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Graphite Bio Reports Recent Business Progress and Fourth Quarter and Fiscal Year 2021 Financial Results

Patient enrollment in Phase 1/2 CEDAR clinical trial of GPH101 for sickle cell disease ongoing at multiple sites; dosing of first patient now planned for second half of 2022, with initial proof-of-concept data anticipated in 2023

Prioritized R&D to maximize capabilities of company's next-generation gene editing platform and significantly impact patient outcomes

Bolstered company leadership with hiring of chief financial officer and chief of staff, and promotion of chief people officer

$378.7 million in cash, cash equivalents and restricted cash as of December 31, 2021; cash runway extended into the fourth quarter of 2024

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)-- Graphite Bio, Inc. (Nasdaq: GRPH), a clinical-stage, next-generation gene editing company focused on developing therapies that harness targeted gene integration to treat or cure serious diseases, today reported recent business progress and fourth quarter and fiscal year 2021 financial results.

"2021 was a pivotal year in our company's history as we established ourselves as a public company and initiated the clinical trial for our lead candidate, GPH101 for sickle cell disease. In 2022, we remain focused on advancing our research and development priorities, in particular the execution of our Phase 1/2 CEDAR clinical trial of GPH101. We continue to work toward demonstrating that our unique gene correction approach to reduce sickle hemoglobin production and restore adult hemoglobin expression has the potential to achieve the ideal genetic outcome and provide a definitive cure for patients with sickle cell disease," said Josh Lehrer, M.D., M.Phil., chief executive officer of Graphite Bio.

"Over the course of this year, we look forward to sharing more information about our research programs, including GPH102, our differentiated gene replacement program for beta-thalassemia," Lehrer continued. "With our updated pipeline priorities, our programs now even more closely align with our goal of transforming the gene therapy treatment paradigm – from how we develop and manufacture these individualized therapies to how we deliver them to patients. Our mission is to make 'one dose, one cure' a reality for as many patients as possible with serious diseases and inadequate treatments."

Program Updates

GPH101 for Sickle Cell Disease

- Continued patient enrollment in the Phase 1/2 CEDAR clinical trial of GPH101, an investigational therapy designed to directly correct the genetic mutation responsible for

sickle cell disease (SCD), at multiple sites across the United States. Due to impacts of the recent COVID-19 Omicron variant surge on patients and site resources and operations, the company now plans to dose its first patient in the second half of 2022, with initial proof-of-concept data anticipated in 2023.
- Presented a trial-in-progress poster about the CEDAR trial at the 63rd American Society of Hematology (ASH) Annual Meeting & Exposition and hosted an event featuring a discussion among key opinion leaders about how the company's differentiated gene correction approach using GPH101 could lead to curative outcomes for SCD patients.
- Presented preclinical data for GPH101 at the 49th Annual Sickle Cell Disease Association of America's National Convention. These data, which support the ability of the company's gene editing platform to precisely and efficiently correct the underlying SCD-causing mutation to reduce sickle hemoglobin production and restore adult hemoglobin expression to levels that are considered potentially curative, are foundational to the company's SCD program and support the evaluation of GPH101 in the CEDAR trial.

Additional R&D Updates

Prioritized and advanced research programs to maximize the capabilities of the company's next-generation gene editing platform and significantly impact patient outcomes, including:

