REGENXBIO Reports Second Quarter 2022 Financial Results and Recent Operational Highlights

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- *RGX-314 program for the treatment of wet AMD and diabetic retinopathy, being developed in collaboration with AbbVie, remains on track for first BLA filing in 2024*
  - *Enrollment ongoing in the pivotal ATMOSPHERE® and ASCENT™ clinical trials of RGX-314 for the treatment of wet AMD using subretinal delivery*
  - *Completed enrollment in Cohort 5 of the AAVIATE® trial of RGX-314 for the treatment of wet AMD using suprachoroidal delivery*
  - *Completed enrollment in ALTITUDE® trial of RGX-314 for the treatment of diabetic retinopathy using suprachoroidal delivery*
- *AFFINITY DUCHENNE™ Phase I/II trial of RGX-202 remains on track for dosing in the first half of 2023*
- *Announced intention to file a BLA in 2024 using the accelerated approval pathway for RGX-121 for the treatment of MPS II; pivotal program is active and enrolling patients*
- *$682 million in cash, cash equivalents and marketable securities of as of June 30, 2022; operational runway into 2025*
- *Conference call Wednesday, August 3rd at 4:30 p.m. ET*

ROCKVILLE, Md., Aug. 3, 2022 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced financial results for the second quarter ended June 30, 2022, and recent operational highlights.

"Our '5x'25' strategy to have five gene therapies either on the market or in late-stage development by 2025 is progressing well," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Earlier today, we announced that the pivotal program for RGX-121, our AAV Therapeutic for the treatment of MPS II, is active and enrolling patients, making this our second program to enter this stage of development. We believe RGX-121 will advance rapidly through the clinic, supporting our intention to file a BLA in 2024 using the accelerated approval pathway. RGX-314, being developed in collaboration with AbbVie, is also progressing well. We have two ongoing pivotal trials evaluating RGX-314 for the treatment of wet AMD and remain on track for a BLA filing in 2024. As previously announced, we also have progressed our Phase II trials evaluating suprachoroidal delivery of RGX-314. With our significant progress over the first half of the year along with a strong balance sheet to support the advancement of our leading AAV-gene therapy pipeline, we reman confident in our ability to deliver meaningful therapies to patients."

Program Highlights and Milestones

RGX-314: RGX-314 is an investigational one-time AAV Therapeutic being developed in collaboration with AbbVie 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® and ASCENT™, two pivotal clinical trials to evaluate the efficacy and safety of RGX-314 in patients with wet AMD using the subretinal delivery approach. 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
  - Completed enrollment of Cohort 5 of the Phase II AAVIATE® trial of RGX-314 for the treatment of wet AMD, which is evaluating RGX-314 at a third dose level of $1x10^{12}$

genomic copies per eye (GC/eye) in patients who are neutralizing antibody (NAb) positive. As in previous cohorts, patients did not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.

- RGX-314 Suprachoroidal Delivery for the Treatment of DR
  - Enrollment is complete in the Phase II ALTITUDE® trial for the treatment of DR. Cohorts 2 and 3 are evaluating RGX-314 at an increased dose level of $5x10^{11}$ GC/eye, with Cohort 3 evaluating RGX-314 in patients who are NAb positive. As in Cohort 1, patients did not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.

RGX-202: RGX-202 is an investigational one-time AAV Therapeutic 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.

- Preparation for the initiation of the AFFINITY DUCHENNE™ Phase I/II trial continues, including readying clinical trial sites and manufacturing additional clinical supply for the trial.
- REGENXBIO anticipates dosing the first patient in this trial in the first half of 2023.

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

- REGENXBIO has announced that, following discussions with the FDA, it intends to file a BLA in 2024 using the accelerated approval pathway for RGX-121 for the treatment of MPS II.
  - The ongoing Phase I/II trial of RGX-121 in children up to five years old has been expanded into a pivotal Phase I/II/III trial, called CAMPSIITE™.
  - CAMPSIITE, a multicenter, open-label trial, is active and enrolling patients. The trial is expected to enroll up to 10 MPS II patients using commercial-scale cGMP material to support the BLA filing, with the potential to enroll additional patients.
  - Glycosaminoglycans (GAGs) in the cerebrospinal fluid (CSF) have the potential to be considered a surrogate biomarker that is reasonably likely to predict clinical benefit in MPS II disease under the accelerated approval pathway, as buildup of GAGs in the CSF of MPS II patients correlates with clinical manifestations, including neurodevelopmental deficits.
- The Phase I/II trial of RGX-121 for the treatment of pediatric patients with MPS II over the age of five years old is also ongoing.

