



Generation Bio Reports Fourth Quarter and Full Year 2020 Financial Results

March 18, 2021

\$262.3 million cash balance at the end of 2020 bolstered by additional net proceeds of \$211.3 million from follow-on offering, funding key milestones into 2024

Ron Cooper appointed to Board of Directors

Company remains on track to begin IND-enabling studies for hemophilia A in 2021

CAMBRIDGE, Mass., March 18, 2021 (GLOBE NEWSWIRE) -- [Generation Bio Co.](#) (Nasdaq: GBIO), an innovative genetic medicines company creating a new class of non-viral gene therapy, reported recent business highlights and fourth quarter and full year 2020 financial results.

"2020 was a foundational year for Generation Bio in which we strengthened the leadership team, advanced our platform science, and added substantially to our balance sheet to fund our lead programs to human data," said Geoff McDonough, M.D., president and chief executive officer of Generation Bio. "We entered 2021 with preclinical data in mice and non-human primates that we believe demonstrate our platform's potential to go beyond what is currently possible with gene therapy for hemophilia A. We expect to announce additional data throughout the remainder of this year that will support the rapid expansion of our pipeline and showcase our differentiated approach to creating a new class of genetic medicine."

Recent Business Highlights

- **Raised \$225.4 million in gross proceeds from follow-on offering:** In January 2021, Generation Bio closed a public offering of 9,200,000 shares of its common stock at a public offering price of \$24.50 per share, including 1,200,000 additional shares of common stock issued upon the exercise in full by the underwriters of their option to purchase additional shares. The gross proceeds of the offering, before deducting the underwriting discount and commissions and other offering expenses payable by Generation Bio, were approximately \$225.4 million.
- **Appointed Ron Cooper to Board of Directors:** In February 2021, Generation Bio announced the appointment of Ron Cooper to its Board of Directors. Mr. Cooper is a global biopharmaceutical executive who currently serves as president, chief executive officer and board member of Albireo Pharma, Inc.
- **Announced preclinical data that support development candidate selection for hemophilia A program:** Also in January 2021, the company reported data from a series of preclinical studies evaluating the expression of factor VIII in both mice and non-human primates (NHP). Data in mice showed that a single dose of a closed-ended DNA (ceDNA) construct delivered via the company's novel, cell-targeted lipid nanoparticle (ctLNP) system led to mean human factor VIII expression levels from 9% to 23% of normal with a dose response across the three cohorts. In two additional separate studies, a mean 2:1 species translation ratio from mice to NHPs was observed, consistent with best-in-class translation ratios seen for other nucleic acids delivered via LNPs.

Selected Anticipated Company Milestones in 2021

- NHP factor VIII expression data for hemophilia A development candidate
- NHP factor IX expression, durability and redosing data
- Initiation of IND-enabling studies for phenylketonuria (PKU)
- Preclinical data for Gaucher disease and Wilson disease
- Preclinical data for expression of therapeutic antibodies

Fourth Quarter and Full Year 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$262.3 million as of December 31, 2020, compared with \$15.1 million as of December 31, 2019. Cash at December 31, 2020 does not include the \$211.3 million in net proceeds received from the follow-on public offering completed in January 2021.
- **R&D Expenses:** Research and development (R&D) expenses were \$16.4 million for the quarter ended December 31, 2020 and \$58.5 million for the year ended December 31, 2020, compared to \$12.7 million for the quarter ended December 31, 2019 and \$50.1 million for the year ended December 31, 2019.
- **G&A Expenses:** General and administrative (G&A) expenses were \$8.0 million for the quarter ended December 31, 2020 and \$22.6 million for the year ended December 31, 2020, compared to \$3.4 million for the quarter ended December 31,

2019 and \$12.2 million for the year ended December 31, 2019.

- **Net Loss:** Net loss was \$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, compared to a net loss of \$16.1 million, or \$3.09 basic and diluted net loss per share, for the quarter ended December 31, 2019 and \$61.3 million, or \$12.96 basic and diluted net loss per share, for the year ended December 31, 2019.

About Generation Bio

Generation Bio is an innovative genetic medicines company focused on creating a new class of non-viral gene therapy to provide durable, redosable treatments for people living with rare and prevalent diseases. The company's non-viral platform incorporates a proprietary, high-capacity DNA construct called closed-ended DNA, or ceDNA; a cell-targeted lipid nanoparticle delivery system, or ctLNP; and an established, scalable capsid-free manufacturing process. The platform is designed to enable multi-year durability from a single dose of ceDNA and to allow titration and redosing if needed. The ctLNP is engineered to deliver large genetic payloads, including multiple genes, to specific tissues to address a wide range of indications. The company's efficient, scalable manufacturing process supports Generation Bio's mission to extend the reach of gene therapy to more people, living with more diseases, in more places around the world.

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 platforms, our research and clinical development plans, 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; 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 quarterly report on Form 10-Q, which is 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, | |
|--|---------------------|-------------|
| | 2020 | 2019 |
| Cash, cash equivalents and marketable securities | \$ 262,327 | \$ 15,076 |
| Working capital | 256,515 | 8,998 |
| Total assets | 294,155 | 42,140 |
| Convertible preferred stock | — | 115,593 |
| Total stockholders' equity (deficit) | 268,013 | (98,592) |

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, | |
|---|------------------------------------|--------------------|-------------------------|--------------------|
| | 2020 | 2019 | 2020 | 2019 |
| Operating expenses: | | | | |
| Research and development | \$ 16,374 | \$ 12,731 | \$ 58,532 | \$ 50,134 |
| General and administrative | 7,971 | 3,427 | 22,582 | 12,168 |
| Total operating expenses | <u>24,345</u> | <u>16,158</u> | <u>81,114</u> | <u>62,302</u> |
| Loss from operations | (24,345) | (16,158) | (81,114) | (62,302) |
| Other income (expense): | | | | |
| Interest income and other income (expense), net | 119 | 95 | 591 | 985 |
| Net loss and net loss attributable to common stockholders | <u>\$ (24,226)</u> | <u>\$ (16,063)</u> | <u>\$ (80,523)</u> | <u>\$ (61,317)</u> |
| Net loss per share attributable to common stockholders, basic and diluted | <u>\$ (0.53)</u> | <u>\$ (3.09)</u> | <u>\$ (2.95)</u> | <u>\$ (12.96)</u> |
| Weighted average common shares outstanding, basic and diluted | <u>45,855,896</u> | <u>5,205,942</u> | <u>27,256,494</u> | <u>4,731,519</u> |
| Comprehensive loss: | | | | |
| Net loss | \$ (24,226) | \$ (16,063) | \$ (80,523) | \$ (61,317) |
| Other comprehensive income: | | | | |
| Unrealized (loss) gain on marketable securities | (17) | (2) | 9 | 9 |
| Comprehensive loss | <u>\$ (24,243)</u> | <u>\$ (16,065)</u> | <u>\$ (80,514)</u> | <u>\$ (61,308)</u> |