease right-of-use assets	42,246	47,156
Restricted cash	1,805	2,030
Other assets	5,300	4,613
Total assets	$ 466,040	$ 453,032
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 13,482	$ 21,358
Accrued expenses and other current liabilities	29,611	38,390
Deferred revenue	5,985	10,452
Operating lease liabilities	6,492	8,286
Royalty monetization liabilities	12,114	39,609
Total current liabilities	67,684	118,095
Deferred revenue	16,221	18,943
Operating lease liabilities	60,054	65,215
Royalty monetization liabilities	173,564	147,408
Other liabilities	475	638
Total liabilities	317,998	350,299
Stockholders' equity		
Preferred stock; no shares issued and outstanding at September 30, 2026 and December 31, 2025	—	—
Common stock; 66,119 and 50,892 shares issued and outstanding at September 30, 2026 and December 31, 2025, respectively	7	5
Additional paid-in capital	1,383,187	1,229,442
Accumulated other comprehensive loss	(1,045)	(687)
Accumulated deficit	(1,234,107)	(1,126,027)
Total stockholders' equity	148,042	102,733
Total liabilities and stockholders' equity	$ 466,040	$ 453,032

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited)
(in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenues				
License and royalty revenue	$ 5,244	$ 23,605	$ 114,175	$ 129,119
Service revenue	3,918	6,128	9,399	10,985
Total revenues	9,162	29,733	123,574	140,104
Operating Expenses (Income)				
Cost of license and royalty revenues	1,141	5,725	13,257	14,370
Research and development	61,009	56,101	174,434	168,688
General and administrative	20,579	20,253	63,501	60,483
Gain on litigation settlement	(38,026)	—	(38,026)	—
Other operating expenses (income)	(7)	65	41	125
Total operating expenses	44,696	82,144	213,207	243,666
Loss from operations	(35,534)	(52,411)	(89,633)	(103,562)
Other Income (Expense)				
Interest income from licensing	16	19	49	65
Investment income	2,252	3,620	5,477	9,500
Interest expense	(7,472)	(13,169)	(23,973)	(32,732)
Total other income (expense)	(5,204)	(9,530)	(18,447)	(23,167)
Net loss	$ (40,738)	$ (61,941)	$ (108,080)	$ (126,729)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	(170)	98	(358)	89
Total other comprehensive income (loss)	(170)	98	(358)	89
Comprehensive loss	$ (40,908)	$ (61,843)	$ (108,438)	$ (126,640)
Net loss per share, basic and diluted	$ (0.62)	$ (1.20)	$ (1.90)	$ (2.46)
Weighted-average common shares outstanding, basic and diluted	65,263	51,689	56,884	51,513

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