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Abeona Therapeutics Details Pathway for Advancing Lead Clinical Programs and Unveils New Cystic Fibrosis Program Born from Next Generation AIM™ Vector Platform at 2018 R&D Day

EB-101 pivotal trial for Recessive Dystrophic Epidermolysis Bullosa planned for mid-2019 enrollment

Expanding Phase I/II study of ABO-102 for Sanfilippo syndrome type A (MPS IIIA)

Novel AIM™ AAV vector with CFTR minigene addresses all mutations of Cystic Fibrosis

NEW YORK and CLEVELAND, Dec. 06, 2018 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel cell and gene therapies for life-threatening rare genetic diseases, today announced key pipeline updates during the Company's 2018 R&D Day.

"The important clinical and preclinical updates we shared today further establish Abeona's pathway to bring long-term value to our shareholders and hope to our patients," said João Siffert, M.D., interim Chief Executive Officer, Chief Medical Officer and Head of R&D. "We are very pleased to share next steps for our lead programs and to unveil the potential of the novel AIM™ AAV vector platform that could be a catalyst for the next generation of gene therapy."

EB-101 for Recessive Dystrophic Epidermolysis Bullosa (RDEB)
Abeona is developing gene-corrected cell therapy EB-101 for the treatment of RDEB, a skin disease characterized by chronic epidermal wounds in which patients suffer from pain, itching, and widespread complications impacting quality-of-life and life expectancy.

The Company expects to initiate a pivotal clinical trial evaluating the potential of EB-101 for the treatment of RDEB in the middle of 2019. The VITAL Study will be a multicenter, randomized, Phase III clinical trial assessing 10-15 patients treated with EB-101 compared to intra-patient untreated wounds. The primary outcome measure of the study will be the proportion of treated wounds with >50% healing at three months, with secondary endpoints of investigator global assessment of wounds and changes in pain and itch from baseline.

The Company also reported that it has established GMP manufacturing capability for EB-

101 at its gene therapy manufacturing facility in Cleveland. The facility, known as the Elisa Linton Center for Rare Disease Therapies, can produce clinical product, and has scalable capacity to support the potential commercial launch of EB-101.

“We believe that we are strongly positioned to initiate a pivotal trial evaluating EB-101 by mid-2019 thanks to the important CMC work undertaken by colleagues at our gene therapy manufacturing facility in Cleveland, which also addressed guidance received through frequent regulatory interactions afforded by the Regenerative Medicine Advanced Therapy and other designations we hold. We believe this work is critical for our path towards BLA filing,” added Dr. Siffert.

ABO-102 for Sanfilippo Syndrome Type A (MPS IIIA)
Abeona is developing novel gene therapy ABO-102 for the treatment of MPS IIIA, a lysosomal storage disease with no approved treatment that is characterized by neurodevelopmental decline, behavior abnormalities, seizures, loss of speech or vision, an inability to sleep, and premature death.

The Company plans to amend its ongoing Phase I/II trial evaluating ABO-102 for MPS IIIA to enroll patients at earlier stages of disease. ABO-102 has been well tolerated to date with no serious drug-related adverse events. The study has also demonstrated a substantial, dose-related improvement in biomarkers, including reductions in cerebrospinal fluid heparan sulfate levels and liver volume in patients treated with ABO-102. Investigators also observed encouraging neurocognitive signals in younger, higher functioning patients enrolled in the higher dose of Cohort 3. Patients unable to participate in the modified Phase I/II study may be eligible to enroll in other studies within our MPS IIIA program.

“The encouraging data generated to date and our interactions with the FDA and EMA have informed the advancement of our Phase I/II trial, which will seek to enroll patients likely to receive the most benefit from treatment,” added Dr. Siffert.

ABO-401 for Cystic Fibrosis
Abeona presented new pre-clinical data today from the Company’s first program produced by the novel AIM^{TM} vector platform for gene therapy delivery. The data suggest that ABO-401, based on the vector AAV204, efficiently targets lung cells and that ABO-401 corrects the underlying cystic fibrosis (CF) chloride channel deficit, regardless of underlying mutations of the CF transmembrane conductance regulators, including the most common CF mutation, delta-F508.

AIM^{TM} Vector Platform Targeting the Eye
The Company presented non-human primate data suggesting that next-generation AIM^{TM} AAV vectors can efficiently target the retinal epithelium after intravitreal injection, creating the potential for new pipeline candidates that can address multiple eye disorders. Also presented were data showing that certain AIM^{TM} capsids with high tropisms for central nervous system tissue can evade neutralizing antibodies against naturally occurring AAV serotype, and potentially enable redosing in patients that have previously received an AAV injection.

“Our AIM™ vector platform enables the potential for gene therapy for patients living with cystic fibrosis, regardless of mutation, which could change the landscape of treatment and alter the course of this progressive, genetic disease,” said Timothy J. Miller, Ph.D., co-Founder, President, and Chief Scientific Officer. “We are very encouraged by the preclinical data presented today demonstrating delivery and correction of the underlying genetic deficit in CF patient cells. Furthermore, we are very excited to show the capability of the AIM™ vectors for delivering genes to the eye and are excited about their potential as the next generation of gene therapy across tissue types.”

ABO-202 for Infantile Batten Disease (CLN1)
Abeona is developing ABO-202 for the treatment of CLN1, a rare and fatal autosomal recessive genetic disorder with no approved treatment, which is characterized by vision impairment and rapid neurological regression.

The Company presented new preclinical data today that will inform the submission of an investigational new drug application (IND) for ABO-202 in the first quarter of 2019. Findings from a combination pre-clinical, dose-escalation study suggest that ABO-202 may have a favorable safety profile, with no significant toxicology findings. Other IND-enabling studies also demonstrated normalized survival, improvement of motor function and cognition in affected mice treated with ABO-202, and that combination dosing of intravenous and intrathecal administrations may enhance the therapeutic potential of ABO-202.

Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates and is the only company with RMAT designation for two investigational therapies (EB-101 and ABO-102).

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 (gene-corrected cell therapy) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV-NAGLU), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type B (MPS IIIB). Abeona is also developing ABO-201 (AAV-CLN3) gene therapy for CLN3 disease, ABO-202 (AAV-CLN1) for treatment of CLN1 disease, EB-201 for epidermolysis bullosa (EB), ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases, and ABO-401 using a novel AAV vector platform, AIM™ with CFTR minigene addresses 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 RMAT designation for two investigational therapies (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 regarding our pipeline and product portfolio, approval by regulatory agencies relative to amendments to INDs and to protocol amendments including related to ABO-102, timelines for initiation of further clinical studies including the pivotal study for EB-101, the establishment and scalability of manufacturing capabilities, the capabilities of the novel AIM™ vector platform in development; the market opportunities for the Company's products and product candidates, the ability to generate long term shareholder value, meet patient expectations, and achieve the company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," will," "anticipate," "believe," "estimate," "expect," "intend," and similar expressions.

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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