May 10, 2019

Abeona Therapeutics Reports First Quarter 2019 Financial Results and Business Highlights

CMC work completed in advance of EB-101 Phase 3 VITAL™ trial expected to begin mid-2019

IND submitted for ABO-202 in CLN1 disease

Investor conference call on Tuesday, May 14 at 10:00 a.m. ET

NEW YORK and CLEVELAND, May 10, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced first quarter 2019 financial results and business highlights, which will be discussed on a conference call scheduled for Tuesday, May 14 at 10:00 a.m. ET. Interested parties are invited to participate in the call by dialing 844-369-8770 (toll-free domestic) or 862-298-0840 (International) or via webcast at https://www.investornetwork.com/event/presentation/48814.

“We’ve had a great start to 2019, with continued progress across our pipeline, including the completion of CMC work in advance of initiating our Phase 3 VITAL™ clinical trial evaluating EB-101, our gene-corrected cell therapy for patients with recessive dystrophic epidermolysis bullosa,” said João Siffert, M.D., Chief Executive Officer. “The study, which will utilize clinical material produced at our Cleveland manufacturing facility, is on track to start mid-2019 following completion of ongoing FDA review.”

“We have continued to advance our lysosomal storage disease programs. Our increased efforts around patient recruitment are starting to bear fruit as we observe an uptick in patient screening for both MPS programs. In addition, we have submitted the IND for our CLN1 program.” added Dr. Siffert.

First Quarter Financial Results:

Cash, cash equivalents and marketable securities as of March 31, 2019 were $68.3 million, compared to $85.0 million as of December 31, 2018. The decrease in cash of $16.7 million was driven primarily by the net cash used for operating activities of $15.1 million.

Research and development expenses for the first quarter ended March 31, 2019 were $11.7 million, compared to $8.2 million for first quarter 2018. The increase in research and development expense was primarily attributable to increased R&D headcount, related facility costs and internal manufacturing costs.

General and administrative expenses for the first quarter ended March 31, 2019 were $5.7 million, compared to $2.9 million for the first quarter 2018. The increase in general and

administrative expenses was primarily attributable to increased headcount and related facility costs.

Net loss was $0.39 per share for the first quarter of 2019, compared to $0.22 per share in the same period of 2018.

First Quarter and Recent Highlights:

- May 8, 2019: Abeona Therapeutics Announces Presentation of EB-101 Data at Society for Investigative Dermatology Annual Meeting
  -- Follow up data showed that three years after treatment with investigational product EB-101, a majority of RDEB patients had durable wound healing and improvements in pain and itching. There were no serious treatment-related adverse events three years post-treatment and no replication competent virus present at any time point.
- May 1, 2019: Reported Preclinical Data Demonstrating Broad Therapeutic Potential of AIM™ Gene Therapy in Retinal Diseases at Association for Research in Vision and Ophthalmology Annual Meeting
  -- Intravitreal administration of the Company's novel AIM™ AAV204 capsid in non-human primates led to robust transgene expression in the inner and outer retina. These preclinical data support the potential use of intravitreal administration to deliver gene therapy in an out-patient setting for a wide range of inherited and acquired retinal diseases.
- April 30, 2019: Reported New Preclinical Data Demonstrating Therapeutic Potential of ABO-401 for Treatment of Cystic Fibrosis at American Society of Gene and Cell Therapy Annual Meeting
  -- ABO-401, the Company's novel gene therapy for cystic fibrosis (CF) efficiently delivered a highly-expressed, functional copy of human mini-CFTR (hCFTR) to the lung of CF mice and restored CFTR function in human CF patient nasal and bronchial epithelial cells.
- April 4, 2019: Received FDA Fast Track Designation for ABO-101 for Treatment of Sanfilippo Syndrome Type B (MPS IIIB)
- February 11, 2019: Appointed João Siffert, M.D. Chief Executive Officer
- January 31, 2019: Presented New Supportive Data for Novel Gene Therapies at WORLDSymposium™
  -- Platform and poster presentations highlighted study results on biodistribution and tissue tropism of the next-generation AIM™ AAV vector platform in Pompe and Fabry diseases, as well as data from programs in MPS IIIA and CLN3 disease.
- January 8, 2019: Strengthened Financial Leadership with New Executive Appointments: Christine Silverstein as Chief Financial Officer and Edward Carr as Chief Accounting Officer

