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Abeona Therapeutics Provides Clinical Update on MPS IIIA Gene Therapy Trial at the 21st Annual ASGCT Meeting

- *ABO-102 18-month efficacy and safety data continue todemonstrate time- and dose-dependent reductions in underlying disease pathology, including decreased CSF and urine GAGs and improved liver volumes*
- *11 subjects enrolled through > 4,200 days cumulative follow up*

NEW YORK and CLEVELAND, May 18, 2018 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel cell and gene therapies for life-threatening, rare genetic diseases, announced today updated clinical data from the Phase 1/2 trial for ABO-102 (AAV-SGSH), the company's clinical gene therapy for the treatment of Sanfilippo syndrome type A (MPS III A) during the 21st Annual Meeting of the ASGCT (American Society for Gene and Cell Therapy) in Chicago, IL. The ongoing ABO-102 (AAV-SGSH) trial results demonstrate robust and durable clinical effects achieved throughout various timepoints post-administration. To date, 11 patients have been dosed with a single intravenous injection of ABO-102. MPS IIIA is a rare, autosomal-recessive, lysosomal storage disease that results in the accumulation of the heparan sulfate.

"Children with MPS IIIA experience devastating quality of life consequences including neurocognitive decline, speech and mobility loss, and premature death," stated Carsten Thiel, Ph.D., CEO of Abeona. "As a leader in gene therapy for MPS IIIA patients, we feel encouraged by the strong data demonstrated thus far in this trial, showing significant dose- and time-dependent improvement of the underlying disease pathology. With the recently granted RMAT designation, we look forward to continuing our regulatory discussions to advance this promising therapy for patients."

Each subject received a single intravenous injection of the gene therapy for systemic delivery of a functional copy of the missing SGSH gene associated with onset and progression of the disease. Select updated data from the presentation are highlighted below:

Biopotency Assessments: ABO-102 continues to demonstrate significant dose-dependent and time-dependent responses in key biomarkers through 18-months post-injection, including sustained reductions of heparan sulfate, the sugar molecule that is the hallmark of MPS IIIA, in the cerebral spinal fluid (CSF) and urine.

CSF heparan sulfate:

- Day 360 assessment
    - Cohort 1 (n=2) demonstrated a reduction of 69.3%
    - Cohort 2 (n=2) demonstrated a reduction of 65.7%
- Day 180 assessment
    - Cohort 1 (n=3) demonstrated a reduction of 58.7%
    - Cohort 2 (n=3) demonstrated a reduction of 60.5%
    - Cohort 3 (n=1) demonstrated a reduction of 83.3%
- Day 30 assessment
    - Cohort 1 (n=3) demonstrated a reduction of 25.8%
    - Cohort 2 (n=3) demonstrated a reduction of 52.1%
    - Cohort 3 (n=4) demonstrated a reduction of 67.1%

Urine heparan sulfate:

- Day 540 assessment
    - Cohort 1 (n=3) demonstrated a reduction of 30.0%
- Day 360 assessment
    - Cohort 1 (n=3) demonstrated a reduction of 29.2%
    - Cohort 2 (n=2) demonstrated a reduction of 45.1%
- Day 180 assessment
    - Cohort 1 (n=3) demonstrated a reduction of 29.2%
    - Cohort 2 (n=2) demonstrated a reduction of 57.6%
    - Cohort 3 (n=1) demonstrated a reduction of 75.0%
- Day 90 assessment
    - Cohort 1 (n=3) demonstrated a reduction of 54.2%
    - Cohort 2 (n=3) demonstrated a reduction of 63.1%
    - Cohort 3 (n=4) demonstrated a reduction of 77.1%
- Day 30 assessment
    - Cohort 1 (n=3) demonstrated a reduction of 64.2%
    - Cohort 2 (n=3) demonstrated a reduction of 54.0%
    - Cohort 3 (n=3) demonstrated a reduction of 90.3%

Biophysical Assessments: A supportive natural history study (Truxal et. al., 2016, Mol. Genet. Metab.) in MPS IIIA demonstrated that subjects showed, on average, 2.2 times increased liver volumes over normal. Results from the Phase 1/2 clinical trial for ABO-102 demonstrate durable biophysical reductions of disease burden including reductions in liver volume.

Safety Assessments: ABO-102 is well-tolerated in all subjects to date, with no drug-related serious adverse events (SAE) reported through over 4,200 cumulative days post-injection.

“MPS IIIA is a serious and deadly lysosomal storage disease with no approved treatments available. The durability observed in the time- and dose-dependent responses reported today provide strong support for a whole-body treatment in this lethal disease,” stated Kevin M. Flanigan, M.D., principal investigator of the trial, director of the Center for Gene Therapy at Nationwide Children’s Hospital and professor of pediatrics and neurology at The Ohio State University College of Medicine. “We are especially pleased to see

sustained decreases in CSF heparan sulfate and liver volumes in all subjects post-injection."

ABO-102 has been granted Regenerative Medicine Advanced Therapy, Rare Pediatric Disease, and Fast Track designations in the United States, and Orphan Product Designation in both the United States and the European Union.

About Abeona: Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV-NAGLU), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type B (MPS IIIB). Abeona is also developing ABO-201 (AAV-CLN3) gene therapy for CLN3 disease, ABO-202 (AAV-CLN1) for treatment of CLN1 disease, 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 is developing 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 Section 21E of the Securities Exchange Act of 1934, as amended and that involve risks and uncertainties. These statements include statements that the ongoing ABO-102 trial results demonstrate robust and durable clinical effects achieved throughout various time points post-administration and that we feel encouraged by the strong data demonstrated thus far in the MPS IIIA gene therapy trial, showing significant dose and time-dependent improvement of the underlying disease pathology, and expectation to continue to have regulatory discussions to advance this promising therapy for patients. We have attempted to identify forward looking statements by such terminology as "may," will," "anticipate," "believe," "estimate," "expect," "intend," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important

factors, 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 secure licenses for any technology that may be necessary to commercialize our products, the ability to achieve or obtain necessary regulatory approvals, the impact of changes in the financial markets and global economic conditions; the results of our ongoing trials; risks associated with data analysis and reporting, and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and quarterly reports on Form 10-Q 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, except as required by the federal securities laws.

Source: Abeona Therapeutics Inc.