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Abeona Therapeutics Announces JAMA Publication of Positive Phase 1 Study Results for EB-101 Gene Therapy Clinical Trial for Epidermolysis Bullosa

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Abeona Therapeutics Inc. (NASDAQ: ABEO):

- *EB-101 Phase 1 clinical trial therapy demonstrated clinical efficacy of 67% healed wounds at 6 month post-treatment, lasting through 12 months, including collagen biomarker expression*
- *EB-101 was well tolerated in patients with recessive dystrophic epidermolysis bullosa (RDEB)*
- *Ongoing Phase 2 clinical trial enrolling at Stanford University*

Abeona Therapeutics Inc. (NASDAQ: ABEO) a clinical-stage biopharmaceutical company focused on delivering gene therapies for life-threatening rare diseases, announced that positive clinical trial results from the EB-101 Phase I gene therapy clinical trial were published as "Safety and wound outcomes following genetically corrected autologous epidermal grafts in patients with recessive dystrophic epidermolysis bullosa" in the Journal of the American Medical Association (JAMA): http://jamanetwork.com/journals/jama/article-abstract/2576610. Abeona recently announced commencing enrollment in the Phase 2 portion of the clinical study (NCT01263379).

Typically, wounds in patients with RDEB, also known as "butterfly skin" syndrome, can remain unhealed for months to years due to the inability of the skin to stay attached to the underlying dermis and can cover a large percentage of the body. Results from the clinical study demonstrated that treatment with EB-101 restored Type VII collagen expression at the dermal-epidermal junction at the graft sites in 90% of the biopsy samples at 3 months post-treatment, in 66% at 6 months post-treatment, and in 42% samples at 12 months post-treatment. Importantly, correct type VII collagen localization was observed at anchoring fibrils. Wounds that demonstrated type VII collagen at graft sites displayed 87% healing at 3 months, 67% at 6 months, 50% at 12 months compared with baseline wound sites.

"Phase 1 data indicate that EB-101 COL7A1 *ex-vivo* gene transfer has a favorable safety profile and capable of cutaneous type C7 delivery, highlighting the potential of durable cell-based RDEB therapy in humans in devastating non-healing chronic wounds associated with high levels of morbidity and mortality," noted Steven H. Rouhandeh, Executive Chairman. "We are looking forward to completing Phase 2 enrollment and exploring approaches to make this potential breakthrough treatment available to RDEB patients."

The Phase 1 clinical trial with gene-corrected skin grafts has shown promising wound healing and safety in patients with RDEB. Investigators at Stanford University are enrolling adolescent and adult patients for the Phase 2 EB-101 trial to determine the safety and efficacy of COL7A1 gene-corrected grafts on wound healing.

"The clinical data demonstrate that EB-101 gene therapy corrected the underlying genetic deficit in RDEB patient wounds for months to over a year, and the wounds closed -- which is remarkable for a disease where the patient's skin can blister and erode every day," said Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. "We are very pleased that JAMA recognized the efforts of Drs. Peter Marinkovich, Jean Tang and the team at Stanford University for a decade of work and publish the clinical study results."

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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