# REGENXBIO Reports Fourth Quarter and Full-Year 2020 Financial Results and Operational Highlights

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- *Pivotal program for subretinal delivery of RGX-314 for the treatment of wet AMD is active and enrolling patients; expected to support BLA filing in 2024*
- *Phase II trials of RGX-314 utilizing in-office suprachoroidal delivery for treatment of wet AMD and diabetic retinopathy are ongoing*
- *IND filing expected in mid-2021 for RGX-202, a novel, advanced microdystrophin gene therapy for the treatment of Duchenne Muscular Dystrophy*
- *Positive interim data from RGX-121 Phase I/II trial demonstrated consistent reductions in CNS biomarkers, continued neurocognitive development, and evidence of systemic effects*
- *Monetized portion of Zolgensma® royalties in December 2020 for $200 million in gross proceeds*
- *$523 million in cash, cash equivalents and marketable securities as of December 31, 2020*
- *Completed public offering of common stock in January 2021 of approximately $230 million in gross proceeds*
- *Conference call Monday, March 1st at 4:30 p.m. ET*

REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the fourth quarter and full year ended December 31, 2020, and recent operational highlights.

"Over the past year, we made great strides in driving our key internal clinical development programs forward while advancing the build-out of our manufacturing capabilities at our new headquarters, and strengthening our balance sheet," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We have initiated our pivotal program for RGX-314 in wet AMD, and ATMOSPHERE, the first of two planned clinical trials, is active and enrolling patients. Our Phase II trials of RGX-314 delivered suprachoroidally for the treatment of wet AMD and Diabetic Retinopathy continue to enroll patients. We also unveiled RGX-202, a potential one-time gene therapy for the treatment of DMD, and we look forward to filing an IND in mid-2021. In addition, we recently announced positive interim data from our ongoing Phase I/II trial of RGX-121 for the treatment of children with MPS II and we look forward to reporting additional data as this program advances."

Mr. Mills added: "Our numerous achievements in 2020, which took place in the backdrop of a challenging pandemic, would not have been possible without the support of our employees, clinical partners, patients and their families, and we are grateful for their commitment."

**Recent Operational Highlights**

***Gene Therapy Using NAV® Vectors for AAV-Mediated Antibody Delivery***

- Pivotal Program for RGX-314 for the Treatment of Wet Age-related Macular Degeneration (wet AMD)
  - REGENXBIO announced in January 2021 that it completed an End of Phase 2 meeting with the U.S. Food and Drug Administration (FDA) to discuss the details of a pivotal program to support a Biologics License Application (BLA). REGENXBIO plans to conduct two randomized, well-controlled clinical trials to evaluate the efficacy and safety of RGX-314 in patients with wet AMD, enrolling approximately 700 patients total. REGENXBIO expects to submit a BLA based on these trials in 2024.
    - ATMOSPHERE™, the first of two planned pivotal trials, is active and enrolling patients. The trial will enroll approximately 300 patients across two RGX-314 dose arms versus ranibizumab. The primary endpoint of the trial is non-inferiority to

ranibizumab based on change from baseline in Best Corrected Visual Acuity (BCVA) at one year.
        - The second pivotal trial is expected to be similar in design to ATMOSPHERE and REGENXBIO plans to initiate the trial in the second half of 2021.
        - The Company plans to incorporate its scalable suspension cell culture manufacturing process to support future commercialization upon completion of a bridging study and the pivotal trials. The bridging study is expected to initiate in the first half of 2021.
    - In February 2021, REGENXBIO presented additional positive data from the patients enrolled in the ongoing Phase I/II trial of RGX-314 for the treatment of wet AMD and its Long-Term Follow-Up study.
        - As of January 22, 2021, RGX-314 continued to be generally well-tolerated across all dose cohorts.
        - Durable treatment effect was observed in patients in Cohorts 4 and 5 at 1.5 years after administration of RGX-314, including stable visual acuity, decreased retinal thickness, and reductions in anti-VEGF injection burden.
        - Long-term, durable treatment effect was demonstrated in Cohort 3 over three years, including mean improvement in vision and stable retinal thickness, and reductions in anti-VEGF treatment burden.

