



## REGENXBIO Reports Third Quarter 2021 Financial Results and Operational Highlights

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- *Announced eye care collaboration with AbbVie to develop and commercialize RGX-314*
  - *AbbVie and REGENXBIO form a strategic partnership combining eye care and gene therapy expertise*
  - *REGENXBIO to receive \$370 million upfront payment*
  - *Transaction expected to close by end of 2021, subject to satisfaction of customary closing conditions, including applicable regulatory approvals*
- *Presented positive initial data from RGX-314 Phase II trials for the treatment of wet AMD and diabetic retinopathy using in-office suprachoroidal delivery*
  - *In AAVIATE<sup>®</sup> trial for the treatment of wet AMD, observed treatment effect with stable visual acuity and retinal thickness and meaningful reduction in anti-VEGF treatment burden*
  - *33% of patients in ALTITUDE<sup>™</sup> trial for the treatment of DR demonstrated a  $\geq 2$  step improvement from baseline on the ETDRS-DRSS*
- *On track to file IND for RGX-202 for the treatment of Duchenne by end of 2021*
- *Cohort 3 of the ongoing Phase I/II trial of RGX-121 for the treatment of patients up to 5 years old diagnosed with MPS II has been expanded to enroll up to 6 additional patients*
- *\$533.5 million in cash, cash equivalents and marketable securities as of September 30, 2021*
- *Conference call Tuesday, November 2<sup>nd</sup> at 4:30 p.m. ET*

REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the third quarter ended September 30, 2021, and recent operational highlights.

"We are encouraged by the clinical profile emerging from ongoing clinical trials evaluating the suprachoroidal delivery of RGX-314 for the treatment of wet AMD and diabetic retinopathy. Preliminary data highlight the potential of one-time, in-office delivery of RGX-314 which could provide sustainable, long-term anti-VEGF protein production in the eye for patients," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO.

"Moreover, our recently announced eye care collaboration with AbbVie, combining our AAV gene therapy expertise with AbbVie's global developmental and commercial infrastructure within eye care, will further support the broad potential of our RGX-314 program utilizing both subretinal and suprachoroidal delivery. We continue to expect this transaction to close by the end of this year."

Mr. Mills added: "For the remainder of 2021, we are focused on continuing to enroll patients in the ATMOSPHERE<sup>™</sup> trial and initiating our second pivotal trial for RGX-314 using subretinal delivery, sharing additional interim data from our AAVIATE<sup>®</sup> trial and filing an IND for RGX-202 for the treatment of Duchenne muscular dystrophy. This year has been transformative for REGENXBIO, and I am grateful to our dedicated employees for their commitment to our mission to develop therapeutics for diseases that have significant unmet needs."

### Eye Care Collaboration with AbbVie

- In September 2021, REGENXBIO announced a strategic partnership with AbbVie to develop and commercialize RGX-314 for the treatment of wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other chronic retinal diseases.
- Under the collaboration, REGENXBIO will be responsible for completion of the ongoing trials of RGX-314. AbbVie and REGENXBIO will collaborate and share costs on additional trials of RGX-314, including the planned second pivotal trial evaluating subretinal delivery for the treatment of wet AMD and future trials. AbbVie will lead the clinical development and commercialization of RGX-314 globally. REGENXBIO shall participate in U.S. commercialization efforts as provided under a mutually agreed upon commercialization plan.

- Under the terms of the agreement, AbbVie will pay REGENXBIO a \$370 million upfront payment, with the potential for REGENXBIO to receive up to \$1.38 billion in additional development, regulatory and commercial milestones. REGENXBIO and AbbVie will share equally in profits from net sales of RGX-314 in the U.S. AbbVie will pay REGENXBIO tiered royalties on net sales of RGX-314 outside the U.S. In addition, REGENXBIO will lead the manufacturing of RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead manufacturing of RGX-314 for commercial supply outside the U.S.
- The transaction is expected to close by the end of 2021, subject to the satisfaction of customary closing conditions, including applicable regulatory approvals.

