Generation Bio Announces the Presentation of Preclinical Data on iqDNA and ctLNP Platforms at the ASGCT 27th Annual Meeting

May 9, 2024

- *Data presented show immune-quiet DNA (iqDNA) is a partially single-stranded DNA cargo that maintains expression while evading innate immune sensors*
- *Rapid enzymatic synthesis continues to improve iqDNA performance through process development and the identification of novel structural elements*
- *Data on cell-targeted LNP (ctLNP)* in vivo *delivery of therapeutic transgenes to T cells to be discussed in an oral presentation on Saturday, May 11*

CAMBRIDGE, Mass., May 09, 2024 (GLOBE NEWSWIRE) -- Generation Bio Co. (Nasdaq:GBIO) a biotechnology company innovating genetic medicines for people living with rare and prevalent diseases, today presented five posters on its immune-quiet DNA (iqDNA) and cell-targeted lipid nanoparticle (ctLNP) platforms as well as its enzymatic synthesis manufacturing at the American Society of Gene and Cell Therapy (ASGCT) 27th Annual Meeting. A sixth presentation on the company's ctLNP platform will be discussed in an oral presentation on Saturday, May 11.

"The data we're presenting at ASGCT showcase some important mechanistic underpinnings of our novel platforms, which we believe have the potential to overcome the core challenges facing the field of genetic medicine," said Matt Stanton, Ph.D., chief scientific officer of Generation Bio. "We believe immune-quiet DNA cargo and highly selective, targeted LNP delivery are the tools required to unlock the full potential of non-viral genetic medicines, and we are applying them to our current portfolio of programs in T cells, hematopoietic stem cells, and hepatocytes."

Data included in the posters describe iqDNA as a modified, structured, partially single-stranded DNA that evades innate immunity while remaining transcriptionally active in the cell. In mouse studies, the immune profile of iqDNA is consistent with avoidance of key innate immune pathways such as cGAS-STING, which is known to have a significantly lower binding affinity for single-stranded DNA than double-stranded DNA. Given the non-immunogenic nature of iqDNA, Generation Bio believes it can now create novel therapies with a wide therapeutic index, which has been a major challenge to the development of *in vivo* DNA-based genetic medicines.

The company invented iqDNA using its proprietary rapid enzymatic synthesis (RES) for DNA production. RES is a cell-free process that permits chemical and structural changes with a high degree of control, facilitating heightened DNA functionality through engineering molecular design and components. Because RES does not rely on cellular or viral components, it consistently produces 99% pure material in approximately one week, marking a significant improvement over biologic methods, which are often lengthy and complex. Further, due to the nature of enzymatic processes, iqDNA can be manufactured at scale to support clinical and global commercial demands without the challenges faced by cell-culture based production.

Generation Bio continues to leverage RES to advance its iqDNA platform by optimizing various structural and chemical elements. In new data presented at ASGCT, a second generation of iqDNA achieved higher luciferase expression than first-generation iqDNA. The company is currently testing formulations of second-generation iqDNA encoding Factor VIII.

The company's ctLNP platform is designed to reach a broad set of target cell types and tissues with exquisite selectivity. Data presented at ASGCT describe the foundation of the platform, Generation Bio's "stealth" LNP, which was engineered in part by making structural modifications to the parental ionizable lipid composition. Cell-specific targeting is achieved by optimizing ligand selection and linker chemistry. Additional data on T cell delivery, including the *in vivo* delivery of therapeutic transgenes via ctLNP, will be discussed in an oral presentation on Saturday, May 11.

Further information on the poster and oral presentations can be found here. A copy of the presentation materials will be added to the "Our Scientific Presentations" section of the company's website here on the day of each presentation.

Forward-Looking Statements

Any statements in this press release about future expectations, plans and prospects for the company, including statements about the company's strategic plans or objectives, technology platforms, research and clinical development plans, and preclinical data 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; uncertainties regarding our novel platforms and related technologies; whether results from preclinical studies will be predictive of the results of later preclinical studies and clinical trials; 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; as well as the other risks and uncertainties set forth in the "Risk Factors" section of the company's most recent annual report on Form 10-K, which is 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.

About Generation Bio

Generation Bio is innovating non-viral genetic medicines to provide durable and redosable treatments for hundreds of millions of patients living with rare and prevalent diseases. The company is developing two distinct and complementary platforms: a potent, highly selective cell-targeted lipid nanoparticle (ctLNP) delivery system and a novel immune-quiet DNA (iqDNA) cargo produced by a scalable capsid-free manufacturing process that uses proprietary cell-free rapid enzymatic synthesis (RES). With these platforms, Generation Bio aims to develop the next wave of non-viral genetic medicines 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.

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