August 9, 2019

# Abeona Therapeutics Reports Second Quarter 2019 Financial Results and Business Updates

- *Five patients with MPS IIIB treated in the Transpher B Study of ABO-101 gene therapy*
- *Positive interim MPS IIIA data showed preservation of neurocognitive development in youngest patients with robust and sustained improvement in clinically relevant biomarkers*
- *Planned initiation of Phase 3 VIITAL™ study evaluating EB-101 in recessive dystrophic epidermolysis bullosa in Q4*
- *Company to host investor conference call Monday, August 12 at 10:00 a.m. ET*

NEW YORK and CLEVELAND, Aug. 09, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced second quarter 2019 financial results and business updates, which will be discussed on a conference call scheduled for Monday, August 12 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/51859.

“The second quarter was highlighted by progress made in both of our MPS III programs,” said João Siffert, M.D., Chief Executive Officer. “Data from our Transpher A study showed that children with MPS IIIA who were treated early with ABO-102 preserved neurocognitive development within the normative range 12-18 months post treatment. Our MPS IIIB program has also progressed, with enrollment of additional patients in cohort 2 of the Transpher B study. Our team remains highly focused on our lead programs, including the start of our VIITAL Phase 3 clinical trial in recessive dystrophic epidermolysis bullosa, continued enrollment in the MPS III programs, and preparations to start the clinical trial in CLN1 disease.”

**Second Quarter Financial Results:**
Cash, cash equivalents and marketable securities as of June 30, 2019, were $62.5 million compared to $68.3 million as of March 31, 2019. The decrease in cash was driven primarily by the net cash used in operating activities of $15.2 million.

Research and development expenses for the second quarter ended June 30, 2019 were $16.3 million compared to $7.9 million in the same period of 2018. The increase in R&D expense was primarily attributable to increased in-house manufacturing activities and related headcount costs.

General and administrative expenses for the second quarter ended June 30, 2019 were $5.6 million compared to $4.6 million in the same period of 2018. The increase in G&A expenses was primarily due to increased headcount and related facility costs.

Net loss was $0.49 per share for the second quarter of 2019 compared to $0.26 per share in the same period of 2018.

**Second Quarter and Recent Highlights:**

- July 25, 2019: Announced positive interim data from the Phase 1/2 AAV9 gene therapy clinical trial in MPS IIIA showing preservation of neurocognitive function for the three youngest patients treated with ABO-102, as well as robust and sustained improvements in biomarkers of the disease. No product-related serious adverse events were reported to date.
- June 26, 2019: Appointed Dr. Victor Paulus as Senior Vice President of Regulatory Affairs and Jodie Gillon as Vice President of Patient Advocacy and Clinical Affairs
- June 18, 2019: Received FDA Fast Track Designation for ABO-202 AAV9 gene therapy in CLN1 disease
- May 21, 2019: Announced FDA clearance of Investigational New Drug application for ABO-202 AAV9 gene therapy in CLN1 disease
- May 14, 2019: Announced treatment of first patient in second cohort of Phase 1/2 clinical trial for ABO-101 AAV9 gene therapy in MPS IIIB

- 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
- April 30, 2019: Reported preclinical data demonstrating therapeutic potential of ABO-401 for treatment of cystic fibrosis at American Society of Gene and Cell Therapy annual meeting
- April 4, 2019: Received FDA Fast Track Designation for ABO-101 AAV9 gene therapy for MPS IIIB

Steven H. Rouhandeh, Abeona's Executive Chairman, said, "Abeona has continued the development of its breakthrough gene and cell therapies for rare genetic diseases through 2019 with important regulatory and clinical achievements secured. We look forward to progressing our MPS programs, and to starting of our Phase 3 VIITAL trial in EB before year end."

**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 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 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. These statements include statements about the Company's clinical trials and its products and product candidates, future regulatory interactions with regulatory authorities, as well as the Company's goals and objectives. 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 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, litigation 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 periodic reports filed by the Company 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 presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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