

May 11, 2018



# Abeona Therapeutics Reports First Quarter 2018 Financial Results and Business Highlights

## Investor Conference Call on Monday, May 14th at 10:00 am ET

NEW YORK and CLEVELAND, May 11, 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, today announced financial results for the first quarter of 2018. The Company will host a call to update investors on recent clinical developments and quarter financial results on Monday, May 14<sup>th</sup> at 10:00 am (Eastern). Interested parties are invited to participate in the call by dialing 877-407-9210 (toll free domestic) or 201-689-8049 (International) or via webcast <http://www.investorcalendar.com/event/29196>.

“Abeona has made significant progress across multiple fronts, including various regulatory achievements, additional enrollments in our MPS IIIA trial, and the reporting of encouraging initial results for our MPS IIIB trial,” stated Carsten Thiel, Ph.D., Abeona’s CEO. “Notably, both our lead clinical programs, EB-101 and ABO-102, were recently granted RMAT designation by the FDA, supporting the strong safety and biopotency evidence from these programs, and their potential to address the unmet medical need for RDEB and MPS IIIA patients.”

### 1<sup>st</sup> Quarter Summary Financial Results:

- Cash position: Cash and cash equivalents as of March 31, 2018 were \$132 million, compared to \$137.8 million as of December 31, 2017.
- Revenues: Revenues were \$2.6 million for the first quarter of 2018, compared to \$186 thousand in the first quarter of 2017. A large portion of the increased quarterly revenues consisted of the recognition of Foundation grants that were announced during the 4th quarter of 2017. A portion of the grants were received in the 4th quarter of 2017 and in the 1st quarter of 2018, and the amount recognized is matched against corresponding expenditures for drug manufacture and clinical readiness. Additional revenues consisted of royalties from marketed products, specifically MuGard. In the quarter, Abeona adopted ASC 606 pertaining to revenue recognition, and therefore there will no longer be any recognition of deferred revenues related to upfront payments from earlier license agreements.
- Loss per share: Loss per share was \$0.18 for the first quarter of 2018, compared to a loss per share of \$0.13 in the comparable period in 2017.

### Abeona Recent Highlights:

April 23, 2018: Announced FDA Grants RMAT Designation to ABO-102 Gene Therapy in

## MPS IIIA

April 20, 2018: Announced EMA Grants Orphan Drug Designation in the European Union for ABO-202 Gene Therapy Program in Batten Disease

April 2, 2018: Announced Appointment of Carsten Thiel, Ph.D., as Chief Executive Officer

March 15, 2018: Announced FDA Grants Rare Pediatric Disease Designation for ABO-202 Gene Therapy Program in CLN1 Disease

February 12, 2018: Announced FDA Grants Orphan Drug Designation for ABO-202 Gene Therapy Program in Infantile Batten Disease

February 8, 2018: Reported Top-Line Data from Phase 1/2 Gene Therapy Trial in MPS IIIA

-- ABO-102 results presented at WORLDSymposium for Lysosomal Diseases show significant time- and dose-dependent reduction of underlying disease pathology, including decreased CSF and urine GAGs (HS fragments) and diminished liver volumes

-- Evidence of cognitive benefit at six months post treatment in Cohort 2 and at one year in Cohort 1

-- Company receives FDA allowance to lower enrollment age to six months

February 7, 2018: Reported on Initial Safety and Biopotency Signals in MPS IIIB Gene Therapy Clinical Trial

-- ABO-101 is well tolerated and demonstrates early biopotency signals with significant disease-specific heparan sulfate (HS) reductions in cerebral spinal fluid, urine, and plasma and greater than 300-fold increase in NAGLU enzyme activity observed in first subject at 30 days post injection

January 29, 2018: Announced FDA Grants Regenerative Medicine Advanced Therapy Designation for EB-101 Gene Therapy in Epidermolysis Bullosa

“2018 continues to be a year of execution for Abeona. With the expanded leadership, we look to a number of milestones in the year including the commencement of our pivotal Phase 3 trial in EB, the completion of enrollment in our Phase 1/2 trial in MPS IIIA, and significant progress on our in-house GMP manufacturing facility,” stated Steven H. Rouhandeh, Abeona’s Executive Chairman. “With the team focusing their efforts and leading the charge on clinical development, we look forward to our continued dialogue with the regulatory bodies and advancing our gene therapies to rare disease patients.”

**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](http://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, without limitation, statements about our ability to develop our products and technologies; our plans for continued development and internationalization of our clinical programs; our expectation to commence our pivotal Phase3 trial in EB; 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; the completion of enrollment in our Phase 1/2 trial in MPS IIIA; we have recently initiated enrollment in our MPS IIIB program; our expectation that we will continue to advance our gene therapy for MPS IIIA patients; and our goal of achieving significant progress on our in-house GMP manufacturing facility. 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.