REGENXBIO Reports Second Quarter 2025 Financial Results and Operational Highlights

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- *RGX-202 in Duchenne muscular dystrophy on track for topline results 1H 2026 and BLA submission mid-2026*
  - *Pivotal trial enrollment accelerated, expected to complete in October 2025 ahead of previous guidance*
- *Clemidsogene lanparvovec (RGX-121) on track to be first gene therapy and one-time treatment for MPS II; FDA inspections completed successfully*
  - *Pre-license inspection (PLI) of in-house manufacturing facility, quality systems and processes completed with no observations*
  - *Bioresearch monitoring information (BIMO) inspection of laboratory and clinical data practices completed with no observations*
- *Surabgene lomparvovec (sura-vec, ABBV-RGX-314) on track to be first gene therapy in chronic retinal disease*
  - *Pivotal data evaluating the safety and efficacy of the subretinal delivery of sura-vec in patients with wet AMD are expected in 2026*
  - *Sura-vec using suprachoroidal delivery for diabetic retinopathy advancing to global pivotal program, supported by positive Phase II trial data*
- *Conference call today at 8:00 a.m. ET*

ROCKVILLE, Md., Aug. 7, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today reported financial results and operational highlights for the second quarter ended June 30, 2025.

"Our REGENXBIO team is accelerating multiple first or best-in-class gene therapies designed to improve the lives of patients and families facing serious diseases like Duchenne, Hunter syndrome, and retinal diseases. We continued our strong momentum in the second quarter of 2025, working with urgency to advance multiple pivotal programs, starting with the potential FDA approval of clemidsogene lanparvovec (RGX-121) this November," said Curran M. Simpson, President and Chief Executive Officer of REGENXBIO. "RGX-202 is progressing rapidly through pivotal study, and today's announcement of our progress in diabetic retinopathy enables another late-stage program with the goal of preventing vision loss in chronic retinal disease that impacts millions of patients."

PROGRAM HIGHLIGHTS AND MILESTONES

Neuromuscular Disease: RGX-202 is a potential best-in-class gene therapy for Duchenne muscular dystrophy (Duchenne). The RGX-202 program uses a novel, differentiated therapeutic approach designed for improved muscle function and safety outcomes for patients.

- The AFFINITY DUCHENNE® pivotal trial of RGX-202 is ongoing in ambulatory patients, and REGENXBIO now expects to complete enrollment of approximately 30 patients aged 1+ in the U.S. and Canada by October 2025.
- Following enrollment completion in the pivotal trial, REGENXBIO expects to continue enrollment to support a planned confirmatory trial. Manufacturing of clinical and confirmatory trial supply of RGX-202 is complete, enabling immediate and broad access to patients seeking next-generation, investigational gene therapy.
- Positive Phase I/II data reported in June 2025 continue to demonstrate the potential of RGX-202 to serve as a best-in-class gene therapy for Duchenne. As of May 7, 2025:
  - Favorable safety profile with no serious adverse events or adverse events of special interest observed in the Phase I/II study; no patients developed signs of liver injury, as assessed by liver function test monitoring.

    - All dose level 2 participants exceeded external natural history controls on all functional measures.
    - Biomarker data continued to demonstrate consistent, robust microdystrophin expression and transduction levels across all treated ages, with all reported microdystrophin levels to date above the threshold of 10% compared to normal control.
- REGENXBIO expects to share topline data in the first half of 2026 and submit a Biologics License Application (BLA) under the accelerated approval pathway in mid-2026. REGENXBIO plans to initiate commercial supply manufacturing in Q3 2025 to support an expected launch in 2027, when vast majority of the prevalent market is expected to be available.

Neurodegenerative Disease: Clemidsogene lanparvovec (RGX-121) 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 May 2025, the U.S. Food and Drug Administration (FDA) granted priority review of the BLA seeking accelerated approval for clemidsogene lanparvovec for MPS II; Prescription Drug User Fee Act (PDUFA) target action date of November 9, 2025.
- The FDA BLA review is progressing as planned; mid-cycle meeting and PLI and BIMO inspections were successfully completed.
    - PLI of in-house REGENXBIO Manufacturing Innovation Center, quality systems and processes completed with no observations.
    - BIMO inspection of laboratory and clinical data practices completed with no observations.
- FDA approval could result in receipt of a Priority Review Voucher (PRV), to which REGENXBIO has full rights.

