



Biogen and AGTC Enter Collaboration to Develop Gene Therapies in Ophthalmology

July 2, 2015

Companies to advance a potentially transformative treatment approach for genetic diseases of the eye

AGTC to receive \$124M upfront, with potential future milestone payments and royalties

AGTC to host conference call today at 8 a.m. EDT

CAMBRIDGE, Mass. & GAINESVILLE, Fla.--(BUSINESS WIRE)--Biogen (NASDAQ: BIIB) and AGTC (NASDAQ: AGTC) today 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," said Olivier Danos, Ph.D., senior vice president, cell & gene therapy at Biogen. "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."

"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," added Sue Washer, president and CEO of AGTC. "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.

Collaboration Overview

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.

Biogen obtains worldwide commercialization rights for the XLRs and XLRP programs. AGTC has an option to share development costs and profits after the initial clinical trial data are available, and an option to co-promote the second of these products to be approved in the United States. AGTC will lead the clinical development programs of XLRs through product approval and of XLRP through the completion of first-in-human trials. Biogen will support the clinical development costs, subject to certain conditions, following the first-in-human study for XLRs and IND-enabling studies for XLRP. Under the manufacturing license, Biogen will receive an exclusive license to use AGTC's proprietary technology platform to make AAV vectors for up to six genes, three of which are in AGTC's discretion, in exchange for payment of milestones and royalties.

The transaction is subject to customary closing conditions, including the expiration of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 in the United States, and is expected to close in the third calendar quarter of 2015.

AGTC will host a live webcast presentation and conference call on July 2 at 8:00 a.m. EDT to discuss the collaboration. The webcast can be accessed at ir.agtc.com/events.cfm or by dialing (888) 427-9419 (US) or (719) 325-2491 (outside of the US) fifteen minutes prior to the start of the call. The passcode is 6785007. The webcast will be archived on the AGTC website.

About Gene Therapy

Gene therapy is an evolving field of medicine in which faulty genes are corrected in cells. Genes control heredity and provide the basic biological code for determining a cell's specific functions. The most common form of gene therapy involves using DNA that encodes a functional, therapeutic gene to replace a defective gene. In gene therapy, the healthy copy of a defective gene is packaged within a vector, a biological delivery mechanism which is used to transport the genetic information into the diseased cells within the body. Once the gene is delivered into the correct cell, a therapeutic protein is naturally made by the cell from the therapeutic gene.

About Adeno-Associated Virus (AAV) Vectors

AAV vectors have emerged as an attractive approach for gene therapy since they can deliver the genes for therapeutic proteins to accessible tissues in the body. Several AAV gene therapy products are in late-stage clinical development, and one product is approved in the EU.

About Biogen

Through cutting-edge science and medicine, Biogen discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hematologic conditions and autoimmune disorders. Founded in 1978, Biogen is one of the world's oldest independent

biotechnology companies, and patients worldwide benefit from its leading multiple sclerosis and innovative hemophilia therapies. For product labeling, press releases and additional information about the company, please visit www.biogen.com.

About AGTC

AGTC is a clinical-stage biotechnology company that uses its proprietary gene therapy platform to develop products designed to transform the lives of patients with severe diseases in ophthalmology. AGTC's lead product candidates focus on X-linked retinoschisis, achromatopsia and X-linked retinitis pigmentosa, which are inherited orphan diseases of the eye, caused by mutations in single genes that significantly affect visual function and currently lack effective medical treatments. AGTC is also using its gene therapy expertise to expand into disease indications with large market opportunity such as wet AMD and other ophthalmology and orphan indications.

Biogen Safe Harbor

This press release contains forward-looking statements, including statements about the potential benefits and advancements that may be achieved through the collaboration with AGTC and the expected timing of the closing of the transactions. These statements may be identified by words such as "believe," "expect," "may," "plan," "potential," "will" and similar expressions, and are based on Biogen's current beliefs and expectations. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements. Risks and uncertainties that may cause actual results to differ materially include, among others: uncertainty inherent in the regulatory review process and satisfaction of other closing conditions relating to the transactions; uncertainty regarding the ability to achieve the expected benefits from the proposed collaboration, including as a result of risks and uncertainties associated with drug development and commercialization, reliance on third parties over which Biogen may not always have full control and other risks associated with collaborations; and other risks and uncertainties that are described in the Risk Factors section of Biogen's most recent annual or quarterly report filed with the Securities and Exchange Commission. Any forward-looking statements speak only as of the date of this press release and Biogen assumes no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

AGTC Safe Harbor

This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs. Forward-looking statements include information concerning the expected timing of the closing of the transactions contemplated by the proposed collaboration, possible or assumed future results of operations, business strategies and operations, preclinical and clinical product development and regulatory progress, potential growth opportunities, potential market opportunities and the effects of competition. Forward-looking statements include all statements that are not historical facts and can be identified by terms such as "anticipates," "believes," "could," "seeks," "estimates," "expects," "intends," "may," "plans," "potential," "predicts," "projects," "should," "will," "would" or similar expressions and the negatives of those terms. Actual results could differ materially from those discussed in the forward-looking statements, due to a number of important factors. Risks and uncertainties that may cause actual results to differ materially include, among others: uncertainty inherent in the regulatory review process and satisfaction of other closing conditions relating to the transactions; uncertainty regarding the ability to achieve the expected benefits from the proposed collaboration, including as a result of risks and uncertainties associated with drug development and commercialization, reliance on third parties over which AGTC may not always have full control and other risks associated with collaborations; and other risks and uncertainties that are described under the heading "Risk Factors" in AGTC's Annual Report on Form 10-K for the fiscal year ended June 30, 2014, as filed with the SEC. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent management's plans, estimates, assumptions and beliefs only as of the date of this release. Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.



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