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Abeona Therapeutics Enrolls First Patient in Phase 2 for EB-101 Gene Therapy Clinical Trial for Epidermolysis Bullosa

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Abeona Therapeutics Inc. (NASDAQ: ABEO)

- Phase 1 clinical trial in five patients demonstrated EB-101 (gene corrected skin grafts) was well tolerated and demonstrated promising clinical efficacy in patients for the treatment of recessive dystrophic epidermolysis bullosa (RDEB)
- Significant improvements in wound healing and gene expression observed up to 12 months post-treatment

Abeona Therapeutics Inc. (NASDAQ: ABEO) a clinical-stage biopharmaceutical company focused on delivering gene therapies for life-threatening rare diseases, announced that the first patient was enrolled in the Phase 2 portion of the 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).

"Treating the first patient in the Phase 2 portion of the EB-101 study marks a significant milestone for Abeona's efforts to advance our lead EB program. Results from the ongoing Phase 1/2 clinical data demonstrates that EB-101 is well tolerated in patients with RDEB, a very debilitating rare skin disease associated with high rates of morbidity and mortality, with no approved FDA treatment options," said Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. "The clinical data on the initial patients showed significant improvement in wound healing and gene expression through 12 months post- grafting, and that data was recently presented at a meeting of the Society for Investigative Dermatology."

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. 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 University 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 on wound healing efficacy.

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 forms, 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 Phase 1/2 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 (JNCL); ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (INCL), 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 has a 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 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.

Investor Contact:
Christine Berni-Silverstein
Vice President, Investor Relations
Abeona Therapeutics Inc
+1 (212)-786-6212
csilverstein@abeonatherapeutics.com

Media Contact:
Andre'a Lucca
Vice President, Communications & Operations Abeona Therapeutics Inc
+1 (212)-786-6208
alucca@abeonatherapeutics.com

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