- Suprachoroidal Delivery of RGX-314 for the Treatment of Wet AMD
    - In January 2021, REGENXBIO announced that it had completed enrollment of patients in Cohort 1 of AAVIATE™, a Phase II trial for the treatment of wet AMD. The Company plans to report interim efficacy data from Cohort 1 in the third quarter of 2021. Enrollment of patients in Cohort 2 has begun and is expected to be complete in the second quarter of 2021.

- Suprachoroidal Delivery of RGX-314 for the Treatment of Diabetic Retinopathy (DR)
    - REGENXBIO expects to report initial data from ALTITUDE, a Phase II trial for the treatment of DR, in 2021.

- Research Program for the Treatment of Hereditary Angioedema (HAE)
    - REGENXBIO expects to provide a program update in 2021.

- Research Program for the Treatment of Neurodegenerative Diseases
    - REGENXBIO continues to collaborate with Neurimmune AG on research programs targeting both alpha synuclein and tau, and expects to provide a program update in 2021.

***Gene Therapy Using NAV Vectors for Rare Genetic Diseases***

- RGX-202 for the Treatment of Duchenne Muscular Dystrophy (DMD)
    - In January 2021, REGENXBIO announced the development of a potential one-time gene therapy for the treatment of DMD. RGX-202 is designed to deliver a novel microdystrophin transgene, which includes an extended coding region of the C-Terminal (CT) domain found in naturally occurring dystrophin, as well as other fundamental improvements.
        - REGENXBIO expects to submit an Investigational New Drug (IND) application to the FDA in mid-2021.

- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
    - In February 2021, REGENXBIO presented additional positive interim data from Cohorts 1 and 2 of the ongoing Phase I/II trial of RGX-121 for the treatment of patients up to 5 years old diagnosed with MPS II.
        - As of January 4, 2021, RGX-121 is reported to be well-tolerated with no drug-related serious adverse events (SAEs) in eight patients dosed with RGX-121.
        - Biomarker data from patients in both cohorts indicate encouraging signals of I2S enzyme activity in the CNS following one-time administration of RGX-121, with consistent reductions of HS and D2S6, a component of HS. Patients in Cohorts 1 and 2 also demonstrated continued neurocognitive development and evidence of I2S enzyme activity in plasma and urine following administration of RGX-121.
        - REGENXBIO plans to evaluate a higher dose of RGX-121 in a third cohort of patients which is expected to begin enrolling in the first quarter of 2021.
    - REGENXBIO expects to begin dosing patients in a Phase I/II multicenter, open-label trial of RGX-121 for the treatment of pediatric patients with MPS II over the age of 5 years old in the first half of 2021.

- RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)
    - In December 2020, REGENXBIO announced dosing of the first patient in the Phase I/II trial of RGX-111 for the treatment of MPS I. Enrollment is ongoing.

- RGX-381 for the Treatment of Ocular Manifestations of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease
    - REGENXBIO plans to submit an IND application, or foreign equivalent, for a Phase I/II study of RGX-381 in patients with CLN2 disease in the first half of 2021.

- RGX-181 for the Treatment of CLN2 Disease
    - REGENXBIO expects to submit an IND for the intracisternal delivery of RGX-181 in the first quarter of 2021 and plans to initiate enrollment in a Phase I/II trial in the first half of 2021.

**Operational Updates**

- Current Good Manufacturing Practice (cGMP) Manufacturing Facility
    - Construction of a new corporate, research and manufacturing headquarters in Rockville, Maryland continues, with plans to begin utilizing the new headquarters in the first half of 2021. The new cGMP production facility is expected to allow for production of NAV vectors at scales up to 2,000 liters using REGENXBIO's platform suspension cell culture process, which will complement REGENXBIO's current external manufacturing network and capabilities. The cGMP facility is expected to be operational starting in the first half of 2022.

**NAV Technology Licensee Program Highlights**

As of December 31, 2020, REGENXBIO's NAV Technology Platform was being applied in one marketed product, and multiple clinical stage programs, with over 20 partnered programs in total. REGENXBIO's NAV Technology Licensees are advancing product candidates in a broad range of therapeutic areas and disease indications. Recent updates from NAV Technology Licensees include:

- In December 2020, Eli Lilly and Company (Lilly) and Prevail Therapeutics Inc. (Prevail) announced a definitive agreement for Lilly to acquire Prevail, which closed in January 2021. Prevail licenses the NAV AAV9 vector for the development of gene therapy candidates for the treatment of Parkinson's disease and other neurodegenerative diseases.

