

Abeona Therapeutics Honored with Partner in Progress Award from DEBRA of America

Recognized for Developing Potential Therapies for Rare Genetic Disorder, Epidermolysis Bullosa

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 11/05/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, today announced the Company was honored with a Partners in Progress Award from the Dystrophic Epidermolysis Bullosa Research Association of America (DEBRA). The award recognizes the company's work developing therapies for patients suffering with recessive dystrophic epidermolysis bullosa (RDEB), a severe form of epidermolysis bullosa (EB), a devastating life-threatening genetic skin disorder impacting children for which there is currently no cure.

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, painful blisters and chronic wounds from minor trauma to their skin and currently there are no FDA approved treatments for RDEB.

"We are honored to be recognized by DEBRA for our work towards developing breakthrough gene therapies for RDEB," said Steven H Rouhandeh, Executive Chairman. "This work is part of our exclusive collaboration with Investigators at Stanford University, who are now expanding enrollment to adolescent and adult patients for the Phase 1/2 trial to determine the safety and efficacy of COL7A1 gene-corrected grafts for wound healing."

The DEBRA of America Benefit marked the start of the annual Epidermolysis Bullosa Awareness Week, October 25-31, which was created to increase awareness of EB and promote the need for a cure, and to spur advocacy on behalf of families suffering with the emotional, financial, and physical burden of the disease.

Abeona Recent Phase 1 EB-101 Gene Therapy Clinical Trial Highlights:

- November 2nd, 2016, <u>Abeona announced JAMA Publication of Positive Phase 1 Study</u> <u>Results for EB-101 Gene Therapy Clinical Trial for Epidermolysis Bullosa</u>
- September 26th, 2016, Abeona enrolled First Patient in Phase 2 for EB-101 Gene Therapy Clinical Trial for Epidermolysis Bullosa
- September 8th, 2016, Abeona enrolled 5th Patient in Phase 1/2 Gene Therapy Clinical Trial for Epidermolysis Bullosa
- August 9, 2016, Abeona announced a collaboration with the EB Research Partnership,

EB Medical Research Foundation and Stanford University for the development of treatments for recessive dystrophic epidermolysis bullosa (RDEB).

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. 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 (C7) owing to mutations in the gene COL7A1 that encodes for C7 and is the main component of anchoring fibrils that attach the dermis to the epidermis. EB patients suffer through intense pain throughout their lives, with 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).

About Abeona: Abeona Therapeutics Inc. is a clinical stage biopharmaceutical 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 syndromes (MPS IIIA and IIIB, respectively). Abeona is also developing EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), EB-201 for epidermolysis bullosa (EB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (INCL), and ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona has a plasma-based protein therapy pipeline, including SDF Alpha[™] (alpha-1 protease inhibitor) for inherited COPD, using its 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 patients will continue to be identified, enrolled, treated and monitored in the EB-101 clinical trial, and that studies will continue to indicate that EB-101 is well-tolerated and may offer significant improvements in wound healing. 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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