#### Recent Operational Highlights

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

- Pivotal Program for RGX-314 for the Treatment of Wet AMD
  - Enrollment is ongoing in ATMOSPHERE™, the first of two planned pivotal trials to evaluate the efficacy and safety of RGX-314 in patients with wet AMD using the subretinal delivery approach.
  - REGENXBIO plans to initiate the second pivotal trial in the fourth quarter of 2021.
- Suprachoroidal Delivery of RGX-314 for the Treatment of Wet AMD
  - In October 2021, REGENXBIO presented positive initial data from patients enrolled in the ongoing Phase II AAVIATE® trial.
    - As of September 13, 2021, RGX-314 was reported to be well tolerated across 50 patients dosed in Cohorts 1 through 3.
    - At six months following one-time administration of RGX-314, stable visual acuity and retinal thickness, as well as a meaningful reduction in anti-VEGF treatment burden, was observed in patients from Cohort 1 (dose level:  $2.5 \times 10^{11}$  genomic copies per eye (GC/eye)).
    - Among patients in Cohort 1, common treatment emergent adverse events (TEAE) in the study eye were generally mild, and none were severe. Mild intraocular inflammation was observed in four out of 15 patients based on slit-lamp examination, and all cases were resolved within days to weeks on topical corticosteroids.
  - In October 2021, REGENXBIO announced that the AAVIATE® trial expanded to include two additional cohorts (Cohorts 4 and 5) to evaluate RGX-314 at a dose level of  $1.0 \times 10^{12}$  GC/eye. Cohort 4 will enroll 15 patients who will be dosed with RGX-314 and Cohort 5 will evaluate the same dose level of RGX-314 in 20 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.
  - REGENXBIO plans to report interim results at six months of follow-up for patients in Cohort 2 (dose level of  $5.0 \times 10^{11}$  GC/eye) at the American Academy of Ophthalmology 2021 Annual Meeting in New Orleans, LA, November 12-15, 2021.
- Suprachoroidal Delivery of RGX-314 for the Treatment of DR
  - In October 2021, REGENXBIO presented positive initial data from patients in Cohort 1 of the ongoing Phase II ALTITUDE™ trial.
    - As of September 29, 2021, RGX-314 was reported to be well tolerated with no drug-related serious adverse events in the 15 patients dosed with RGX-314 in Cohort 1 (dose level:  $2.5 \times 10^{11}$  GC/ eye). No intraocular inflammation was

observed on slit-lamp examination.

- Three months following one-time administration of RGX-314, five patients (33%) demonstrated a two-step or greater improvement from baseline on the Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (ETDRS-DRSS), including one patient who had a four-step improvement. None of the five patients in the observation control portion of the study demonstrated a two-step or greater improvement from baseline on the ETDRS-DRSS.
- Enrollment of patients in Cohorts 2 and 3 is ongoing. Both Cohorts will evaluate RGX-314 at a dose level of  $5.0 \times 10^{11}$  GC/eye. Cohort 2 will include 20 patients randomized to receive RGX-314 versus observational control at a 3:1 ratio. Cohort 3 will evaluate RGX-314 at the same dose level as Cohort 2 in 20 patients who are NAb positive. As in Cohort 1, patients in Cohorts 2 and 3 will not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.
- REGENXBIO continues to conduct research and preclinical studies to advance a gene therapy candidate for the treatment of Hereditary Angioedema (HAE).
- REGENXBIO and Neurimmune AG are collaborating on research studies to develop novel gene therapies for neurodegenerative diseases targeting both alpha synuclein and tau.

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

- RGX-202 for the Treatment of Duchenne muscular dystrophy (Duchenne)
  - REGENXBIO is on track to submit an Investigational New Drug application (IND) to the U.S. Food and Drug Administration (FDA) for RGX-202 for the treatment of Duchenne by the end of 2021.
- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
  - REGENXBIO continues to enroll patients in the ongoing Phase I/II trial of RGX-121 for the treatment of patients up to 5 years old diagnosed with MPS II. REGENXBIO today announced that Cohort 3 has been expanded to enroll up to six additional patients.
    - Additional data from this trial is expected to be reported in the first half of 2022.
  - Enrollment continues in the Phase I/II trial of RGX-121 for the treatment of pediatric patients with MPS II over the age of 5 years old.
- RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)
  - Enrollment is ongoing in Cohort 2 of the Phase I/II trial of RGX-111 for the treatment of MPS I.
  - REGENXBIO expects to share initial data from this trial in the first half of 2022.
- Programs for the Treatment of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease
  - REGENXBIO is continuing to evaluate the path forward for RGX-181 for the treatment of CLN2 disease and plans to provide a program update in 2022.
  - REGENXBIO is conducting additional preclinical studies of RGX-381 for the treatment of ocular manifestations of CLN2 disease and is in discussions with regulatory agencies. REGENXBIO plans to provide a program update in 2022.

#### *Operational Updates*

- Current Good Manufacturing Practice (cGMP) Manufacturing Facility
  - REGENXBIO has begun utilizing its new headquarters in Rockville, Maryland. The

headquarters include a 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 and is on track to be fully operational starting in the first half of 2022.

