Generation Bio Presents Preclinical Data Demonstrating Broad Potential of Gene Therapy Platform at ASGCT and Reports First Quarter Financial Results

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Durable, therapeutically relevant levels of anti-SARS-CoV-2 spike antibodies produced from the liver in a mouse model

Next-generation rapid enzymatic synthesis of closed-ended DNA further increases the efficiency and scale of the manufacturing platform

Potent factor VIII construct optimized by taking advantage of the increased cargo capacity of closed-ended DNA

CAMBRIDGE, Mass., May 12, 2021 (GLOBE NEWSWIRE) -- Generation Bio Co. (Nasdaq: GBIO), an innovative genetic medicines company creating a new class of non-viral gene therapy, today reported data from multiple digital presentations during the ongoing 24th American Society of Gene and Cell Therapy (ASGCT) Annual Meeting as well as first quarter 2021 financial results.

"We are excited to highlight two significant new developments for our novel, non-viral gene therapy platform that showcase its potential to address a broad range of diseases at a manufacturing scale unprecedented for gene therapy," said Geoff McDonough, M.D., president and chief executive officer of Generation Bio. "We have long thought that therapeutic antibodies could be produced by the liver, potentially expanding the durability and reach for certain classes of biologics. Our data demonstrate endogenous therapeutic antibody production from the livers of mice for anti-SARS-CoV-2 spike antibodies. Combining these data with our proprietary rapid enzymatic synthesis method for closed-ended DNA (ceDNA) could open the door to address a number of highly prevalent infectious diseases. 2021 is set to be milestone-rich for the company, and we remain on track to execute our goals for the year."

Highlights from Digital Presentations at ASGCT

- **Novel non-viral gene therapy unlocks significant potential for genetic medicine:** In a digital presentation entitled "Non-Viral Gene Delivery of Human FVIII to Hemophilia A Mice and Non-Human Primates," the company presented insights into the scientific breakthroughs underpinning its unique non-viral approach, specifically the on-target biodistribution of its cell-targeted lipid nanoparticle (ctLNP) and the access to the nucleus afforded by ceDNA. To view the digital presentation, please visit Generation Bio's website.

- **Durable expression of therapeutically relevant levels of anti-SARS-CoV-2 spike monoclonal antibody (mAb) expressed from the liver in mice:** Building on its core technologies, Generation Bio has designed a ceDNA-based approach to generate persistent expression of therapeutic antibodies from the liver. Endogenous therapeutic antibody production may provide an alternative means of delivering specific mAbs, equipping patients with the ability to produce their own biotherapeutics for an extended period of time per dose. In a digital presentation entitled, "Vector Optimization for Non-Viral Antibody Gene Therapy and Expression of Human Monoclonal Antibodies in Mice," the data presented show that ceDNA delivered via LNP enabled mice to generate persistent anti-spike protein human antibody concentrations reaching peak expression of 8 µg/ml, which corresponds to a level that may be therapeutically relevant in humans. Importantly, endogenously produced antibodies in the serum of ceDNA-treated mice retained binding and functional activity, neutralizing SARS-CoV-2 *ex vivo* at the same level as recombinantly produced monoclonal antibodies. Generation Bio developed these data as part of its collaboration with Vir Biotechnology, Inc. To view the digital presentation, please visit Generation Bio's website.

- **Next-generation rapid enzymatic synthesis further expands efficiency and scale of manufacturing platform:** In the digital presentation on optimization of non-viral endogenous therapeutic antibody production, Generation Bio outlined a proprietary, next-generation rapid enzymatic synthesis of ceDNA directly from nucleic acid components, without relying on Sf9 cells. The structure and sequence of ceDNA from enzymatic synthesis is comparable to Sf9-produced ceDNA but shortens the ceDNA production cycle from 28 days to one day. This enzymatic process is similar to the one used to manufacture messenger RNA (mRNA) vaccines, with what the company believes will be a comparable trajectory of cost and scale.

- **Construct optimization improves potency of ctLNP-ceDNA in mouse models of hemophilia A**: Generation Bio's ceDNA constructs are not constrained by the 4.7 kb transgene size limitation faced by adeno-associated virus (AAV) gene therapy. In a poster entitled, "When Size Matters: FVIII Construct Optimization Leveraging ceDNA, a Non-Viral Gene Therapy Platform," the company described its semi-combinatorial approach to optimizing factor VIII expressing ceDNA, which resulted in 34-times higher expression than an analogous wild-type factor VIII construct. This demonstrates one aspect of the potential that derives from ceDNA's greater cargo capacity, which includes improved construct potency, larger or multiple genes and the inclusion of regulatory elements that can modulate transgene expression. To view the digital presentation, please visit Generation Bio's website.

First Quarter 2021 Financial Results

- **Cash Position**: Cash, cash equivalents and marketable securities were $451.1 million as of March 31, 2021, compared with $262.3 million as of December 31, 2020.
- **R&D Expenses**: Research and development (R&D) expenses were $18.8 million for the quarter ended March 31, 2021, compared to $13.4 million for the quarter ended March 31, 2020.
- **G&A Expenses**: General and administrative (G&A) expenses were $6.9 million for the quarter ended March 31, 2021, compared to $4.6 million for the quarter ended March 31, 2020.
- **Net Loss:** Net loss was $25.6 million, or $0.46 basic and diluted net loss per share, for the quarter ended March 31, 2021, compared to a net loss of $17.7 million, or $3.22 basic and diluted net loss per share, for the quarter ended March 31, 2020.

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 annual report on Form 10-K, 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)

Earnings Release Balance Sheet	March 31, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	$ 451,083	$ 262,327
Working capital	444,991	256,515
Total assets	514,081	294,155
Total stockholders' equity	458,736	268,013

GENERATION BIO CO.
CONSOLIDATED STATEMENTS OF OPERATIONS
(Unaudited)

(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2021	**2020**
Operating expenses:		
Research and development	$ 18,753	$ 13,394
General and administrative	6,902	4,642
Total operating expenses	25,655	18,036
Loss from operations	(25,655)	(18,036)
Other income:		
Interest income	93	319
Net loss and net loss attributable to common stockholders	$ (25,562)	$ (17,717)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.46)	$ (3.22)
Weighted average common shares outstanding, basic and diluted	55,366,238	5,495,013
Comprehensive loss:		
Net loss	$ (25,562)	$ (17,717)
Other comprehensive income:		
Unrealized gains on marketable securities	1	—
Comprehensive loss	$ (25,561)	$ (17,717)