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Abeona Therapeutics Enrolls 5th Patient in Phase 1/2 Gene Therapy Clinical Trial for Epidermolysis Bullosa

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 09/08/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO)

- ***EB-101 (gene-corrected skin grafts) has demonstrated promising clinical efficacy in patients for the treatment of recessive dystrophic epidermolysis bullosa (RDEB)***
- ***Phase 1/2 clinical trial (n=5 subjects) enrolled with data indicating significant improvements in wound healing and gene expression 12 months post-grafting (n=4)***

Abeona Therapeutics Inc. (NASDAQ: ABEO) a clinical-stage biopharmaceutical company focused on delivering gene therapy for life-threatening rare diseases, announced that the fifth patient was enrolled in the Phase 1/2 clinical trial for EB-101 (gene-corrected skin grafts). EB-101 is the Company's lead gene therapy program for patients suffering with recessive dystrophic epidermolysis bullosa (RDEB), a severe form of epidermolysis bullosa (EB), a group of devastating, life-threatening genetic skin disorders impacting children that is characterized by skin blisters and erosions all over the body.

"With currently no approved FDA treatments for RDEB, the strong Phase 1/2 clinical data demonstrates that EB-101 is well-tolerated, and the efficacy reported in patients after one year of follow-up assessments is encouraging and supports our ongoing clinical strategy for children suffering from this deadly disease," said Timothy J. Miller, Ph.D, President and CEO of Abeona Therapeutics.

Also known as "Butterfly skin" syndrome, RDEB is a rare genetic skin disease that is caused by the absence of a gene (COL7A1) which encodes a protein known as type VII collagen (C7). Patients with RDEB develop large, severely painful blisters and chronic wounds from minor trauma to their skin and currently no FDA approved treatments for RDEB. The Phase 1/2 clinical trial with gene-corrected skin grafts has shown promising wound healing and safety in patients with RDEB. Investigators at Stanford are now expanding enrollment to adolescent patients for the Phase 1/2 trial to determine the safety and efficacy of COL7A1 gene-corrected grafts on wound healing efficacy. Clinical data on the initial four patients in the Phase 1/2 trial were recently presented at the opening Plenary Session of the Society for Investigative Dermatology.

"Building on and leveraging our strengths in gene therapy, rare diseases and patient partnerships, we are pleased to advance and expand enrollment for EB-101, a program that represents a significant potential treatment for patients suffering with RDEB," said Steven H.

Rouhandeh, Executive Chairman of Abeona Therapeutics.

About Epidermolysis Bullosa (EB): EB is a group of devastating, life-threatening genetic skin disorders impacting children that is characterized by skin blisters and erosions all over the body. One of the most severe form, recessive dystrophic epidermolysis bullosa (RDEB), is characterized by chronic skin blistering, open and painful wounds, joint contractures, esophageal strictures, pseudosyndactyly, corneal abrasions, and a shortened life span. Patients with RDEB lack functional type VII collagen owing to mutations in the gene COL7A1 that encodes for C7. C7 is the main component of anchoring fibrils that attach the dermis to the epidermis. EB patients suffer through intense pain throughout their lives, with few or no effective treatments available to reduce the severity of their symptoms. Along with the life-threatening infectious complications associated with this disorder, many individuals often develop an aggressive form of squamous cell carcinoma (SCC). Abeona's lead EB product, EB-101 (gene-corrected skin grafts), is a gene therapy currently in clinical trials for the treatment of RDEB patients.

About Abeona: Abeona Therapeutics Inc. is a clinical stage company developing gene and plasma-based therapies for life-threatening rare genetic diseases. Abeona's lead programs are ABO-102 (AAV-SGSH) and ABO-101 (AAV-NAGLU), adeno-associated virus (AAV) based gene therapies for Sanfilippo syndrome (MPS IIIA and IIIB), respectively. Abeona is also developing EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JBD); and ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona's has plasma-based protein therapy pipeline, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD, using our proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com.

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 that involve risks and uncertainties. These statements include, without limitation, our plans for continued development and internationalization of our clinical programs, that are looking forward to advancing multiple important new therapeutic candidates for the treatment of epidermolysis bullosa, that we plan to accelerate up to three new promising EB product candidates toward commercialization, that encouraging signs of early biopotency had been observed in urinary and CSF GAG (heparan sulfate) measurements as well as potential disease-modifying effects in the liver and spleen in our ABO-102 program, management plans for the Company, and general business outlook. These statements are subject to 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 impact of competition; the ability to develop our products and technologies; the ability to achieve or obtain necessary regulatory approvals; the impact of changes in the financial markets and global economic conditions; and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

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