October 11, 2017

Abeona Therapeutics Enrolls First Two Patients in Pivotal Expansion of Phase 1/2 Clinical Trial in MPS IIIA

- ***ABO-102 Patients Enrolled in Cohort 3 at 3E13 vg/kg***
- ***Accelerated enrollments begin with first Patient Enrolled at clinical site in Australia; Patient Screening at Spain site underway***
- ***Day 30 results demonstrate 67% Heparan sulfate reduction in the CNS and 92% reduction in urinary heparan sulfate and normalization of 76% points in liver volume***

NEW YORK and CLEVELAND, Oct. 11, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene therapies for life-threatening rare diseases, announced today that two patients were enrolled in the Company's ABO-102 Phase 1/2 clinical trial, at sites in Australia and the US. Both patients have been treated with the Company's Cohort 3 dose of ABO-102 (3×10^{13} vg/kg).

"The recently announced one-year data on our Cohort 1 patients showed durable, time-dependent responses as measured in reductions of heparan sulfate storage pathology in the CSF and urine, reduction in liver volume, stabilization of deep brain architecture and signals of stabilization of neurocognitive decline one-year post-administration. After seeing dose-dependent improvements in Cohort 2, with ABO-102 being well tolerated to date, Abeona, together with our principal investigators, dose-escalated to potentially enhance clinical benefits and prolong durability; a decision supported by the regulatory agencies across the three countries supporting our trial," stated Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. He continued, "ABO-102 continues to be well-tolerated at all doses at all follow up timeframes, and has enabled an accelerated enrollment schedule over the coming months. We look forward to reporting additional clinical data in the ABO-102 global trial later this year."

Per the design of the pivotal expansion, subjects in the ABO-102 trial receive a single, intravenous injection of ABO-102 to systemically deliver the AAV viral vector throughout the body and CNS, introducing a corrective copy of the SGSH gene that underlies the cause of the MPS IIIA disease. Subjects are evaluated at multiple time points post-injection for safety assessments and initial signals of biopotency and clinical activity, which indicate that ABO-102 successfully reached target tissues throughout the body, including the central nervous system.

Biopotency Assessments: ABO-102 demonstrated a significant reduction of the sugar molecule that is the hallmark of the diseases in the CNS, heparan sulfate (HS) in the first Cohort 3 subject that has reached 30 days post-injection:

--66.7% reduction in CSF heparan sulfate
--92.3% reduction in urinary heparan sulfate

Biophysical Assessments: Hepatomegaly – the first Cohort 3 subject through 30 days post-injection demonstrated a 76% reduction in liver volume.

"From numerous pre-clinical, clinical and natural history studies of many rare neurodegenerative conditions, including MPS IIIA and other lysosomal storage diseases, it is strongly suggested that treatment outcomes will be improved by earlier intervention, at the highest possible tolerated doses," stated Juan Ruiz, M.D., Ph.D., Abeona's Chief Medical Officer. "We remain encouraged by the early outcome data of all enrolled patients that shows a clear dose-dependent and time-dependent response to ABO-102, and a good safety profile to date," he continued.

Abeona enrolled the first patient at Adelaide Women's and Children's Hospital in Adelaide, Australia and the additional patient at the US site, Nationwide Children's Hospital. The Company also commenced patient screening at the Spain clinical site, Clinico Universitario de Santiago de Compostela.

"We are pleased to initiate our enrollment in the ABO-102 trial," stated Nick Smith, M.D., Ph.D, Principal Investigator and Department Head of Neurology at the Adelaide Women's & Children's Hospital in Australia. "MPS IIIA is a profoundly disabling and progressive neurodegenerative disease with no approved treatments available. The encouraging clinical data from all cohorts to date, continues to support a whole-body treatment approach using an intravenously delivered AAV to deliver and remove disease pathology in multiple organs of the body, particularly the brain. We are grateful to the many patient foundations and parents who have supported the research needed to advance a potential treatment for this devastating unmet medical need."

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.

Investor Contact:
Christine Silverstein
Vice President, Investor Relations
Abeona Therapeutics Inc.
+1 (212)786-6212
csilverstein@abeonatherapeutics.com

Media Contact:

Lynn Granito
Berry & Company Public Relations
+1 (212) 253-8881
lgranito@berrypr.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 expectation that we will continue to advance our gene therapy for MPS IIIA patients, our expectation of accelerating enrollment with our active global sites in Spain and Australia, and that we remain encouraged by signs of tolerability and biological effects observed in Cohort 1 post injection. Such 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 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; our belief that initial signals of biopotency and clinical activity, which suggest that ABO-102 successfully reached target tissues throughout the body, including the central nervous system and the increased reductions in CNS GAG support our approach for intravenous delivery for subjects with Sanfilippo syndromes, 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.

Source: Abeona Therapeutics Inc