



REGENXBIO Reports Fourth Quarter and Full-Year 2021 Financial Results and Recent Operational Highlights

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- *Closed eye care collaboration agreement with AbbVie to develop and commercialize RGX-314*
 - *Received upfront payment of \$370 million and eligible to receive up to \$1.38 billion in additional development, regulatory and commercial milestones*
- *Continue to advance RGX-314 program for the treatment of wet AMD and diabetic retinopathy*
 - *Two pivotal trials active for the treatment of wet AMD using subretinal delivery*
 - *Today announced Cohort 4 enrollment complete in RGX-314 Phase II trial for the treatment of wet AMD using in-office suprachoroidal delivery; presented positive six-month data*
 - *Presented positive six-month data from RGX-314 Phase II trial for the treatment of diabetic retinopathy using in-office suprachoroidal delivery*
- *Received IND clearance and Orphan Drug Designation for RGX-202 for the treatment of Duchenne*
 - *Today announced that FDA has granted Rare Pediatric Disease Designation for RGX-202*
 - *AFFINITY DUCHENNE™ Phase I/II clinical trial on-track to be initiated in the first half of 2022*
- *Presented positive data from RGX-121 and RGX-111 clinical trials for the treatment of MPS II and MPS I*
- *\$849 million current cash and cash equivalents of as of December 31, 2021; including upfront payment from closing of the AbbVie eye care collaboration agreement; operational runway into 2025*
- *Conference call Tuesday, March 1st at 4:30 p.m. ET*

REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the fourth quarter ended December 31, 2021, and recent operational highlights.

"I am pleased with our performance as an organization over this past quarter, which was highlighted by the successful closing of our eye care collaboration agreement with AbbVie," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We believe this collaboration, along with the continued positive interim data we presented at recent medical meetings, provides strong validation supporting the development of RGX-314 as a potential one-time gene therapy for the treatment of some of the largest ophthalmic markets. We look forward to working with AbbVie and providing additional data from our Phase II trials in diabetic retinopathy and wet AMD."

Mr. Mills continued: "Our 2021 momentum has continued into the new year with a number of recent events that we believe raise the profile of our exciting neurodegenerative and neuromuscular pipeline. Most recently, we presented positive interim data at the 18th Annual WORLDSymposium that demonstrated that RGX-121 and RGX-111 were well tolerated with encouraging evidence of CNS biomarker activity and improvements in neurodevelopmental function. We are looking forward to advancing RGX-202 for the treatment of Duchenne this year, following the FDA's clearance of our Investigational New Drug application and granting of Rare Pediatric Disease Designation and Orphan Drug Designation, which mark important milestones and acknowledgement of the need for new treatment options for patients with Duchenne. RGX-202 is our first home-grown program leveraging our proprietary NAV AAV8 vector to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal (CT) domain. We believe that RGX-202 has the potential to address the limitations of other treatment approaches for this severe, degenerative disease, and we look forward to initiating this clinical trial."

