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# **Abeona Therapeutics and Beacon Therapeutics Announce Non-Exclusive Agreement for Beacon to Evaluate Therapeutic Potential of Abeona's Patented AAV204 Capsid for Select Ophthalmology Indications**

CLEVELAND and LONDON, July 11, 2024 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a cell and gene therapy company with proprietary adeno-associated virus (AAV)-based capsids, and Beacon Therapeutics, an ophthalmic gene therapy company and Syncona portfolio company, today announced an agreement by which Beacon will evaluate Abeona's patented AAV204 capsid for the development and commercialization of potential gene therapies for select ophthalmology indications.

"This agreement with Beacon underscores the potential of AAV204 to enable efficient targeting in the eye of novel AAV-based gene therapies for patients with rare and prevalent ophthalmic diseases with high unmet medical need, and we are looking forward to a fruitful collaboration," said Dr. Madhav Vasanthavada, Chief Commercial Officer and Head of Business Development at Abeona Therapeutics.

AAV204, a novel AAV capsid from the AIM™ capsid library licensed by Abeona from the University of North Carolina at Chapel Hill, has been shown to achieve high macular and optic nerve transduction levels after para-retinal administration and has also been shown to facilitate transduction of both the inner and outer retina after intravitreal administration in mice and non-human primates.

Abraham Scaria, Chief Scientific Officer of Beacon Therapeutics, said, "We are looking forward to evaluating AAV204 for its ability to transduce various layers of the retina in order to develop therapies for multiple retinal diseases with high unmet need."

Under the terms of the agreement, Beacon will have the right to evaluate, for a 12-month period, the AAV204 capsid for potential use in certain ophthalmology indications, with an option to take a worldwide, non-exclusive license to use AAV204 in connection with up to five gene or disease targets. Beacon will also have the right to use AAV204 for up to four additional nominated gene or disease targets subject to certain conditions. Under the agreement, Abeona will receive an upfront payment upon Beacon's exercise of its option to license AAV204, with additional payments upon the achievement of certain development, regulatory, and sales milestones, along with tiered royalties on worldwide net sales for licensed products incorporating AAV204. Beacon will be responsible for the development

and commercialization of all licensed products. The targets for which Beacon would receive rights under its non-exclusive license agreement are distinct from those currently in development at Abeona.

### **About the AIM™ capsid library**

The AIM™ capsid library is a collection of novel AAV serotypes that target delivery of genetic payloads to key tissues implicated in devastating genetic diseases, including the central nervous system (including the retina), lungs, eye, muscle, liver and other tissues, with potentially improved tropism profiles. AIM™ vectors have shown the potential to evade the immune response generated by exposure to naturally-occurring AAV vectors in preclinical studies. AAV204 is covered by U.S. Patent Nos. 10,532,110 and 10,561,743.

### **About Abeona Therapeutics**

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for serious diseases. Prademagene zamikeracel (pz-cel) is Abeona's investigational autologous, COL7A1 gene-corrected epidermal sheets currently in development for recessive dystrophic epidermolysis bullosa. The Company's fully integrated cell and gene therapy cGMP manufacturing facility served as the manufacturing site for pz-cel used in its Phase 3 VIITAL™ trial, and is capable of supporting commercial production of pz-cel upon FDA approval. 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. For more information, visit [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com).

### **About Beacon Therapeutics**

Beacon Therapeutics is an ophthalmic gene therapy company founded in 2023 to save and restore the vision of patients with a range of prevalent and rare retinal diseases that result in blindness. Beacon has an established scientific foundation that combines a late-stage development candidate to treat X-linked retinitis pigmentosa (XLRP), as well as two preclinical programs, one targeting dry age-related macular degeneration (AMD) and another targeting cone-rod dystrophy (CRD), an inherited retinal disease. The company is supported by funds from Syncona, Forbion, Oxford Science Enterprises, TCGX, Advent Life Sciences, and additional investors. To learn more, please visit [www.beacontx.com](http://www.beacontx.com).

### **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, the timing and results of ongoing testing and other corrective actions being performed in response to the FDA's identified deficiencies, which could delay the Company's BLA resubmission; the timing and outcome of the FDA's review of our resubmission; the FDA's grant of a Priority Review Voucher upon approval; continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of future meetings with the*

*FDA or other regulatory agencies, including those relating to preclinical programs; the ability to achieve or obtain necessary regulatory approvals; 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 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.*

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