

March 24, 2021



# Abeona Therapeutics Announces 2020 Financial Results and Recent Operational Progress

*Fourth patient treated in pivotal Phase 3 VIITAL™ study of EB-101 in RDEB after successful Type B meeting with FDA*

*Positive new interim data from MPS IIIA and MPS IIIB programs presented at 17<sup>th</sup> Annual WORLDSymposium*

*\$95 million in cash, cash equivalents and short-term investments as of December 31, 2020*

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

NEW YORK and CLEVELAND, March 24, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced financial results for the fourth quarter and full year 2020, and provided an update on recent operational progress.

“In 2020, Abeona advanced our three clinical programs toward bringing urgently needed treatments to patients with recessive dystrophic epidermolysis bullosa (RDEB) and Sanfilippo syndrome type A (MPS IIIA) and type B (MPS IIIB) despite the macro disruptions that impacted the world,” said Michael Amoroso, Chief Executive Officer of Abeona. “We remain laser focused on executing our strategy and achieving upcoming milestones. Our recent momentum is highlighted by a successful Type B meeting with the FDA where we aligned on co-primary endpoints for the pivotal Phase 3 VIITAL study of EB-101 in RDEB, treatment of the fourth patient in the VIITAL study, and reporting new positive clinical data for both ABO-102 in MPS IIIA and ABO-101 in MPS IIIB at the *WORLD Symposium*. We look forward to continuing to propel our clinical programs forward and bringing our gene and cell therapies to patients who currently have no approved treatment options. We believe we have sufficient cash resources to build on our momentum and fund our current development and operating plan through the achievement of key anticipated milestones, including the potential for multiple regulatory submissions.”

## Recent Highlights

### *Corporate Developments*

- In March 2021, Michael Amoroso, Executive Vice President, Chief Operating Officer (COO) and principal executive officer at Abeona, was promoted to President, Chief Executive Officer (CEO) and a member of the company’s Board of Directors.

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

- The fourth patient was treated in Abeona's EB-101 pivotal Phase 3 VIITAL™ study for recessive dystrophic epidermolysis bullosa (RDEB). The company currently anticipates completing study enrollment in 2021 of 10 to 15 patients with RDEB, comprising approximately 35 large chronic wound sites treated in total.
- Abeona held a successful Type B meeting with the U.S. Food and Drug Administration (FDA) to align with the Agency on the company's proposal regarding co-primary endpoints of partial wound closure and mean pain reduction for the Phase 3 VIITAL™ study of EB-101 in RDEB.

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

- Reported new positive interim data from the ABO-102 Transpher A study for MPS IIIA and the ABO-101 Transpher B study for MPS IIIB. The data was presented in late-breaking platform oral presentations at the 17<sup>th</sup> Annual *WORLDSymposium™* in February 2021. The presented results from the high dose cohort 3 in the Transpher A study showed evidence of preservation of neurocognitive development within normal range of a non-afflicted child for 2.5 years to 3 years after treatment with ABO-102 in the three young patients treated before 30 months of age. In addition, the data showed a dose-related and sustained reduction in cerebrospinal fluid (CSF) levels of heparan sulfate, denoting transgene expression in the CNS, and a durable reduction of liver volume. The presented results from the Transpher B study continued to show signals of biologic effect with reduction of disease-specific biomarkers in the CSF, plasma and urine and reduction in liver volumes. Abeona expects additional follow-up visits and neurocognitive assessments of patients treated in the high dose cohort 3 in the Transpher A study and additional clinical updates from the Transpher B study in 2021.
- The FDA accepted Abeona's request for a meeting to discuss the data-to-date from the Transpher A study and the potential path to a Biologics License Application (BLA) submission for ABO-102 in MPS IIIA.
- As previously reported, target enrollment in the ABO-102 Transpher A study has been achieved. Abeona continues to enroll patients into the study given the lack of treatment options for MPS IIIA and encouraging efficacy and safety data from the high dose cohort 3. To date, 19 patients have been dosed in the Transpher A study, including 13 patients dosed in cohort 3.
- The ABO-101 Transpher B study for MPS IIIB is ongoing, and to date, 11 patients have been dosed, including 4 patients dosed in cohort 3.

#### *Preclinical Pipeline*

- Initiated preclinical research in December 2020 and are assessing AAV capsids in six undisclosed ophthalmic disorders and intend to advance toward IND-enabling studies in two to three indications in 2022. Previously reported preclinical data showed the potential for AIM™ AAV vectors to efficiently target the retinal epithelium after intravitreal injection, creating the potential for new pipeline candidates that can address multiple ophthalmic disorders.

#### **Fourth Quarter and Full Year 2020 Financial Results**

Cash, cash equivalents and short-term investments totaled \$95.0 million as of December 31, 2020, compared to \$129.3 million as of December 31, 2019. Net cash used in operating

activities was \$35.0 million for the full year of 2020, compared to \$62.8 million for the full year 2019.

License and other revenues for the fourth quarter and full year of 2020 were \$3.0 million and \$10.0 million, respectively, compared to zero revenues in the same periods in 2019. The increase in revenue was comprised of initial proceeds from agreements with Taysha Gene Therapies in August 2020 relating to ABO-202, a potential gene therapy for CLN1 disease, and in October 2020 relating to intellectual property directed to a potential gene therapy for Rett syndrome.

