



REGENXBIO Reports Third Quarter 2020 Financial Results and Operational Highlights

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- **RGX-314 subretinal pivotal wet AMD trial expected to initiate in Q1 2021**
- **Enrollment continues in RGX-314 suprachoroidal Phase II AAVIATE study; initial safety data expected in early 2021**
- **RGX-314 suprachoroidal Phase II ALTITUDE trial is active and expects to initiate enrollment by end of 2020; initial data expected in 2021**
- **Additional patients enrolled in expanded Phase I/II study of RGX-121 for the treatment of MPS II; further updates from ongoing trial anticipated by end of 2020**
- **\$80 million milestone payment from Novartis received in October 2020**
- **\$290 million in cash, cash equivalents and marketable securities as of September 30, 2020**
- **Q3 earnings conference call Wednesday, November 4 at 4:30 p.m. ET**

ROCKVILLE, Md., Nov. 4, 2020 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV[®] Technology Platform, today announced financial results for the quarter ended September 30, 2020, and recent operational highlights.

"We have made several important advancements in our clinical pipeline during the third quarter of 2020, with continued focus on execution during the COVID-19 pandemic. I'm pleased that two Phase II trials are underway for the treatment of wet AMD and diabetic retinopathy using the suprachoroidal approach for the delivery of RGX-314. In addition, we have expanded our RGX-121 program for patients with MPS II to gain additional insight into the potential treatment effects of this one-time gene therapy candidate in more patients, and we have dosed additional patients in the second cohort of the ongoing Phase I/II trial. I look forward to providing additional updates on all of our clinical programs in 2021, as we continue to focus on the significant unmet medical needs in both large indications and rare diseases," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO.

"The overall progress of gene therapy treatments continues to be encouraging, as we see additional patients around the world treated with Zolgensma[®], the first approved gene therapy based on REGENXBIO's NAV Technology Platform," continued Mr. Mills. "We maintain our focus on our important relationships with partners and licensees in the gene therapy space, with a strong focus on patient needs and innovative approaches to treat disease."

Recent Operational Highlights

Gene Therapy Using NAV Vectors for AAV-Mediated Antibody Delivery

- **RGX-314 for the Treatment of Wet Age-related Macular Degeneration (wet AMD)**
 - REGENXBIO expects to initiate the pivotal program for the subretinal delivery of RGX-314 for the treatment of wet AMD in the first quarter of 2021.
 - On September 9, 2020, REGENXBIO announced that the first patient had been dosed in the Phase II AAVIATE trial to evaluate the suprachoroidal delivery of RGX-314 using the SCS Microinjector[®] for the treatment of wet AMD. REGENXBIO expects to complete enrollment of the first cohort by the end of 2020, and report initial safety data from the first cohort in early 2021.
- **RGX-314 for the Treatment of Diabetic Retinopathy (DR)**
 - The Phase II trial, ALTITUDE, to evaluate the targeted, in-office suprachoroidal delivery of RGX-314 in patients with DR is active and REGENXBIO expects to begin enrolling

patients by the end of 2020. REGENXBIO plans to report interim data from this trial 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 REGENXBIO expects to provide program updates in 2021.

