



REGENXBIO Reports Second Quarter 2021 Financial Results and Operational Highlights

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- *Enrollment in RGX-314 programs is on-track, including the pivotal program for the treatment of wet AMD utilizing subretinal delivery, and the Phase II trials for the treatment of wet AMD and DR utilizing in-office suprachoroidal delivery*
- *Interim data from RGX-314 Phase II trial for the treatment of wet AMD utilizing in-office suprachoroidal delivery will be presented at Retina Society 54th Annual Scientific Meeting*
- *Reported additional positive interim data in May 2021 from RGX-121 Phase I/II trial for the treatment of patients up to 5 years old with MPS II; enrollment continues in Cohort 3 at increased dose*
- *Expects to file IND for RGX-202 for the treatment of Duchenne by end of 2021*
- *\$593.0 million in cash, cash equivalents and marketable securities as of June 30, 2021*
- *Conference call Monday, August 9th at 4:30 p.m. ET*

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

"We are well positioned to expand upon the progress we have made in our gene therapy programs over the first half of 2021, including clinical advancements in our RGX-314 programs for the treatment of wet AMD and diabetic retinopathy," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Earlier this year, we reported additional positive interim data from Cohorts 1 and 2 of our Phase I/II trial of RGX-121 in patients up to 5 years old with MPS II, which included safety, biomarker data, and emerging evidence of systemic enzyme expression and activity in urine and plasma. Patient enrollment in this program continues. In addition, we expect to submit an IND for RGX-202 for the treatment of patients with Duchenne by the end of 2021."

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)
 - 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 is on-track to initiate the second pivotal trial in the fourth quarter of 2021.
- Suprachoroidal Delivery of RGX-314 for the Treatment of Wet AMD
 - Interim data from Cohort 1 (dose level: 2.5×10^{11} genome copies per eye (gc/eye)) of AAVIATE®, a Phase II trial of RGX-314 for the treatment of wet AMD, will be presented by a trial investigator at the Retina Society 54th Annual Scientific Meeting in Chicago, IL, September 29-October 2, 2021.
 - REGENXBIO expects to report interim data from Cohort 2 (5.0×10^{11} gc/eye) in the fourth quarter of 2021.
 - Patient dosing in Cohort 3 (5.0×10^{11} gc/eye) is complete.
- Suprachoroidal Delivery of RGX-314 for the Treatment of Diabetic Retinopathy (DR)
 - REGENXBIO has completed enrollment of patients in Cohort 1 (2.5×10^{11} gc/eye) in ALTITUDE™, a Phase II trial for the treatment of DR, and expects to report initial data in

the fourth quarter of 2021.

- Enrollment of patients in Cohort 2 (5.0×10^{11} gc/eye) has begun.
- In addition, similar to the AAVIATE trial, REGENXBIO has expanded ALTITUDE and plans to enroll patients in a third cohort.
 - Cohort 3 will evaluate the efficacy, safety and tolerability of RGX-314 in up to 20 patients who are neutralizing antibody positive.
 - The same dose evaluated in Cohort 2 will be delivered to patients in Cohort 3 (5.0×10^{11} gc/eye) and, as in previous cohorts, patients will not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.
- Research Program for the Treatment of Hereditary Angioedema
 - REGENXBIO expects to provide a program update by the end of 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 by the end of 2021.

Gene Therapy Using NAV Vectors for Rare Genetic Diseases

- RGX-202 for the Treatment of Duchenne Muscular Dystrophy
 - REGENXBIO expects 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)
 - In May 2021, REGENXBIO reported additional positive interim data from the ongoing Phase I/II trial of RGX-121 for the treatment of patients up to 5 years old diagnosed with MPS II. REGENXBIO continues to enroll patients in Cohort 3 at an increased dose of 2.0×10^{11} GC/g brain mass.
 - 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 at an increased dose of 5.0×10^{10} GC/g brain mass.
- RGX-181 for the Treatment of Late-infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease
 - REGENXBIO plans to provide a program update by the end of 2021.
- RGX-381 for the Treatment of Ocular Manifestations of CLN2 Disease
 - REGENXBIO plans to provide a program update by the end of 2021.

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.

NAV Technology Licensee Program Highlights

As of June 30, 2021, REGENXBIO's NAV® Technology Platform was being applied in one marketed product, and multiple clinical stage programs, with 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 May 2021, Ultragenyx Pharmaceutical Inc. reported positive multi-year durability data from its Phase I/II trials of DTX301 for the treatment of Ornithine Transcarbamylase Deficiency and DTX401 for the treatment of Glycogen Storage Disease Type Ia, both of which use REGENXBIO's AAV8 vector. Ultragenyx plans to dose the first patient in the Phase 3 studies for both programs in the second half of 2021.
- In June 2021, Corlieve Therapeutics announced it had entered into a definitive agreement for uniQure N.V. to acquire Corlieve and its lead program for the treatment of temporal lobe epilepsy, which utilizes REGENXBIO's AAV9 vector and will be known as AMT-260. The transaction closed in July 2021. Under the license and collaboration agreement, REGENXBIO received equity in Corlieve and is eligible to receive milestones, as well as royalties on net sales of AMT-260. As a result of the acquisition, REGENXBIO receives a portion of the €46.3 million in upfront cash uniQure paid to acquire Corlieve, and is eligible to receive a portion of the €203.7 million in additional potential milestones that may be paid to Corlieve shareholders by uniQure.

