REGENXBIO Reports Fourth Quarter 2022 and Full-Year 2022 Financial Results and Recent Operational Highlights

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- *Continued progress on '5x'25' strategy to advance five AAV Therapeutics from REGENXBIO's 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*
    - *Enrollment ongoing in the pivotal ATMOSPHERE® and ASCENT™ clinical trials using subretinal delivery, expected to support BLA filing in 2024*
    - *Recently announced interim data from the Phase II pharmacodynamic study supports use of NAVXpress™ platform process in future commercialization plans*
    - *Recently announced cohort expansions in AAVIATE® and ALTITUDE® suprachoroidal clinical trials on track to be completed in the first half of 2023; expected interim trial updates in the second half of 2023*
- *AFFINITY DUCHENNE™ Phase I/II trial of RGX-202 is active and recruiting patients; anticipated to report initial trial data in the second half of 2023*
- *CAMPSIITE™ clinical trial of RGX-121 for the treatment of MPS II on track to complete pivotal program enrollment in the first half of 2023; recent updates continue to support plan to file BLA in 2024 using the accelerated approval pathway*
- *$565 million in cash, cash equivalents and marketable securities as of December 31, 2022; operational runway into 2025*
- *Conference call Tuesday, February 28th at 4:30 p.m. ET*

ROCKVILLE, Md., Feb. 28, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the fourth quarter and full-year ended December 31, 2022, and recent operational highlights.

"REGENXBIO enters the year with a strong pipeline of AAV Therapeutics. One year ago, we introduced our '5x'25' strategy and I remain confident that we will achieve this goal," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Our lead candidate, RGX-314, is being developed in partnership with AbbVie for the treatment of wet AMD and diabetic retinopathy, which present opportunities to make an impact in eye care for millions of patients. In 2022, we made meaningful progress with our RGX-314 trial enrollment objectives and geographic expansion planning. We also established in-house manufacturing to meet global clinical and commercial regulatory standards. We remain committed to developing treatments for rare diseases and made significant advancements with our pipeline of treatments for diseases such as Batten, Duchenne, Hurler and Hunter Syndrome, the latter of which we expect will support a second BLA filing using the accelerated approval pathway. Our history, science, resources, people and values combine to make us an industry leader in gene therapy and in the development of potentially ground-breaking therapies. We look forward to providing additional updates about our progress in 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. Material from REGENXBIO's NAVXpress™ platform process has been incorporated in the pivotal trials and is expected to be produced at REGENXBIO's Manufacturing Innovation Center for future commercialization of

RGX-314. The pivotal trials are expected to support a Biologics License Application (BLA) submission in 2024.
    - In February 2023, REGENXBIO announced data from the Phase II pharmacodynamic study, demonstrating that RGX-314 produced by the NAVXpress platform process was well tolerated and exhibited a similar clinical profile to the initial adherent cell culture process used in the Phase I/IIa trial. The Phase II pharmacodynamic study is designed to evaluate the same dose levels being used in the two pivotal trials.
    - In October 2022, REGENXBIO announced data from the Phase I/IIa Long-term Follow-up study demonstrating that RGX-314 continued to be generally well-tolerated and showed a long-term, durable treatment effect in Cohort 3 up to four years and in Cohort 4 up to three years. Doses similar to those used in Cohort 3 and Cohort 4 of the Phase I/IIa trial were advanced into the pivotal trials.

- RGX-314 Suprachoroidal Delivery for the Treatment of Wet AMD
    - The Phase II AAVIATE® trial continues to enroll patients in Cohort 6 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 are enrolled in Cohort 6 regardless of neutralizing antibody (NAb) status. REGENXBIO expects to complete enrollment of Cohort 6 in the first half of 2023 and report additional interim trial data, including initial data from Cohort 6, in the second half of 2023.
    - In October 2022, REGENXBIO announced new interim data from the Phase II AAVIATE trial, demonstrating that RGX-314 using suprachoroidal delivery was well tolerated across 85 patients. Meaningful reduction in treatment burden at six months was observed across all dose levels, and no meaningful differences in outcomes were observed at six months for patients who are NAb positive. Some patients had mild to moderate intraocular inflammation, all of which resolved with topical corticosteroids.

- RGX-314 Suprachoroidal Delivery for the Treatment of DR
    - The Phase II ALTITUDE® trial continues to enroll patients in two new cohorts at a higher third dose level, with patients stratified by Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (DRSS) levels and all receiving short-course prophylactic ocular steroids following RGX-314 administration. REGENXBIO expects to complete enrollment of both cohorts from the higher third dose level in the first half of 2023 and report additional interim trial data, including initial data from the third dose level, in the second half of 2023.
    - In November 2022, REGENXBIO announced new data from the Phase II ALTITUDE trial demonstrating that RGX-314 was well tolerated across 50 patients and BCVA remained stable through six months. Patients treated with RGX-314 demonstrated clinically meaningful improvements in disease severity and less disease worsening versus observation control at six months as measured by DRSS. No meaningful differences in safety outcomes were observed for patients who are NAb positive. Some patients had mild intraocular inflammation, all of which resolved with topical corticosteroids.

