July 28, 2021

Abeona Therapeutics Reports Second Quarter Financial Results

Second clinical trial site activated in EB-101 pivotal Phase 3 VIITAL™ study

Successful Type B meeting with U.S. FDA; Transpher A study will serve as the pivotal study for ABO-102 in MPS IIIA; Alignment with FDA on primary study endpoint

Focusing resources on completing EB-101 and ABO-102 pivotal, registration-enabling studies

Conference call scheduled for Thursday, July 29, 2021 at 8:30 a.m. ET

NEW YORK and CLEVELAND, July 28, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced financial results for the second quarter 2021 and recent business progress.

“The second quarter was a highly effective and productive quarter for Abeona, and we remain committed to delivering operational excellence and bringing our gene therapies to patients with no approved treatments,” said Michael Amoroso, Chief Executive Officer of Abeona. “As we continue to advance our clinical programs, we are delivering on meaningful milestones. We are focusing our resources on completing registration-enabling studies for patients with RDEB and MPS IIIA, and are preparing for the potential of two BLA filings.”

Second Quarter and Recent Highlights

Corporate Updates

- Appointed Vishwas Seshadri, Ph.D., M.B.A., as Senior Vice President, Head of Research & Clinical Development. Dr. Seshadri joins Abeona from Celgene Corporation, now a subsidiary of Bristol-Myers Squibb Company, and brings substantial experience in the life sciences industry overseeing product development, regulatory submissions, and commercialization for novel therapies including personalized, autologous cell therapies.

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

- Activated UMass Memorial Medical Center in Worcester, MA as the second clinical trial site in the pivotal Phase 3 VIITAL™ study of its investigational EB-101 treatment for recessive dystrophic epidermolysis bullosa (RDEB).
- Presented updated Phase 1/2a clinical trial results at the Society for Pediatric Dermatology (SPD) 46th Annual Meeting, with EB-101 treatment of large, chronic RDEB wounds continuing to show a considerable reduction in both wound burden and associated long-term pain for up to six years.

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

- Abeona completed a successful Type B meeting with the U.S. Food and Drug Administration (FDA) regarding the pivotal trial to support filing and approval for ABO-102 for the treatment of patients with Sanfilippo syndrome type A (MPS IIIA). Based on the Type B meeting, the ongoing Transpher A study will serve as the pivotal study for ABO-102 and could potentially support a Biologics License Application (BLA) submission depending on the data set. In addition, Abeona also aligned with the FDA on the definition of the primary endpoint for the study, neurocognitive assessment using the raw score from the Bayley Scales of Infant and Toddler Development (BSITD) and the Kauffman Assessment Battery for Children (KABC-2), which are already part of the assessment plan in the Transpher A protocol.
- Presented new brain MRI data during an oral presentation at the 16th International Symposium on MPS and Related Diseases, held during July 23-25, 2021. The MRI data indicated 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 MRI data is consistent with previously reported results of preservation of neurocognitive development in these three young patients in the Transpher A study.
- The Company is in the process of closing enrollment for the Transpher B trial. To date, four patients have been treated in the higher dose cohort of Transpher B. An additional three patients have been treated and a fourth patient will be treated with the higher dose through the Named Patient Program (NPP) in Germany, a compassionate use program that allows for patients with high unmet need to be treated at the request of the treating physician. The patients treated in the NPP are followed for safety and efficacy with the same rigor and frequency as patients in Transpher B. The Company intends to pool all patients' data from Transpher B and the NPP to assess therapeutic effect going forward. The Company expects 2-year neurocognitive data for the first patients treated in the high dose cohort of Transpher B beginning in the first half of 2022.

Preclinical Pipeline

- Presented new data at the Association for Research in Vision and Ophthalmology (ARVO) 2021 Annual Meeting supporting the potential of Cre-mediated dual AAV vector technology to enable delivery of large genes targeted for treatment of Stargardt disease.
- Completed non-human primate (NHP) studies comparing several of the company's AAV capsids with AAV8, the industry standard for intraocular administration. 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 AAV214D5 capsids demonstrated nearly identical levels of transduction compared with AAV8 of photoreceptor and retinal pigmented epithelium cells, which are the cell types most frequently affected in inherited retinal diseases. The NHP results support Abeona's strategy to advance multiple preclinical eye programs onward toward the clinic.

