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Abeona Therapeutics Reinitiates Enrollment in EB-101 Pivotal Phase 3 VIITAL™ Study in RDEB after COVID-19 Related Pause and Announces Progress in Patient Enrollment in MPS III Studies

NEW YORK and CLEVELAND, July 08, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced recent updates on its clinical programs, highlighted by the restart of patient enrollment in the Company's pivotal Phase 3 VIITAL™ study of EB-101, its autologous, gene-corrected cell therapy, for the treatment of recessive dystrophic epidermolysis bullosa (RDEB). Enrollment in the VIITAL™ study at Stanford University Medical Center was paused in March 2020 in order to redirect healthcare resources to COVID-19 patients and to ensure the safety of study participants and site staff.

"RDEB is a debilitating and life-threatening rare genetic disorder without an approved treatment option. Reinitiating enrollment in the VIITAL study is important for patients with RDEB and brings us a step closer toward concluding the clinical development of EB-101," said João Siffert, M.D., Chief Executive Officer of Abeona. "The unprecedented COVID-19 global health crisis has had a broad impact across our industry, and we will continue to monitor the pandemic to ensure the safety of trial participants, healthcare professionals and our employees."

Abeona's MPS III Phase 1/2 Clinical Trials Continue Despite COVID-19 Pandemic

Abeona also announced that two ongoing MPS III Phase 1/2 clinical trials of its investigational adeno-associated virus (AAV)-based gene therapies, ABO-102 and ABO-101, have continued to treat patients, with additional enrollment expected in the programs in the coming weeks. In June 2020, the 10th patient was dosed in cohort 3 of the ABO-102 Transpher A study for MPS IIIA (Sanfilippo syndrome type A), bringing the total to 16 patients. The Company previously reported in May 2020 that additional patients were treated in dose cohort 3 of the Transpher A study and the ABO-101 Transpher B study for MPS IIIB (Sanfilippo syndrome type B), bringing the total enrollment in the Transpher B study to 9 children to date.

Dr. Siffert added, "We have worked closely with sites in our MPS III clinical studies to continue to enroll patients given the urgent need to treat children with Sanfilippo syndrome as early as possible. We are encouraged by the number of new pre-screened patients that may meet enrollment criteria and could allow dosing in the coming weeks. We also hope to resume on-site follow-up study visits that include neurocognitive assessments of already

enrolled patients."

About Recessive Dystrophic Epidermolysis Bullosa
Recessive dystrophic epidermolysis bullosa (RDEB) is a rare connective tissue disorder characterized by severe skin wounds that cause pain and can lead to systemic complications impacting the length and quality of life. People with RDEB have a defect in the COL7A1 gene, leaving them unable to produce functioning type VII collagen which is necessary to anchor the dermal and epidermal layers of the skin. There is currently no approved treatment for RDEB.

About EB-101
EB-101 is an autologous, gene-corrected cell therapy currently being investigated in the pivotal Phase 3 VIITAL™ study for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a rare connective tissue disorder without an approved therapy. The EB-101 VIITAL™ study is a multi-center, randomized clinical trial enrolling 10 to 15 RDEB patients with approximately 30 large, chronic wound sites treated in total. Treatment with EB-101 involves using gene transfer to deliver COL7A1 genes into a patient's own skin cells (keratinocytes and its progenitors) and transplanting them back to the patient to enable normal Type VII collagen expression and facilitate wound healing. Abeona produces EB-101 for the VIITAL™ study at its fully-functional gene and cell therapy manufacturing facility in Cleveland, OH. In a Phase 1/2a clinical trial, EB-101 provided durable wound healing for RDEB patients lasting 2+ to 5+ years, including for the largest, most challenging wounds that affect the majority of the RDEB population. More information on the clinical trials of EB-101 can be found at https://www.abeonatherapeutics.com/clinical-trials/rdeb and ClinicalTrials.gov (Identifier: NCT04227106).

About the VIITAL™ Phase 3 Study
The VIITAL™ Phase 3 study is a multi-center, randomized clinical trial assessing EB-101 in up to 15 RDEB patients, with approximately 30 large, chronic wound sites treated in total. The primary outcome measure is wound healing, comparing treated with untreated wound sites on the same patient. Secondary endpoints include the assessments of pain, as well as other patient reported outcomes. Investigators at Stanford University Medical Center are currently enrolling eligible patients into the VIITAL™ study. Additional information about the trial is available at https://www.abeonatherapeutics.com/clinical-trials/rdeb and ClinicalTrials.gov (Identifier: NCT04227106).

About The Transpher A Study
The Transpher A Study (NCT02716246) is an ongoing, two-year, open-label, dose-escalation, Phase 1/2 global clinical trial assessing ABO-102 for the treatment of patients with Sanfilippo syndrome type A (MPS IIIA). The study, also known as ABT-001, is intended for patients 6 months to 2 years of age, or patients older than 2 years with a cognitive Developmental Quotient of 60% or above. The gene therapy ABO-102 is delivered using AAV9 technology via a single-dose intravenous infusion. The study primary endpoints are neurodevelopment and safety, with secondary endpoints including behavior evaluations, quality of life, enzyme activity in cerebrospinal fluid (CSF) and plasma, heparan sulfate levels in CSF, plasma and urine, and brain and liver volume.

About The Transpher B Study
The Transpher B Study (NCT03315182) is an ongoing, two-year, open-label, dose-

escalation, Phase 1/2 global clinical trial assessing ABO-101 for the treatment of patients with Sanfilippo syndrome type B (MPS IIIB). The study, also known as ABT-002, is intended for patients from birth to 2 years of age, or patients older than 2 years with a cognitive Developmental Quotient of 60% or above. The gene therapy ABO-101 is delivered using AAV9 technology via a single-dose intravenous infusion. The study primary endpoints are neurodevelopment and safety, with secondary endpoints including behavior evaluations, quality of life, enzyme activity in cerebrospinal fluid (CSF) and plasma, heparan sulfate levels in CSF, plasma and urine, and brain and liver volume.

About Abeona Therapeutics

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. Abeona's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel AAV-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively, in Phase 1/2 development. The Company's portfolio of AAV-based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Abeona's library of novel, next-generation AIM™ capsids have shown potential to improve tropism profiles for a variety of devastating diseases. Abeona's fully functional, gene and cell therapy GMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and commercial production of AAV-based gene therapies. 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 the potential impacts of the COVID-19 pandemic on our business, operations, and financial condition, 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.

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