- Announced a new research program, GPH102 for the treatment of beta-thalassemia, a genetic blood disorder that reduces the production of hemoglobin and leads to severe anemia and, in severe cases, transfusion dependency. Using the company's gene replacement technology, GPH102 is designed to replace the entire mutated beta-globin gene and restore adult hemoglobin expression to levels similar to individuals who do not have disease or have beta-thalassemia trait. The company expects to submit an IND for this program by mid-2024, pending feedback from health authorities.
- Disclosed an early-stage research program for the treatment of alpha-1 antitrypsin (AAT) deficiency, a severe inherited genetic disorder that can cause progressive lung and liver disease. The program leverages the company's targeted gene insertion technology to permanently increase AAT protein production, offering a potentially differentiated approach to treating the disease. The company expects to provide updates about this program over the course of 2022.
- Initiated discovery research efforts to develop non-genotoxic hematopoietic stem cell (HSC) targeted conditioning (NGTC) regimens, which could greatly broaden the number of diseases and patients who can be treated with precision engineered, one-time treatments and cures. The company also intends to leverage industry advances to accelerate its efforts in this area.
- Announced intentions to develop GPH201 for X-linked combined deficiency syndrome (XSCID) with an academic partner. The company believes GPH201 has the potential to be an important treatment option for this ultra-rare disease, and data from this program could be informative to the company's platform and pipeline.
- Reassessed the development strategy for GPH301 for Gaucher disease. The company believes GPH301 can have the greatest impact as a potential one-time curative treatment and alternative to chronic enzyme replacement therapy in patients with Type 1 Gaucher disease, when combined with a potential NGTC regimen. The company is assessing a strategy and timeline for GPH301 to enter clinical testing with an NGTC

regimen.

Business Updates

- Appointed Alethia Young as chief financial officer. Ms. Young has more than 20 years of experience in healthcare and biotechnology equity research and investing. She joins the company from Cantor Fitzgerald, where she served as senior biotechnology analyst and head of research, managing the equity research department covering companies across the biotechnology industry.
- Welcomed Christine Garrett, Ph.D., as chief of staff and senior vice president of operations. Dr. Garrett has more than 20 years of industry experience leading cross-functional teams comprised of research, development and commercial leaders across various phases of drug development.
- Promoted Julia Tran to the role of chief people officer. Ms. Tran has more than 20 years of experience building and growing passionate, mission-driven organizations that are committed to developing innovative solutions to tackle some of the biggest global challenges in biotech, cleantech and cybersecurity.

Fourth Quarter Financial Highlights

- **Cash, Cash Equivalents and Restricted Cash:** As of December 31, 2021, cash, cash equivalents and restricted cash totaled $378.7 million. The company expects this will fund its planned operations into the fourth quarter of 2024.
- **R&D Expenses:** Research and development expenses were $11.2 million for the fourth quarter of 2021, which includes $1.1 million in stock-based compensation expense.
- **G&A Expenses:** General and administrative expenses were $7.7 million for the fourth quarter of 2021, which includes $1.4 million in stock-based compensation expense.
- **Net Loss:** Net loss was $18.9 million, or $0.35 per basic and diluted share, for the fourth quarter of 2021.

Fiscal Year 2021 Financial Highlights

- **R&D Expenses:** Research and development expenses were $37.9 million for fiscal year 2021, which includes $2.7 million in stock-based compensation expense.
- **G&A Expenses:** General and administrative expenses were $22.5 million for fiscal year 2021, which includes $5.2 million in stock-based compensation expense.
- **Net Loss:** Net loss was $70.8 million, or $2.45 per basic and diluted share, for fiscal year 2021. This includes a change in the fair value of our Series A redeemable convertible preferred stock, which was fully converted upon our initial public offering in June 2021.

About Graphite Bio

Graphite Bio is a clinical-stage, next-generation gene editing company harnessing high efficiency targeted gene integration to develop a new class of therapies to potentially cure a wide range of serious and life-threatening diseases. Graphite Bio is pioneering a precision gene editing approach that could enable a variety of applications to transform human health through its potential to achieve one of medicine’s most elusive goals: to precisely “find & replace” any gene in the genome. Graphite Bio’s platform allows it to precisely correct

mutations, replace entire disease-causing genes with normal genes or insert new genes into predetermined, safe locations. The company was co-founded by academic pioneers in the fields of gene editing and gene therapy, including Maria Grazia Roncarolo, M.D., and Matthew Porteus, M.D., Ph.D.

Learn more about Graphite Bio by visiting www.graphitebio.com and following the company on LinkedIn.

