March 18, 2019

Abeona Therapeutics Reports Fourth Quarter and Full Year 2018 Financial Results

On track to initiate Phase 3 VITAL™ trial for treatment of RDEB in mid-2019; EB-101 to be manufactured in-house

Manufacturing scaled to support AAV based programs; GMP readiness expected 2H19

Investor conference call on Tuesday, March 19 at 10:00 a.m. ET

NEW YORK and CLEVELAND, March 18, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a leading clinical-stage biopharmaceutical company developing novel cell and gene therapies for serious diseases, today announced fourth quarter and full year 2018 financial results, and provided business highlights. The Company will host a conference call on Tuesday, March 19 at 10:00 a.m. ET to discuss fourth quarter and full year results, and to provide business highlights. 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/44847.

“The diligent work conducted over the past nine months at our GMP manufacturing facility puts us on track to initiate the pivotal VITAL™ study evaluating EB-101, our gene-corrected cell therapy for the treatment of recessive dystrophic epidermolysis bullosa, in mid-2019. We will produce EB-101 at our Cleveland facility, which is an important milestone for Abeona. We are also advancing our manufacturing capabilities to support our AAV gene therapy programs and expect to be at scale for GMP production in the second half of this year,” said João Siffert, M.D., Chief Executive Officer. “On the lysosomal storage disease programs, we have stepped up efforts to accelerate patient enrollment in the MPS III programs and recently implemented the protocols to enroll younger, higher functioning patients.”

“We believe that these important steps have positioned Abeona for success in 2019 as we focus on advancing our clinical programs and developing our pipeline utilizing novel AAV capsids,” added Dr. Siffert.

Fourth Quarter and Full Year Summary Financial Results:

Cash, cash equivalents and marketable securities as of December 31, 2018 were $85.0 million, compared to $112.2 million as of September 30, 2018. The decrease in cash of $27.2 million was driven primarily by the net cash used for operating activities of $17.0 million and cash used for the acquisition of the REGENXBIO license of $10 million.

Revenues were $0.5 million for the fourth quarter of 2018 compared with $0.2 million for the fourth quarter of 2017. The increased quarterly revenues resulted from the recognition of Foundation grants that were announced during the fourth quarter of 2017.

Net loss was $0.36 per share for the fourth quarter of 2018, compared to $0.19 per share in the comparable period in 2017. For the twelve months ended December 31, 2018, net loss was $1.19 per share compared to $0.66 per share in the same period in 2017.

Fourth Quarter and Recent Highlights:

- December 6, 2018: Provided lead program updates and unveiled data from AIM™ AAV vector platform in cystic fibrosis and retinal diseases at R&D Day
- January 8, 2019: Appointed Christine Silverstein as Chief Financial Officer and Ed Carr as Chief Accounting Officer
- February 5-6, 2019: Presented new supportive data for novel gene therapies, including new proof-of-concept data for the AIM™ vector platform at WORLD*Symposium*
- February 11, 2019: Appointed João Siffert, M.D. as Chief Executive Officer

“As a fully-integrated organization, Abeona is on the forefront of cell and gene therapy thanks to in-house manufacturing facilities, the AIM™ AAV vector platform, and two programs in the clinic that have exclusive license to the AAV9 vector,” said Steven H. Rouhandeh, Chairman of the Board and Executive Chairman. “Under João’s leadership, the Company is focused on maximizing these end-to-end capabilities as it prepares for important near-

term milestones and beyond."

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include EB-101, its gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa, and ABO-102, a novel AAV9 based gene therapy for Sanfilippo syndrome type A (MPS IIIA). The Company's portfolio of AAV9 based gene therapies also features ABO-101 for Sanfilippo syndrome type B (MPS IIIB), and ABO-201 and ABO-202 for CLN3 disease and CLN1 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 investigational therapies (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 including but not limited to statements about our existing cash and cash equivalents; our belief that we have a rich pipeline of products and product candidates; our belief in our ability to continue to develop our novel AAV-vector gene therapy platform technology; our belief that EB-101 could potentially benefit patients with recessive dystrophic epidermolysis bullosa ("RDEB"); our ability to initiate a Phase III clinical trial for patients with RDEB and complete enrollment of patients into the trial; our belief that AAV treatment could potentially benefit patients with MPS IIIA and B; our ability to add clinical sites and identify additional patients for our Phase I/II clinical trial for patients with MPS IIIA and B; 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. 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.

Investor Contact:
Sofia Warner
Director, Investor Relations
Abeona Therapeutics
+1 (646) 813-4707
swarner@abeonatherapeutics.com

Media Contact:
Scott Santiamo
Director, Corporate Communications
Abeona Therapeutics
+1 (718) 344-5843
ssantiamo@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.