

November 15, 2021



# Abeona Therapeutics Reports Third Quarter Financial Results

*Phase 3 VIITAL™ study on track to complete enrollment in first quarter 2022 with final patients identified and under screening*

*Added gene therapy and biopharmaceutical industry veterans to regulatory and quality teams to prepare for two Biologics License Application (BLA) submissions*

*Formally settled dispute with REGENXBIO Inc., eliminating uncertainty from arbitration outcome and facilitating planning of resources to reach next value inflection points*

*Conference call scheduled for Wednesday, November 17, 2021 at 8:30 a.m. ET*

NEW YORK and CLEVELAND, Nov. 15, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced financial results for the third quarter 2021 and recent business progress.

“This is an exciting and critical time in Abeona’s life cycle as we advance two late-stage pivotal assets with rare pediatric designations, transformational potential, and key milestones ahead,” said Vish Seshadri, Ph.D., Chief Executive Officer of Abeona. “In the coming months, we expect to finish patient accrual for the EB-101 Phase 3 VIITAL™ study in the first quarter of 2022 with top-line data readout in the third quarter of 2022. We are focused on bringing our gene therapies to patients who suffer from diseases with no approved treatments, and we look forward to completing the registration-enabling studies and submitting BLA filings for both EB-101 and ABO-102.”

“The settlement with REGENXBIO provides for payments by Abeona over a 3 year period and, importantly, allows us to focus all our resources on advancing our pipeline of therapies to treat rare debilitating diseases,” said Edward Carr, Chief Financial Officer of Abeona. “With our existing financial resources, we are continuing to drive our lead programs toward key milestones. To further strengthen the balance sheet and prepare for BLA filings, commercial launch readiness and AAV facility build out, we are exploring various options including strategic partnering of pipeline assets and non-dilutive funding.”

## Third Quarter and Recent Highlights

*EB-101 for RDEB: Enrollment completion for Phase 3 VIITAL™ study expected in Q1 2022 with top-line data readout expected in Q3 2022*

- Continued to enroll and treat patients in the pivotal Phase 3 VIITAL™ study for RDEB and identified patients required to complete accrual.
- UMass Memorial Medical Center was activated as the second clinical trial site in the VIITAL™ study and is actively recruiting patients.

- Presented updated Phase 1/2a results showing considerable reduction in both wound burden and associated long-term pain up to six years after EB-101 treatment at the Society for Pediatric Dermatology 46<sup>th</sup> Annual Meeting.
- Continued to prepare Abeona's cGMP commercial facility in Cleveland, OH for the manufacturing of EB-101 drug product to support BLA activities. BLA filing anticipated in late-2022 to early-2023.

*ABO-102 for MPS IIIA: Pivotal Transpher A study top-line data readout expected between Q4 2022 and Q2 2023*

- Having aligned with the FDA on the definition of the primary endpoint for registration at the Type B meeting in June 2021, submitted an amended protocol for the Transpher A study to reflect these endpoints.
- Collaborated with experts in MPS IIIA to finalize the statistical analysis plan (SAP) which the Company is scheduled to discuss with the FDA in the first quarter of 2022 through the RMAT mechanism. The Company expects top-line data for Transpher A between the fourth quarter of 2022 and the second quarter of 2023.
- Presented brain MRI data from the Transpher A study indicating that ABO-102 increased grey matter, corpus callosum, and amygdala volumes in the brain in the three young patients with MPS IIIA at 24 months as compared to afflicted patients without treatment. The findings were presented at the 16<sup>th</sup> International Symposium on MPS and Related Diseases and corroborate previously reported results of preservation of neurocognitive development in these three young patients in the Transpher A study.
- Manufacturing six GMP lots of ABO-102 in the second half of 2021 using animal-free materials; Analytical comparability studies to establish equivalence with drug product sourced from Nationwide Children's Hospital is expected to be completed in the first half of 2022.
- Initiated the construction of a 12,000 square foot commercial AAV manufacturing facility at the Company's Cleveland site to support its AAV-based needs, including the production of ABO-102 for an anticipated BLA filing and subsequent commercial launch.