Forward-Looking Statements

Statements we make in this press release may include statements which are not historical facts and are considered forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). These statements may be identified by words such as "aims," "anticipates," "believes," "could," "estimates," "expects," "forecasts," "goal," "intends," "may," "plans," "possible," "potential," "seeks," "will," and variations of these words or similar expressions that are intended to identify forward-looking statements. Any such statements in this press release that are not statements of historical fact, including statements regarding the clinical and therapeutic potential of our gene editing platform and our product candidates, the timing for treating the first patient in our Phase 1/2 clinical trial of GPH101 and the availability of initial proof-of-concept data, our research and development plans, including our GPH102 research program for the treatment of beta-thalassemia and our plans to submit an IND for this program, our research program for the treatment of AAT and our plans to provide updates regarding this program, our plans to develop GPH201 for XSCID and GPH301 for Gaucher disease with an NGTC regimen, and the timing of these activities, and our anticipated cash runway, may be deemed to be forward-looking statements. We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Exchange Act and are making this statement for purposes of complying with those safe harbor provisions.

Any forward-looking statements in this press release are based on Graphite Bio's current expectations, estimates and projections only as of the date of this release and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements, including the risk that we may encounter delays in patient enrollment and in the initiation, conduct and completion of our planned clinical trials. These risks concerning Graphite Bio's programs and operations are described in additional detail in its periodic filings with the SEC, including its quarterly report on Form 10-Q filed with the SEC on November 10, 2021, and subsequent filings thereafter. Graphite Bio explicitly disclaims any obligation to update any forward-looking statements except to the extent required by law.

GRAPHITE BIO, INC.
Statements of Operations and Comprehensive Loss
(In thousands, except share and per share data)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2021	**2020**	**2021**	**2020**
Operating expenses*:				
Research and development	$ 11,205	$ 6,390	$ 37,932	$ 9,123
General and administrative	7,736	1,933	22,511	4,377
Total operating expenses	18,941	8,323	60,443	13,500
Loss from operations	(18,941)	(8,323)	(60,443)	(13,500)
Other income (expense), net:				
Other income, net	10	—	24	—
Related party convertible note interest expense	—	—	—	(40)
Change in fair value of the Series A redeemable convertible preferred stock tranche liability	—	(55,213)	(10,341)	(54,833)
Total other income (expense), net	10	(55,213)	(10,317)	(54,873)
Net loss and comprehensive loss	$ (18,931)	$ (63,536)	$ (70,760)	$ (68,373)
Net loss per common share – basic and diluted	$ (0.35)	$ (18.68)	$ (2.45)	$ (29.93)
Weighted-average number of common shares outstanding – basic and diluted	53,429,766	3,401,912	28,919,255	2,284,087
*** Includes stock-based compensation as follows:**				
Research and development	$ 1,051	$ 117	$ 2,685	$ 123
General and administrative	1,396	24	5,186	54
Total stock-based compensation expense	$ 2,447	$ 141	$ 7,871	$ 177

GRAPHITE BIO, INC.
Balance Sheets
(In thousands)

	December 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 376,976	$ 19,782
Restricted cash	-	35
Prepaid expenses and other current assets	4,760	1,286
Total current assets	381,736	21,103
Restricted cash, non-current	1,716	—
Property, plant, and equipment, net	6,507	1,461
Operating lease right-of-use assets	11,574	—
Other assets	454	—
Total assets	$ 401,987	$ 22,564
Liabilities, redeemable convertible preferred stock, and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 2,453	$ 630
Accrued compensation	2,689	466
Accrued research costs	633	1,764
Accrued expenses and other current liabilities	886	126
Current portion of operating lease liabilities	5,482	—
Series A redeemable convertible preferred stock tranche liability	—	29,062
Total current liabilities	12,143	32,048
Non-current operating lease liabilities	5,794	—
Other liabilities	—	316
Total liabilities	17,937	32,364
Series A redeemable convertible preferred stock	—	55,608
Stockholders' equity (deficit):		
Common stock	1	—
Additional paid-in capital	525,400	5,183
Accumulated deficit	(141,351)	(70,591)
Total stockholders' equity (deficit)	384,050	(65,408)
Total liabilities, redeemable convertible preferred stock, and stockholders' equity (deficit)	$ 401,987	$ 22,564

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