Gene Therapy Using NAV Vectors for Rare Genetic Diseases

- RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)
 - Eight patients have now been dosed across two dose cohorts in the ongoing Phase I/II trial of RGX-121 in severe MPS II patients under the age of 5 years old. The first two patients in the expanded Cohort 2 were dosed in October 2020, via intracisternal delivery of RGX-121 at a dose of 6.5×10^{10} genome copies per gram (GC/g) of brain mass. REGENXBIO anticipates further updates from this trial by the end of 2020.
 - On September 30, 2020, REGENXBIO announced the expansion of the RGX-121 program for MPS II to gain additional insight into the neurodegenerative manifestations of the disease and evaluate RGX-121 in a broader patient population:
 - REGENXBIO plans to begin a second Phase I/II multicenter, open-label trial of RGX-121 for the treatment of pediatric patients with severe MPS II over the age of 5 years old. Up to six patients will be enrolled, and RGX-121 will be administered at a dose level of 6.5×10^{10} GC/g of brain mass.
 - REGENXBIO also announced a new prospective observational study designed to provide detailed characterization of neurocognitive development and key biomarkers in patients with severe MPS II.
- RGX-111 for the Treatment of Mucopolysaccharidosis Type I (MPS I)
 - Recruitment and patient screening are ongoing in REGENXBIO's Phase I/II clinical trial evaluating RGX-111 for the treatment of MPS I.
 - REGENXBIO expects to provide a program update by the end of 2020.
- RGX-381 for the Treatment of Ocular Manifestations of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease
 - REGENXBIO is on track to submit an Investigational New Drug (IND) application for a Phase I/II study of RGX-381 in patients with CLN2 disease by the end of 2020 and plans to initiate enrollment in the first half of 2021.
 - Data from non-human primates demonstrate elevated and sustained levels of TPP1, the enzyme deficient in patients with CLN2 disease, in the vitreous following a single subretinal injection of RGX-381.
- 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.
- Research Program for the Treatment of Neuromuscular Disorders
 - REGENXBIO expects to announce a program update in 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 September 30, 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.

REGENXBIO recently collaborated with Kurma Partners, a leading European venture capital firm, to form Corlieve Therapeutics, a new company focused on severe neurological conditions. Under the collaboration and license agreement with Corlieve, REGENXBIO has licensed Corlieve rights to the NAV AAV9 vector for the treatment of refractory temporal lobe epilepsy. In return for these rights, REGENXBIO received equity in Corlieve and is eligible to receive milestone payments and royalties. In addition, REGENXBIO's Chief Scientific Officer, Olivier Danos, Ph.D., has joined Corlieve's Board of Directors. Idivest Partners and Pureos Bioventures are also investors in Corlieve.

Marketed NAV Technology Product Highlights

REGENXBIO's NAV Technology Platform is being applied in one marketed product, Zolgensma. On October 27, 2020, Novartis AG reported third quarter 2020 global Zolgensma sales revenue of \$291 million, and REGENXBIO recognized royalty revenue of \$18.8 million in the third quarter of 2020 as a result of these sales. Novartis AG has recorded over \$1.0 billion in cumulative net sales of Zolgensma since launch in 2019.

Financial Results

Cash Position: Cash, cash equivalents and marketable securities were \$289.8 million as of September 30, 2020, compared to \$400.0 million as of December 31, 2019. The decrease was primarily attributable to net cash used in operating activities of \$93.5 million and cash used to purchase property and equipment of \$14.0 million. Cash, cash equivalents and marketable securities reported as of September 30, 2020 excludes the \$80.0 million milestone payment received from Novartis AG in October 2020, which was recorded as accounts receivable as of September 30, 2020.

Revenues: Revenues were \$98.9 million for the three months ended September 30, 2020, compared to \$14.7 million for the three months ended September 30, 2019. The increase was primarily attributable to a \$9.6 million increase in Zolgensma royalty revenue for the third quarter of 2020 as compared to the third quarter of 2019, as well as 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. Net sales of Zolgensma for the third quarter of 2020 increased by 82% as compared to the third quarter of 2019.

Research and Development Expenses: Research and development expenses were \$44.0 million for the three months ended September 30, 2020, compared to \$35.7 million for the three months ended September 30, 2019. The increase was primarily attributable to personnel-related costs as a result of increased headcount, expenses associated with conducting clinical trials for our lead product candidates, and externally sourced services for preclinical, regulatory and manufacturing-related activities.

General and Administrative Expenses: General and administrative expenses were \$15.9 million for the three months ended September 30, 2020, compared to \$12.4 million for the three months ended September 30, 2019. The increase was primarily attributable to personnel-related costs as a result of increased headcount, and professional fees for advisory and other services.

