Generation Bio Presents Data Demonstrating First Lipid Nanoparticle to Achieve Uniform Retinal Transduction and Tolerability via Sub-Retinal Delivery of ceDNA and mRNA at the European Society of Gene and Cell Therapy 2021 Annual Virtual Congress

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Sub-retinal delivery of closed-ended DNA (ceDNA) using a retina-specific cell-targeted LNP (ctLNP) demonstrated broad photoreceptor distribution, durable expression and tolerability in rodents; potential to address inherited retinal diseases with full gene replacement

Uniform retinal transduction and tolerability also demonstrated for ctLNP delivery of mRNA following sub-retinal injection in non-human primates; potential best-in-class non-viral delivery of mRNA for gene editing in the retina

CAMBRIDGE, Mass., Oct. 22, 2021 (GLOBE NEWSWIRE) -- Generation Bio Co. (Nasdaq: GBIO), a biotechnology company innovating genetic medicines for people living with rare and prevalent diseases, today presented new preclinical data demonstrating widespread delivery of multiple nucleic acid cargos to photoreceptors using the company's cell-targeted lipid nanoparticle (ctLNP). The findings were shared in an oral presentation at the European Society of Gene and Cell Therapy (ESGCT) 2021 Annual Virtual Congress.

"We're excited to extend the benefits of our highly specific, cell-targeted LNP to the retina, where non-viral delivery of nucleic acids has long been held back by poor tolerability and low expression. Our ability to selectively deliver multiple nucleic acid cargos to the retina using ctLNP may allow us to address a variety of inherited retinal diseases using full gene replacement or gene editing," said Matthew Stanton, Ph.D., chief scientific officer of Generation Bio.

Sub-retinal delivery of Generation Bio's proprietary closed-ended DNA (ceDNA) using ctLNP demonstrated broad photoreceptor distribution and durable expression in rodents. Expression was comparable to AAV5 delivery, and ctLNP-ceDNA was well-tolerated without evidence of photoreceptor degeneration, supporting the potential for full gene replacement to address inherited retinal diseases.

Data were also presented for sub-retinal delivery of mRNA using ctLNP, representing the first-ever demonstration of species translation from rodents to non-human primates with tolerability and uniform photoreceptor expression. Distribution with ctLNP was broader and more uniform than that achieved with AAV5 in mice, and total expression was comparable to AAV5. These findings suggest ctLNP as a best-in-class non-viral delivery system for mRNA, potentially enabling gene editing in the retina.

"Diseases such as Stargardt cannot be addressed with traditional viral-based genetic therapies due to the limited cargo capacity of the viral vector. We believe these data using our non-viral genetic medicine platform provide a promising path to treat this challenging disease and others like it, and may expand our platform's potential to enable multiple therapeutic modalities, including full gene replacement and gene editing," said Tracy Zimmermann, Ph.D., chief development officer of Generation Bio. "We are excited for the potential for our non-viral delivery technology to expand therapeutic opportunities in the retina as well as to target other tissue types for the treatment of a broad range of diseases."

To view the digital presentation, please visit Generation Bio's website.

About Generation Bio
Generation Bio is innovating genetic medicines to provide durable, redosable treatments for people living with rare and prevalent diseases. The company's non-viral genetic medicine platform incorporates a novel DNA construct called closed-ended DNA, or ceDNA; a unique cell-targeted lipid nanoparticle delivery system, or ctLNP; and a highly scalable capsid-free manufacturing process that uses proprietary cell-free rapid enzymatic synthesis, or RES, to produce ceDNA. The platform is designed to enable multi-year durability from a single dose, to deliver large genetic payloads, including multiple genes, to specific tissues, and to allow titration and redosing to adjust or extend expression levels in each patient. RES has the potential to expand Generation Bio's manufacturing scale to hundreds of millions of doses to support its mission to extend the reach of genetic medicine to more people, living with more diseases, around the world.

For more information, please visit www.generationbio.com.

Forward-Looking Statements
Any statements in this press release about future expectations, plans and prospects for the company, including statements about our strategic plans or objectives, our technology platform, our research and clinical development plans, the expected timing of the submission of IND applications and preclinical data, our manufacturing plans, our expectations regarding our new facility and other statements containing the words "believes," "anticipates," "plans," "expects," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: uncertainties inherent in the identification and development of product candidates, including the conduct of research activities, the initiation and completion of preclinical studies and clinical trials and clinical development of the company's product candidates; uncertainties as to the availability and timing of results from preclinical studies and clinical trials; whether results from preclinical studies will be predictive of the results of later preclinical studies and clinical trials; uncertainties regarding the timing and ability to complete the build-out of the company's manufacturing facility and regarding the new manufacturing process; expectations regarding the timing of submission of IND applications; expectations for regulatory approvals to conduct trials or to market products; challenges in the manufacture of genetic medicine products; whether the company's cash resources are sufficient to fund the company's operating expenses and capital expenditure requirements for the period anticipated; the impact of the COVID-19 pandemic on the company's business and operations; as well as the other risks and uncertainties set forth in the "Risk Factors" section of our most recent annual report on Form 10-K and quarterly report on Form 10-Q, which are on file with the Securities and Exchange Commission, and in subsequent filings the company may make with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the company's views as of the date hereof. The company anticipates that subsequent events and developments will cause the company's views to change. However, while the company may elect to update these forward-looking statements at some point in the future, the company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the company's

views as of any date subsequent to the date on which they were made.

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