Program Highlights and Milestones

RGX-314: RGX-314 is an investigational one-time gene therapy being developed 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™, the first of two pivotal trials to evaluate the efficacy and safety of RGX-314 in patients with wet AMD using the subretinal delivery approach.
 - REGENXBIO recently initiated ASCENT™, the second of two pivotal trials to evaluate the efficacy and safety of subretinal delivery of RGX-314 in patients with wet AMD. 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 Licensing Application (BLA) submission for RGX-314 in 2024.
- RGX-314 Suprachoroidal Delivery for the Treatment of Wet AMD
 - In November 2021, REGENXBIO presented positive interim data from the Phase II AAVIATE™ trial for the treatment of wet AMD at the 2021 American Academy of Ophthalmology (AAO).
 - As of November 4, 2021, suprachoroidal delivery of RGX-314 continued to be well tolerated in 50 patients from Cohorts 1-3 with no drug-related serious adverse events (SAEs).
 - At six months following one-time administration of RGX-314, stable visual acuity and retinal thickness, as well as a meaningful reduction (>70%) in anti-VEGF treatment burden, was observed in patients in Cohort 2, with 40% of patients anti-VEGF injection-free (dose level 5×10^{11} GC/eye).
 - Mild intraocular inflammation observed on slit-lamp examination was reported at similar incidence across both dose levels in Cohorts 1 and 2, with four out of 15 patients in Cohort 1 and three out of 15 patients in Cohort 2. All cases of inflammation in both cohorts were resolved within days to weeks on topical corticosteroids.
 - Enrollment is complete in Cohort 4 and expected to be completed in Cohort 5 in the first half of 2022. These cohorts are evaluating RGX-314 at a third dose level of 1×10^{12} GC/eye. Cohort 5 is evaluating RGX-314 in patients who are neutralizing antibody (NAb) positive. As in previous cohorts, patients will not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.
- RGX-314 Suprachoroidal Delivery for the Treatment of DR
 - In February 2022, REGENXBIO presented positive interim data from the Phase II ALTITUDE™ trial for the treatment of DR at the 2022 Angiogenesis, Exudation, and Degeneration conference.
 - As of January 18, 2022, suprachoroidal delivery of RGX-314 continued to be well tolerated in the 15 patients dosed with RGX-314 in Cohort 1, with no drug-related SAEs, and no intraocular inflammation observed.
 - Of the patients dosed with RGX-314 in Cohort 1, 47% demonstrated a two-step or greater improvement from baseline on the Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (ETDRS-DRSS) at six months, compared to 0% in the observational control group. One patient (7%) dosed with RGX-314 continued to demonstrate a four-step improvement. The percentage of Cohort 1 patients dosed with RGX-314 achieving at least two-step improvement at six months in RGX-314 treated eyes (47%) increased from the previously reported three-month results (33%).
 - Enrollment is expected to be completed in the first half of 2022 for Cohorts 2 and 3 at an increased dose level of 5×10^{11} GC/eye. Cohort 3 is evaluating RGX-314 in patients who are neutralizing antibody (NAb) positive. As in Cohort 1, patients will not receive

prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.

RGX-202: RGX-202 is an investigational one-time gene therapy 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.

- REGENXBIO today announced that the U.S. Food and Drug Administration (FDA) granted Rare Pediatric Disease Designation for RGX-202, which may entitle REGENXBIO to receive a priority review voucher which can be redeemed to obtain priority review for any subsequent marketing application and may be sold or transferred should a new BLA for RGX-202 be approved.
- In January 2022, REGENXBIO announced Investigational New Drug (IND) clearance by the FDA. REGENXBIO plans to initiate AFFINITY DUCHENNE, a Phase I/II clinical trial, in the first half 2022.
- In November 2021, REGENXBIO announced that the FDA granted Orphan Drug Designation for RGX-202.

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

- In February 2022, REGENXBIO announced new data from the Phase I/II trial of RGX-121 for the treatment of MPS II at the 18th Annual *WORLD Symposium*.
 - As of December 20, 2021, RGX-121 continued to be well-tolerated with no drug-related SAEs across three dose levels with preliminary results indicating dose-dependent reductions in key CSF biomarkers, with patients in Cohort 3 approaching normal levels of the D2S6 biomarker.
 - Measures of neurodevelopmental function from patients in Cohorts 1 and 2 demonstrated continued developmental skill acquisition up to 2 years after RGX-121 administration.
 - Evidence of systemic enzyme expression and biomarker activity continued to be observed.
 - Cohort 3 expansion using commercial-scale cGMP material is planned to start in the first quarter of 2022.
- The Phase I/II trial of RGX-121 for the treatment of pediatric patients with MPS II over the age of 5 years old is ongoing.

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

- In February 2022, REGENXBIO announced new interim data from the Phase I/II trial and the single-patient IND of RGX-111 at the 18th Annual *WORLD Symposium*.
 - As of December 20, 2021, RGX-111 was well tolerated across two dose levels in the Phase I/II trial and in the single-patient IND, with no drug-related SAEs.
 - Biomarker and neurodevelopmental assessments indicated encouraging CNS profile in patients dosed with RGX-111, with emerging evidence of systemic biomarker activity observed.
 - REGENXBIO plans to enroll additional patients in a Cohort 2 expansion in the first half of 2022.

Operational Updates

- In November 2021, REGENXBIO closed the eye care collaboration agreement with AbbVie to develop and commercialize RGX-314.

- REGENXBIO received an upfront payment from AbbVie of \$370 million in the fourth quarter 2021. In addition, REGENXBIO is eligible to receive up to \$1.38 billion in additional development, regulatory and commercial milestones.
- Current Good Manufacturing Practice (cGMP) Manufacturing Facility
 - REGENXBIO's cGMP facility, which is expected to allow for production of NAV vectors at scales up to 2,000 liters using REGENXBIO's platform suspension cell culture process, is on track to be fully operational starting in the first half of 2022.

