

November 9, 2018



Abeona Therapeutics Reports Third Quarter 2018 Financial Results and Business Highlights

- *Investor Conference Call on Monday, November 12th at 10:00 a.m. ET*

NEW YORK and CLEVELAND, Nov. 09, 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 third quarter of 2018.

The Company will host a conference call on Monday, November 12th at 10:00 a.m. ET to discuss third quarter results and business highlights. 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/40030>

"In the third quarter our team has worked very hard to advance our lead programs and to further strengthen our pipeline," stated Carsten Thiel, Ph.D., Chief Executive Officer of Abeona. "Our recently announced agreement with REGENXBIO is an exciting development, as we build on our existing expertise and the success we've seen to date with the use of the AAV9 vector in our clinical and preclinical trials. We are now one step closer to bringing these transformative therapies to the patient communities that need them most."

3rd Quarter Summary Financial Results:

- Cash position: Cash, cash equivalents and marketable securities as of September 30, 2018 were \$112.2 million, compared to \$120 million as of June 30, 2018.
- Revenues: Revenues were \$1.7 million for the third quarter of 2018 compared with \$219 thousand for the similar quarter the year prior. A portion of the increased quarterly revenues consisted of the recognition of Foundation grants that were announced during the fourth quarter of 2017. A portion of the grants were received in the third quarter of 2018, and the amount recognized is matched against corresponding expenditures. Additional revenues consisted of royalties from marketed products, primarily MuGard®.
- Loss per share: Loss per share was \$0.34 per share for the third quarter of 2018 compared to \$0.13 per share in the comparable period in 2017.

3rd Quarter and Recent Highlights:

- The Company is announcing today that Edward Carr will join as Vice President, Controller, effective as of November 26, 2018, and will serve as principal accounting officer, replacing Stephen Thompson, who will retire from the Company at the end of

the year

- November 05, 2018: Abeona Therapeutics and REGENXBIO Announce Worldwide Exclusive Licenses for the Treatment of Four Rare Lysosomal Storage Disorders Using NAV AAV9 Vector, MPS IIIA, MPS IIIB, CLN1, and CLN3
- October 18, 2018: Announced Appointment of João Siffert, M.D. as Head of Research and Development and Chief Medical Officer
- September 12, 2018: Announced Authorization to Commence Phase 1/2 Gene Therapy Clinical Study for Patients with MPS IIIB in Spain
- July 26, 2018: Announced Leadership Appointments with Max Colao as Chief Commercial Officer

“As we look to yearend, we anticipate expanding on the continued progress and momentum of our clinical and pre-clinical gene therapy pipeline and further advancing the commercial readiness of our in-house manufacturing facility,” stated Steven H. Rouhandeh, Executive Chairman of Abeona. “We believe we are well-positioned as a leader in the development of gene and cell therapies with significant potential to treat life-threatening, rare genetic diseases.”

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 regarding our continued progress and momentum of our

clinical and pre-clinical gene therapy pipeline, our ability to advance our programs, commercial readiness of our in-house manufacturing facility and that we are well-positioned as a leader in the development of gene and cell therapies. 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, 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; 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.