



Generation Bio Reports Business Highlights and Third Quarter 2021 Financial Results

November 10, 2021

New preclinical retinal data illustrating potential for expansive therapeutic applications of ctLNP delivery presented at ESGCT 2021 Annual Virtual Congress

CAMBRIDGE, Mass., Nov. 10, 2021 (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, reported recent business highlights and third quarter 2021 financial results.

"We are building a leading non-viral genetic medicine company with the potential to transform treatments within multiple therapeutic areas," said Geoff McDonough, M.D., chief executive officer of Generation Bio. "We continue to advance our closed-ended DNA (ceDNA) and next-generation rapid enzymatic synthesis technologies, and plan to provide data on factor VIII expression in non-human primates for our hemophilia A program by year-end. We also continue to develop our cell-targeted lipid nanoparticle (ctLNP) delivery platform, and with the data recently presented at the ESGCT Congress, demonstrated a breakthrough for the retinal field, potentially paving the way for a wave of novel retinal therapies."

Business Highlights

- **Reported Preclinical Data Demonstrating Broad Delivery of ceDNA and mRNA to Photoreceptors Using ctLNP:** In October 2021, Generation Bio presented preclinical data at the European Society of Gene and Cell Therapy (ESGCT) Annual Virtual Congress showing that sub-retinal delivery of closed-ended DNA using cell-targeted lipid nanoparticle (ctLNP-ceDNA) in rodents demonstrated broad distribution and durable expression that was comparable to that achieved with adeno-associated virus Type 5 (AAV5) (the current standard of delivery) in rodents, and tolerability. These findings support the use of ctLNP-ceDNA to potentially achieve full gene replacement for inherited retinal diseases. Similar results were observed for sub-retinal delivery of mRNA using ctLNP in both rodents and non-human primates. This first-ever demonstration of species translation with comparable tolerability and uniform photoreceptor expression across species using ctLNP suggests ctLNP could be a best-in-class non-viral delivery system for mRNA to the retina, potentially enabling gene editing in the retina.

Third Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$398.4 million as of September 30, 2021, compared with \$262.3 million as of December 31, 2020.
- **R&D Expenses:** Research and development (R&D) expenses were \$22.0 million for the quarter ended September 30, 2021, compared to \$15.3 million for the quarter ended September 30, 2020.
- **G&A Expenses:** General and administrative (G&A) expenses were \$9.7 million for the quarter ended September 30, 2021, compared to \$5.7 million for the quarter ended September 30, 2020.
- **Net Loss:** Net loss was \$31.9 million, or \$0.56 basic and diluted net loss per share, for the quarter ended September 30, 2021, compared to a net loss of \$20.8 million for the quarter ended September 30, 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 tissues, 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, the expected timing of the submission of investigational new drug, or IND, applications and preclinical data, our manufacturing plans, our expectations regarding our new facility 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 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 regarding the timing of submission of IND applications; 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)

Earnings Release Balance Sheet

	September 30, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	\$ 398,363	\$ 262,327
Working capital	388,764	256,515
Total assets	464,033	294,155
Total stockholders' equity	407,210	268,013

GENERATION BIO CO. CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS (Unaudited)

(in thousands, except share and per share data)

	Three Months Ended September 30,	
	2021	2020
Operating expenses:		
Research and development	\$ 21,991	\$ 15,308
General and administrative	9,667	5,661
Total operating expenses	31,658	20,969
Loss from operations	(31,658)	(20,969)
Other (expense) income:		
Other (expense) and interest income, net	(197)	120
Net loss and net loss attributable to common stockholders	\$ (31,855)	\$ (20,849)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.56)	\$ (0.46)
Weighted average common shares outstanding, basic and diluted	56,629,193	45,468,838
Comprehensive loss:		
Net loss	\$ (31,855)	\$ (20,849)
Other comprehensive loss:		
Unrealized (losses) gains on marketable securities	(3)	30
Comprehensive loss	\$ (31,858)	\$ (20,819)

