March 16, 2020

Abeona Therapeutics Reports Fourth Quarter and Full Year 2019 Financial Results and Business Updates

On track to treat first RDEB patient in pivotal Phase 3 VIITAL™ study of EB-101 in Q1 2020

Positive interim data from MPS IIIA and MPS IIIB programs presented at WORLDSymposium™;
Completion of cohort 2 and first patient enrolled in cohort 3 of MPS IIIB study

Issuance of U.S. Patents for AIM™ AAV capsids

Closing of $103.5 million underwritten public offering

Company to host investor conference call Tuesday, March 17 at 10:00 a.m. ET

NEW YORK and CLEVELAND, March 16, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced fourth quarter and full year 2019 financial results, as well as business updates, which will be discussed on a conference call scheduled for Tuesday, March 17 at 10:00 a.m. ET. Interested parties are invited to participate in the call by dialing 844-369-8770 (domestic) or 862-298-0840 (international) or via webcast at https://www.webcaster4.com/Webcast/Page/1818/33644.

“We have started 2020 strongly by opening enrollment in the pivotal Phase 3 VIITAL™ study evaluating EB-101 for RDEB and we remain on track to dose the first patient in the first quarter,” said João Siffert, M.D., Chief Executive Officer of Abeona. “A majority of study participants have been pre-screened and interest from the RDEB community has been high. The recently published long-term follow-up data leave us confident that VIITAL™ will demonstrate EB-101 has the unique potential to durably heal the most challenging large and chronic wounds faced by RDEB patients.”

Dr. Siffert continued, “We have also made great strides in our MPS III gene therapy programs. Positive interim data from the ongoing Phase 1/2 Transpher A study demonstrated that ABO-102 preserved neurocognitive skills up to two years after treatment in MPS IIIA patients treated early in life. In MPS IIIB, dose-dependent and sustained reductions in disease-specific biomarkers demonstrated a clear biologic effect of ABO-101 treatment in the Phase 1/2 Transpher B study, which is now enrolling a third dose cohort. Finally, our $103.5 million gross underwritten public offering fully funds operations well into 2021 and enables the continued progress and momentum across our robust clinical pipeline.”

Fourth Quarter and Recent Highlights

Opening of enrollment in the Phase 3 VIITAL™ study evaluating EB-101 for RDEB, which is on track to treat the first patient in Q1. The majority of study participants have been pre-screened and preparations for an additional clinical site initiation are ongoing.

Presentation of positive interim data from ABO-102 gene therapy program in MPS IIIA at WORLDSymposium™:

- Three young patients treated in high-dose cohort 3 (at ages 27 months, 19 months, and 12 months) continued to show preserved neurocognitive skills 18 months to two years post treatment, compared with natural history.
- Across all cohorts (n=14), biomarker improvements included rapid and sustained, dose-related reductions in CSF-HS that reached lower limit of quantitation in Cohort 3 (n=2); a reduction in plasma HS levels; and a durable, dose-dependent reduction in liver volume with up to 2 years of follow up.
- ABO-102 has been well-tolerated, with long-term safety remaining favorable 15-45 months post treatment. There have been no treatment-related severe adverse events and no clinically-significant adverse events reported.

Presentation of positive interim data from ABO-101 gene therapy program in MPS IIIB at WORLDSymposium™:

- Initial improvements in multiple disease-specific biomarkers including decreased cerebrospinal fluid heparan sulfate (HS) levels, reduced plasma and urine HS and glycosaminoglycans, reduced liver volume.
- ABO-101 has been well-tolerated to date, with no treatment-related severe adverse events and no clinically-significant adverse events reported (n=8).

Completion of cohort 2 and enrollment in cohort 3 of the ABO-101 Transpher B study in MPS IIIB.

Closing of $103.5 million underwritten public offering strengthening balance sheet to support the VIITAL™ study through data readouts and the advancement of additional clinical programs.

Receipt of EMA PRIME designation for ABO-102 program in MPS IIIA.

Issuance of two U.S. Patents for AIM™ AAV capsids.

Completion of strategic review.

Fourth Quarter and Full Year Summary Financial Results

Cash, cash equivalents and marketable securities as of December 31, 2019 were $129.3 million, compared to $47.9 million as of September 30, 2019. The increase in cash of $81.4 million was driven primarily by $103.5 million gross underwritten public offering.

Net loss was $0.30 per share for the fourth quarter of 2019, compared to $0.36 per share in the comparable period in 2018. For the twelve months ended December 31, 2019, net loss was $1.51 per share compared to $1.19 per share in the same period in 2018.

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. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates, including Regenerative Medicine Advanced Therapy designation for two candidates (EB-101 and ABO-102). 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, 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.

Investor Contact:
Dan Ferry
LifeSci Advisors, LLC
+1 (617) 535-7746
daniel@lifesciadvisors.com

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

Source: Abeona Therapeutics Inc.