Abeona Therapeutics, EB Research Partnership and EB Medical Research Foundation to Collaborate on Epidermolysis Bullosa (EB) Treatments

New York, NY and Cleveland, OH – August 9, 2016 – Abeona Therapeutics Inc. (Nasdaq: ABEO)

- ***Collaboration will focus on gene therapy treatments for epidermolysis bullosa (EB)***
- ***EB-101, an ex vivo gene therapy for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), demonstrated promising Phase 1 results***
- ***Phase 2 clinical trials expected to begin in 3Q2016 for EB-101***
- ***EB-201 is a pre-clinical candidate targeting a novel, AAV-mediated gene editing and delivery approach to correct gene mutations in skin cells (keratinocytes)***

Abeona Therapeutics Inc. (Nasdaq: ABEO), a clinical-stage biopharmaceutical company focused on delivering gene and plasma-based therapy for life-threatening rare diseases, EB Research Partnership (EBRP) and EB Research Medical Foundation (EBMRF) announced today a collaboration focusing on gene therapy treatments for epidermolysis bullosa (EB), a group of devastating rare genetic skin disorders impacting children; characterized by skin blisters and erosions all over the body.

“The addition of the EB gene therapy programs to our clinical pipeline advances our mission of serving those impacted by rare disease. The strong Phase 1 clinical data demonstrate safety and initial efficacy one year post treatment, and support a follow-on Phase 2 trial for children suffering from EB,” said Timothy Miller, PhD., President and CEO of Abeona Therapeutics.

“This collaboration builds on our strengths in developing gene therapies for devastating rare diseases in partnership with patient groups and academic research centers,” said Steven H. Rouhandeh, Executive Chairman of Abeona Therapeutics. “We are proud to work with the EB Research Partnership, EB Medical Research Foundation and Stanford University to accelerate these promising product candidates towards commercialization.”

Phase 1 clinical trial results for the lead EB program, EB-101 for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), were recently presented at the opening Plenary Session of the Society for Investigative Dermatology in May 2016. Investigators at Stanford are recruiting patients for a Phase 2 trial to begin soon. These novel gene therapy products were developed at the Stanford University School of Medicine and are exclusively licensed to Abeona.

"This collaboration exemplifies the mission of EBRP to advance commercially sustainable research aimed at treating and ultimately curing epidermolysis bullosa," stated Alexander Silver, co-founder and Chairman, EBRP. "We believe that Abeona can fully realize our mission of progressing research insights from academia into life-changing treatment solutions for EB patients and their families. This partnership also validates EBRP's venture philanthropy model, which is important in getting treatments to patients as soon as possible. We are thankful to the team at Stanford for all their hard work and assistance in forming this partnership."

Recessive dystrophic epidermolysis bullosa (RDEB) is a severe inherited blistering skin disease caused by absence of a protein known as type VII collagen. Patients with RDEB develop large, severely painful blisters and chronic wounds from minor trauma to their skin and there are currently no FDA approved treatments. The Phase 1 clinical trial with gene-corrected skin grafts has shown promising wound healing and safety in adult patients with RDEB. Investigators at Stanford are now recruiting patients for a Phase 2 trial with EB-101 in adolescents age 13 and older to determine the effect of type VII collagen 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 is recessive dystrophic epidermolysis bullosa (RDEB) 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 will 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 therapy and plasma-based therapies for severe and 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. We are also developing 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 program for rare blood diseases. In addition, Abeona is developing plasma protein therapies, 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.

About EB Research Partnership (EBRP): EBRP is the largest 501(c)(3) nonprofit dedicated to funding research aimed at treating and ultimately curing Epidermolysis Bullosa, a group of devastating and life-threatening skin disorders that affect children from birth. EBRP uses a sustainable philanthropic model via venture philanthropy for all of its research commitments. To learn more, please visit ebresearch.org.

About EB Medical Research Foundation (EBMRF): The Epidermolysis Bullosa Medical Research Foundation was founded in 1991 with the mission of funding a cure for EB and is currently headed by The Joseph Family in Los Angeles. The Foundation is dedicated to raising funds and awareness for critical EB research, as well as providing outreach through the media and various fundraising programs. To learn more, please visit www.ebkids.org

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, management plans for the Company, our expectation to accelerate our product candidates towards commercialization, 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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