



## Generation Bio Outlines 2023 Strategic Priorities and Reports Fourth Quarter and Full Year 2022 Financial Results

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— Company expects to announce data for Factor VIII expression in non-human primates in 2023

— Strong cash balance of \$279.1M provides expected runway into 2025

CAMBRIDGE, Mass., Feb. 23, 2023 (GLOBE NEWSWIRE) -- [Generation Bio Co.](https://www.generationbio.com) (Nasdaq: GBIO), a biotechnology company innovating genetic medicines for people living with rare and prevalent diseases, outlined its strategic priorities for 2023 and reported fourth quarter and full year 2022 financial results.

"Generation Bio is aiming to be the new modality leader for non-viral DNA therapeutics. In the past year, we've made significant progress across our non-viral DNA therapeutics platform, most importantly in optimizing the biodistribution profile of our cell-targeted lipid nanoparticle (ctLNP) delivery system," said Geoff McDonough, M.D., president and chief executive officer of Generation Bio. "We are currently engineering novel ctLNPs with two distinct biodistribution profiles that could each enable a broad pipeline of liver disease programs, as well as programs for other tissues and cell types. Our focus for 2023 is to achieve proof-of-concept for either of these ctLNP types, which could unlock tremendous value for our pipeline."

### 2023 Strategic Priorities

Generation Bio expects to demonstrate Factor VIII expression in non-human primates (NHPs) with a novel ctLNP that supports the subsequent nomination of a development candidate for the company's lead program in hemophilia A.

The company is currently pursuing development of two types of ctLNP delivery systems. The first is a GalNAc-conjugated ctLNP designed for highly specific uptake by hepatocytes. Traditional, non-targeted LNPs are evenly distributed to the liver and the spleen, triggering immune system activation with DNA cargo. A liver-directed ctLNP could minimize immune stimulation by reducing distribution to the spleen and delivering DNA cargo more directly to hepatocytes. Beyond application to a new therapy for hemophilia A, these ctLNPs could also potentially be used in treatments for many other rare and prevalent liver diseases.

Generation Bio is also developing a "stealth" LNP, designed to avoid clearance by the spleen and liver, enabling targeting to extrahepatic tissues by conjugating a tissue- or cell-specific ligand to the LNP. This type of ctLNP could extend the reach of nucleic acid therapeutics to new tissues and cell types beyond the liver. In 2023, Generation Bio expects to demonstrate proof-of-concept for re-targeting its stealth LNP system to reach immune cells and plans to evaluate the potential to pursue non-liver programs independently and/or with one or more partners.

### Fourth Quarter and Full Year 2022 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$279.1 million as of December 31, 2022, compared with \$375.1 million in cash, cash equivalents, and marketable securities as of December 31, 2021. The company continues to believe that its cash position will fund its operating plan into 2025.
- **R&D Expenses:** Research and development (R&D) expenses were \$21.6 million for the quarter ended December 31, 2022, and \$96.7 million for the year ended December 31, 2022, compared to \$21.8 million for the quarter ended December 31, 2021, and \$85.2 million for the year ended December 31, 2021.
- **G&A Expenses:** General and administrative (G&A) expenses were \$13.1 million for the quarter ended December 31, 2022, and \$44.5 million for the year ended December 31, 2022, compared to \$9.1 million for the quarter ended December 31, 2021, and \$33.9 million for the year ended December 31, 2021.
- **Net Loss:** Net loss was \$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, compared to a net loss of \$30.9 million, or \$0.54 basic and diluted net loss per share, for the quarter ended December 31, 2021 and \$119.2 million, or \$2.12 basic and diluted net loss per share, for the year ended December 31, 2021.

### 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 cell types, 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](https://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 platform, 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; whether results from earlier preclinical studies will be predictive of the results of later preclinical studies and clinical trials; uncertainties regarding the RES manufacturing process; uncertainties regarding the company’s ability to assign or sublease its manufacturing property; 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; expectations for regulatory approvals to conduct trials or to market products; 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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#### GENERATION BIO CO. CONSOLIDATED BALANCE SHEET DATA (Unaudited) (In thousands)

#### Earnings Release Balance Sheet

	December 31, 2022	December 31, 2021
Cash, cash equivalents and marketable securities	\$ 279,091	\$ 375,145
Working capital	267,866	360,378
Total assets	376,264	476,771
Total stockholders' equity	282,493	381,746

#### 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,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	\$ 21,607	\$ 21,847	\$ 96,718	\$ 85,247
General and administrative	13,081	9,099	44,464	33,854
Total operating expenses	<u>34,688</u>	<u>30,946</u>	<u>141,182</u>	<u>119,101</u>
Loss from operations	(34,688)	(30,946)	(141,182)	(119,101)
Other income (expense):				
Other income (expense) and interest income, net	2,283	3	4,543	(50)
Net loss and net loss attributable to common stockholders	<u>\$ (32,405)</u>	<u>\$ (30,943)</u>	<u>\$ (136,639)</u>	<u>\$ (119,151)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.55)</u>	<u>\$ (0.54)</u>	<u>\$ (2.35)</u>	<u>\$ (2.12)</u>
Weighted average common shares outstanding, basic and diluted	<u>59,407,296</u>	<u>56,881,041</u>	<u>58,114,893</u>	<u>56,295,409</u>
Comprehensive loss:				
Net loss	\$ (32,405)	\$ (30,943)	\$ (136,639)	\$ (119,151)
Other comprehensive income:				
Unrealized gains (losses) on marketable securities	219	(1)	(83)	(9)
Comprehensive loss	<u>\$ (32,186)</u>	<u>\$ (30,944)</u>	<u>\$ (136,722)</u>	<u>\$ (119,160)</u>