- In December 2020, Rocket Pharmaceuticals, Inc. announced positive gene expression, clinical biomarker and preliminary functional data from their Phase I trial of RP-A501 for the treatment of Danon Disease. RP-A501 uses the NAV AAV9 vector.

***Marketed NAV Technology Products***

REGENXBIO's NAV Technology Platform is being applied in one marketed product, Zolgensma®. On January 26, 2021, Novartis AG reported fourth quarter 2020 global Zolgensma sales revenue of $254 million and full year 2020 revenue of $920 million.

In December 2020, REGENXBIO entered into an agreement to monetize a portion of the royalty rights due from the net sales of Zolgensma to entities managed by Healthcare Royalty Management, LLC (HCR) for gross proceeds of $200 million.

**Financial Results**

*Cash Position:* Cash, cash equivalents and marketable securities were $522.5 million as of December 31, 2020, compared to $400.0 million as of December 31, 2019. The increase was primarily attributable to gross proceeds of $200.0 million received from the monetization of REGENXBIO's Zolgensma royalty rights, partially offset by net cash used in operating activities of $54.1 million and cash used to purchase property and equipment of $26.9 million in 2020.

*Revenues:* Revenues were $21.4 million and $154.6 million for the three months and year ended December 31, 2020, respectively, compared to $11.8 million and $35.2 million for the three months and year ended December 31, 2019, respectively. The increases were primarily attributable to Zolgensma royalty revenue, which increased by $10.2 million and $40.8 million for the three months and year ended December 31, 2020, respectively, as compared to the same periods in 2019. The increase in revenue for the year ended December 31, 2020 also includes an $80.0 million milestone fee 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 AG, sales of Zolgensma for the fourth quarter of 2020 increased by 37% (USD) as compared to the fourth quarter of 2019.

*Research and Development Expenses:* Research and development expenses were $47.2 million and $166.3 million for the three months and year ended December 31, 2020, respectively, compared to $33.8 million and $124.2 million for the three months and year ended December 31, 2019, respectively. The increases were primarily attributable to personnel costs as a result of increased headcount and expenses associated with manufacturing services and conducting clinical trials for our lead product candidates.

*General and Administrative Expenses:* General and administrative expenses were $17.6 million and $63.8 million for the three months and year ended December 31, 2020, respectively, compared to $14.5 million and $51.8 million for the three months and year ended December 31, 2019, respectively. The increases were primarily attributable to personnel costs as a result of increased headcount and professional fees for advisory and other services.

*Net Loss:* Net loss was $46.2 million, or $1.24 basic and diluted net loss per share, and $111.3 million, or $2.98 basic and diluted net loss per share, for the three months and year ended December 31, 2020, respectively, compared to net loss of $26.5 million, or $0.72 basic and diluted net loss per share, and $94.7 million, or $2.58 basic and diluted net loss per share, for the three months and year ended December 31, 2019, respectively.

On January 12, 2021, REGENXBIO announced the closing of an underwritten public offering of 4,260,000 shares of its common stock at a price to the public of $47.00 per share, as well as the exercise in full of the underwriters' option to purchase 639,000 additional shares at the public offering price. Including the option exercise, the total gross proceeds received by REGENXBIO from the offering were approximately $230.3 million, before deducting the underwriting discounts and commissions and other offering expenses.

**Financial Guidance**

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $522.5 million as of December 31, 2020, as well as the $230.3 million of gross proceeds received from its public offering of common stock completed in January 2021, to fund its operations, including the completion of its internal manufacturing capabilities and clinical advancement of its product candidates, into the second half of 2023.

**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 6298809. 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, 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, 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, 2020, which will be filed with the U.S. Securities and Exchange Commission (SEC) in the first quarter of 2021, 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.