- To further REGENXBIO's mission to improve lives through the curative potential of gene therapy, the Company has supported the founding of two important consortia.
  - In November 2021, REGENXBIO, in collaboration with Solid Biosciences Inc., announced the launch of the Pathway Development Consortium (PDC), a multistakeholder initiative which aims to identify, develop, expand and maintain pathways to effective therapies for patients diagnosed early in life with rare diseases. The PDC seeks to achieve these goals by bringing together a broad and diverse group of stakeholders from the rare disease and AAV gene therapy communities, including patients, industry, regulators, academia and payers, among others, for meaningful scientific and policy discussions.
  - In October 2021, REGENXBIO joined industry, NIH, FDA and not-for-profit partners to form the Accelerating Medicines Partnership Bespoke Gene Therapy Consortium, a public-private collaboration, which aims to accelerate the delivery of promising new gene therapies to patients with rare diseases that currently lack effective treatments. The consortium will support a series of research projects and clinical trials of new gene therapies to be conducted by the NIH. These projects will create new tools and resources for AAV clinical development and regulatory evaluation of future AAV therapies.

#### **NAV Technology Licensee Program Highlights**

As of September 30, 2021, REGENXBIO's NAV<sup>®</sup> 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 August 2021, Novartis announced plans to initiate STEER, a global pivotal Phase 3 registration-enabling study of OAV-101 administered intrathecally in treatment-naïve patients with spinal muscular atrophy (SMA) Type 2 aged between 2 and 18 years old. OAV-101 is being developed as a one-time gene therapy utilizing REGENXBIO's AAV9 vector.
- In October 2021, Ultragenyx announced that it has successfully screened and enrolled multiple patients with Wilson disease into the baseline monitoring period prior to dosing in its pivotal, seamless Phase 1/2/3 study of UX701 for the treatment of Wilson disease. UX701 is being developed as a one-time therapy utilizing REGENXBIO's AAV9 vector.

#### **Marketed NAV Technology Products**

REGENXBIO's NAV Technology Platform is being applied in one marketed product, Zolgensma<sup>®</sup>. On October 26, 2021, Novartis AG reported third quarter 2021 global Zolgensma sales revenue of \$375 million.

#### **Financial Results**

**Cash Position:** Cash, cash equivalents and marketable securities were \$533.5 million as of September 30, 2021, compared to \$522.5 million as of December 31, 2020. The increase was primarily attributable to \$216.1 million of aggregate net proceeds received from the Company's follow-on public offering of common stock completed in January 2021, and was partially offset by net cash used in operating activities of \$107.4 million, cash used to purchase property and equipment of \$69.6 million, and Zolgensma royalties paid to Healthcare Royalty Management, LLC of \$33.3 million during the nine months ended September 30, 2021.

**Revenues:** Revenues were \$30.8 million for the three months ended September 30, 2021, compared to \$98.9 million for the three months ended September 30, 2020. The decrease was primarily attributable to an \$80.0 million milestone fee recognized as revenue in the third quarter of 2020 upon the achievement of \$1.0 billion of cumulative net sales of Zolgensma, and was partially offset by an increase in Zolgensma royalty revenues, which increased by \$11.5 million, from \$18.8 million for the third quarter of 2020 to \$30.3 million for the third quarter of 2021. As reported by Novartis, sales of Zolgensma for the third quarter of 2021 increased by 29% as compared to the third quarter of 2020, driven by geographic expansion of product access.

**Research and Development Expenses:** Research and development expenses were \$47.9 million for the three months ended September 30, 2021, compared to \$44.0 million for the three months ended September 30, 2020. The increase was primarily attributable to personnel costs as a result of increased headcount, as well as laboratory and facilities costs.

**General and Administrative Expenses:** General and administrative expenses were \$21.0 million for the three months ended September 30, 2021, compared to \$15.9 million for the three months ended September 30, 2020. The increase was primarily attributable to personnel costs as a result of increased headcount and professional fees for advisory and other services

**Net Loss:** Net loss was \$58.4 million, or \$1.37 basic and diluted net loss per share, for the three months ended September 30, 2021, compared to net income of \$8.8 million, or \$0.24 basic and \$0.23 diluted net income per share, for the three months ended September 30, 2020.

## Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of \$533.5 million as of September 30, 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, excluding the effect of any potential payments that may be received under REGENXBIO's collaboration with AbbVie, which is expected to close by the end of 2021, subject to the satisfaction of customary closing conditions, including applicable regulatory approvals.

