REGENXBIO Reports Third Quarter 2022 Financial Results and Recent Operational Highlights

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- *Continued progress on 5x25 strategy to advance five AAV Therapeutics from our internal pipeline and licensed programs into pivotal-stage or commercial products by 2025*
- *RGX-314 program for the treatment of wet AMD and diabetic retinopathy, being developed in collaboration with AbbVie, remains on track for first BLA filing in 2024*
    - *Enrollment ongoing in the pivotal ATMOSPHERE® and ASCENT™ clinical trials of RGX-314 for the treatment of wet AMD using subretinal delivery*
    - *Presented positive interim data from RGX-314 trials for the treatment of wet AMD - the Phase II AAVIATE® trial using suprachoroidal delivery and the Phase I/IIa Long-term Follow-up study using subretinal delivery*
        - *Announced expansion of AAVIATE trial to include Cohort 6 at third dose level with short-course prophylactic ocular steroids following RGX-314 administration*
    - *Presented additional positive interim data from Phase II ALTITUDE® trial of RGX-314 for the treatment of diabetic retinopathy using suprachoroidal delivery*
        - *Announced dose escalation and expansion of ALTITUDE trial to include Cohorts 4 and 5 at new, third dose level with short-course prophylactic ocular steroids following RGX-314 administration*
- *AFFINITY DUCHENNE™ Phase I/II trial of RGX-202 remains on track for dosing in the first half of 2023*
- *CAMPSIITE™ pivotal program of RGX-121 is active and enrolling patients for the treatment of MPS II; remains on track to file a BLA in 2024 using the accelerated approval pathway*
- *$617 million in cash, cash equivalents and marketable securities of as of September 30, 2022; operational runway into 2025*
- *Conference call Thursday, November 3rd at 8:30 a.m. ET*

ROCKVILLE, Md., Nov. 3, 2022 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the third quarter ended September 30, 2022, and recent operational highlights.

"REGENXBIO has seen continued progress since our last earnings update, with encouraging data updates across four of our key gene therapy programs that make up our '5x'25' strategy," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We shared multiple updates for our lead program, RGX-314, that is being developed in collaboration with AbbVie, including new long-term follow-up data for RGX-314 subretinal delivery that supports our anticipated 2024 BLA filing. We also shared new Phase II data for RGX-314 suprachoroidal delivery in both wet AMD and diabetic retinopathy. We are expanding these trials in both indications as we continue developing this novel approach to delivering gene therapy with the potential to expand patient access to the in-office setting. Within our rare disease pipeline, we also announced new data for RGX-121, our gene therapy for the treatment of MPS II, and we continue enrolling in the pivotal CAMPSIITE trial that we expect to support a planned BLA filing in 2024 using the accelerated approval pathway. We remain well funded to execute upon our goals and look forward to keeping you updated on the advancement of our programs as we finish the year and look ahead to 2023."

Program Highlights and Milestones

RGX-314: RGX-314 is an investigational one-time AAV Therapeutic being developed in collaboration with AbbVie for the treatment of wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other additional chronic retinal conditions. RGX-314 uses the NAV® AAV8 vector to deliver a gene encoding a therapeutic antibody fragment to inhibit vascular endothelial growth factor (VEGF).

- RGX-314 Subretinal Delivery for the Treatment of Wet AMD
    - Enrollment is ongoing in ATMOSPHERE® and ASCENT™, two pivotal clinical trials to