Research and development (R&D) expenses were \$9.2 million for the fourth quarter of 2020 and \$30.1 million for the full year of 2020, compared to \$9.6 million and \$48.6 million in the same periods in 2019. The decrease in R&D expenses was primarily due to decreased clinical and development work for the company's gene and cell therapy product candidates as a result of scaled back manufacturing, clinical and non-clinical development activities impacted by the COVID-19 pandemic, and cost savings from the decision to internally manufacture retrovirus for the EB-101 program.

General and administrative (G&A) expenses were \$7.4 million for the fourth quarter of 2020 and \$23.8 million for the full year of 2020, compared to \$4.7 million and \$20.7 million in the same periods in 2019. The increase in G&A expenses in the fourth quarter of 2020 was primarily due to increased professional fees and increased share-based compensation expense. The increase in G&A expenses for full year 2020 was primarily due to severance costs associated with management changes and increased professional fees.

Net loss was \$15.8 million for the fourth quarter of 2020 and \$84.2 million for the full year of 2020, compared to net loss of \$16.4 million and \$76.3 million for the same periods in 2019. The increase in the full year 2020 net loss is primarily due to the licensed technology non-cash impairment charge of \$32.9 million related to the termination of the license agreement with REGENXBIO, partially offset by increased license and other revenues along with decreased clinical and development expenses.

## **Conference Call Details**

Abeona Therapeutics will host a conference call and webcast tomorrow, Thursday, March 25, 2021 at 8:30 a.m. ET, to discuss its fourth quarter 2020 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: 251720 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**  
**Consolidated Statements of Operations and Comprehensive Loss**  
*(unaudited)*

	For the three months ended December 31,		For the years ended December 31,	
	2020	2019	2020	2019
<b>Revenues:</b>				
License and other revenues	\$ 3,000,000	\$ -	\$ 10,000,000	\$ -
Total revenues	3,000,000	-	10,000,000	-
<b>Expenses:</b>				
Research and development	9,243,000	9,605,000	30,139,000	48,566,000
General and administrative	7,397,000	4,734,000	23,779,000	20,705,000

Depreciation and amortization	840,000	2,072,000	4,586,000	7,819,000
Licensed technology impairment charge	-	-	32,916,000	-
Total expenses	<u>17,480,000</u>	<u>16,411,000</u>	<u>91,420,000</u>	<u>77,090,000</u>
Loss from operations	(14,480,000)	(16,411,000)	(81,420,000)	(77,090,000)
Interest and miscellaneous income	40,000	380,000	1,301,000	1,208,000
Interest and other expense	(1,388,000)	(400,000)	(4,115,000)	(400,000)
Net loss	<u>\$(15,828,000)</u>	<u>\$(16,431,000)</u>	<u>\$(84,234,000)</u>	<u>\$(76,282,000)</u>
Basic and diluted loss per common share	\$ (0.17)	\$ (0.30)	\$ (0.91)	\$ (1.51)
Weighted average number of common shares outstanding – basic and diluted	92,869,775	54,718,776	92,663,574	50,354,596
Other comprehensive loss: Change in unrealized losses related to available-for-sale debt securities	(27,000)	-	(10,000)	-
Comprehensive loss	<u>\$(15,855,000)</u>	<u>\$(16,431,000)</u>	<u>\$(84,244,000)</u>	<u>\$(76,282,000)</u>

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

<b>ASSETS</b>	<b>December 31, 2020</b>	<b>December 31, 2019</b>
Current assets:		
Cash and cash equivalents	\$ 12,596,000	\$ 129,258,000
Short-term investments	82,438,000	-
Prepaid expenses and other current assets	2,708,000	3,132,000
Total current assets	<u>97,742,000</u>	<u>132,390,000</u>
Property and equipment, net	11,322,000	13,157,000
Right-of-use lease assets	7,032,000	8,047,000
Licensed technology, net	1,500,000	36,178,000
Goodwill	32,466,000	32,466,000

Other assets and restricted cash	1,136,000	1,144,000
Total assets	<u>\$ 151,198,000</u>	<u>\$ 223,382,000</u>

## LIABILITIES AND STOCKHOLDERS' EQUITY

### Current liabilities:

Accounts payable	\$ 4,695,000	\$ 3,763,000
Accrued expenses	3,410,000	5,543,000
Current portion of lease liability	1,713,000	1,699,000
Current portion of loan payable	330,000	-
Payable to licensor	31,515,000	27,400,000
Deferred revenue	296,000	296,000
Total current liabilities	<u>41,959,000</u>	<u>38,701,000</u>

Loan payable	1,428,000	-
Long-term lease liabilities	5,260,000	6,251,000
Total liabilities	<u>48,647,000</u>	<u>44,952,000</u>

Commitments and contingencies - -

### Stockholders' equity:

Common stock - \$0.01 par value; authorized 200,000,000 shares; issued and outstanding 96,131,678 at December 31, 2020; issued and outstanding 83,622,135 at December 31, 2019	961,000	836,000
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Additional paid-in capital	672,304,000	664,064,000
Accumulated deficit	(570,704,000)	(486,470,000)
Accumulated other comprehensive loss	(10,000)	-
Total stockholders' equity	<u>102,551,000</u>	<u>178,430,000</u>
Total liabilities and stockholders' equity	<u>\$ 151,198,000</u>	<u>\$ 223,382,000</u>

### Investor and Media Contact:

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Source: Abeona Therapeutics Inc.