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

- REGENXBIO continues with plans to enroll additional patients in a Cohort 2 expansion arm of the Phase I/II trial.

Operational Updates

- Opened state-of-the-art gene therapy manufacturing facility
  - REGENXBIO's cGMP facility, called the REGENXBIO Manufacturing Innovation Center, is designed to meet global clinical and commercial regulatory standards and enable the Company to efficiently advance its AAV-based gene therapy pipeline from research and early development to clinical programs to commercial readiness.
  - REGENXBIO is one of only a few gene therapy companies worldwide with a GMP facility capable of production at scales up to 2,000 liters.

NAV Technology Licensee Program Highlights

As of June 30, 2022, 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.

- Zolgensma®, a one-time AAV Therapeutic for the treatment of spinal muscular atrophy (SMA), is a marketed product utilizing REGENXBIO's NAV AAV9 vector. In July 2022, Novartis AG reported second quarter global sales of Zolgensma of $379 million (>2,300 patients treated worldwide.)
- In May 2022, Rocket Pharmaceuticals, Inc. announced RP-A501 for the treatment of Danon disease was well-tolerated in both patients from the low-dose (6.7 x 1013GC/kg; n=2) pediatric cohort. RP-A501 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.
- In May 2022, Prevail Therapeutics Inc., a wholly owned subsidiary of Eli Lilly, announced that the FDA accepted an IND application to study PR001 (LY3884961) for Type 1 Gaucher disease (GD1) in a Phase 1/2 clinical trial. PR001 (LY3884961) is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.
- In May 2022, Ultragenyx Pharmaceutical Inc. announced dosing and enrollment of the Phase 3 study of DTX401 for Glycogen Storage Disease Type Ia (GSDIa) is ongoing. Ultragenyx expects to initiate the Phase 3 eNH3ance study of DTX301 for Ornithine Transcarbamylase (OTC) Deficiency in mid-2022, and is dosing patients in the Phase 1/2 stage of the seamless Phase 1/2/3 Cyprus2+ study of UX701 for Wilson Disease. DTX401 and DTX301 are both being developed as one-time gene therapies utilizing REGENXBIO's NAV AAV8 vector. UX701 is being developed as a one-time gene therapy utilizing REGENXBIO's NAV AAV9 vector.

Financial Results

Cash Position: Cash, cash equivalents and marketable securities were $682.0 million as of June 30, 2022, compared to $849.3 million as of December 31, 2021. The decrease was primarily driven by cash used to fund operating activities and capital expenditures and temporary unrealized losses on marketable debt securities during the six months ended June 30, 2022.

Revenues: Revenues were $32.6 million for the three months ended June 30, 2022, compared to $22.0 million for the three months ended June 30, 2021. The increase was primarily attributable to Zolgensma royalty revenues, which increased by $10.0 million, from $18.4 million for the second quarter of 2021 to $28.4 million for the second quarter of 2022. As reported by Novartis, sales of Zolgensma for the second quarter of 2022 increased by 20% as compared to the second quarter of 2021, driven by geographic expansion of product access outside the United States.

Research and Development Expenses: Research and development expenses were $61.0 million for the three months ended June 30, 2022, compared to $45.9 million for the three months ended June 30, 2021. The increase was primarily attributable to personnel costs as a result of increased headcount, and costs associated with clinical trials and manufacturing-related activities for our lead product candidates.

General and Administrative Expenses: General and administrative expenses were $20.8 million for the three months ended June 30, 2022, compared to $18.4 million for the three months ended June 30, 2021. The increase was primarily attributable to personnel costs and corporate overhead expenses.