evaluate the efficacy and safety of RGX-314 in patients with wet AMD using the subretinal delivery approach. The ASCENT trial is the first trial to be initiated by REGENXBIO under the eye care collaboration with AbbVie.
    - Pivotal trials are expected to support Biologics License Application (BLA) submission for RGX-314 in 2024.
    - In October 2022, REGENXBIO announced data from the Phase I/IIa Long-term Follow-up study of RGX-314 for the treatment of wet AMD using subretinal delivery at the American Academy of Ophthalmology (AAO) Annual Meeting.
        - As of August 29, 2022, RGX-314 continued to be generally well-tolerated in the long-term follow-up study (n=37).
        - Patients treated with RGX-314 continued to demonstrate a long-term, durable treatment effect in Cohort 3 up to four years and in Cohort 4 up to three years. Stable to improved visual acuity was observed, with a mean best corrected visual acuity (BCVA) of +12 letters from baseline at four years for Cohort 3 patients and -5 letters from baseline at three years for Cohort 4 patients following RGX-314 administration.
- RGX-314 Suprachoroidal Delivery for the Treatment of Wet AMD
    - In October 2022, REGENXBIO announced new data from the Phase II AAVIATE® trial of RGX-314 for the treatment of wet AMD using suprachoroidal delivery.
        - As of August 1, 2022, RGX-314 suprachoroidal delivery was reported to be well tolerated across 85 patients dosed in Cohorts 1-5. Mild intraocular inflammation was reported at similar incidence in the first and second dose levels, with an increase in incidence in mild to moderate inflammation seen at the third dose level (Cohort 4). All intraocular inflammation resolved with topical corticosteroids.
        - Patients treated in the RGX-314 arms and the ranibizumab control arm both continued to demonstrate stable BCVA and central retina thickness (CRT) at 6 months. In addition, a meaningful reduction in anti-VEGF treatment burden following administration of RGX-314 was observed and ranged from -64% to -85% across all cohorts. The highest reduction in treatment burden was observed in the third dose level, with patients receiving a mean of 1.3 injections over six months following administration of RGX-314, which represents an 85% reduction in anti-VEGF treatment burden.
        - Ten out of 15 patients (67%) in the third dose level received no anti-VEGF injections over six months following RGX-314 administration.
        - Interim data from the second dose level (Cohorts 2 and 3) suggested there was no meaningful difference in safety and vision outcomes for patients who are neutralizing antibody (NAb) positive.
        - The AAVIATE trial has expanded to include a new cohort at the third dose level with short-course prophylactic ocular steroids following RGX-314 administration to evaluate the ability to prevent or reduce the occurrence of the mild to moderate intraocular inflammation seen in previous cohorts. Patients will be enrolled in Cohort 6 regardless of NAb status.
- RGX-314 Suprachoroidal Delivery for the Treatment of DR
    - The Phase II ALTITUDE® trial has expanded to include a higher third dose level ($1x10^{12}$ GC/eye), with patients stratified by Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (DRSS) levels across cohorts and all receiving short-course prophylactic ocular steroids following RGX-314 administration.
    - As of October 17, 2022, RGX-314 was reported to be well tolerated across 50 patients dosed in Cohorts 1-3 at two dose levels (D1 and D2). Three patients had intraocular

inflammation, all of which were mild and resolved on topical corticosteroids.
    - No meaningful differences in safety outcomes were observed at six months for patients who are NAb positive.
    - BCVA remained stable in Cohorts 1-3 through six months.
    - Patients treated with RGX-314 in Cohorts 1-3 demonstrated clinically meaningful improvements in disease severity and less disease worsening versus observation control at six months as measured by DRSS. Specifically:
        - 20% (D1: 40%, D2: 11%) achieved ≥2-step DRSS improvement vs. 10% in control
        - 54% (D1: 60%, D2: 51%) achieved any DRSS improvement vs. 20% in control
        - 0% (D1: 0%, D2: 0%) worsened ≥2 steps vs. 20% in control

RGX-202: RGX-202 is an investigational one-time AAV Therapeutic for the treatment of Duchenne Muscular Dystrophy (Duchenne), using the NAV AAV8 vector to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal (CT) domain as well as a muscle specific promoter to support a targeted therapy for improved resistance to muscle damage associated with Duchenne.

- Preparation for the initiation of the AFFINITY DUCHENNE™ Phase I/II trial continues, including readying clinical trial sites and manufacturing additional clinical supply for the trial.
- REGENXBIO anticipates dosing the first patient in this trial in the first half of 2023.

RGX-121: RGX-121 is an investigational one-time AAV Therapeutic for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter Syndrome, using the NAV AAV9 vector to deliver the gene that encodes the iduronate-2-sulfatase (I2S) enzyme.

