Abeona THERAPEUTICS

Abeona Therapeutics (Nasdaq: ABEO) is a fully-integrated gene and cell therapy company at the forefront of the rapidly-advancing field of genetic medicine. The Company's multi-platform expertise across the research, development, manufacture, and discovery of novel gene and cell therapies has it uniquely positioned to bring new medicines to patients in need.

Our Commitment

Our strategy to harness the promise of genetic medicine to transform the lives of patients is inspired by our vision, to realize a world where cure is the new standard of care. We are working together to deliver gene and cell therapies for people impacted by serious diseases.

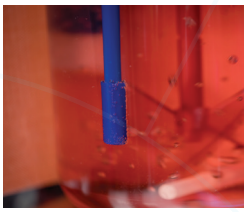
Manufacturing cGMP-compliant Gene and Cell Therapies

Abeona is underpinned by its fully-operational, cGMP-compliant manufacturing facility where the Company is currently producing therapies and vectors used in its clinical and preclinical studies. The in-house facility will manufacture product for Abeona's upcoming Phase 3 clinical trial of EB-101, its gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa (RDEB). The Elisa Linton Center for Rare Disease Therapies is expected to be scaled for GMP production of Abeona's adeno-associated virus (AAV)-based gene therapies in the second half of 2019. Complementing manufacturing are established CMC capabilities in process and assay development that are validated and governed by comprehensive quality systems, and expansion is underway to establish state-of-the-art labs housing this expertise. In total, centralizing manufacturing and surrounding disciplines strengthens control of supply, timelines, and costs and reduces the risks of outsourcing this highly-specialized work. These are critical steps toward transitioning from clinical- to commercial-scale production and delivering gene and cell therapies to patients.

Fostering the Next Generation of AAV Gene Therapy

Abeona is developing the AIM™ Vector Platform: next-generation AAV capsids for use in gene therapies. The AIM™ capsid library can utilize AAV biology to selectively target delivery of genetic payloads to the central nervous system, lungs, eye, muscle, liver and other tissues. AIM™ vectors are non-replicating and have shown the

potential to evade the immune responses generated by exposure to naturally-occurring AAV vectors. The Company's AIM™ library contains more than 100 capsids with tissue tropisms selected for their potential to target a wide range of organs and multiple routes of delivery.



Advancing a Robust and Diverse Pipeline

Abeona's therapeutic candidates use a range of technologies and delivery platforms intended to address a variety of diseases. The pipeline includes an autologous, gene-corrected cell therapy, one-time gene therapies using the AAV9 vector, and preclinical programs born from the AIM™ AAV platform.

- The Company is planning to initiate a Phase 3 study evaluating EB-101 for the treatment of RDEB in Q4 2019.
- Novel, one-time AAV-based therapies ABO-102 and ABO-101 have shown early promise in ongoing Phase 1/2 trials in Sanfilippo syndrome types A and B.
- Clinical development of single-dose gene therapy ABO-202 for infantile Batten disease, or CLN1, is on the horizon.
- Preclinical studies of ABO-201 for the juvenile form of Batten disease are ongoing.
- Preclinical candidate ABO-401 is the first investigational therapy born from the AIM™ platform and may address all mutations of cystic fibrosis.
- In preclinical studies, the AIM™ AAV204 capsid has shown potential to deliver gene therapy in an out-patient setting for a wide range of inherited and acquired retinal diseases.

20 Regulatory Designations Across Five Programs

		EB-101		ABO-102		ABO-101		ABO-202		ABO-201	
Development Phase		Entering Phase 3		Ongoing Phase 1/2		Ongoing Phase 1/2		Entering Phase 1/2		Preclinical	
🇺🇸	Regenerative Medicine Advanced Therapy	✓		✓							
🇺🇸	Breakthrough Therapy	✓									
🇺🇸	Fast Track			✓		✓		✓			
🇺🇸	Rare Pediatric Disease	✓		✓		✓		✓			
🇺🇸 🇪🇺	Orphan Drug	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓

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