November 12, 2019

Abeona Therapeutics Reports Third Quarter 2019 Financial Results and Business Updates

- *Submitted additional EB-101 transport stability data to FDA in response to Clinical Hold Letter; CMC clearance for pivotal VIITAL™ Phase 3 trial anticipated in Q4 2019*
- *Publication of positive long-term data for EB-101 demonstrated sustained wound healing, relief of pain and itch, and favorable safety profile three years post-treatment*
- *Ongoing strategic review of broad range of initiatives*
- *Company to host investor conference call Wednesday, November 13 at 10:00 a.m. ET*

NEW YORK and CLEVELAND, Nov. 12, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced third quarter 2019 financial results and business updates, which will be discussed on a conference call scheduled for Wednesday, November 13 at 10:00 a.m. ET. Interested parties are invited to participate in the call by dialing 844-602-0380 (toll-free domestic) or 862-298-0970 (international) or via webcast at https://www.investornetwork.com/event/presentation/56788.

“Abeona has made progress on our lead clinical program, EB-101, for the treatment of recessive dystrophic epidermolysis bullosa,” said João Siffert, M.D., Chief Executive Officer of Abeona. “We continue to anticipate receiving CMC clearance for this pivotal trial by year-end following the recent submission of additional transport stability data for EB-101, in response to the Clinical Hold Letter received in the quarter, as well as the submission of updated clinical and comparability protocols for the VIITAL™ Phase 3 study.”

Dr. Siffert continued, “We were also very pleased with the publication of positive long-term data from our Phase 1/2a clinical trial, reinforcing the potential of EB-101 to safely provide durable healing for the most challenging to treat, large and painful RDEB wounds. Three years post treatment, the majority of EB-101-treated wounds remained healed and without pain. We believe that EB-101 is uniquely positioned to address the needs of the majority of RDEB patients who suffer from these types of chronic wounds and we remain dedicated to delivering this therapy to the community.”

Third Quarter Financial Results:
Cash, cash equivalents and marketable securities as of September 30, 2019, were $47.9 million compared to $62.5 million as of June 30, 2019. The decrease in cash was driven primarily by the net cash used in operating activities of $18.3 million.

Research and development expenses for the third quarter ended September 30, 2019 were $10.9 million compared to $13.2 million in the same period of 2018. The decrease in R&D expense was primarily attributable to decreased clinical and development work, partially

offset by increased salary and related costs from the hiring of additional clinical, regulatory, manufacturing and quality staff.

General and administrative expenses for the third quarter ended September 30, 2019 were $4.7 million compared to $5.0 million in the same period of 2018. The decrease in G&A expenses was primarily due to decreased rental, recruiting, professional fee and salary related costs.

Net loss was $0.35 per share for the third quarter of 2019 compared to $0.34 per share in the same period of 2018.

Third Quarter and Recent Highlights:

- Submitted additional transport stability data for EB-101 in response to September 17 FDA Clinical Hold Letter regarding the planned Phase 3 VIITAL™ study.
- Submitted Phase 3 VIITAL™ clinical trial protocol with updated PRO assessments, and submitted the retrovirus comparability protocol to FDA.
- Presentation of data from the Transpher A Study, the Company's ongoing Phase 1/2 clinical trial evaluating ABO-102 for the treatment of MPS IIIA, and research updates from its library of novel AIM™ adeno-associated virus capsids at the 27th European Society of Gene and Cell Therapy Congress.
- Publication of positive long-term safety and efficacy data from the Phase 1/2a clinical trial of EB-101 in *JCI Insight* with collaborators from Stanford University School of Medicine.
  • Three years after treatment with EB-101, a majority of RDEB patients had sustained wound healing, with 80% (16/20) of wounds achieving ≥50% healing, and 70% (14/20) achieving ≥75%
  • Two years after treatment, only 1 of 6 untreated (17%), prospectively selected control wounds, had ≥50% healing
  • 50% or greater wound healing was associated with no pain (0/16) and no itch (0/16) at treated sites three years post-treatment, compared with presence of pain in 53% (20/38) and itch in 61% (23/38) of wound sites at baseline
  • EB-101 was associated with long-term molecular expression of type VII collagen protein, which plays an important role in anchoring the dermal and epidermal layers of the skin
  • No serious treatment-related adverse events were observed during the three-year observation period
- Retained Jefferies LLC as its financial advisor to assist with the review of strategic initiatives focused on advancing the Company's mission and maximizing stakeholder value.

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 twenty regulatory designations from the FDA and EMA for its pipeline candidates. 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 timing for CMC clearance for the VIITAL™ trial and the Company's beliefs relating thereto, the Company's ability to provide additional transport stability data points in response to the FDA clinical hold letter and the timing thereof, the Company's belief that completion of its CMC work and the durable safety and efficacy data will ultimately be critical to support a future Biologics License Application, the ability of its management team to lead the Company and deliver on key strategies, 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, risk of whether or when the FDA will lift the clinical hold respecting the Company's planned Phase 3 clinical trial for EB-101, 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.

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