- CAMPSIITE™, a multicenter, open-label, Phase I/II/III trial for the treatment of patients up to 5 years old diagnosed with MPS II, is active and enrolling patients. The trial is expected to enroll up to 10 MPS II patients using commercial-scale cGMP material to support a BLA filing in 2024 using the accelerated approval pathway, with the potential to enroll additional patients.
- In August 2022, REGENXBIO announced positive interim data from the CAMPSIITE trial of RGX-121
    - As of August 1, 2022, RGX-121 was reported to be well-tolerated across all cohorts in 14 patients dosed with RGX-121.
    - Patients in all three cohorts demonstrated encouraging, dose-dependent reductions of cerebrospinal fluid (CSF) glycosaminoglycans (GAGs), key biomarkers of I2S enzyme activity, following one-time administration of RGX-121.
    - Improvements in neurodevelopmental function and caregiver reported outcomes in Cohorts 1 and 2 demonstrated CNS activity up to 2 years after RGX-121 administration.
    - GAGs in the CSF have the potential to be considered a surrogate biomarker that is reasonably likely to predict clinical benefit in MPS II disease under the accelerated approval pathway, as buildup of GAGs in the CSF of MPS II patients correlates with clinical manifestations, including neurodevelopmental deficits.
- The Phase I/II trial of RGX-121 for the treatment of pediatric patients with MPS II over the age of five years old is also ongoing.

RGX-111: RGX-111 is an investigational one-time AAV Therapeutic for the treatment of severe Mucopolysaccharidosis Type I (MPS I), using the NAV AAV9 vector to deliver the α-l-iduronidase (IDUA) gene.

- REGENXBIO continues with plans to enroll additional patients in a Cohort 2 expansion arm of the Phase I/II trial.

Operational Updates

- The REGENXBIO Manufacturing Innovation Center is fully operational, producing GMP bulk substance lots to support programs using the NAVXpress™ process platform.
    - State-of-the-art cGMP gene therapy manufacturing facility is designed to meet global clinical and commercial regulatory standards and enable the Company to efficiently

advance its AAV-based gene therapy pipeline from research and early development to clinical programs to commercial readiness.
- REGENXBIO is one of only a few gene therapy companies worldwide with a GMP facility capable of production at scales up to 2,000 liters.

NAV Technology Licensee Program Highlights

As of September 30, 2022, REGENXBIO's NAV Technology Platform was being applied in one marketed product and multiple clinical stage partnered programs, with the potential to impact a broad range of therapeutic areas and disease indications.

- Zolgensma®, a one-time AAV Therapeutic for the treatment of spinal muscular atrophy (SMA), is a marketed product utilizing REGENXBIO's NAV AAV9 vector. In October 2022, Novartis AG reported third quarter global sales of Zolgensma of $319 million (>2,500 patients treated worldwide).
- In September 2022, Rocket Pharmaceuticals, Inc. announced positive updates from the Phase 1 clinical trial for RP-A501 in Danon Disease. Pediatric efficacy data showed initial improvements across clinical, functional and biomarker endpoints with six to nine months of follow-up; positive results including protein expression obtained at three and six months were consistent with adult cohorts at similar timeframe. The clinical data presented to date is expected to support a Phase 2 pivotal study. RP-A501 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.
- In August 2022, uniQure N.V. announced the initiation of an IND-enabling GLP toxicology study in non-human primates to support an IND submission for AMT-260 in rTLE. AMT-260 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.
- In July 2022, Ultragenyx Pharmaceutical Inc. announced that dosing and enrollment of the Phase 3 study of DTX401 for Glycogen Storage Disease Type Ia (GSDIa) is ongoing, it expects to initiate the Phase 3 study of DTX301 for Ornithine Transcarbamylase (OTC) Deficiency in the second half of 2022, and it is dosing patients in the first stage of the Cyprus2+ study of UX701 for Wilson Disease. DTX401 and DTX301 are both being developed as one-time gene therapies utilizing REGENXBIO's NAV AAV8 vector and UX701 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.

Financial Results

Cash Position: Cash, cash equivalents and marketable securities were $617.0 million as of September 30, 2022, compared to $849.3 million as of December 31, 2021. The decrease was primarily driven by cash used to fund operating activities and capital expenditures and temporary unrealized losses on marketable debt securities during the nine months ended September 30, 2022.

Revenues: Revenues were $26.5 million for the three months ended September 30, 2022, compared to $30.8 million for the three months ended September 30, 2021. The decrease was primarily attributable to Zolgensma royalty revenues, which decreased from $30.3 million for the third quarter of 2021 to $25.2 million for the third quarter of 2022. Zolgensma royalty revenues for the nine months ended September 30, 2022 were $75.1 million for the nine months ended September 30, 2022, compared to $66.9 million for the nine months ended September 30, 2021.

