Generation Bio Reports Business Highlights and Fourth Quarter and Full Year 2023 Financial Results

March 6, 2024

- Company is leveraging proprietary cell-targeted lipid nanoparticle delivery to develop wholly-owned in vivo *program for sickle cell disease and beta-thalassemia*

- Development of breakthrough immune-quiet DNA for hemophilia A program continues

- Cash balance of $264.4 million expected to fund operations into 2H 2027

CAMBRIDGE, Mass., March 06, 2024 (GLOBE NEWSWIRE) -- Generation Bio Co. (Nasdaq:GBIO), a biotechnology company innovating genetic medicines for people living with rare and prevalent diseases, reported business highlights and fourth quarter and full year 2023 financial results.

“Late last year we announced important advances in our cell-targeted lipid nanoparticle and immune-quiet DNA platforms that we are now translating to our portfolio strategy,” said Geoff McDonough, M.D., chief executive officer of Generation Bio. “The potency and selectivity of our ctLNP platform open up significant opportunities for *in vivo* delivery of T cell and hematopoietic stem cell therapies, and this year we are laying the foundation for a wholly-owned program in sickle cell disease and beta-thalassemia. We are also continuing to optimize iqDNA to progress our hemophilia A program and to explore therapeutic applications in cell types beyond hepatocytes. With our current cash balance, we believe we have sufficient runway to reach meaningful development milestones for our platforms and programs.”

Business Highlights

- **Developing ctLNP for *in vivo* T cell and hematopoeitc stem cell (HSC) programs:** Generation Bio’s ctLNP platform has demonstrated a set of key characteristics that could make possible *in vivo* genetic medicine approaches to treating diseases for which only *ex vivo* therapies are currently available. The company has developed ctLNPs that selectively target and transduce 75% of circulating and splenic T cells and maintain high levels of delivery at doses as low as 0.005 mg/kg in humanized mice. In addition to continuing this work in T cells in collaboration with Moderna, Generation Bio is developing a wholly-owned *in vivo* program targeting HSCs to address sickle cell disease and beta-thalassemia. *In vivo* approaches could offer important advantages over current *ex vivo* approaches, such as the lack of pre-treatment chemotherapy, redosing, on-demand availability and lower cost. In 2024, the company intends to show highly selective *in vivo* delivery to T cells with therapeutic transgenes, and proof of concept for specific and potent *in vivo* HSC targeting in humanized mouse models.

- **Developing iqDNA for programs in hepatocytes and other cell types**: In 2023, Generation Bio announced that it had developed a novel DNA cargo that was shown to avoid innate immune detection in both mice and NHPs. In addition to these immune quiet characteristics, iqDNA also exhibited robust and durable expression in mice. This profile could lead to differentiated therapeutics utilizing hepatocytes to treat a wide range of genetic diseases, including hemophilia A, as well extrahepatic tissues and cell types, such as T cells and HSCs. In 2024, the company intends to optimize iqDNA for application in hepatocytes and T cells with a focus on improving potency.

Fourth Quarter and Full Year 2023 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $264.4 million as of December 31, 2023, compared with $279.1 million in cash, cash equivalents, and marketable securities as of December 31, 2022. The company continues to believe that its cash, cash equivalents and marketable securities will fund its operating expenditures and capital expenditure requirements into the second half of 2027.

- **R&D Expenses**: Research and development (R&D) expenses were $27.9 million for the quarter ended December 31, 2023, including a one-time $5.1 million charge related to the company’s reduction in force, and $93.6 million for the year ended December 31, 2023, compared to $21.6 million for the quarter ended December 31, 2022, and $96.7 million for the year ended December 31, 2022.

- **G&A Expenses**: General and administrative (G&A) expenses were $13.4 million for the quarter ended December 31, 2023, including a one-time $1.9 million charge related to the company’s reduction in force, and $50.9 million for the year ended December 31, 2023, compared to $13.1 million for the quarter ended December 31, 2022, and $44.5 million for the year ended December 31, 2022.

- **Net Loss:** Net loss was $35.2 million, or $0.53 basic and diluted net loss per share, for the quarter ended December 31, 2023, and $126.6 million, or $1.96 basic and diluted net loss per share, for the year ended December 31, 2023, compared to a net loss of $32.4 million, or $0.55 basic and diluted net loss per share, for the quarter ended December 31, 2022, and

$136.6 million, or $2.35 basic and diluted net loss per share, for the year ended December 31, 2022.

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

Earnings Release Balance Sheet	December 31, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 264,364	$ 279,091
Working capital	232,704	267,866
Total assets	374,758	376,264
Total stockholders' equity	203,128	282,493

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

	Three Months Ended December 31,		Year Ended December 31,	
	2023	2022	2023	2022
Revenues:				
Collaboration Revenue	$ 2,878	$ —	$ 5,904	$ —
Operating expenses:				
Research and development	27,923	21,607	93,617	96,718
General and administrative	13,376	13,081	50,850	44,464
Total operating expenses	41,299	34,688	144,467	141,182
Loss from operations	(38,421)	(34,688)	(138,563)	(141,182)
Other income:				
Other income and interest income, net	3,235	2,283	11,951	4,543

Net loss	$ (35,186)	$ (32,405)	$ (126,612)	$ (136,639)
Net loss per share, basic and diluted	$ (0.53)	$ (0.55)	$ (1.96)	$ (2.35)
Weighted average common shares outstanding, basic and diluted	66,062,208	59,407,296	64,483,520	58,114,893
Comprehensive loss:				
Net loss	$ (35,186)	$ (32,405)	$ (126,612)	$ (136,639)
Other comprehensive income (loss):				
Unrealized gains (losses) on marketable securities	278	219	357	(83)
Comprehensive loss	$ (34,908)	$ (32,186)	$ (126,255)	$ (136,722)