Second Quarter Financial Results

Cash, cash equivalents and short-term investments totaled $77.6 million as of June 30, 2021, compared to $86.8 million as of March 31, 2021. Net cash used in operating activities was $11.5 million for the second quarter of 2021.

“While pursuing our key strategic priorities, we have thoughtfully and carefully managed our spending decisions. Across the organization, there is a balanced approach to not only focusing on moving towards our key milestones, but also utilizing our cash resources prudently and on time to deliver results,” said Edward Carr, Chief Accounting Officer of Abeona.

Total research and development (R&D) spending was $7.4 million for the second quarter of 2021, which is consistent with the $7.2 million spent in the first quarter of 2021. R&D expenses include the cost of clinical development of the EB-101 and MPS programs, manufacturing of the drug products for EB-101 and ABO-102, and preclinical ophthalmic research activities. Total general and administrative (G&A) spending was $5.5 million in the second quarter of 2021, down from the $6.6 million spent in the first quarter of 2021. The decrease in G&A in the second quarter of 2021 is primarily due to lower professional fees. Net loss was $15.2 million for the second quarter of 2021.

Conference Call Details

Abeona Therapeutics will host a conference call and webcast on Thursday, July 29, 2021 at 8:30 a.m. ET, to discuss its second 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: 7184522 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. 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.

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)

	For the three months ended June 30,		For the six months ended June 30,	
	2021	**2020**	**2021**	**2020**
Revenues	$ -	$ -	$ -	$ -
Expenses:				
Research and development	7,434,000	6,109,000	14,646,000	12,927,000
General and administrative	5,457,000	5,538,000	12,025,000	11,950,000
Depreciation and amortization	824,000	834,000	1,641,000	2,899,000
Licensed technology impairment charge	-	-	-	32,916,000
Total expenses	13,715,000	12,481,000	28,312,000	60,692,000
Loss from operations	(13,715,000)	(12,481,000)	(28,312,000)	(60,692,000)
Interest and miscellaneous income	8,000	271,000	23,000	923,000
Interest expense	(1,500,000)	(800,000)	(2,920,000)	(1,400,000)
Net loss	$(15,207,000)	$(13,010,000)	$(31,209,000)	$(61,169,000)

Basic and diluted loss per common share	$ (0.16)	$ (0.14)	$ (0.33)	$ (0.66)
Weighted average number of common shares outstanding – basic and diluted	96,509,783	92,704,203	95,378,503	92,533,354
Other comprehensive (loss)/income:				
Change in unrealized (losses)/gains related to available-for-sale debt securities	(4,000)	(253,000)	9,000	133,000
Comprehensive loss	$(15,211,000)	$(13,263,000)	$(31,200,000)	$(61,036,000)

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

ASSETS	**June 30, 2021**	**December 31, 2020**
Current assets:		
Cash and cash equivalents	$ 27,179,000	$ 12,596,000
Short-term investments	50,380,000	82,438,000
Prepaid expenses and other current assets	1,321,000	2,708,000
Total current assets	78,880,000	97,742,000
Property and equipment, net	10,240,000	11,322,000
Right-of-use lease assets	6,489,000	7,032,000
Licensed technology, net	1,442,000	1,500,000
Goodwill	32,466,000	32,466,000
Other assets and restricted cash	1,158,000	1,136,000
Total assets	$ 130,675,000	$ 151,198,000
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,469,000	$ 4,695,000
Accrued expenses	2,190,000	3,410,000
Current portion of lease liability	1,720,000	1,713,000
Current portion of loan payable	1,758,000	330,000
Payable to licensor	34,434,000	31,515,000

Contract liability	296,000	296,000
Total current liabilities	41,867,000	41,959,000
Loan payable	-	1,428,000
Long-term lease liabilities	4,722,000	5,260,000
Total liabilities	46,589,000	48,647,000
Commitments and contingencies	-	-
Stockholders' equity:		
Common stock - $0.01 par value; authorized 200,000,000 shares; issued and outstanding 101,251,023 at June 30, 2021; issued and outstanding 96,131,678 at December 31, 2020	1,013,000	961,000
Additional paid-in capital	684,987,000	672,304,000
Accumulated deficit	(601,913,000)	(570,704,000)
Accumulated other comprehensive loss	(1,000)	(10,000)
Total stockholders' equity	84,086,000	102,551,000
Total liabilities and stockholders' equity	$ 130,675,000	$ 151,198,000

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