November 9, 2022

Graphite Bio Reports Recent Business Progress and Third Quarter 2022 Financial Results

Dosed first sickle cell patient with nula-cel; initial proof-of-concept data on track for mid-2023

Two abstracts accepted for 64th ASH Annual Meeting and Exposition in December

Entered strategic partnership with Philadelphia-based WuXi Advanced Therapies for the manufacturing of nula-cel

Bolstered company leadership with hiring of senior vice president of development

$305.1 million in cash, cash equivalents and investments in marketable securities as of September 30, 2022; cash runway into fourth quarter 2024

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)-- Graphite Bio, Inc. (Nasdaq: GRPH), a clinical-stage, next-generation gene editing company harnessing the power of high-efficiency precision gene repair to develop therapies with the potential to treat or cure serious diseases, today reported recent business progress and third quarter 2022 financial results.

“Over the third quarter, we have continued to focus on execution of our Phase 1/2 CEDAR clinical trial of nula-cel for sickle cell disease. We are pleased to have recently dosed our first patient with nula-cel, marking the first time an investigational therapy designed to correct a genetic mutation has been administered to a patient. We continue to enroll patients in CEDAR and look forward to reporting initial clinical proof-of-concept data in mid-2023,” said Josh Lehrer, M.D., M.Phil., chief executive officer of Graphite Bio. “Additionally, we are excited to partner with WuXi Advanced Therapies to support the manufacturing of nula-cel as we scale and work to develop and deliver our potentially curative therapy for sickle cell disease to those who desperately need it. Finally, we are thrilled to welcome Darren Hart to the Graphite Bio team, whose expertise in overseeing clinical trials, including those for sickle cell disease, will play a pivotal role in bringing our next-generation therapies to patients.”

Program Updates

Nulabeglogene autogedtemcel (nula-cel), formerly GPH101, for sickle cell disease

- Dosed the first patient with nula-cel in the Phase 1/2 CEDAR clinical trial. Nula-cel is an investigational gene-editing therapy designed to directly correct the genetic mutation that causes sickle cell disease. Enrollment in CEDAR is ongoing at multiple sites across the United States. The company is on track to share initial proof-of-concept data in mid-2023.
- Received acceptance of two abstracts at the 64th American Society of Hematology

(ASH) Annual Meeting and Exposition, which will take place December 10-13 in New Orleans. Graphite Bio will present details about the development of a single-cell RNA sequencing (scRNAseq) method to assess the gene correction outcomes in patients treated with nula-cel. In addition, an abstract highlighting the application of the company's UltraHDR™ gene editing platform for the treatment of beta-thalassemia was accepted for online publication in *Blood*.

Business Updates

- Entered into a strategic partnership with WuXi Advanced Therapies, a global contract testing, development and manufacturing organization (CTDMO) based in Philadelphia, to support the manufacturing, testing and validation of nula-cel. WuXi's expertise in manufacturing cell and gene therapies will support Graphite Bio's efforts to scale for the continued development of nula-cel, particularly as the company prepares for late-stage clinical testing and potential commercialization.
- Welcomed Darren Hart, B.S.N., J.D., as senior vice president of development. Mr. Hart has more than 25 years of experience overseeing clinical trials, including those for sickle cell disease. He joined Graphite Bio from Denali Therapeutics where he oversaw development operations.

Second Quarter Financial Highlights

- **Cash Position:** As of September 30, 2022, cash, cash equivalents and investments in marketable securities totaled $305.1 million. The company continues to expect this will fund its planned operations into the fourth quarter of 2024.
- **R&D Expenses:** Research and development expenses were $18.3 million for the third quarter of 2022, which includes $1.2 million in stock-based compensation expense.
- **G&A Expenses:** General and administrative expenses were $7.9 million for the third quarter of 2022, which includes $2.0 million in stock-based compensation expense.
- **Net Loss:** Net loss was $24.7 million, or $0.45 per basic and diluted share, for the quarter ended September 30, 2022.

About nulabeglogene autogedtemcel (nula-cel)

Nula-cel, formerly GPH101, is an investigational next-generation gene-editing autologous hematopoietic stem cell (HSC) therapy designed to directly correct the genetic mutation that causes sickle cell disease (SCD). A serious, life-threatening inherited blood disorder, SCD affects approximately 100,000 people in the United States and millions of people around the world, making it the most prevalent monogenic blood disease worldwide. Nula-cel is the first investigational therapy to use a highly differentiated gene correction approach that seeks to efficiently and precisely correct the mutation in the beta-globin gene to decrease sickle hemoglobin (HbS) production and restore adult hemoglobin (HbA) expression, thereby potentially curing SCD. The U.S. Food and Drug Administration (FDA) granted Fast Track and Orphan Drug designations to nula-cel for the treatment of SCD.

Graphite Bio is evaluating nula-cel in the CEDAR trial, an open-label, multi-center Phase 1/2 clinical trial designed to assess safety, engraftment success, gene correction rates, total hemoglobin, as well as other clinical and exploratory endpoints and pharmacodynamics in patients with severe SCD.