Net Loss: Net loss was $68.2 million, or $1.58 basic and diluted net loss per share, for the three months ended June 30, 2022, compared to a net loss of $57.6 million, or $1.36 basic and diluted net loss per share, for the three months ended June 30, 2021.

Financial Guidance

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $682.0 million as of June 30, 2022 to fund its operations into 2025.

Conference Call

In connection with this announcement, REGENXBIO will host a conference call and webcast today at 4:30 p.m. ET. A live audio webcast will be available at regenxbio.com/investors. Interested parties may also pre-register for the earnings conference call here. Once registration is completed, participants will be provided a dial-in number with a personalized conference code to access the call. Those who plan on participating are advised to dial in 15 minutes prior to the start time.

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, including late-stage and commercial programs, in multiple therapeutic areas. REGENXBIO is committed to a "5x'25" strategy to progress five AAV Therapeutics from our internal pipeline and licensed programs into pivotal-stage or commercial products by 2025.

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, 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, 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.

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

	June 30, 2022	December 31, 2021
Assets		
Current assets		
Cash and cash equivalents	$ 121,374	$ 345,209
Marketable securities	281,588	112,230
Accounts receivable, net	37,500	32,439
Prepaid expenses	16,245	18,752
Other current assets	8,717	10,196
Total current assets	465,424	518,826
Marketable securities	279,073	391,907
Accounts receivable, net	2,034	2,262
Property and equipment, net	138,815	131,547
Operating lease right-of-use assets	60,163	60,904
Restricted cash	2,030	2,030
Other assets	5,905	6,428
Total assets	$ 953,444	$ 1,113,904
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 16,985	$ 11,387
Accrued expenses and other current liabilities	47,014	76,111
Deferred revenue	6,636	3,333
Operating lease liabilities	2,832	1,752
Liability related to sale of future royalties	41,089	37,889
Total current liabilities	114,556	130,472
Operating lease liabilities	85,672	84,929
Liability related to sale of future royalties	114,171	133,460
Other liabilities	8,526	745
Total liabilities	322,925	349,606
Stockholders' equity		
Preferred stock; no shares issued and outstanding at June 30, 2022 and December 31, 2021	—	—
Common stock; 43,171 and 42,831 shares issued and outstanding at June 30, 2022 and December 31, 2021, respectively	4	4
Additional paid-in capital	951,412	928,095
Accumulated other comprehensive loss	(14,763)	(2,569)
Accumulated deficit	(306,134)	(161,232)
Total stockholders' equity	630,519	764,298
Total liabilities and stockholders' equity	$ 953,444	$ 1,113,904

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,	
	2022	2021	2022	2021
Revenues				
License and royalty revenue	$ 32,649	$ 22,035	$ 54,867	$ 40,919
Total revenues	32,649	22,035	54,867	40,919
Operating Expenses				
Cost of revenues	12,951	9,819	28,668	14,670
Research and development	61,008	45,882	116,635	85,604
General and administrative	20,832	18,425	43,150	36,263
Credit losses and other	391	135	474	650
Total operating expenses	95,182	74,261	188,927	137,187
Loss from operations	(62,533)	(52,226)	(134,060)	(96,268)
Other Income (Expense)				
Interest income from licensing	153	554	247	583
Investment income	1,061	399	1,860	979
Interest expense	(6,860)	(6,366)	(12,990)	(13,068)
Total other income (expense)	(5,646)	(5,413)	(10,883)	(11,506)
Loss before income taxes	(68,179)	(57,639)	(144,943)	(107,774)
Income Tax Benefit (Expense)	—	—	41	(4)
Net loss	$ (68,179)	$ (57,639)	$ (144,902)	$ (107,778)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	(2,813)	113	(12,194)	(895)
Total other comprehensive income (loss)	(2,813)	113	(12,194)	(895)
Comprehensive loss	$ (70,992)	$ (57,526)	$ (157,096)	$ (108,673)
Net loss per share, basic and diluted	$ (1.58)	$ (1.36)	$ (3.37)	$ (2.56)
Weighted-average common shares outstanding, basic and diluted	43,111	42,510	43,028	42,170

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