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Biogen and AGTC have announced a broad collaboration and license agreement to develop gene-based therapies for multiple ophthalmic diseases. The collaboration will focus on the development of a portfolio of AGTC's therapeutic programs, including both a clinical stage candidate and a pre-clinical candidate for orphan diseases of the retina that can lead to blindness in children and adults. The agreement also includes options for early stage discovery programs in two ophthalmic diseases and one non-ophthalmic condition, as well as an equity investment in AGTC by Biogen and a license agreement for manufacturing rights.

"With this collaboration, we hope to advance gene therapies to open possibilities for patients who suffer from diseases that are well understood, but have no adequate treatment. AGTC is an exceptional partner to help us advance our gene therapy capabilities by targeting diseases of the eye, an organ that provides an ideal setting for the localized, selective delivery of gene-based therapies," said Dr Olivier Danos,senior vice-president, cell and gene therapy at Biogen.

"We expect this collaboration will further validate our novel adeno-associated virus (AAV) gene therapy platform and support the development of new therapies that may allow for transformative treatments for these rare inherited eye diseases and other clinical indications," said Ms Sue Washer, president and CEO of AGTC. She added, "Biogen's significant commitment to advancing gene therapies and demonstrated success in developing innovative therapies to treat complex diseases, combined with our proprietary manufacturing technology and extensive gene therapy experience, makes this an ideal partnership."

The lead development programs in the collaboration include a clinical candidate for X-linked Retinoschisis (XLRS) and a pre-clinical candidate for the treatment of X-Linked Retinitis Pigmentosa (XLRP). XLRS, a disease affecting young males beginning during the teenage years, can lead to serious complications such as vitreous hemorrhage or retinal detachment during adulthood. XLRP usually causes night blindness by the age of ten and progresses to legal blindness by an individual's early forties. Both conditions represent significant unmet needs that may be addressed by replacing the single, faulty gene

causing each disease.

Biogen will make an upfront payment in the amount of $124 million to AGTC, which includes a $30 million equity investment in AGTC at a price equal to $20.63 per share and certain prepaid research and development expenditures. Biogen will be granted a license to the XLRS and XLRP programs and the option to license discovery programs for three additional indications at the time of clinical candidate selection.

Under the collaboration, AGTC is eligible to receive upfront and milestone payments exceeding $1 billion. This includes up to $472.5 million collectively for the two lead programs, which also will carry royalties in the high single digit to mid-teen percentages of annual net sales. In addition, Biogen will make payments up to $592.5 million across the discovery programs, along with royalties in the mid single digits to low teen percentages of annual net sales.