Research and Development Expenses: Research and development expenses were $63.3 million for the three months ended September 30, 2022, compared to $47.9 million for the three months ended September 30, 2021. The increase was primarily attributable to personnel costs as a result of increased headcount, and costs associated with clinical trials and manufacturing-related activities for our lead product candidates.

General and Administrative Expenses: General and administrative expenses were $20.9 million for the three months ended September 30, 2022, compared to $21.0 million for the three months ended September 30, 2021.

Net Loss: Net loss was $75.5 million, or $1.75 basic and diluted net loss per share, for the three months ended September 30, 2022, compared to a net loss of $58.4 million, or $1.37 basic and diluted net loss per share, for the three months ended September 30, 2021.

Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $617.0 million as of September 30, 2022 to fund its operations into 2025.

Conference Call

In connection with this announcement, REGENXBIO will host a conference call and webcast today at 8:30 a.m. ET. 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 leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates, including late-stage and commercial programs, in multiple therapeutic areas. REGENXBIO is committed to a "5x'25" strategy to progress five AAV Therapeutics from our internal pipeline and licensed programs into pivotal-stage or commercial products by 2025.

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 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, the impact of the COVID-19 pandemic or similar public health crises on REGENXBIO's business, 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, 2021, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (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.

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

	September 30, 2022	December 31, 2021
Assets		
Current assets		
Cash and cash equivalents	$ 118,544	$ 345,209
Marketable securities	263,874	112,230
Accounts receivable, net	32,549	32,439
Prepaid expenses	14,824	18,752
Other current assets	6,848	10,196
Total current assets	436,639	518,826
Marketable securities	234,594	391,907
Accounts receivable, net	1,803	2,262
Property and equipment, net	140,906	131,547
Operating lease right-of-use assets	59,471	60,904
Restricted cash	2,030	2,030
Other assets	8,350	6,428
Total assets	$ 883,793	$ 1,113,904
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 25,210	$ 11,387
Accrued expenses and other current liabilities	44,462	76,111
Deferred revenue	5,903	3,333
Operating lease liabilities	3,608	1,752
Liability related to sale of future royalties	44,365	37,889
Total current liabilities	123,548	130,472
Operating lease liabilities	84,673	84,929
Liability related to sale of future royalties	103,084	133,460
Other liabilities	8,664	745
Total liabilities	319,969	349,606
Stockholders' equity		
Preferred stock; no shares issued and outstanding at September 30, 2022 and December 31, 2021	—	—

Common stock; 43,292 and 42,831 shares issued and outstanding at September 30, 2022 and December 31, 2021, respectively	4	4
Additional paid-in capital	963,694	928,095
Accumulated other comprehensive loss	(18,256)	(2,569)
Accumulated deficit	(381,618)	(161,232)
Total stockholders' equity	563,824	764,298
Total liabilities and stockholders' equity	$ 883,793	$ 1,113,904

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,	
	2022	2021	2022	2021
Revenues				
License and royalty revenue	$ 26,512	$ 30,773	$ 81,379	$ 71,692
Total revenues	26,512	30,773	81,379	71,692
Operating Expenses				
Cost of revenues	13,094	14,105	41,762	28,775
Research and development	63,313	47,855	179,948	133,459
General and administrative	20,921	21,030	64,071	57,293
Credit losses and other	229	5,131	703	5,781
Total operating expenses	97,557	88,121	286,484	225,308
Loss from operations	(71,045)	(57,348)	(205,105)	(153,616)
Other Income (Expense)				
Interest income from licensing	18	117	265	700
Investment income	1,497	5,535	3,357	6,514
Interest expense	(5,954)	(6,709)	(18,944)	(19,777)
Total other income (expense)	(4,439)	(1,057)	(15,322)	(12,563)
Loss before income taxes	(75,484)	(58,405)	(220,427)	(166,179)
Income Tax Benefit (Expense)	—	—	41	(4)
Net loss	$ (75,484)	$ (58,405)	$ (220,386)	$ (166,183)
Other Comprehensive Loss				
Unrealized loss on available-for-sale securities, net	(3,493)	(30)	(15,687)	(925)
Total other comprehensive loss	(3,493)	(30)	(15,687)	(925)
Comprehensive loss	$ (78,977)	$ (58,435)	$ (236,073)	$ (167,108)
Net loss per share, basic and diluted	$ (1.75)	$ (1.37)	$ (5.11)	$ (3.93)
Weighted-average common shares outstanding, basic and diluted	43,251	42,629	43,103	42,324

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