"We expect 2019 to be a transformative year for Abeona, as we prepare to execute on a number of milestones in the clinic, in our mission to develop gene and cell therapies that could transform the treatment of serious diseases," said Steven H. Rouhandeh, Chairman of the Board and Executive Chairman. "We are encouraged to continue advancing the proprietary technology of our AIM™ vector platform, especially following recent promising data presented across multiple indications, for which we continue to evaluate avenues of development internally, and with potential partners."

About Abeona Therapeutics

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. The Company's clinical programs include EB-101, its

autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa, as well as ABO-102 and ABO-101, novel NAV® AAV9-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively. The Company's portfolio of AAV9-based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Its preclinical assets include ABO-401, which uses the novel AIM™ AAV vector platform to address all mutations of cystic fibrosis. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates and is the only company with Regenerative Medicine Advanced Therapy designation for two candidates (EB-101 and ABO-102). For more information, visit www.abeonatherapeutics.com.

Forward Looking Statement

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. These statements include but are not limited to statements related to our estimates regarding expenses, future revenues, capital requirements, and needs for additional financing; our ability to raise capital; our ability to fund our operating expenses and capital expenditure requirements for at least the next 12 months with our existing cash and cash equivalents; our expectation that we will continue to incur losses; our belief that we will expend substantial funds to conduct research and development programs; our future ability to achieve profitability at all or on a sustained basis; our cash burn rate; the dilutive effect that raising additional funds by selling additional equity securities would have on the relative equity ownership of our existing investors; our belief that we have a rich pipeline of products and product candidates; our ability to continue to develop our novel adeno-associated virus ("AAV")-based gene therapy platform technology to treat neurologic disorders, cystic fibrosis and eye disorders in human subjects; our belief that EB-101 could potentially benefit patients with recessive dystrophic epidermolysis bullosa ("RDEB"); positive feedback from regulators on our manufacturing of clinical trial product for EB-101; our ability to initiate a Phase III clinical trial for patients with RDEB; our ability to complete enrollment of patients into clinical trials to secure sufficient data to assess efficacy and safety; our belief that AAV treatment could potentially benefit patients with Sanfilippo syndrome type A ("MPS IIIA") and Sanfilippo syndrome type B ("MPS IIIB"); our ability to add clinical sites and identify additional patients for our Phase I/II clinical trial for patients with MPS IIIA and MPS IIIB; our ability to continue to secure and maintain regulatory designations for our product candidates; our ability to develop manufacturing capability compliant with current good manufacturing practices for our product candidates; our ability to manufacture gene therapy products and produce an adequate product supply to support clinical trials and potentially future commercialization; our ability to secure timely regulatory review related to our clinical program; our belief in the adequacy of the data from clinical trials in EB-101 and expansion cohort of our Phase I/II clinical trial in ABO-102 (AAV-SGSH) for MPS IIIA, together with the data generated in the program to date, to support regulatory approvals; our intellectual property position and our ability to obtain, maintain and enforce intellectual property protection and exclusivity for our proprietary assets; the rate and degree of market acceptance of our product candidates for any indication once approved; our estimates regarding the size of the potential markets for our product candidates, the strength of our commercialization strategies and our ability to serve and supply those markets; our ability to meet our obligations contained in license agreements to which we are party; and the terms of future licensing arrangements or collaborations. We have attempted to identify forward looking statements by such terminology as "may," "will," "anticipate," "believe," "estimate," "expect," "intend," 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: continued interest in our rare disease portfolio, our ability to submit protocols and protocol amendments to regulatory agencies, our ability to initiate and enroll patients in clinical trials, the adequacy of manufacturing capabilities, the impact of competition, the ability to secure licenses or establish intellectual property rights for any technology that may be necessary to continue to develop and 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 as may be detailed from time to time in the Company's annual reports on Form 10-K and quarterly reports on Form 10-Q and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or update them to reflect events or circumstances occurring after the date of this presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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