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.

- The Phase I/II AFFINITY DUCHENNE™ trial is active and recruiting patientsREGENXBIO plans to use commercial-scale cGMP material from the REGENXBIO Manufacturing Innovation Center in the clinical trial. REGENXBIO expects to report initial data from the trial in

the second half of 2023.
- Additionally, REGENXBIO is recruiting patients in the AFFINITY BEYOND™ trial, an observational screening study to evaluate the prevalence of AAV8 antibodies in patients with Duchenne up to 12 years of age. Information collected in this study may be used to identify potential participants for the AFFINITY DUCHENNE trial and potential future trials of RGX-202.

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.

- The Phase I/II/III CAMPSIITE™ trial is ongoing, and is expected to incorporate material from the NAVXpress platform process manufactured at the REGENXBIO Manufacturing Innovation Center to support the future commercialization of RGX-121. CAMPSIITE is expected to complete enrollment of 10 MPS II patients in the first half of 2023 to support a BLA filing in 2024 using the accelerated approval pathway.
- In February 2023, REGENXBIO announced additional interim data from the Phase I/II part of the CAMPSIITE trial, demonstrating that RGX-121 continued to be well-tolerated across 15 patients. Patients receiving the pivotal program dose level continued to demonstrate the largest reductions in CSF GAGs and continued to approach normal levels at 48 weeks. CSF GAGs 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. Longer-term clinical measures demonstrated continued improvement in neurodevelopmental and daily activity skill acquisition up to three years after RGX-121 administration.
- A 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.

- A Phase I/II trial of RGX-111 for the treatment of MPS I is fully enrolled with follow-up ongoing. REGENXBIO is on track to manufacture commercial-scale cGMP material using the NAVXpress platform process in the first half of 2023 to support the continued development of RGX-111. REGENXBIO expects to share additional updates on program plans in the second half of 2023.
- In February 2023, REGENXBIO announced additional positive interim data from the Phase I/II trial demonstrating that RGX-111 was well tolerated in eight patients. Biomarker and neurodevelopmental assessments indicated an encouraging CNS profile in patients dosed with RGX-111.

RGX-181: RGX-181 is an investigational one-time AAV Therapeutic for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, a form of Batten disease, using the NAV AAV9 vector to deliver the TPP1 gene directly to the central nervous system.

- In December 2022, REGENXBIO announced that physician investigators in Brazil have dosed the first child with CLN2 disease with RGX-181 in a single-patient, investigator-initiated study.

RGX-381: RGX-381 is an investigational one-time AAV Therapeutic for the treatment of the ocular manifestations of CLN2 disease using the NAV AAV9 vector to deliver the TPP1 gene directly to the retina.

- In December 2022, REGENXBIO announced a clinical trial application (CTA) has been accepted by the UK Health Authority to support a first-in-human, open-label, dose-escalation Phase I/II clinical trial to evaluate the safety and tolerability, as well as the effect on retinal anatomic and functional outcomes, of the subretinal delivery of RGX-381 for the treatment of

ocular manifestations of CLN2 disease.
- REGENXBIO continues to expect to initiate the Phase I/II clinical trial in the first half of 2023.

Operational Updates

- The REGENXBIO Manufacturing Innovation Center in Maryland is fully operational, producing GMP bulk substance lots to support programs using the NAVXpress™ platform process.
  - The 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 and 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.
  - In 2023, REGENXBIO plans to utilize the facility to continue to produce commercial-scale cGMP material for its entire clinical pipeline and performance qualification lots to support planned BLA filings in 2024 for RGX-314 and RGX-121.

NAV Technology Licensee Program Highlights

As of December 31, 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 February 2023, Novartis AG reported fourth quarter and full year 2022 global sales of Zolgensma of $309 million and $1.37 billion, respectively.
- In January 2023, Rocket Pharmaceuticals, Inc. announced positive updates from its Phase I trial of RP-A501 for the treatment of Danon Disease. The Phase II pivotal trial remains on track for initiation in the second quarter of 2023 based on ongoing and productive FDA interactions. RP-A501 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.
- In January 2023, Ultragenyx Pharmaceutical Inc. (Ultragenyx) announced that in the Phase III study of DTX401 for Glycogen Storage Disease Type Ia (GSDIa), the last patient has entered the baseline screening period and data readout is expected in the first half of 2024. Ultragenyx also expects to initiate the Phase III study of DTX301 for Ornithine Transcarbamylase (OTC) Deficiency in the first quarter of 2023. DTX401 and DTX301 are both being developed as one-time gene therapies utilizing REGENXBIO's NAV AAV8 vector.

Financial Results

Cash Position: Cash, cash equivalents and marketable securities were $565.2 million as of December 31, 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 year ended December 31, 2022.

Revenues: Revenues were $31.3 million and $112.7 million for the three months and year ended December 31, 2022, respectively, compared to $398.7 million and $470.3 million for the three months and year ended December 31, 2021, respectively. The decreases were primarily attributable to non-recurring revenue of $370.0 million recognized in the fourth quarter of 2021 upon the effectiveness of our eye care collaboration with AbbVie. The decrease in revenues for the year ended December 31, 2022 was partially offset by Zolgensma royalty revenues, which increased from $95.0 million in 2021 to $101.9 million in 2022.