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

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

- REGENXBIO today announced new data from the Phase II ALTITUDE® trial and plans to initiate a pivotal program.
    - Data demonstrate durable safety and efficacy profile observed in patients with non-proliferative DR through two years with a single, in-office injection. As of June 9, 2025, sura-vec was well tolerated at dose levels 1, 2, and 3, with no drug-related serious adverse events. No intraocular inflammation was observed through two years at dose level 3 ($1.0x10^{12}$ GC/eye) (n=15) with short-course topical prophylactic steroids.
    - A two-part placebo-controlled Phase IIb/III trial will be initiated; the primary endpoint will be 2-step DRSS improvement. Site selection is in progress.
- The Phase II ALTITUDE® trial cohort evaluating a one-time, in-office injection of sura-vec at dose level 4 (1.5x10e12 GC/eye) in patients with center-involved diabetic macular edema (DME) is fully enrolled.

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

- Enrollment is ongoing in the ATMOSPHERE® and ASCENT™ pivotal trials. Topline results are expected in 2026. Sura-vec is on track to be the first approved gene therapy for wet AMD.

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

- The Phase II AAVIATE® trial continues enrolling a new cohort to evaluate sura-vec at dose level 4 (1.5x10e12 GC/eye). Patients in this cohort will also receive short-course prophylactic

steroid eye drops.

CORPORATE UPDATES

Royalty Monetization

- In May 2025, REGENXBIO announced the closure of a non-dilutive, limited recourse royalty bond agreement of up to $250 million with Healthcare Royalty (HCRx). Per the agreement, REGENXBIO received $150 million at closing and is eligible to receive an additional $100 million consisting of two separate $50 million tranches.

AbbVie Eye Care Collaboration

- In August 2025, AbbVie and REGENXBIO executed an amendment to the collaboration and license agreement established between the two companies on September 10, 2021. The amendment includes an updated milestone structure for the DR program, under the terms of which AbbVie will pay REGENXBIO $100 million upon first subject dosed in the Phase IIb/III trial and an additional $100 million upon first subject dosed in a second Phase III clinical trial. REGENXBIO will pay for all costs for Phase IIb of the Phase IIb/III trial.
- The amendment also reflects AbbVie's continued investment across the broader sura-vec program. AbbVie will independently advance and pay all costs for a new Phase III ACHIEVE trial in wet AMD. This randomized controlled trial will assess the potential reduction in injection burden and preservation of long-term vision of sura-vec compared to standard of care.

FINANCIAL RESULTS

Cash Position: Cash, cash equivalents and marketable securities were $363.6 million as of June 30, 2025, compared to $244.9 million as of December 31, 2024. The increase was primarily attributable to the $110.0 million upfront payment received under the Nippon Shinyaku partnership in March 2025 and $144.5 million in net proceeds received from the royalty monetization with HCRx in May 2025, and was partially offset by cash used to fund operating activities during the first half of 2025.

Revenues: Revenues were $21.4 million for the three months ended June 30, 2025, compared to $22.3 million for the three months ended June 30, 2024. The decrease was primarily attributable to Zolgensma royalties, which decreased from $21.8 million for the second quarter of 2024 to $18.4 million for the second quarter of 2025. The decrease was partially offset by an increase in service revenues, driven primarily by $2.7 million of development service revenue under the Nippon Shinyaku partnership in the second quarter of 2025.

Research and Development Expenses: Research and development expenses were $59.5 million for the three months ended June 30, 2025, compared to $48.9 million for the three months ended June 30, 2024. The increase was primarily attributable to manufacturing-related expenses and other clinical supply costs and clinical trial expenses for sura-vec and RGX-202 pivotal trials.

General and Administrative Expenses: General and administrative expenses were $19.9 million for the three months ended June 30, 2025, compared to $18.9 million for the three months ended June 30, 2024. The increase was primarily attributable to personnel-related costs and expenses for consulting and professional services.

Net Income: Net loss was $70.9 million, or $1.38 basic and diluted net loss per share, for the three months ended June 30, 2025, compared to a net loss of $53.0 million, or $1.05 basic and diluted net loss per share, for the three months ended June 30, 2024.