Marketed NAV Technology Products

REGENXBIO's NAV Technology Platform is being applied in one marketed product, Zolgensma®. On July 21, 2021, Novartis AG reported second quarter 2021 global Zolgensma sales revenue of \$315 million.

Financial Results

Cash Position: Cash, cash equivalents and marketable securities were \$593.0 million as of June 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, including the full exercise of the underwriters' option to purchase additional shares in connection with the offering. The increase was partially offset by net cash used in operating activities of \$71.0 million, cash used to purchase property and equipment of \$50.9 million, and Zolgensma royalties paid to Healthcare Royalty Management, LLC of \$22.0 million during the six months ended June 30, 2021.

Revenues: Revenues were \$22.0 million for the three months ended June 30, 2021, compared to \$16.6 million for the three months ended June 30, 2020. The increase was primarily attributable to Zolgensma royalty revenues, which increased by \$6.5 million, from \$11.9 million for the second quarter of 2020 to \$18.4 million for the second quarter of 2021. As reported by Novartis, sales of Zolgensma for the second quarter of 2021 increased by 54% as compared to the second quarter of 2020, driven by geographic expansion of product access.

Research and Development Expenses: Research and development expenses were \$45.9 million for the three months ended June 30, 2021, compared to \$38.1 million for the three months ended June 30, 2020. The increase was primarily attributable to clinical trial expenses for our lead product candidates, personnel costs as a result of increased headcount, laboratory and facilities costs, preclinical studies and other early-stage research and development activities.

General and Administrative Expenses: General and administrative expenses were \$18.4 million for the three months ended June 30, 2021, compared to \$15.6 million for the three months ended June 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 \$57.6 million, or \$1.36 basic and diluted net loss per share, for the three months ended June 30, 2021, compared to net loss of \$33.8 million, or \$0.91 basic and diluted net loss per share, for the three months ended June 30, 2020.

Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of \$593.0 million as of June 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.

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 8655773. 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, 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. 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)

	<u>June 30, 2021</u>	<u>December 31, 2020</u>
Assets		
Current assets		
Cash and cash equivalents	\$ 257,072	\$ 338,426
Marketable securities	117,665	137,314
Accounts receivable, net	44,394	42,999
Prepaid expenses	13,092	10,505
Other current assets	5,164	1,953
Total current assets	437,387	531,197
Marketable securities	218,220	46,809
Accounts receivable, net	2,808	3,267
Property and equipment, net	106,685	56,467
Operating lease right-of-use assets	62,280	63,815
Restricted cash	1,330	1,330
Other assets	7,692	5,279
Total assets	<u>\$ 836,402</u>	<u>\$ 708,164</u>
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 9,354	\$ 10,622
Accrued expenses and other current liabilities	46,761	49,082
Deferred revenue	395	449
Operating lease liabilities	1,709	2,500
Liability related to sale of future royalties	33,335	18,794
Total current liabilities	91,554	81,447
Deferred revenue	3,630	3,783
Operating lease liabilities	82,383	70,153
Liability related to sale of future royalties	151,076	174,504
Other liabilities	514	524
Total liabilities	329,157	330,411
Stockholders' equity		
Preferred stock; no shares issued and outstanding at June 30, 2021 and December 31, 2020	—	—
Common stock; 42,555 and 37,476 shares issued and outstanding at June 30, 2021 and December 31, 2020, respectively	4	4
Additional paid-in capital	905,346	667,181
Accumulated other comprehensive loss	(1,255)	(360)
Accumulated deficit	(396,850)	(289,072)
Total stockholders' equity	507,245	377,753
Total liabilities and stockholders' equity	<u>\$ 836,402</u>	<u>\$ 708,164</u>

REGENXBIO INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited)
(in thousands, except per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Revenues				
License and royalty revenue	\$ 22,035	\$ 16,566	\$ 40,919	\$ 34,210
Total revenues	22,035	16,566	40,919	34,210
Operating Expenses				
Cost of revenues	9,819	4,684	14,670	8,093
Research and development	45,882	38,111	85,604	75,146
General and administrative	18,425	15,554	36,263	30,387
Provision for credit losses and other	135	50	650	117
Total operating expenses	74,261	58,399	137,187	113,743
Loss from operations	(52,226)	(41,833)	(96,268)	(79,533)
Other Income (Expense)				
Interest income from licensing	554	1,849	583	2,697
Investment income	399	5,722	979	2,536
Interest expense	(6,366)	—	(13,068)	—
Total other income (expense)	(5,413)	7,571	(11,506)	5,233
Loss before income taxes	(57,639)	(34,262)	(107,774)	(74,300)
Income Tax Benefit (Expense)				
Net loss	\$ (57,639)	\$ (33,762)	\$ (107,778)	\$ (73,800)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	113	1,330	(895)	545
Total other comprehensive income (loss)	113	1,330	(895)	545
Comprehensive loss	\$ (57,526)	\$ (32,432)	\$ (108,673)	\$ (73,255)
Net loss per share, basic and diluted	\$ (1.36)	\$ (0.91)	\$ (2.56)	\$ (1.98)
Weighted-average common shares outstanding, basic and diluted	42,510	37,257	42,170	37,180

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