

May 18, 2021



# Abeona Therapeutics Reports First Quarter Financial Results

*Patient enrollment ongoing for EB-101 pivotal Phase 3 VIITAL™ study*

*Positive interim MPS III data presented at 17<sup>th</sup> Annual WORLDSymposium; additional neurocognitive assessments of patients treated in high dose cohort 3 in MPS IIIA Transpher A study and additional clinical data from MPS IIIB Transpher B study expected in 2021*

*Strengthened management and board to support the company's focus on driving future growth, enhancing corporate governance, and creating additional shareholder value*

*Conference call scheduled for Tuesday, May 25, 2021 at 8:30 a.m. ET*

NEW YORK and CLEVELAND, May 18, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced financial results for the first quarter 2021 and recent business progress.

"We are off to a fast start in 2021, reflecting our intense focus on execution," said Michael Amoroso, Chief Executive Officer of Abeona. "We are focused on completing enrollment in the EB-101 Phase 3 pivotal VIITAL™ study, gaining clarity on a regulatory path for ABO-102 in MPS IIIA, producing the first lot of Abeona-produced clinical grade product for ABO-102, and reporting additional neurocognitive and biomarker data from both the ABO-102 Transpher A and the ABO-101 Transpher B studies. We also have a robust preclinical pipeline, and we are conducting research assessing AAV capsids with the aim of IND-enabling studies in two to three eye indications. Importantly, our focus on building the right talent and experience on our leadership team positions us well to continue to advance our clinical programs toward delivering meaningful milestones later this year."

## First Quarter and Recent Highlights

### *Corporate Updates*

- Appointed Michael Amoroso as President, Chief Executive Officer (CEO) and a member of the company's Board of Directors.
- Abeona strengthened its Board of Directors with the appointment of four new independent members who bring relevant operational leadership experience with life sciences companies, including in the areas of clinical development, manufacturing of cell therapy and gene therapy products, and corporate and financial compliance, to support the company's focus on driving future growth and creating additional shareholder value.

*EB-101 (Autologous, Gene-Corrected Cell Therapy)*

- Patient enrollment is ongoing for the EB-101 pivotal Phase 3 VIITAL™ study for RDEB. The company continues to expect to complete enrollment in the VIITAL™ study in 2021, depending upon the impact from the COVID-19 pandemic, including travel restrictions and safety concerns.
- To support ongoing enrollment and commercial preparation, Abeona continues to work toward adding a second clinical site in the VIITAL™ study by the third quarter of 2021.
- Presented data on long-term patient-reported outcomes following EB-101 treatment of RDEB wounds at the Society for Investigative Dermatology (SID) Virtual Meeting 2021, held from May 3-8, 2021. The results showed durable wound healing and reduction in pain through 6 years after treatment.

#### *ABO-102 and ABO-101 (AAV-based Gene Therapies)*

- Presented new positive data from two ongoing Phase 1/2 clinical trials of ABO-102 in MPS IIIA and ABO-101 in MPS IIIB in late-breaking platform oral presentations at the 17<sup>th</sup> Annual WORLDSymposium™ in February 2021.
- The FDA granted Abeona's request and scheduled a Type B meeting in June 2021 to discuss the data-to-date from the ABO-102 Transpher A study and the potential path to a Biologics License Application (BLA) submission for ABO-102 in MPS IIIA.

#### *Preclinical Pipeline*

- Presented new data supporting the potential of Cre-mediated dual AAV vector technology to enable delivery of large genes targeted for treatment of Stargardt disease during an oral presentation at the Association for Research in Vision and Ophthalmology (ARVO) 2021 Annual Meeting, held virtually from May 1-7, 2021.
- Abeona recently completed non-human primate (NHP) studies comparing several capsids with AAV8, the industry standard for intraocular administration, in order to further understand and characterize the company's AAV capsids. The results showed that AAV204, part of Abeona's in-licensed AIM™ capsid library, was superior to AAV8 using a recently developed route of ocular administration.
- In a separate NHP experiment, the company's AAV214 and AAVV214D5 capsids were tested versus AAV8 administered subretinally. Both capsids demonstrated nearly identical levels of transduction of photoreceptor and retinal pigmented epithelium (RPE) cells, which are the cell types most frequently affected in inherited retinal diseases, when compared with AAV8.
- The results from the recently completed NHP studies support Abeona's strategy to advance multiple preclinical eye programs into the clinic.

#### **First Quarter Financial Results**

Cash, cash equivalents and short-term investments totaled \$86.8 million as of March 31, 2021, compared to \$95.0 million as of December 31, 2020. Net cash used in operating activities was \$13.6 million for the first quarter of 2021.

Research and development (R&D) expenses were \$7.2 million for the first quarter of 2021, compared to \$6.8 million in the comparable period in 2020. The increase in R&D expenses was primarily due to increased clinical and development work for the company's gene and cell therapy product candidates, and increased salary and related costs. General and administrative (G&A) expenses were \$6.6 million for the first quarter of 2021, compared to

\$6.4 million in the same period in 2020. The increase in G&A expenses was primarily due to increased professional fees, partially offset by decreased salary and related costs, and decreases in net other G&A expenses.

