February 24, 2016

Abeona Therapeutics, Inc. to Present on CRISPR/CAS9 Technology Platform Progress at CRISPR Precision Gene Editing Congress

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 02/24/16 -- Abeona Therapeutics, Inc. (NASDAQ: ABEO), a biopharmaceutical company focused on developing and delivering gene therapy and plasma-based products for severe and life-threatening rare diseases, today announced being featured in both a Poster and Abstract Presentation at the 2nd Annual CRISPR Precision Gene Editing Congress in Boston, MA. The Poster and Abstract session will focus on CRISPR/Cas9 as a Promising Gene Editing Tool for Fanconi anemia and other rare blood disorders.

CRISPR/CAS9:

Introduction: Fanconi anemia (FA) is a rare inherited disease manifested by bone marrow failure and increased risk of malignancy. The c.456 + 4A>T (IVS4 + 4A>T) point mutation in FA complementation group C (FA-C) gene results in a cryptic splice site and causes aberrant splicing and in-frame deletion of FANCC exon 4. Gene editing is highly desirable alternative to allogeneic hematopoietic cell transplantation (HCT) for FA. In the present study, we have generated a CRISPR/Cas9 system for FANCC locus and demonstrated its usefulness in repairing the FANCC c.456+4A>T mutation.

Methods and Results: To test the ability of our custom-designed CRISPR/Cas9 reagents to mediate FANCC gene HDR, a transformed skin fibroblast culture was derived from an FA-C patient homozygous for the c.456+4 A>T mutation. The cells were treated with a donor plasmid and either the CRISPR/Cas9 nuclease or nickase. To determine whether genome editing by CRISPR/Cas9 resulted in restoration of exon 4 expression, HDR-specific PCR was performed using an allele specific RT-PCR. Interestingly, CRISPR/Cas9 nuclease and nickase clones each identified correction of c.456+4A>T compared to untreated and WT controls. Furthermore, to evaluate functional capability of our gene editing method, H2AX staining clearly demonstrated inability of untreated FA-C cells to phosphorylate γ-H2AX, however, the clones that were corrected by the nickase or the nuclease showed clear evidence to phosphorylate γ-H2AX. These findings confirm correction of the c.456+4A>T mutation at DNA, RNA and protein level.

An important safety concern of gene editing based correction strategy is potential for off target (OT) effects. To assess this important safety issue, a surveyor assay and an integration deficient lenti virus (IDLV) reporter gene trapping assay was performed and no OT activity for the nuclease or nickase was observed. Moreover, to identify the sites of integration of the IDLV, the samples were tested using LAM PCR and nonrestrictive (nr)

LAM PCR, these results documented only on target events. In total, the data suggests highly specific CRISPR/Cas9 reagents.

Conclusions: To summarize, this data show that CRISPR/Cas9 mediated direct c.456 + 4A>T mutation repair resulted in normalization of the FANCC gene. This study also demonstrates that nickase was more efficient and reliable compared to nuclease. Furthermore, the gene editing model system established here provides support for a favorable safety profile using these synthetic molecules for correction of FA and other genetic disease in human cells.

About Abeona: Abeona Therapeutics, Inc. develops and delivers gene therapy and plasma-based products for severe and life-threatening rare diseases. Abeona's lead programs are AB0-101 (AAV NAGLU) and ABO-102 (AAV SGSH), adeno-associated virus (AAV)-based gene therapies for Sanfilippo syndrome (MPS IIIB and IIIA). 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, we are also developing rare 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.

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, 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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Source: Abeona Therapeutics, Inc.