**REGENXBIO INC.**
**CONSOLIDATED BALANCE SHEETS**
**(unaudited)**
**(in thousands, except per share data)**

| | December 31, 2020 | December 31, 2019 |
|---|---|---|
| **Assets** | | |
| Current assets | | |
| Cash and cash equivalents | $ 338,426 | $ 69,514 |
| Marketable securities | 137,314 | 226,696 |
| Accounts receivable, net | 42,999 | 38,148 |
| Prepaid expenses | 10,505 | 6,475 |
| Other current assets | 1,953 | 4,199 |
| Total current assets | 531,197 | 345,032 |
| Marketable securities | 46,809 | 103,785 |
| Accounts receivable | 3,267 | 4,155 |
| Property and equipment, net | 56,467 | 28,973 |
| Operating lease right-of-use assets | 63,815 | 10,078 |
| Restricted cash | 1,330 | 1,330 |
| Other assets | 5,279 | 4,555 |
| Total assets | $ 708,164 | $ 497,908 |
| **Liabilities and Stockholders' Equity** | | |
| Current liabilities | | |
| Accounts payable | $ 10,622 | $ 6,409 |
| Accrued expenses and other current liabilities | 49,082 | 24,846 |
| Deferred revenue | 449 | — |
| Operating lease liabilities | 2,500 | 2,421 |
| Liability related to sale of future royalties | 18,794 | — |
| Total current liabilities | 81,447 | 33,676 |
| Deferred revenue | 3,783 | 3,333 |
| Operating lease liabilities | 70,153 | 8,874 |
| Liability related to sale of future royalties | 174,504 | — |
| Other liabilities | 524 | 1,828 |
| Total liabilities | 330,411 | 47,711 |
| Stockholders' equity | | |
| Preferred stock; no shares issued and outstanding at December 31, 2020 and December 31, 2019 | — | — |
| Common stock; 37,476 and 36,992 shares issued and outstanding at December 31, 2020 and December 31, 2019, respectively | 4 | 4 |
| Additional paid-in capital | 667,181 | 627,810 |
| Accumulated other comprehensive income (loss) | (360) | 205 |
| Accumulated deficit | (289,072) | (177,822) |
| Total stockholders' equity | 377,753 | 450,197 |
| Total liabilities and stockholders' equity | $ 708,164 | $ 497,908 |

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

| | Three Months Ended December 31, | | Years Ended December 31, | |
|---|---|---|---|---|
| | 2020 | 2019 | 2020 | 2019 |
| **Revenues** | | | | |
| License and royalty revenue | $ 21,445 | $ 11,768 | $ 154,567 | $ 35,233 |
| Total revenues | 21,445 | 11,768 | 154,567 | 35,233 |
| **Operating Expenses** | | | | |

| | | | | |
|---|---|---|---|---|
| Cost of revenues | 10,257 | 3,791 | 35,714 | 8,241 |
| Research and development | 47,180 | 33,807 | 166,294 | 124,185 |
| General and administrative | 17,571 | 14,450 | 63,817 | 51,815 |
| Provision for credit losses and other | 88 | 44 | 7,975 | (10) |
| Total operating expenses | 75,096 | 52,092 | 273,800 | 184,231 |
| Loss from operations | (53,651) | (40,324) | (119,233) | (148,998) |
| **Other Income** | | | | |
| Interest income from licensing | 130 | 860 | 4,271 | 2,951 |
| Investment income | 13,794 | 10,609 | 9,723 | 48,559 |
| Interest expense | (771) | — | (771) | — |
| Total other income | 13,153 | 11,469 | 13,223 | 51,510 |
| Loss before income taxes | (40,498) | (28,855) | (106,010) | (97,488) |
| **Income Tax Benefit (Expense)** | (5,743) | 2,391 | (5,240) | 2,755 |
| Net loss | $ (46,241) | $ (26,464) | $ (111,250) | $ (94,733) |
| **Other Comprehensive Income (Loss)** | | | | |
| Unrealized gain (loss) on available-for-sale securities, net | (623) | (158) | (565) | 885 |
| Total other comprehensive income (loss) | (623) | (158) | (565) | 885 |
| Comprehensive loss | $ (46,864) | $ (26,622) | $ (111,815) | $ (93,848) |
| | | | | |
| Net loss per share: | | | | |
| Basic | $ (1.24) | $ (0.72) | $ (2.98) | $ (2.58) |
| Diluted | $ (1.24) | $ (0.72) | $ (2.98) | $ (2.58) |
| Weighted-average common shares outstanding: | | | | |
| Basic | 37,418 | 36,905 | 37,281 | 36,690 |
| Diluted | 37,418 | 36,905 | 37,281 | 36,690 |

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