FINANCIAL GUIDANCE

REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $363.6 million as of June 30, 2025 to fund its operations into early 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 potential monetization of a PRV that would be received upon potential approval of clemidsogene lanparvovec.

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 access the live call by dialing (646) 307-1963 (domestic) or (800) 715-9871 (international) and enter the passcode 9571992. 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; clemidsogene lanparvovec (RGX-121) for the treatment of MPS II and RGX-111 for the treatment of MPS I, both in partnership with Nippon Shinyaku; and surabgene lomparvovec (ABBV-RGX-314) for the treatment of wet AMD and diabetic retinopathy, in collaboration with AbbVie. Thousands of patients have been treated with REGENXBIO's AAV platform, including those receiving Novartis' ZOLGENSMA®. 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, 2024, 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® is a registered trademark of Novartis Gene Therapies. All other trademarks referenced herein are registered trademarks of REGENXBIO.

CONTACTS:
Dana Cormack
Corporate Communications
Dcormack@regenxbio.com

George E. MacDougall
Investor Relations
IR@regenxbio.com

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

	June 30, 2025	December 31, 2024
Assets		
Current assets		
Cash and cash equivalents	$ 79,558	$ 57,526
Marketable securities	243,740	177,161
Accounts receivable	20,199	20,473
Prepaid expenses	10,776	9,067
Other current assets	19,479	13,774
Total current assets	373,752	278,001
Marketable securities	40,296	10,179
Accounts receivable	1,584	474
Property and equipment, net	111,017	117,589
Operating lease right-of-use assets	50,469	53,716
Restricted cash	2,030	2,030
Other assets	1,879	4,000
Total assets	$ 581,027	$ 465,989
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 21,655	$ 22,798
Accrued expenses and other current liabilities	35,557	38,070
Deferred revenue	13,977	115
Operating lease liabilities	8,049	7,902
Royalty monetization liabilities	40,302	34,309
Total current liabilities	119,540	103,194
Deferred revenue	23,804	—
Operating lease liabilities	69,647	74,131
Royalty monetization liabilities	153,693	25,378
Other liabilities	664	3,635
Total liabilities	367,348	206,338

Stockholders' equity		
Preferred stock; no shares issued and outstanding at June 30, 2025 and December 31, 2024	—	—
Common stock; 50,389 and 49,549 shares issued and outstanding at June 30, 2025 and December 31, 2024, respectively	5	5
Additional paid-in capital	1,211,361	1,192,536
Accumulated other comprehensive loss	(750)	(741)
Accumulated deficit	(996,937)	(932,149)
Total stockholders' equity	213,679	259,651
Total liabilities and stockholders' equity	$ 581,027	$ 465,989

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
Revenues				
License and royalty revenue	$ 18,465	$ 21,846	$ 105,514	$ 37,190
Service revenue	2,894	449	4,857	727
Total revenues	21,359	22,295	110,371	37,917
Operating Expenses				
Cost of license and royalty revenues	5,209	10,579	8,645	14,862
Research and development	59,500	48,869	112,587	103,713
General and administrative	19,883	18,855	40,230	37,146
Impairment of long-lived assets	—	—	—	2,101
Other operating expenses (income)	45	29	60	(5)
Total operating expenses	84,637	78,332	161,522	157,817
Loss from operations	(63,278)	(56,037)	(51,151)	(119,900)
Other Income (Expense)				
Interest income from licensing	21	29	46	66
Investment income	3,379	3,468	5,880	5,937
Interest expense	(10,993)	(449)	(19,563)	(2,422)
Total other income (expense)	(7,593)	3,048	(13,637)	3,581
Net loss	$ (70,871)	$ (52,989)	$ (64,788)	$ (116,319)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	12	963	(9)	2,163
Total other comprehensive income (loss)	12	963	(9)	2,163
Comprehensive loss	$ (70,859)	$ (52,026)	$ (64,797)	$ (114,156)
Net loss per share, basic and diluted	$ (1.38)	$ (1.05)	$ (1.26)	$ (2.41)
Weighted-average common shares outstanding, basic and diluted	51,483	50,601	51,423	48,167



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