Research and Development Expenses: Research and development expenses were $62.5 million and $242.5 million for the three months and year ended December 31, 2022, respectively, compared to $48.0 million and $181.4 million for the three months and year ended December 31, 2021, respectively. The increases were 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 $21.2 million and $85.3 million for the three months and year ended December 31, 2022, respectively, compared to $22.0 million and $79.3 million for the three months and year ended December 31, 2021, respectively. The increase in general and administrative expenses for the year ended December 31, 2022 was primarily attributable to corporate overhead expenses.

Net Income (Loss): Net loss was $59.9 million, or $1.38 basic and diluted net loss per share, for the three months ended December 31, 2022, compared to net income of $294.0 million, or $6.87 basic and $6.67 diluted net income per share, for the three months ended December 31, 2021. Net

loss was $280.3 million, or $6.50 basic and diluted net loss per share, for the year ended December 31, 2022, compared to net income of $127.8 million, or $3.01 basic and $2.91 diluted net income per share, for the year ended December 31, 2021.

Financial Guidance

REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $565.2 million as of December 31, 2022 to fund its operations into 2025. This cash runway guidance is based on the Company's current operational plans and excludes the impact of any payments that may be received from AbbVie upon the achievement of development or commercial milestones under our RGX-314 collaboration.

Conference Call

In connection with this announcement, REGENXBIO will host a conference call and webcast today at 4:30 p.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 and AAV9. 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, 2022, 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)

	December 31, 2022	December 31, 2021
Assets		
Current assets		
Cash and cash equivalents	$ 96,952	$ 345,209
Marketable securities	267,690	112,230
Accounts receivable, net	28,082	32,439
Prepaid expenses	13,900	18,752
Other current assets	9,352	10,196
Total current assets	415,976	518,826
Marketable securities	200,560	391,907
Accounts receivable, net	1,504	2,262
Property and equipment, net	141,685	131,547
Operating lease right-of-use assets	65,116	60,904
Restricted cash	2,030	2,030
Other assets	6,397	6,428
Total assets	$ 833,268	$ 1,113,904
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 27,213	$ 11,387
Accrued expenses and other current liabilities	46,794	76,111
Deferred revenue	1,829	3,333

Operating lease liabilities	5,997	1,752
Liability related to sale of future royalties	48,601	37,889
Total current liabilities	130,434	130,472
Operating lease liabilities	88,802	84,929
Liability related to sale of future royalties	89,005	133,460
Other liabilities	8,832	745
Total liabilities	317,073	349,606
Stockholders' equity		
Preferred stock; no shares issued and outstanding at December 31, 2022 and 2021	—	—
Common stock; 43,299 and 42,831 shares issued and outstanding at December 31, 2022 and 2021, respectively	4	4
Additional paid-in capital	973,145	928,095
Accumulated other comprehensive loss	(15,401)	(2,569)
Accumulated deficit	(441,553)	(161,232)
Total stockholders' equity	516,195	764,298
Total liabilities and stockholders' equity	$ 833,268	$ 1,113,904

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

	Three Months Ended December 31,		Years Ended December 31,	
	2022	2021	2022	2021
Revenues				
License and royalty revenue	$ 31,345	$ 398,655	$ 112,724	$ 470,347
Total revenues	31,345	398,655	112,724	470,347
Operating Expenses				
Cost of revenues	12,783	23,058	54,545	51,833
Research and development	62,505	47,978	242,453	181,437
General and administrative	21,210	22,040	85,281	79,333
Credit losses (recoveries)	—	(8,102)	—	(2,569)
Other operating expenses (income)	(7,382)	85	(6,679)	333
Total operating expenses	89,116	85,059	375,600	310,367
Income (loss) from operations	(57,771)	313,596	(262,876)	159,980
Other Income (Expense)				
Interest income from licensing	77	19	342	719
Investment income	2,026	311	5,383	6,825
Interest expense	(4,310)	(6,500)	(23,254)	(26,277)
Total other income (expense)	(2,207)	(6,170)	(17,529)	(18,733)
Income (loss) before income taxes	(59,978)	307,426	(280,405)	141,247
Income Tax Benefit (Expense)	43	(13,403)	84	(13,407)
Net income (loss)	$ (59,935)	$ 294,023	$ (280,321)	$ 127,840
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	2,855	(1,284)	(12,832)	(2,209)
Total other comprehensive income (loss)	2,855	(1,284)	(12,832)	(2,209)
Comprehensive income (loss)	$ (57,080)	$ 292,739	$ (293,153)	$ 125,631
Net income (loss) per share:				
Basic	$ (1.38)	$ 6.87	$ (6.50)	$ 3.01
Diluted	$ (1.38)	$ 6.67	$ (6.50)	$ 2.91
Weighted-average common shares outstanding:				
Basic	43,296	42,774	43,152	42,438
Diluted	43,296	44,084	43,152	43,913

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