## 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 7878814. To access a live or recorded webcast of the call, please visit the "Investors" section of the REGENXBIO website at [www.regenxbio.com](http://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<sup>®</sup> 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 proposed collaboration with AbbVie and 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 anticipated completion of REGENXBIO's proposed transaction with AbbVie, the outcome of REGENXBIO's proposed collaboration with AbbVie, 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, 2020 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](http://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<sup>®</sup> 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)

	<u>September 30, 2021</u>	<u>December 31, 2020</u>
<b>Assets</b>		
Current assets		
Cash and cash equivalents	\$ 228,387	\$ 338,426
Marketable securities	111,473	137,314
Accounts receivable, net	46,017	42,999
Prepaid expenses	18,401	10,505
Other current assets	5,886	1,953
Total current assets	410,164	531,197
Marketable securities	193,640	46,809
Accounts receivable, net	2,730	3,267
Property and equipment, net	122,231	56,467
Operating lease right-of-use assets	61,742	63,815
Restricted cash	1,330	1,330

Other assets	8,558	5,279
Total assets	<u>\$ 800,395</u>	<u>\$ 708,164</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities		
Accounts payable	\$ 19,845	\$ 10,622
Accrued expenses and other current liabilities	49,694	49,082
Deferred revenue	395	449
Operating lease liabilities	1,329	2,500
Liability related to sale of future royalties	<u>35,508</u>	<u>18,794</u>
Total current liabilities	106,771	81,447
Deferred revenue	3,531	3,783
Operating lease liabilities	83,880	70,153
Liability related to sale of future royalties	144,315	174,504
Other liabilities	<u>484</u>	<u>524</u>
Total liabilities	338,981	330,411
Stockholders' equity		
Preferred stock; no shares issued and outstanding at September 30, 2021 and December 31, 2020	—	—
Common stock; 42,752 and 37,476 shares issued and outstanding at September 30, 2021 and December 31, 2020, respectively	4	4
Additional paid-in capital	917,950	667,181
Accumulated other comprehensive loss	(1,285)	(360)
Accumulated deficit	<u>(455,255)</u>	<u>(289,072)</u>
Total stockholders' equity	461,414	377,753
Total liabilities and stockholders' equity	<u>\$ 800,395</u>	<u>\$ 708,164</u>

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

	Three Months		Nine Months	
	Ended September 30,		Ended September 30,	
	2021	2020	2021	2020
<b>Revenues</b>				
License and royalty revenue	\$ 30,773	\$ 98,912	\$ 71,692	\$ 133,122
Total revenues	30,773	98,912	71,692	133,122
<b>Operating Expenses</b>				
Cost of revenues	14,105	17,364	28,775	25,457
Research and development	47,855	43,968	133,459	119,114
General and administrative	21,030	15,859	57,293	46,246
Provision for credit losses and other	5,131	7,770	5,781	7,887
Total operating expenses	<u>88,121</u>	<u>84,961</u>	<u>225,308</u>	<u>198,704</u>
Income (loss) from operations	(57,348)	13,951	(153,616)	(65,582)
<b>Other Income (Expense)</b>				
Interest income from licensing	117	1,444	700	4,141
Investment income (loss)	5,535	(6,607)	6,514	(4,071)
Interest expense	(6,709)	—	(19,777)	—
Total other income (expense)	<u>(1,057)</u>	<u>(5,163)</u>	<u>(12,563)</u>	<u>70</u>
Income (loss) before income taxes	(58,405)	8,788	(166,179)	(65,512)
<b>Income Tax Benefit (Expense)</b>				
Net income (loss)	<u>\$ (58,405)</u>	<u>\$ 8,791</u>	<u>\$ (166,183)</u>	<u>\$ (65,009)</u>
<b>Other Comprehensive Income (Loss)</b>				
Unrealized gain (loss) on available-for-sale securities, net	(30)	(487)	(925)	58
Total other comprehensive income (loss)	<u>(30)</u>	<u>(487)</u>	<u>(925)</u>	<u>58</u>
Comprehensive income (loss)	<u>\$ (58,435)</u>	<u>\$ 8,304</u>	<u>\$ (167,108)</u>	<u>\$ (64,951)</u>
<b>Net income (loss) per share:</b>				
Basic	\$ (1.37)	\$ 0.24	\$ (3.93)	\$ (1.75)
Diluted	\$ (1.37)	\$ 0.23	\$ (3.93)	\$ (1.75)
<b>Weighted-average common shares outstanding:</b>				
Basic	42,629	37,342	42,324	37,234
Diluted	42,629	38,877	42,324	37,234

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