*ABO-101 for MPS IIIB: Completed dosing seven patients at the highest dose; 2-year neurocognitive data expected in H2 2022 for efficacy evaluation*

- Seven patients, of whom four were dosed in Transpher B and three in the Named Patient Program in Germany, are currently in follow up for safety and efficacy of the high dose of ABO-101. The Company expects 2-year neurocognitive data to assess efficacy of ABO-101 in the second half of 2022.

*Preclinical Novel AAV-based Therapies for Ocular Diseases: Advancing programs toward pre-IND meetings in late-2022*

- Work is ongoing in advancing multiple preclinical programs for undisclosed eye indications toward the clinic, with animal proof-of-concept data anticipated by mid-2022.

*Corporate Updates*

- As of October 15, 2021, Michael Amoroso transitioned to the role of Chairman of the Board following the retirement of Steven Rouhandeh. As part of the transition plan, Vish Seshadri, Ph.D., M.B.A., transitioned to the role of President and CEO, and member of the Board of Directors.
- Added gene therapy and biopharmaceutical industry veterans with deep operational expertise across regulatory and quality to prepare for two BLA submissions for the Company's lead clinical programs in RDEB and MPS IIIA.
- Entered into a settlement with REGENXBIO Inc. that resolves the previously disclosed dispute over an arbitration award to REGENXBIO related to the parties' former license agreement. As part of the settlement, Abeona has agreed to make payments to REGENXBIO of \$20 million in the fourth quarter of 2021, \$5 million in November 2022, and \$5 million no later than November 2024. The settlement allows Abeona to eliminate ongoing legal expenses, deployment of resources and risks related to the dispute.

### **Third Quarter Financial Results**

Cash, cash equivalents and short-term investments totaled \$67.0 million as of September 30, 2021. Net cash used in operating activities was \$10.3 million for the third quarter of 2021.

License and other revenues for the third quarter of 2021 were nil, as compared to \$7.0 million for the third quarter of 2020. The revenues in the third quarter of 2020 resulted from the sublicense and inventory purchase agreements for ABO-202 entered into with Taysha Gene Therapies in August 2020.

Total research and development (R&D) spending was \$8.0 million for the third quarters of 2021 and 2020.

Total general and administrative (G&A) spending was \$6.1 million in the third quarter of 2021, compared to \$4.4 million spent in the third quarter of 2020. The increase in G&A is primarily due to increased stock-based compensation and professional fees, partially offset by decreased salary and related costs.

Gain on settlement with licensor was \$6.7 million in the third quarter of 2021, as compared to nil in the same period of 2020. As noted above, Abeona entered into a settlement agreement on November 12, 2021 with REGENXBIO to resolve all current disputes relating to the license agreement between the parties. The accounting for the settlement agreement resulted in a \$6.7 million gain on settlement with REGENXBIO in the third quarter of 2021.

PPP (Paycheck Protection Program) loan payable forgiveness income was \$1.8 million in the third quarter of 2021, as compared to nil in the same period of 2020, as a result of the Small Business Administration's forgiveness of Abeona's PPP loan in July 2021.

Net loss was \$7.0 million for the third quarter of 2021 or a \$0.07 basic and diluted loss per common share as compared to a net loss of \$7.2 million, or a \$0.08 basic and diluted loss per common share, for the same period in 2020.