NAV Technology Licensee Program Highlights

As of December 31, 2021, 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.

- REGENXBIO's NAV Technology Platform is being applied in one marketed product, Zolgensma®. In February 2022, Novartis AG reported fourth quarter and full year 2021 global sales of Zolgensma of \$342 million and \$1.35 billion, respectively (>1,800 patients dosed).
- In November 2021, Rocket Pharmaceuticals, Inc. announced sustained benefit across clinical, functional and biomarker endpoints in all four patients with long-term follow up in the Phase I clinical trial of RP-A501 for the treatment on Danon Disease. Rocket anticipates initiating Phase II pivotal study activities for RP-A501 in the fourth quarter 2022. RP-A501 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.
- In November 2021, Ultragenyx Pharmaceutical Inc. announced long-term durability data with sustained responses lasting up to three years since treatment from the Phase I/II study of DTX401 for the treatment of Glycogen Storage Disease Type Ia (GSDIa), and durable metabolic control and sustained responses from the Phase I/II study of DTX301 for the treatment of Ornithine Transcarbamylase (OTC) deficiency. In February 2022, Ultragenyx announced it has dosed the first patients in the Phase III study of DTX401 and anticipates initiating the Phase III study for DTX301 in the first half of 2022. 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 \$849.3 million as of December 31, 2021, compared to \$522.5 million as of December 31, 2020. The increase was primarily attributable to a \$370.0 million upfront payment received upon the closing of the AbbVie eye care collaboration agreement in November 2021 and \$216.1 million of aggregate net proceeds received from a follow-on public offering of common stock completed in January 2021, and was partially offset by cash used to fund operating activities and capital expenditures during the year ended December 31, 2021.

Revenues: Revenues were \$398.7 million and \$470.3 million for the three months and year ended December 31, 2021, respectively, compared to \$21.4 million and \$154.6 million for the three months and year ended December 31, 2020, respectively. The increases were primarily attributable to revenue of \$370.0 million recognized in the fourth quarter of 2021 under our eye care collaboration agreement with AbbVie, and increases in Zolgensma royalty revenues, which increased by \$7.1 million and \$33.3 million for the three months and year ended December 31, 2021, respectively, as compared to the same periods in 2020. The increase in revenues for the year ended December 31, 2021 was partially offset by an \$80.0 million milestone payment recognized as revenue in the third quarter of 2020 upon the achievement of \$1.0 billion in cumulative net sales of Zolgensma. As reported by Novartis, sales of Zolgensma for the fourth quarter of 2021 increased by 35% as compared to the fourth quarter of 2020, driven by geographic expansion of product access.

Research and Development Expenses: Research and development expenses were \$48.0 million and \$181.4 million for the three months and year ended December 31, 2021, respectively, compared to \$47.2 million and \$166.3 million for the three months and year ended December 31, 2020, respectively. The increases were primarily attributable to personnel costs as a result of increased headcount, laboratory and facilities costs, and costs associated with clinical trial and regulatory activities for our lead product candidates.

General and Administrative Expenses: General and administrative expenses were \$22.0 million and \$79.3 million for the three months and year ended December 31, 2021, respectively, compared to \$17.6 million and \$63.8 million for the three months and year ended December 31, 2020. The increases were primarily attributable to personnel costs as a result of increased headcount and professional fees for advisory and other services.

Net Income (Loss): Net income was \$294.0 million, or \$6.87 basic and \$6.67 diluted net income per share, for the three months ended December 31, 2021, compared to a net loss of \$46.2 million, or \$1.24 basic and diluted net loss per share, for the three months ended December 31, 2020. Net income was \$127.8 million, or \$3.01 basic and \$2.91 diluted net income per share, for the year ended December 31, 2021, compared to a net loss of \$111.3 million, or \$2.98 basic and diluted net loss per share, for the year ended December 31, 2020.

Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of \$849.3 million as of December 31, 2021 to fund its operations, including the completion of its internal manufacturing capabilities and clinical advancement of its product candidates, into 2025.

Conference Call

In connection with this announcement, REGENXBIO will host a conference call and webcast today at 4:30 p.m. ET. To access the live call by phone, dial (855) 422-8964 (domestic) or (210) 229-8819 (international) and enter the passcode 7635958. To access a live or recorded webcast of the call, please visit the "Investors" section of the REGENXBIO website at www.regenxbio.com. The recorded webcast will be available for approximately 30 days following the call.

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 in multiple therapeutic areas.