About GPH102

GPH102 is Graphite Bio's research program for the treatment of beta-thalassemia, one of the most common autosomal recessive disorders with approximately 68,000 people worldwide born with the disease each year. Beta-thalassemia is a genetic blood disorder characterized by reduced production of beta-globin, a protein that forms oxygen-carrying hemoglobin with alpha-globin. Individuals with the most severe form of beta-thalassemia fail to produce functional beta-globin, which results in severe anemia and transfusion dependency. Using Graphite Bio's gene replacement approach, GPH102 is designed to replace the mutated beta-globin gene with a functional gene and restore adult hemoglobin (HbA) expression to levels similar to individuals who do not have the disease.

About Graphite Bio

Graphite Bio is a clinical-stage, next-generation gene editing company driven to discover and develop cures for a wide range of serious and life-threatening diseases. The company is pioneering a precision gene editing approach that has the potential to transform human health by achieving one of medicine's most elusive goals: to precisely "find & replace" any gene in the genome. Graphite Bio's UltraHDR™ gene editing platform takes CRISPR beyond cutting and harnesses the power of high-efficiency precision DNA repair, also known as homology directed repair (HDR), to precisely correct genetic mutations, replace entire disease-causing genes with functional 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 and Twitter.

Forward-Looking Statements

Statements we make in this press release may include statements that 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 our projected cash runway, our nula-cel (formerly GPH101) product candidate, its clinical and therapeutic potential, our plans to advance nula-cel in our Phase 1/2 CEDAR trial, the availability of proof-of-concept data from the trial, the progress and success of our strategic partnership with WuXi Advanced Therapies, including our plans to scale for the continued development of nula-cel, and the timing of these events, 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 views about our plans, intentions, expectations, strategies and prospects 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 regulatory hurdles or delays, for example, in patient enrollment and dosing, and in the progress, conduct and completion of our Phase 1/2 CEDAR trial and our other planned clinical trials, as well as risks associated with the maintenance of our strategic partnership with WuXi Advanced Therapies. These risks concerning Graphite Bio's programs and operations are described in additional detail in our periodic filings with the SEC, including our most recently filed periodic report, 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.
Condensed Statements of Operations and Comprehensive Loss
(unaudited)
(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	**2021**	**2022**	**2021**
Operating expenses*:				
Research and development	$ 18,302	$ 8,683	$ 54,325	$ 26,727
General and administrative	7,852	5,919	24,563	14,776
Total operating expenses	26,154	14,602	78,888	41,503
Loss from operations	(26,154)	(14,602)	(78,888)	(41,503)
Other income (expense), net:				
Interest income, net	1,472	10	2,435	14
Change in fair value of the Series A redeemable convertible preferred stock tranche liability	—	—	—	(10,341)
Total other income (expense), net	1,472	10	2,435	(10,327)
Net loss	$ (24,682)	$ (14,592)	$ (76,453)	$ (51,830)
Unrealized loss on investments	(563)	—	(1,596)	—
Comprehensive loss	$ (25,245)	$ (14,592)	$ (78,049)	$ (51,830)
Net loss per share attributable to common stockholders—basic and diluted	$ (0.45)	$ (0.28)	$ (1.40)	$ (2.51)
Weighted-average shares used in computing net loss per share—basic and diluted	55,206,139	52,769,916	54,591,593	20,668,560
*** Includes stock-based compensation as follows:**				
Research and development	$ 1,249	$ 824	$ 3,881	$ 1,635
General and administrative	1,961	1,550	6,031	3,790
	$ 3,210	$ 2,374	$ 9,912	$ 5,425

GRAPHITE BIO, INC.
Condensed Balance Sheets
(in thousands)

	September 30, 2022	December 31, 2021
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 56,391	$ 376,976
Investments in marketable securities, current	235,294	—
Prepaid expenses and other current assets	6,807	4,760
Total current assets	298,492	381,736
Restricted cash, non-current	1,716	1,716
Investments in marketable securities, non-current	13,403	—
Property and equipment, net	13,270	6,507
Operating lease right-of-use assets	7,120	11,574
Other assets	844	454
Total assets	$ 334,845	$ 401,987
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,972	$ 2,453
Accrued compensation	3,346	2,689
Accrued research costs	353	633
Accrued expenses and other current liabilities	1,297	886
Operating lease liabilities, current	4,556	5,482
Total current liabilities	13,524	12,143
Operating lease liabilities, non-current	2,451	5,794
Other liabilities	2,417	
Total liabilities	18,392	17,937
Stockholders' equity:		
Common stock	1	1
Additional paid-in-capital	535,852	525,400
Accumulated other comprehensive loss	(1,596)	—
Accumulated deficit	(217,804)	(141,351)
Total stockholders' equity	316,453	384,050
Total liabilities and stockholders' equity	$ 334,845	$ 401,987

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Investors:
Stephanie Yao
Graphite Bio
ir@graphitebio.com

Media:
Sheryl Seapy
Real Chemistry
media@graphitebio.com

Source: Graphite Bio, Inc.