### **Conference Call Details**

Abeona Therapeutics will host a conference call and webcast on Wednesday, November 17,

2021 at 8:30 a.m. ET, to discuss its third 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: 512059 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 investigational autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel investigational 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 development portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical need. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases. Abeona's fully integrated gene and cell therapy cGMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and planned 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 timing of data readouts, 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.*

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

	For the three months ended September 30,		For the nine months ended September 30,	
	2021	2020	2021	2020
<b>Revenues</b>	\$ -	\$ 7,000,000	\$ -	\$ 7,000,000
<b>Expenses:</b>				
Research and development	7,978,000	7,969,000	22,624,000	20,896,000
General and administrative	6,092,000	4,432,000	18,117,000	16,382,000
Depreciation and amortization	802,000	847,000	2,443,000	3,746,000
Licensed technology impairment charge	-	-	-	32,916,000
Total expenses	<u>14,872,000</u>	<u>13,248,000</u>	<u>43,184,000</u>	<u>73,940,000</u>
Loss from operations	(14,872,000)	(6,248,000)	(43,184,000)	(66,940,000)
PPP loan payable forgiveness income	1,758,000	-	1,758,000	-
Gain on settlement with licensor	6,743,000	-	6,743,000	-
Interest and miscellaneous income	10,000	338,000	33,000	1,261,000
Interest expense	<u>(683,000)</u>	<u>(1,327,000)</u>	<u>(3,603,000)</u>	<u>(2,727,000)</u>
Net loss	\$ (7,044,000)	\$ (7,237,000)	\$ (38,253,000)	\$ (68,406,000)
Basic and diluted loss per common share	\$ (0.07)	\$ (0.08)	\$ (0.40)	\$ (0.74)
Weighted average number of common shares outstanding – basic and diluted	97,990,338	92,714,983	96,258,681	92,594,339
Other comprehensive income/(loss):				

Change in unrealized gains/(losses) related to available-for-sale debt securities	1,000	(116,000)	10,000	17,000
Foreign currency translation adjustments	(9,000)	-	(9,000)	-
Comprehensive loss	\$ (7,052,000)	\$ (7,353,000)	\$ (38,252,000)	\$ (68,389,000)

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

<b>ASSETS</b>	<b>September 30, 2021</b>	<b>December 31, 2020</b>
Current assets:		
Cash and cash equivalents	\$ 43,781,000	\$ 12,596,000
Short-term investments	23,217,000	82,438,000
Prepaid expenses and other current assets	907,000	2,708,000
Total current assets	<u>67,905,000</u>	<u>97,742,000</u>
Property and equipment, net	9,869,000	11,322,000
Right-of-use lease assets	6,207,000	7,032,000
Licensed technology, net	1,413,000	1,500,000
Goodwill	32,466,000	32,466,000
Other assets and restricted cash	1,159,000	1,136,000
Total assets	<u>\$ 119,019,000</u>	<u>\$ 151,198,000</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 1,861,000	\$ 4,695,000
Accrued expenses	2,593,000	3,410,000
Current portion of lease liability	1,723,000	1,713,000
Current portion of PPP loan payable	-	330,000
Current portion of payable to licensor	20,000,000	31,515,000
Contract liability	296,000	296,000
Total current liabilities	<u>26,473,000</u>	<u>41,959,000</u>
PPP loan payable	-	1,428,000
Payable to licensor	8,360,000	-

Long-term lease liabilities	4,442,000	5,260,000
Total liabilities	<u>39,275,000</u>	<u>48,647,000</u>
Commitments and contingencies	-	-
Stockholders' equity:		
Common stock - \$0.01 par value; authorized 200,000,000 shares;		
issued and outstanding 101,867,539 at September 30, 2021;		
issued and outstanding 96,131,678 at December 31, 2020	1,019,000	961,000
Additional paid-in capital	687,691,000	672,304,000
Accumulated deficit	(608,957,000)	(570,704,000)
Accumulated other comprehensive loss	(9,000)	(10,000)
Total stockholders' equity	<u>79,744,000</u>	<u>102,551,000</u>
Total liabilities and stockholders' equity	<u>\$ 119,019,000</u>	<u>\$ 151,198,000</u>



Source: Abeona Therapeutics Inc.