Net Income (Loss): Net income was \$8.8 million, or \$0.24 basic and \$0.23 diluted net income per share, for the three months ended September 30, 2020, compared to net loss of \$34.6 million, or \$0.94 basic and diluted net loss per share, for the three months ended September 30, 2019. Net income for the three months ended September 30, 2020 includes a non-cash charge of \$7.7 million for a provision for credit losses recognized related to accounts receivable during the period.

Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of \$289.8 million as of September 30, 2020, in addition to the \$80.0 million received from Novartis AG in October 2020, to fund its operations, including the completion of its internal manufacturing capabilities and clinical advancement of its product candidates, until mid-2022.

Q3 Earnings 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 4107819. 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," "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, 2019, 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. 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.

SCS Microinjector[®] is a registered trademark of Clearside Biomedical, Inc. 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)

	September 30, December 31,	
	2020	2019
Assets		
Current assets		
Cash and cash equivalents	\$ 93,220	\$ 69,514
Marketable securities	148,305	226,696
Accounts receivable, net	122,116	38,148
Prepaid expenses	16,112	6,475
Other current assets	7,197	4,199
Total current assets	386,950	345,032
Marketable securities	48,272	103,785
Accounts receivable, net	3,564	4,155
Property and equipment, net	38,871	28,973
Operating lease right-of-use assets	57,827	10,078
Restricted cash	1,330	1,330
Other assets	4,360	4,555
Total assets	\$ 541,174	\$ 497,908
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 15,244	\$ 6,409
Accrued expenses and other current liabilities	44,356	24,846
Deferred revenue	449	—
Operating lease liabilities	3,564	2,421
Total current liabilities	63,613	33,676
Deferred revenue	3,895	3,333
Operating lease liabilities	57,461	8,874
Other liabilities	545	1,828
Total liabilities	125,514	47,711
Stockholders' equity		
Preferred stock; \$0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at September 30, 2020 and December 31, 2019	—	—
Common stock; \$0.0001 par value; 100,000 shares authorized at September 30, 2020 and December 31, 2019; 37,404 and 36,992 shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively	4	4
Additional paid-in capital	658,224	627,810
Accumulated other comprehensive income	263	205
Accumulated deficit	(242,831)	(177,822)
Total stockholders' equity	415,660	450,197
Total liabilities and stockholders' equity	\$ 541,174	\$ 497,908

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,	
	2020	2019	2020	2019
Revenues				
License and royalty revenue	\$ 98,912	\$ 14,700	\$ 133,122	\$ 23,465
Total revenues	98,912	14,700	133,122	23,465
Operating Expenses				
Cost of revenues	17,364	2,494	25,457	4,450
Research and development	43,968	35,692	119,114	90,378
General and administrative	15,859	12,402	46,246	37,365
Provision for credit losses and other	7,770	8	7,887	(54)
Total operating expenses	84,961	50,596	198,704	132,139
Income (loss) from operations	13,951	(35,896)	(65,582)	(108,674)
Other Income (Loss)				
Interest income from licensing	1,444	716	4,141	2,091
Investment income (loss)	(6,607)	431	(4,071)	37,950
Total other income (loss)	(5,163)	1,147	70	40,041
Income (loss) before income taxes	8,788	(34,749)	(65,512)	(68,633)
Income Tax Benefit				
	3	165	503	364
Net income (loss)	\$ 8,791	\$ (34,584)	\$ (65,009)	\$ (68,269)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	(487)	(108)	58	1,043
Total other comprehensive income (loss)	(487)	(108)	58	1,043
Comprehensive income (loss)	\$ 8,304	\$ (34,692)	\$ (64,951)	\$ (67,226)
Net income (loss) per share:				
Basic	\$ 0.24	\$ (0.94)	\$ (1.75)	\$ (1.86)
Diluted	\$ 0.23	\$ (0.94)	\$ (1.75)	\$ (1.86)
Weighted-average common shares outstanding:				
Basic	37,342	36,813	37,234	36,618
Diluted	38,877	36,813	37,234	36,618

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