Generation Bio to Apply ctLNP Delivery Technology to Develop siRNA Therapeutics for T Cell-Driven Autoimmune Diseases

January 6, 2025

- *Novel programs will combine validated cell-targeted LNP (ctLNP) delivery with siRNA to selectively modulate T cells* in vivo
- *Programs to focus on silencing hard-to-drug targets of high therapeutic value in T cell-driven autoimmune diseases*
- *Company reorganization supports evolution of ctLNP-enabled strategy and buildout of clinical capabilities ahead of first IND expected in 2H 2026*

CAMBRIDGE, Mass., Jan. 06, 2025 (GLOBE NEWSWIRE) -- Generation Bio Co. (Nasdaq:GBIO) a biotechnology company working to change what is possible for people living with T cell-driven autoimmune diseases, announced it is leveraging its validated T cell-directed lipid nanoparticle (ctLNP) to develop siRNA therapeutics to silence disease-driving targets in T cells.

“We are excited to move Generation Bio toward the clinic by deploying our ctLNP to deliver siRNA to T cells. By precisely modulating T cell activity *in vivo* we believe we can address high-value, currently undruggable targets involved in the inflammation and tissue damage associated with T cell-driven autoimmune diseases,” said Geoff McDonough, M.D., chief executive officer of Generation Bio. “Our aim is to silence therapeutic T cell targets without impacting other immune cell types, unlocking a powerful new application for siRNA in the field. We plan to submit our first IND in the second half of 2026 and to enter the clinic within our cash runway, which is into the second half of 2027.”

siRNA delivery to T cells has historically been limited by the inability to achieve selective cell targeting combined with efficient access to the cytoplasm where siRNA operates, challenges that Generation Bio has designed its ctLNP to overcome. The company recently presented non-human primate data demonstrating that its ctLNPs work by targeting T cells through a target receptor of interest with a strong selectivity for CD8+ and CD4+ effector T cells and NK cells, all of which are involved in auto-reactive tissue damage in a number of autoimmune diseases.

siRNA delivered with ctLNP aims to modulate T cell function without impacting the function of the broader immune system. ctLNP-siRNA could reach targets that change how auto-reactive T cells activate, differentiate, migrate, and damage tissues. Generation Bio will provide further details about its lead ctLNP-siRNA programs in upcoming quarters and expects to submit its first IND in the second half of 2026. The company is reorganizing to support the clinical development of T cell-directed medicines.

In addition, the company is announcing changes to its executive leadership team. Phillip Samayoa, Ph.D., chief strategy officer, will succeed Matthew Stanton, Ph.D. as chief scientific officer. Dr. Stanton will remain with the company through mid-2025 and transition to its Scientific Advisory Board thereafter. Kevin Conway, the company’s head of finance, has been elected chief financial officer to succeed Matthew Norkunas, M.D. Generation Bio also expects to appoint a chief medical officer in 2025.

“I am grateful for the passion and expertise each person at Generation Bio has contributed to enable our transition toward the clinic,” said Dr. McDonough. “Matt Stanton has been instrumental in the invention of our technologies, and I look forward to our continued work together. Likewise, Matt Norkunas has built a terrific team here, which Kevin will continue to build upon. With a strong foundation in place, we are well-positioned to execute on our mission to bring highly differentiated T cell-directed therapies to patients.”

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, strategic reorganization, cash resources, technology platforms, research and clinical development plans and timelines, 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: the potential impact of the strategic reorganization on the company’s operations and development timeline; 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 the company’s 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 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.

About Generation Bio
Generation Bio is a biotechnology company changing what is possible for people living with T cell-driven autoimmune diseases. The company is developing a new modality of therapeutics leveraging its T cell selective cell-targeted lipid nanoparticle (ctLNP) to deliver siRNA *in vivo*, enabling modulation of T cell activity that causes inflammation and auto-reactive tissue destruction. By selectively modulating T cells that drive disease pathology, ctLNP-siRNA therapeutics could potently block target function with sequence-level specificity while sparing the broader immune system. This potent new modality is designed to reach targets that are poorly drugged by other approaches, opening a broad indication space of T cell driven

autoimmune diseases.

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

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