Net loss was \$16.0 million for the first quarter of 2021, compared to net loss of \$48.2 million for the comparable period in 2020. The decrease in net loss was primarily due to the non-cash impairment charge of \$32.9 million related to the termination of the license agreement with REGENXBIO in the first quarter of 2020.

## **Conference Call Details**

Abeona Therapeutics will host a conference call and webcast on Tuesday, May 25, 2021 at 8:30 a.m. ET, to discuss its first quarter 2021 financial results and business update. To access the call, dial 888-506-0062 (U.S. toll-free) or 973-528-0011 (international) and Entry Code: 552097 five minutes prior to the start of the call. A live, listen-only webcast and archived replay of the call can be accessed on the Investors & Media section of Abeona's website at [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com). The archived webcast replay will be available for 30 days following the call.

## **About Abeona Therapeutics**

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. Abeona's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel AAV-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively, in Phase 1/2 development. The Company's portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical needs. Abeona's novel, next-generation AIM™ capsids have shown potential to improve tropism profiles for a variety of devastating diseases. Abeona's fully functional, gene and cell therapy GMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and commercial production of AAV-based gene therapies. For more information, visit [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com).

## **Forward-Looking Statements**

*This press release contains certain statements that are forward-looking 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, and that involve risks and uncertainties. We have attempted to identify forward-looking statements by such terminology as "may," "will," "believe," "estimate," "expect," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, numerous risks and uncertainties, including but not limited to the potential impacts of the COVID-19 pandemic on our business, operations, and financial condition, continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies, the impact of competition, the ability to secure licenses for any technology that may be necessary to commercialize our products, the ability to achieve or obtain necessary regulatory approvals, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, and other risks disclosed in the*

*Company's most recent Annual Report on Form 10-K and subsequent quarterly reports on Form 10-Q and other periodic reports filed with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to update them to reflect events or circumstances occurring after the date of this press release, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.*

**Abeona Therapeutics Inc. and Subsidiaries**  
**Condensed Consolidated Statements of Operations and Comprehensive Loss**  
*(unaudited)*

	<b>For the three months ended March 31,</b>	
	<b>2021</b>	<b>2020</b>
<b>Revenues</b>	\$ -	\$ -
<b>Expenses:</b>		
Research and development	7,212,000	6,818,000
General and administrative	6,568,000	6,412,000
Depreciation and amortization	817,000	2,065,000
Licensed technology impairment charge	-	32,916,000
Total expenses	14,597,000	48,211,000
Loss from operations	(14,597,000)	(48,211,000)
Interest and miscellaneous income	15,000	652,000
Interest expense	(1,420,000)	(600,000)
Net loss	\$(16,002,000)	\$(48,159,000)
Basic and diluted loss per common share	\$ (0.17)	\$ (0.52)
Weighted average number of common shares outstanding – basic and diluted	94,234,653	92,362,505
Other comprehensive income:		
Change in unrealized gains related to available-for-sale debt securities	13,000	386,000
Comprehensive loss	\$(15,989,000)	\$(47,773,000)

**Abeona Therapeutics Inc. and Subsidiaries**  
**Condensed Consolidated Balance Sheets**  
*(unaudited)*

	<b>March 31, 2021</b>	<b>December 31, 2020</b>
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 14,265,000	\$ 12,596,000
Short-term investments	72,506,000	82,438,000
Prepaid expenses and other current assets	1,826,000	2,708,000
Total current assets	88,597,000	97,742,000
Property and equipment, net	10,978,000	11,322,000
Right-of-use lease assets	6,764,000	7,032,000
Licensed technology, net	1,471,000	1,500,000
Goodwill	32,466,000	32,466,000
Other assets and restricted cash	1,156,000	1,136,000
Total assets	\$ 141,432,000	\$ 151,198,000
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 2,476,000	\$ 4,695,000
Accrued expenses	2,868,000	3,410,000
Current portion of lease liability	1,716,000	1,713,000
Current portion of loan payable	549,000	330,000
Payable to licensor	32,934,000	31,515,000
Deferred revenue	296,000	296,000
Total current liabilities	40,839,000	41,959,000
Loan payable	1,209,000	1,428,000
Long-term lease liabilities	4,994,000	5,260,000
Total liabilities	47,042,000	48,647,000
Commitments and contingencies	-	-
Stockholders' equity:		
Common stock - \$0.01 par value; authorized 200,000,000 shares;		
issued and outstanding 99,038,933 at March 31, 2021;		
issued and outstanding 96,131,678 at December 31, 2020	990,000	961,000
Additional paid-in capital	680,103,000	672,304,000
Accumulated deficit	(586,706,000)	(570,704,000)
Accumulated other comprehensive income/(loss)	3,000	(10,000)

Total stockholders' equity	<u>94,390,000</u>	<u>102,551,000</u>
Total liabilities and stockholders' equity	<u>\$ 141,432,000</u>	<u>\$ 151,198,000</u>

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Source: Abeona Therapeutics Inc.