Generation Bio Announces Recent Business Highlights and First Quarter 2024 Financial Results

May 13, 2024

- Oral presentation at ASGCT described selective, high levels of therapeutic transgene delivery to T cells in vivo *by cell-targeted lipid nanoparticle (ctLNP)*

- ASGCT poster presentations described immune-quiet DNA (iqDNA) as partially single-stranded, produced by flexible, scalable, proprietary rapid enzymatic synthesis

- Cash balance of $233.9 million still expected to fund operations into 2H 2027

CAMBRIDGE, Mass., May 13, 2024 (GLOBE NEWSWIRE) -- Generation Bio Co. (Nasdaq: GBIO), a biotechnology company innovating genetic medicines for people living with rare and prevalent diseases, reported additional data on its cell-targeted lipid nanoparticle (ctLNP) platform presented at the American Society of Gene and Cell Therapy (ASGCT) 27th Annual Meeting as well as first quarter 2024 financial results.

“The data presented on our ctLNP and iqDNA platforms highlight their maturity and differentiation as the foundation for building programs in hematology,” said Geoff McDonough, M.D., chief executive officer of Generation Bio. “Our unique non-viral delivery and cargo may unlock a series of non-viral *in vivo* therapeutic candidates, starting with sickle cell disease and hemophilia A. We believe our medicines could have a drug-like, redosable *in vivo* profile, meaning they can surpass what is possible for current viral, biologic and *ex vivo* approaches to reach people on a global scale.”

Recent Highlights:

- **Presented New Data Demonstrating *In Vivo* Delivery of a Therapeutic Transgene to T cells with ctLNP**: During an oral presentation at ASGCT, Generation Bio discussed *in vivo* data demonstrating selective delivery of a T cell-targeted ctLNP carrying mRNA cargo encoding a CAR. In these studies, CAR expression was efficient and dose-dependent with robust surface presentation on T cells. Transduced CAR T cells demonstrated preliminary efficacy in an *in vitro* tumor cell killing assay. The data were generated as part of the company’s collaboration with Moderna, Inc.

  Generation Bio has engineered its ctLNPs to avoid clearance by the liver and spleen, allowing selective targeting of tissues and cell types with the addition of a receptor-specific targeting ligand. The next steps for the platform include assessing efficacy in mouse disease models and evaluating the delivery of immune-quiet DNA (iqDNA) with T cell ctLNPs.

  The company is also developing a ctLNP targeting hematopoietic stem cells (HSCs) as part of its sickle cell disease and beta-thalassemia program. At ASGCT, the company showed preliminary data demonstrating selective uptake and expression in human HSCs delivered by an HSC-targeted ctLNP.

  “The demonstration of selective delivery with a therapeutic cargo outside the liver is an important proof point for the ability of our ctLNPs to reach previously unreachable cell types and tissues,” said Phillip Samayoa, Ph.D., chief strategy officer of Generation Bio. “We look forward to advancing our ctLNP platform in T cells and continuing to expand the application into other cell types, beginning with a wholly-owned program in sickle cell disease and beta-thalassemia.”

- **Disclosed New Information About iqDNA Platform and RES:** Also at ASGCT, Generation Bio described its iqDNA as a modified, structured, partially single-stranded DNA that is non-immunogenic while remaining transcriptionally active. The company invented iqDNA using its proprietary rapid enzymatic synthesis (RES) method. RES is a cell-free process that allows for precise chemical and structural changes to DNA. This high degree of control enables the enhancement of DNA functionality by engineering molecular design and components.

  Generation Bio is advancing its iqDNA platform with the application of RES. The findings presented at ASGCT demonstrated that a second generation of iqDNA achieved greater luciferase expression than a first-generation iqDNA. The company is currently testing formulations of iqDNA encoding Factor VIII delivered by LNP.

First Quarter 2024 Financial Results

- **Cash Position**: Cash, cash equivalents, and marketable securities were $233.9 million as of March 31, 2024, compared to $264.4 million in cash, cash equivalents, and marketable securities as of December 31, 2023. The company continues to believe that its cash, cash equivalents, and marketable securities will fund its operating plan into the second half of 2027.
- **R&D Expenses**: Research and development (R&D) expenses were $14.3 million for the quarter ended March 31, 2024, compared to $22.0 million for the quarter ended March 31, 2023.
- **G&A Expenses**: General and administrative (G&A) expenses were $10.4 million for the quarter ended March 31, 2024, compared to $12.9 million for the quarter ended March 31, 2023.

- **Loss on lease termination**: During the three months ended March 31, 2024, we recognized a non-cash charge of $56.9 million in connection with the termination of the Seyon Lease.
- **Net Loss:** Net loss was $74.5 million, or $1.12 basic and diluted net loss per share, for the quarter ended March 31, 2024, compared to a net loss of $ 32.1 million, or $0.53 basic and diluted net loss per share, for the quarter ended March 31, 2023.

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.

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, cash resources, technology platforms, research and clinical development plans, and preclinical data, including those relating to immune-quiet DNA, 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.

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GENERATION BIO CO.
CONSOLIDATED BALANCE SHEET DATA
(Unaudited)
(In thousands)

Earnings Release Balance Sheet	March 31, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	$ 233,937	$ 264,364
Working capital	188,851	232,704
Total assets	285,879	374,758
Total stockholders' equity	131,991	203,128

GENERATION BIO CO.
CONSOLIDATED STATEMENTS OF OPERATIONS
(Unaudited)
(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2024	2023
Revenues:		
Collaboration Revenue	$ 4,059	$ —
Operating expenses:		
Research and development	14,335	22,000
General and administrative	10,428	12,866
Loss on lease termination	56,930	—

Total operating expenses	81,693	34,866
Loss from operations	(77,634)	(34,866)
Other income:		
Other income and interest income, net	3,093	2,772
Net loss	$ (74,541)	$ (32,094)
Net loss per share, basic and diluted	$ (1.12)	$ (0.53)
Weighted average common shares outstanding, basic and diluted	66,433,640	60,230,077
Comprehensive loss:		
Net loss	$ (74,541)	$ (32,094)
Other comprehensive (loss) income:		
Unrealized (losses) gains on marketable securities	(471)	117
Comprehensive loss	$ (75,012)	$ (31,977)