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 and clinical trials. 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, which will be filed with the U.S. Securities and Exchange Commission (SEC) in the first quarter of 2022, 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.

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

	December 31, 2021	December 31, 2020
Assets		
Current assets		
Cash and cash equivalents	\$ 345,209	\$ 338,426
Marketable securities	112,230	137,314
Accounts receivable, net	32,439	42,999
Prepaid expenses	18,752	10,505
Other current assets	10,196	1,953
Total current assets	518,826	531,197
Marketable securities	391,907	46,809
Accounts receivable, net	2,262	3,267
Property and equipment, net	131,547	56,467
Operating lease right-of-use assets	60,904	63,815
Restricted cash	2,030	1,330
Other assets	6,428	5,279
Total assets	\$ 1,113,904	\$ 708,164
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 11,387	\$ 10,622
Accrued expenses and other current liabilities	76,111	49,082
Deferred revenue	3,333	449
Operating lease liabilities	1,752	2,500
Liability related to sale of future royalties	37,889	18,794
Total current liabilities	130,472	81,447
Deferred revenue	—	3,783
Operating lease liabilities	84,929	70,153
Liability related to sale of future royalties	133,460	174,504
Other liabilities	745	524
Total liabilities	349,606	330,411
Stockholders' equity		

Preferred stock; no shares issued and outstanding at December 31, 2021 and 2020	—	—
Common stock; 42,831 and 37,476 shares issued and outstanding at December 31, 2021 and 2020, respectively	4	4
Additional paid-in capital	928,095	667,181
Accumulated other comprehensive loss	(2,569)	(360)
Accumulated deficit	(161,232)	(289,072)
Total stockholders' equity	<u>764,298</u>	<u>377,753</u>
Total liabilities and stockholders' equity	<u>\$ 1,113,904</u>	<u>\$ 708,164</u>

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

	Three Months		Years	
	Ended December 31,		Ended December 31,	
	2021	2020	2021	2020
Revenues				
License and royalty revenue	\$ 398,655	\$ 21,445	\$ 470,347	\$ 154,567
Total revenues	<u>398,655</u>	<u>21,445</u>	<u>470,347</u>	<u>154,567</u>
Operating Expenses				
Cost of revenues	23,058	10,257	51,833	35,714
Research and development	47,978	47,180	181,437	166,294
General and administrative	22,040	17,571	79,333	63,817
Credit losses (recoveries) and other	(8,017)	88	(2,236)	7,975
Total operating expenses	<u>85,059</u>	<u>75,096</u>	<u>310,367</u>	<u>273,800</u>
Income (loss) from operations	<u>313,596</u>	<u>(53,651)</u>	<u>159,980</u>	<u>(119,233)</u>
Other Income (Expense)				
Interest income from licensing	19	130	719	4,271
Investment income	311	13,794	6,825	9,723
Interest expense	(6,500)	(771)	(26,277)	(771)
Total other income (expense)	<u>(6,170)</u>	<u>13,153</u>	<u>(18,733)</u>	<u>13,223</u>
Income (loss) before income taxes	<u>307,426</u>	<u>(40,498)</u>	<u>141,247</u>	<u>(106,010)</u>
Income Tax Expense	<u>(13,403)</u>	<u>(5,743)</u>	<u>(13,407)</u>	<u>(5,240)</u>
Net income (loss)	<u>\$ 294,023</u>	<u>\$ (46,241)</u>	<u>\$ 127,840</u>	<u>\$ (111,250)</u>
Other Comprehensive Loss				
Unrealized loss on available-for-sale securities, net	(1,284)	(623)	(2,209)	(565)
Total other comprehensive loss	<u>(1,284)</u>	<u>(623)</u>	<u>(2,209)</u>	<u>(565)</u>
Comprehensive income (loss)	<u>\$ 292,739</u>	<u>\$ (46,864)</u>	<u>\$ 125,631</u>	<u>\$ (111,815)</u>
Net income (loss) per share:				
Basic	<u>\$ 6.87</u>	<u>\$ (1.24)</u>	<u>\$ 3.01</u>	<u>\$ (2.98)</u>
Diluted	<u>\$ 6.67</u>	<u>\$ (1.24)</u>	<u>\$ 2.91</u>	<u>\$ (2.98)</u>
Weighted-average common shares outstanding:				
Basic	<u>42,774</u>	<u>37,418</u>	<u>42,438</u>	<u>37,281</u>
Diluted	<u>44,084</u>	<u>37,418</u>	<u>43,913</u>	<u>37,281</u>

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