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Abeona Therapeutics Receives Orphan Drug Designation in the European Union for EB-101 Gene Therapy Clinical Trial for Epidermolysis Bullosa

Abeona's Fourth Gene Therapy Program to Receive EMA Orphan Designation

EB-101 Gene Therapy for Recessive Dystrophic Epidermolysis Bullosa (RDEB) has Demonstrated Promising Efficacy and Safety in Ongoing Phase 1/2 Clinical Trial

NEW YORK and CLEVELAND, March 08, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing gene therapies for life-threatening rare diseases, announced today that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products has granted Orphan Drug Designation for Abeona's EB-101 gene therapy program for patients with recessive dystrophic epidermolysis bullosa (RDEB), a devastating, life-threatening genetic skin disorder that is characterized by skin blisters and erosions all over the body.

"EB-101 is Abeona's fourth gene therapy program to be granted EMA Orphan Designation and it further builds on our commercial portfolio of clinical-stage gene therapies that have received FDA and EMA orphan drug designations, which is an important validation of the clinical translation of these treatments for severely underserved patient populations," stated Timothy J. Miller, Ph.D., President & CEO of Abeona Therapeutics Inc. "The orphan designation also provides 10 years of market exclusivity in the European Union from similar medicines for similar indications, an important value driver for Abeona as we expand our clinical programs in Europe."

The ongoing phase 1/2 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 portion of the EB-101 clinical trial to determine the efficacy of COL7A1 gene-corrected grafts on wound healing (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 initial four patients of the clinical study demonstrated that treatment with EB-101 restored type VII collagen (C7) 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, and 50% at 12 months compared with

baseline wound sites.

“The encouraging EB-101 clinical results advances our support to address the significant unmet medical needs that RDEB patients experience and underscores our commitment to collaborating with outstanding research groups such as Stanford University,” noted Steven H. Rouhandeh, Executive Chairman of Abeona Therapeutics. “We are also very proud to collaborate with dedicated patient advocacy groups such as EB Research Partnership (EBRP) and EB Medical Research Foundation (EBMRF) to advance EB-101 for the RDEB community.”

About European Union (EU) Orphan Drug Designation: The European Commission grants orphan drug designation status to provide incentives to develop medicinal products to treat, prevent or diagnose diseases or conditions that affect no more than five in 10,000 persons in the European Union. Authorised orphan medicines, such as EB-101, benefit from ten years of protection from market competition with similar medicines for similar indications once they are approved, and provides Abeona with incentives and benefits in the EU that include protocol assistance and reduced fees once EB-101 is approved for EB patients.

About EB-101: EB-101 is an autologous, *ex-vivo* gene therapy in which COL7A1 is transduced into autologous keratinocytes for the treatment of recessive dystrophic epidermolysis bullosa (RDEB). EB-101 has been well tolerated to date and demonstrated promising efficacy in the ongoing phase 1/2 clinical trial in RDEB patients (NCT01263379).

About Epidermolysis Bullosa (EB): EB is a group of devastating, life-threatening genetic skin disorders 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 therapies for life-threatening rare genetic diseases. Abeona's lead programs include 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), 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 belief that the designation by the EMA is an important validation of the scientific and clinical translation of our products for severely underserved patient populations. 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 ability to successfully continue our 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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