March 14, 2023

Abeona Therapeutics Announces Update on AAV Ophthalmology Program

Advancing AAV-based gene therapy candidates toward IND studies on encouraging animal proof of concept data in Stargardt Disease, X-linked Retinoschisis (XLRS), and Autosomal Dominant Optic Atrophy (ADOA)

To submit first pre-Investigational New Drug (IND) application meeting request this month

NEW YORK and CLEVELAND, March 14, 2023 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced three internally developed investigational preclinical gene therapy product candidates from its ophthalmology program. Abeona's preclinical programs are investigating the use of novel adeno-associated virus (AAV) capsids in therapies for serious genetic eye diseases.

"We are excited by the broad potential for treating serious eye diseases with new AAV-based therapies using novel AAV capsids from our in-licensed AIM™ capsid library and internal research," said Brian Kevany, Ph.D., Chief Technical Officer and Head of Research at Abeona. "In 2022, we evaluated the ability of our gene constructs and capsids to deliver and express the recombinant protein in target eye tissues and rescue mutant phenotypes in mouse disease models. Based on encouraging findings from these animal proof of concept experiments, we are looking forward to reporting new data from these programs at a scientific congress in the second quarter of 2023 and gaining alignment with the U.S. FDA on the clinical development plans for these programs."

Preclinical Product Candidate ABO-504 for Stargardt Disease

Abeona's internal research and development team developed ABO-504, which is designed to efficiently reconstitute the full-length ABCA4 gene by implementing a dual AAV vector strategy using the Cre-LoxP recombinase system. In May 2021 at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting, Abeona reported preclinical data demonstrating the ability of the dual AAV vector system to produce full length ABCA4 protein in cell culture. Recent proof-of-concept studies have extended these findings by showing expression of ABCA4 mRNA and full-length ABCA4 protein in the retina of subretinally dosed *abca4*$^{-/-}$ knockout mice, at levels similar to endogenous ABCA4 in wild-type animals.

Preclinical Product Candidate ABO-503 for X-linked Retinoschisis (XLRS)

ABO-503, composed of a functional human RS1 packaged in the novel AIM™ capsid AAV204, has shown preclinical efficacy following delivery to the retina in a mouse model of XLRS. Preclinical studies have demonstrated robust RS1 expression in the retina, improved cone photoreceptor density and overall photoreceptor cell survival, as well as a restoration of

outer retina architecture.

Preclinical Product Candidate ABO-505 for Autosomal Dominant Optic Atrophy (ADOA)

ABO-505 is designed to express a functional copy of human Opa1 in the retina following para-retinal injection. AB0-505 aims to take advantage of the robust optic nerve and retinal ganglion cell (RGC) transduction ability of AAV204 to deliver its genetic payload to the cells most affected by ADOA. Preclinical studies have confirmed expression of Opa1 in both cell culture and the retinas of dosed wild-type and disease model animals. Initial efficacy results suggest an improvement in retinal signaling to the brain, and improved visual acuity in treated mutant mice.

About Stargardt Disease
Autosomal recessive Stargardt disease, the most common form of juvenile macular degeneration with estimated incidence of 1 in 8,000 to 10,000 people[1], causes vision loss in children and young adults. The most common form of Stargardt disease is caused by mutations in the ABCA4 gene, which prevent removal of toxic compounds from photoreceptor cells that results in photoreceptor cell death and progressive vision loss. There are currently no FDA approved treatments available, and to date, development of investigational gene modifying therapies has remained challenging in part due to the large size of the ABCA4 gene, which exceeds the encapsidation capacity of a single AAV vector.

About X-linked Retinoschisis (XLRS)
XLRS is a rare, monogenic retinal disease that results in the irreversible loss of photoreceptor cells and severe visual impairment. XLRS is caused by mutations in the RS1 protein, which is normally secreted by retinal photoreceptors and bipolar neurons and functions to mediate cell-cell adhesion. XLRS is characterized by abnormal splitting of the layers of the retina, resulting in poor visual acuity, which can progress to legal blindness. The incidence of XLRS is estimated to be between 1 in 5,000 and 1 in 20,000 in males, with an estimated prevalence of 35,000 in the United States and Europe combined.[2,3] There are currently no disease modifying therapies approved for XLRS, but because the genetics of the disease are well understood, early intervention via gene therapy has significant potential to reverse or stabilize disease progression at early stages and prevent vision loss.

About Autosomal Dominant Optic Atrophy (ADOA)
ADOA, a form of hereditary vision loss associated with RGC death, is predominantly caused by mutations in the Opa1 gene. Opa1, a dynamin-related GTPase, acts to stabilize the inner mitochondrial membrane and acts in mitochondrial fusion and inner membrane remodeling. Mutant phenotypes present with a progressive loss of RGCs that results in optic nerve degeneration and legal blindness with a loss of visual acuity, optic disc pallor, and color vision deficits. ADOA affects approximately 1 in 30,000 people worldwide.[4] Currently, there is no approved treatment for people living with ADOA.

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for serious diseases. Abeona's lead clinical program is EB-101, its investigational autologous, engineered cell therapy currently in development for recessive dystrophic epidermolysis bullosa. Abeona is currently in the process of preparing a Biologics

License Application (BLA) for submission to the FDA. The Company's development portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical need. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases. Abeona's fully integrated cell and gene therapy cGMP manufacturing facility produced EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and potential commercial production of AAV-based gene therapies. For more information, visit www.abeonatherapeutics.com.

References:

1. MedlinePlus [Internet]. Bethesda (MD): National Library of Medicine (US); [updated 2020 Aug 18]. Stargardt macular degeneration; [updated 2020 Aug 18; reviewed 2010 November 1; cited 2021 Oct 11]; [about 5 p.]. Available from https://medlineplus.gov/genetics/condition/stargardt-macular-degeneration/
2. Molday RS, Kellner U, Weber BH. X-linked juvenile retinoschisis: clinical diagnosis, genetic analysis, and molecular mechanisms. Prog Retin Eye Res. 2012;31:195-212.
3. Sikkink SK, Biswas S, Parry NR, Stanga PE, Trump D. X-linked retinoschisis: an update. J Med Genet. 2007;44:225-232.
4. Lenaers G, Hamel C, Delettre C, Amati-Bonneau P, Procaccio V, Bonneau D, Reynier P, Milea D. Dominant optic atrophy. Orphanet J Rare

Dis. 2012 Jul 9;7:46. doi: 10.1186/1750-1172-7-46. PMID: 22776096; PMCID: PMC3526509

Forward-Looking Statements

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. We have attempted to identify forward-looking statements by such terminology as "may," "will," "believe," "anticipate," "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, our ability to continue as a going concern; the timing and outcome of our Biologics License Application submission to the FDA for EB-101; our ability to successfully develop our AAV ophthalmology programs, continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of any future meetings with the FDA or other regulatory agencies; 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 disclosed in the Company's most recent Annual Report on Form 10-K and other periodic reports filed with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to update them to reflect events or circumstances occurring after the date of this press release, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

Investor and Media Contact:

Greg Gin
VP, Investor Relations and Corporate Communications
Abeona Therapeutics
ir@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.