



Generation Bio Reports Business Highlights and Third Quarter 2023 Financial Results

November 9, 2023

- Immune-quiet DNA evades innate immune sensors in both mice and non-human primates addressing a central challenge for non-viral DNA therapeutics
- Proprietary cell-targeted LNP delivery system showed highly selective T cell transduction in humanized mouse model, demonstrating *in vivo* targeting for T cells and potentially for other extrahepatic tissues and cell types
- Third quarter 2023 cash balance of \$291.0 million expected to fund operations into 2026

CAMBRIDGE, Mass., Nov. 09, 2023 (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 third quarter 2023 financial results.

"The recent advances in our immune-quiet DNA (iqDNA) and cell-targeted lipid nanoparticle (ctLNP) platforms represent two breakthroughs in the field of genetic medicine," said Geoff McDonough, chief executive officer of Generation Bio. "Our iqDNA has been shown to evade host innate immune sensors in mice and non-human primates (NHPs), addressing a central challenge for non-viral DNA therapeutics. Our ctLNP delivery system overcomes an equally central challenge for delivery, which is the highly selective targeting of DNA and/or RNA cargo to extrahepatic tissues and cell types. The foundational properties of iqDNA and our ctLNP have translated across mice and NHPs, and we now look forward to generating additional NHP data with therapeutic payloads for each platform."

Business Highlights

- **Developed iqDNA to Evade Innate Immune Sensors:** Generation Bio announced results from preclinical studies in NHPs showing that an intravenous infusion of iqDNA delivered by an LNP triggered little to no activation of a panel of cytokines associated with the innate immune response. Innate immunity has been a key challenge for DNA-based non-viral genetic medicines as it has typically limited their therapeutic index.

Given the improved tolerability profile of iqDNA versus prior closed-ended DNA (ceDNA) constructs, the company is advancing iqDNA as its lead cargo across all programs, including its lead program in hemophilia A. The company believes that iqDNA's ability to evade detection by key innate immune sensors while driving protein expression will allow it to take advantage of a broader array of LNP delivery systems. Planned next steps for the platform include scaling iqDNA encoding Factor VIII protein for testing in NHPs.

- **Demonstrated Highly Selective T Cell Transduction:** At the European Society for Gene and Cell Therapy (ESGCT) 30th Annual Congress, Generation Bio announced data showing that 70% of circulating and splenic T cells were positive for mRNA expression with less than 8% delivery to off-target immune cells following dosing of its ctLNP in a humanized mouse model. Furthermore, the profile of the underlying stealth LNP translated to NHPs, with less than 0.1% of the dose taken up by off-target tissues including the liver and spleen. The studies were conducted as part of the company's collaboration with Moderna, Inc. and provide initial proof of concept for *in vivo* delivery to T cells and the potential ability to apply the ctLNP platform to a broader range of extrahepatic tissues and cell types.

Third Quarter 2023 Financial Results

- **Cash Position:** Cash, cash equivalents, and marketable securities were \$291.0 million as of September 30, 2023, compared to \$279.1 million in cash, cash equivalents, and marketable securities as of December 31, 2022. The company continues to believe that its cash position will fund its operating plan into 2026.
- **R&D Expenses:** Research and development (R&D) expenses were \$21.9 million for the quarter ended September 30, 2023, compared to \$21.2 million for the quarter ended September 30, 2022.
- **G&A Expenses:** General and administrative (G&A) expenses were \$11.6 million for the quarter ended September 30, 2023, compared to \$11.5 million for the quarter ended September 30, 2022.
- **Net Loss:** Net loss was \$28.3 million, or \$0.43 basic and diluted net loss per share, for the quarter ended September 30, 2023, compared to a net loss of \$31.3 million, or \$0.53 basic and diluted net loss per share, for the quarter ended September 30, 2022.

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 novel immune-quiet DNA, or iqDNA; 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 iqDNA. This approach 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.

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; uncertainties regarding our novel technologies; 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; 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 | September 30, 2023 | December 31, 2022 |
|--|---------------------------|--------------------------|
| Cash, cash equivalents and marketable securities | \$ 291,025 | \$ 279,091 |
| Working capital | 264,990 | 267,866 |
| Total assets | 381,492 | 376,264 |
| Total stockholders' equity | 231,914 | 282,493 |

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

| | Three Months Ended September 30, | |
|---|---|-------------|
| | 2023 | 2022 |
| Revenues: | | |
| Collaboration Revenue | \$ 2,146 | \$ — |
| Operating expenses: | | |
| Research and development | 21,862 | 21,192 |
| General and administrative | 11,641 | 11,477 |
| Total operating expenses | 33,503 | 32,669 |
| Loss from operations | (31,357) | (32,669) |
| Other income: | | |
| Other income and interest income, net | 3,091 | 1,363 |
| Net loss and net loss attributable to common stockholders | \$ (28,266) | \$ (31,306) |
| Net loss per share attributable to common stockholders, basic and diluted | \$ (0.43) | \$ (0.53) |
| Weighted average common shares outstanding, basic and diluted | 65,907,000 | 58,872,334 |

Comprehensive loss:

| | | | | |
|---|----|-----------------|----|-----------------|
| Net loss | \$ | (28,266) | \$ | (31,306) |
| Other comprehensive loss: | | | | |
| Unrealized gains on marketable securities | | 19 | | 37 |
| Comprehensive loss | \$ | <u>(28,247)</u> | \$ | <u>(31,269)</u> |