

April 25, 2017



Abeona Therapeutics Announces EB-101 Clinical Update to be Presented at the Society for Investigative Dermatology Annual Meeting

Oral Presentation and Poster Sessions Highlighting EB-101 Gene Therapy Clinical Trial Updates and Natural History Study Data for Patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB)

NEW YORK and CLEVELAND, April 25, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene therapies for life-threatening rare genetic diseases, today announced that updated Phase 1/2 clinical trial data on the EB-101 gene therapy program and supportive natural history data for patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB) will be highlighted at the upcoming Society for Investigative Dermatology (SID) conference to be held April 26-29, 2017 in Portland, OR.

“Recessive Dystrophic Epidermolysis Bullosa, also known as butterfly skin syndrome, is a painful and ultimately disfiguring disease that results in early deaths. The EB-101 clinical data update highlights the wound closure and collagen biomarker expression continues for over two years in multiple patients, which are critically important parameters of efficacy in patients with RDEB,” said Timothy J. Miller, Ph.D., President and CEO.

EB-101 was administered to non-healing chronic wounds [mean length of time wounds were unhealed (unclosed) was 8.5 years] on each subject and assessed for wound healing at predefined time points over years. The primary endpoint of the clinical trial is to assess safety and evaluate wound closure compared to untreated wounds. Secondary endpoints include expression of full-length C7 and restoration of anchoring fibrils at three and six months post-administration.

Significant wound healing, defined as >75% closure from baseline, was observed in 94% (27/36 grafts) at 3 months, 67% (16/24 grafts) at 6 months and 50% (12/24 grafts) at 12 months post-administration. In addition, C7 collagen expression and morphologically normal NC2 reactive anchoring fibrils – the “zipper” that holds skin onto the underlying tissue and the primary deficit in RDEB patients - have been observed in tissue biopsies in the 4 subjects that are through two years of follow-up. By comparison, all untreated control wounds remained unhealed (0% wound closure) over the same time frame. Importantly, all subjects (n=35) in the supportive natural history study failed to close wounds (0% wound healing) using a comparison product of non-gene corrected keratinocyte graft (Apligraf®) treatment by 7 weeks post-application. This is a significant finding of the natural history study, as there are no approved therapies for RDEB patients that demonstrate significant

wound closure after two months post-application.

Details for the oral presentations and poster sessions are listed below:

Presentation Title: "Phase I/IIa Clinical Trial for Recessive Dystrophic Epidermolysis Bullosa Using Genetically Corrected Autologous Keratinocytes"

Presenter: Zurab Siprashvili, Ph.D.

Abstract Final ID: 519

Oral Presentation, Friday, April 28th, 2:00 p.m. – 4:30 p.m. PT

Room: Oregon Ballroom 204

Abstract Title: "Quality of Life in Recessive Dystrophic Epidermolysis Bullosa: The AltaVoice Patient Registry, 2012-2015"

Presenter: Sara Choi

Abstract Final ID: 224

Poster Session I: Thursday, April 27th, 2017, 10:15 a.m. – 12:15 p.m. PT

Room: Exhibit Hall A

Abstract Title: "Natural History of Chronic Wounds in Patients with Recessive Dystrophic Epidermolysis Bullosa"

Presenter: Daniel C. Solis, BA

Abstract Final ID: 219

Poster Session II: Friday, April 28th, 2017, 11:30 a.m. – 1:30 p.m. PT

Room: Exhibit Hall A

Abstract Title: Phase I/IIa Clinical Trial for Recessive Dystrophic Epidermolysis Bullosa Using Genetically Corrected Autologous Keratinocytes"

Presenter: Zurab Siprashvili, Ph.D.

Abstract Final ID: 519

Poster Session III: Saturday, April 29th, 10:15 a.m. – 12:15 p.m. PT

Room: Exhibit Hall A

About EB-101: Recessive Dystrophic Epidermolysis Bullosa (RDEB) is a subtype of an inherited genetic skin disorder 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 and is the main component of anchoring fibrils, which stabilize the dermal-epidermal basement membrane. 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 Phase 2 portion of the ongoing Phase 1/2 clinical trial in RDEB patients (NCT01263379).

About Society of Investigative Dermatology (SID): The Society for Investigative Dermatology was founded in 1937 to highlight and promote discovery in investigative dermatology and to better represent skin-related scientific investigation amongst medical societies. Soon after, it launched the Journal of Investigative Dermatology (JID), a source for all advancements in cutaneous biology. The first Annual Meeting was held in New York City in 1938. The 76th Annual SID Meeting, like those before it, will bring together researchers,

lecturers, and industry leaders in dermatology. Throughout the four-day conference, cutting-edge research, findings and trends in skin health and disease will be shared. All abstracts will be published in upcoming editions of the JID. For more information, please visit the conference website at <http://www.sidnet.org/>.

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.

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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.



Source: Abeona Therapeutics Inc