



Generation Bio Outlines 2022 Strategic Priorities and Reports Fourth Quarter and Full Year 2021 Financial Results

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Well-capitalized with \$375.1 million at the end of 2021, funding planned operations into 2024

CAMBRIDGE, Mass., Feb. 24, 2022 (GLOBE NEWSWIRE) -- [Generation Bio Co.](#) (Nasdaq: GBIO), a biotechnology company innovating genetic medicines for people living with rare and prevalent diseases, outlined its strategic priorities for 2022 and reported fourth quarter and full year 2021 financial results.

"Our primary focus is on diseases of the liver, where we are working to complete optimization of our cell-targeted lipid nanoparticle, or ctLNP, for the liver to advance our lead hemophilia A program as well as subsequent liver programs," said Geoff McDonough, M.D., president and chief executive officer of Generation Bio. "In parallel, we are advancing our retina ctLNP to support our Stargardt program, and our vaccine ctLNP to enable our work in vaccines. We have an exceptional team and a strong balance sheet to drive toward achieving our vision."

2022 Strategic Priorities

Generation Bio has created a unique non-viral genetic medicine platform comprised of closed-ended DNA (ceDNA), rapid enzymatic synthesis (RES) manufacturing for ceDNA and a proprietary portfolio of ctLNPs. The company is applying its platform technologies in three areas: the liver, retina and vaccines.

- **Liver:** In December 2021, the company [reported preclinical data](#) demonstrating that enhanced manufacturing and production controls for its ceDNA and ctLNPs achieved peak mean human factor VIII expression of 205% in mice with a single dose, a near nine-fold improvement from studies using materials produced by prior processes. In non-human primates (NHPs), peak human factor VIII expression reached 2%. The demonstration of therapeutic protein expression in mice and NHPs represents a milestone achievement for a non-viral DNA delivery platform. The difference in expression between species is attributed to differences in biodistribution. Generation Bio plans to continue to optimize the biodistribution profile of its liver ctLNP in NHP to support the selection of a development candidate for its hemophilia A program and to advance additional liver programs.
- **Retina:** Generation Bio has developed ctLNPs for the retina that may allow the company to address a variety of inherited retinal diseases using full gene replacement or gene editing. In October 2021, the company presented [preclinical findings at the European Society of Gene and Cell Therapy \(ESGCT\)](#) demonstrating that sub-retinal delivery of messenger RNA (mRNA) or ceDNA via retina ctLNP resulted in broad photoreceptor distribution, expression, and good tolerability in rodents and in NHPs. Generation Bio plans to evaluate more advanced generations of its retina ctLNP in NHPs in order to enable potential selection of a development candidate for its lead Stargardt disease program, as well as for use in subsequent retina programs.
- **Vaccines:** Generation Bio aims to develop a novel vaccine platform using a vaccine ctLNP to deliver ceDNA or mRNA that could meet or exceed the efficacy of existing mRNA vaccines. In January 2022, the company [reported preclinical findings](#) from studies of the company's mRNA-vaccine ctLNP. At a dose of 10µg, mRNA delivered via vaccine ctLNP demonstrated a neutralizing antibody response in mice similar to the benchmark established by Moderna Therapeutics' mRNA1273, the COVID-19 vaccine approved for individuals ages 18 and older. These murine results translated to NHP a similar neutralizing antibody response was observed at a dose of 100µg. The company is also exploring ceDNA delivered with its vaccine ctLNP, which could enable more durable protection and be stored at ambient temperatures potentially allowing for greater shelf stability than current mRNA-LNP vaccines. Generation Bio plans to evaluate a next generation vaccine ctLNP in NHPs that has demonstrated enhanced potency in preclinical murine studies for both mRNA and ceDNA.

Fourth Quarter and Full Year 2021 Financial Results

- **Cash Position:** Cash and cash equivalents were \$375.1 million as of December 31, 2021, compared with \$262.3 million in cash, cash equivalents and marketable securities as of December 31, 2020. The company continues to believe that its cash position is expected to fund its operating plan into 2024.
- **R&D Expenses:** Research and development (R&D) expenses were \$21.8 million for the quarter ended December 31, 2021, and \$85.2 million for the year ended December 31, 2021, compared to \$16.4 million for the quarter ended December 31, 2020, and \$58.5 million for the year ended December 31, 2020.
- **G&A Expenses:** General and administrative (G&A) expenses were \$9.1 million for the quarter ended December 31, 2021, and \$33.9 million for the year ended December 31, 2021, compared to \$8.0 million for the quarter ended December 31, 2020, and \$22.6 million for the year ended December 31, 2020.
- **Net Loss:** Net loss was \$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, compared to a net loss of \$24.2 million, or \$0.53 basic and diluted net loss per share, for the quarter ended December 31, 2020 and \$80.5 million, or \$2.95 basic and diluted net loss per share, for the year ended December 31, 2020.

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 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.

Forward-Looking Statements

Any statements in this press release about future expectations, plans and prospects for the company, including statements about our strategic plans or objectives, our technology platform, our research and clinical development plans, applications 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 timing and ability to complete the build-out of the company's manufacturing facility and regarding the new manufacturing process; expectations for regulatory approvals to conduct trials or to market products; 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; 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)

	December 31,	
	2021	2020
Cash, cash equivalents and marketable securities	\$ 375,145	\$ 262,327
Working capital	360,378	256,515
Total assets	476,771	294,155
Total stockholders' equity	381,746	268,013

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,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	\$ 21,847	\$ 16,374	\$ 85,247	\$ 58,532

General and administrative	<u>9,099</u>	<u>7,971</u>	<u>33,854</u>	<u>22,582</u>
Total operating expenses	<u>30,946</u>	<u>24,345</u>	<u>119,101</u>	<u>81,114</u>
Loss from operations	(30,946)	(24,345)	(119,101)	(81,114)
Other income (expense):				
Other income (expense) and interest income, net	<u>3</u>	<u>119</u>	<u>(50)</u>	<u>591</u>
Net loss and net loss attributable to common stockholders	\$ <u>(30,943)</u>	\$ <u>(24,226)</u>	\$ <u>(119,151)</u>	\$ <u>(80,523)</u>
Net loss per share attributable to common stockholders, basic and diluted	\$ <u>(0.54)</u>	\$ <u>(0.53)</u>	\$ <u>(2.12)</u>	\$ <u>(2.95)</u>
Weighted average common shares outstanding, basic and diluted	<u>56,881,041</u>	<u>45,855,896</u>	<u>56,295,409</u>	<u>27,256,494</u>
Comprehensive loss:				
Net loss	\$ (30,943)	\$ (24,226)	\$ (119,151)	\$ (80,523)
Other comprehensive income:				
Unrealized (losses) gains on marketable securities	<u>(1)</u>	<u>(17)</u>	<u>(9)</u>	<u>9</u>
Comprehensive loss	\$ <u>(30,944)</u>	\$ <u>(24,243)</u>	\$ <u>(119,160)</u>	\$ <u>(80,514)</u>