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Abeona Announces $13.85M Grant from Leading Sanfilippo Syndrome Foundations for Clinical Development of MPS III Gene Therapies

- ***Nine global foundations collaborate to grant $13.85 million for the continued advancement of lead Sanfilippo gene therapy programs***
- ***Company receives infusion of an additional $5 million from exercise of outstanding warrants, totaling $18.85 million in proceeds***

NEW YORK and CLEVELAND, Oct. 16, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene and cell therapies for life-threatening rare diseases, announced today a collaborative agreement between nine Sanfilippo foundations to provide approximately $13.85 million of grants to Abeona in installments for the advancement of the Company's clinical stage gene therapies for Sanfilippo Syndrome Type A (MPS IIIA) and Sanfilippo Syndrome Type B (MPS IIIB).

"Abeona is pleased to continue our global collaboration with the Sanfilippo foundations to help further advance our gene therapy programs for MPS III disease," said Timothy J. Miller, Ph.D., president and chief executive officer of Abeona Therapeutics. "The effort and expertise that we continue to commit to the ABO-102 and ABO-101 programs puts us in a strong position to further extend the important progress reported to date. We are grateful to the foundations for their ongoing commitment to identifying and facilitating the development of clinical innovation to treat patients with MPS III disease."

"Stop Sanfilippo considers that, based on the very good clinical data recently published by Abeona on the Phase I/II trial, this is a great opportunity to support a further step on this program making it possible to treat more patients and allowing a broader clinical indication for this potential gene therapy potential treatment," said Emilio Lopez Alvarez, President of Stop Sanfilippo in Spain.

Team Sanfilippo Foundation, Stop Sanfilippo Fundación, Fundación Sanfilippo B, Sanfilippo Children's Foundation, the National MPS Society, the Red Sanfilippo Foundation, the Children's Medical Research Foundation, Abby Grace Foundation, and Fondation Sanfilippo Suisse collectively collaborated on the grant to Abeona.

"The importance of reducing the heparan sulfate as a cause of disease burden cannot be understated, and the clinical data demonstrated by Abeona enabled us to provide additional support in the pursuit of finding new paradigms to treat all children with Sanfilippo syndrome," stated Carl Kapes, Board Member of Team Sanfilippo.

Additionally, Abeona received $5.0 million through the cash exercise of 625,000 common stock purchase warrants. Each warrant was exercised to purchase one share of common stock for $8.00 per share. The warrants were issued as part of a $10.0 million financing completed in May 2015 and would have otherwise been exercisable until November 2017.

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), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB). Abeona is also developing ABO-101 (AAV-NAGLU) for Sanfilippo syndrome type B (MPS IIIB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) for treatment of infantile Batten disease (INCL), EB-201 for epidermolysis bullosa (EB), 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 proprietary vector platform, AIM™, for next generation product candidates. 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 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 and licenses; 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.