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Abeona Therapeutics® Announces ABO-503 Gene Therapy for X-linked Retinoschisis (XLRS) Selected by FDA for Rare Disease Endpoint Advancement (RDEA) Pilot Program

RDEA Pilot Program provides sponsors enhanced communication and collaboration with FDA to accelerate development of rare disease therapies

CLEVELAND, Oct. 13, 2025 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a commercial-stage biopharmaceutical company developing cell and gene therapies for serious diseases, today announced that its ABO-503 gene therapy for X-linked retinoschisis (XLRS) has been selected to participate in the U.S. Food and Drug Administration (FDA) Rare Disease Endpoint Advancement (RDEA) Pilot Program. The RDEA program facilitates the development and timely approval of rare disease therapies by supporting the use of novel efficacy endpoints in clinical trials. As part of the RDEA program, Abeona will have opportunities for enhanced communication and collaboration with the FDA, including frequent advice and regular ad-hoc conversations to accelerate the development and validation of product-specific novel efficacy endpoints for Abeona's XLRS program.

"XLRS is an underserved area with a large unmet need," said Vish Seshadri, Chief Executive Officer of Abeona. "We are honored that ABO-503 gene therapy for XLRS has been chosen for the FDA's highly competitive RDEA pilot program. We believe our participation will meaningfully improve the success rate of our XLRS clinical development efforts, and more broadly, could help facilitate pipeline innovation by using novel efficacy endpoints in new therapy development across other inherited retinal diseases."

ABO-503 is composed of a functional human *RS1* gene packaged in the novel AIM™ capsid AAV204. ABO-503 has shown preclinical efficacy following delivery to the retina in a mouse model of XLRS. Preclinical studies have demonstrated structural and functional improvements following robust RS1 expression throughout the retina, including improved cone photoreceptor density and overall photoreceptor cell survival, restoration of outer retina architecture by eliminating cysts characteristic of XLRS, and improvements in visual function as demonstrated by electroretinogram (ERG). Abeona anticipates completing IND-enabling studies in the second half of 2026.

The FDA launched the RDEA Pilot Program to support the development of novel efficacy endpoints for rare disease treatments. Under the pilot program, between 2023 and 2027, the

FDA will accept up to one RDEA proposal per quarter with a maximum of three proposals per year. To be considered, sponsors must submit a proposal detailing the data they plan to collect, the novelty of the endpoint, and its potential to establish substantial evidence of effectiveness.

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.[1,2] 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 Abeona Therapeutics

Abeona Therapeutics Inc. is a commercial-stage biopharmaceutical company developing cell and gene therapies for serious diseases. Abeona's ZEVASKYN® (prademagene zamikeracel) is the first and only autologous cell-based gene therapy for the treatment of wounds in adults and pediatric patients with recessive dystrophic epidermolysis bullosa (RDEB). The Company's fully integrated cell and gene therapy cGMP manufacturing facility in Cleveland, Ohio serves as the manufacturing site for ZEVASKYN commercial production. The Company's development portfolio features adeno-associated virus (AAV)-based gene therapies for ophthalmic diseases with high unmet medical need. Abeona's novel, next-generation AAV capsids are being evaluated for a variety of devastating diseases. For more information, visit www.abeonatherapeutics.com.

References:

1. 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.
2. Sikkink SK, Biswas S, Parry NR, Stanga PE, Trump D. X-linked retinoschisis: an update. J Med Genet. 2007;44:225-232.

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," "potential," and similar words and 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 commercialize ZEVASKYN; the therapeutic potential of ZEVASKYN; whether the unmet need and market opportunity for ZEVASKYN are consistent with the Company's expectations; continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of future meetings with and inspections by the FDA or other regulatory agencies, including those relating to preclinical programs and to the cGMP manufacturing of ZEVASKYN; the ability to achieve or obtain necessary regulatory approvals for our pre-clinical programs; the impact of any 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 subsequent periodic reports filed with the Securities and Exchange Commission. The Company undertakes no obligation to revise these 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.

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