

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

Dosed first patient with nulabeglogene autogedtemcel (nula-cel), formerly known as GPH101, in Phase 1/2 CEDAR clinical trial in people with sickle cell disease; initial proof-of-concept data now anticipated in mid-2023

Presented preclinical gene replacement data for GPH102 in beta-thalassemia at ASGCT 25th Annual Meeting

\$328.3 million in cash, cash equivalents and investments in marketable securities as of June 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 cure serious diseases, today reported recent business progress and second quarter 2022 financial results.

“We have made significant progress in advancing CRISPR-based gene editing beyond cutting and disruption toward precision repair as we work to unlock the full promise of gene editing. Earlier today, we announced that we dosed the first sickle cell disease patient in our Phase 1/2 CEDAR trial of nula-cel, the first investigational therapy designed to correct a mutated gene to normal. We look forward to reporting initial proof-of-concept data for nula-cel in mid-2023,” said Josh Lehrer, M.D., M.Phil., chief executive officer of Graphite Bio. “In addition, we continue to advance our pipeline and platform capabilities and were pleased to present positive preclinical data from our beta-thalassemia program at the ASGCT 25th Annual Meeting, which support further advancement of GPH102 and highlight the curative potential of our gene replacement approach.”

Program Updates

Nulabeglogene autogedtemcel (nula-cel), formerly known as GPH101, for Sickle Cell Disease

- [Announced](#) the first patient has been dosed in the Phase 1/2 CEDAR clinical trial evaluating nula-cel in patients with severe sickle cell disease (SCD). Nula-cel is an investigational gene editing therapy designed to directly correct the genetic mutation that causes SCD. The company now anticipates sharing initial proof-of-concept data in mid-2023.
- [Received](#) U.S. Food and Drug Administration (FDA) Fast Track designation, which facilitates the expedited development and review of new drugs or biologics that are intended to treat serious or life-threatening conditions and demonstrate the potential to

address unmet medical needs. Nula-cel also has FDA Orphan Drug designation.

GPH102 for Beta-Thalassemia

- [Presented](#) data highlighting the discovery and preclinical development of GPH102, the company's gene replacement program for beta-thalassemia, at the American Society of Gene and Cell Therapy (ASGCT) 25th Annual Meeting. GPH102 is designed to directly replace the mutated beta-globin gene with a functional gene and restore adult hemoglobin expression to levels seen in individuals who do not have the disease. The company expects to submit an investigational new drug application (IND) for this program by mid-2024, pending feedback from regulatory authorities.

Second Quarter Financial Highlights

- **Cash Position:** As of June 30, 2022, cash, cash equivalents and investments in marketable securities totaled \$328.3 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 \$17.8 million for the second quarter of 2022, which includes \$1.3 million in stock-based compensation expense.
- **G&A Expenses:** General and administrative expenses were \$9.0 million for the second quarter of 2022, which includes \$2.1 million in stock-based compensation expense.
- **Net Loss:** Net loss was \$25.9 million, or \$0.48 per basic and diluted share, for the quarter ended June 30, 2022.

About nula-cel

Nula-cel, formerly known as 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 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, expectations with respect to the clinical development of nula-cel, including the availability of initial proof-of-concept data and potential benefits conferred by Fast Track designation, 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, the timing of these events, 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 regulatory challenges or delays in patient enrollment and in the initiation, conduct and completion of our ongoing and planned clinical trials, and that our

operating expenses may exceed our current estimates. These risks concerning Graphite Bio's programs and operations are described in additional detail in its periodic filings with the SEC, including its 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 June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Operating expenses*:				
Research and development	\$ 17,777	\$ 12,667	\$ 36,023	\$ 18,044
General and administrative	8,999	4,866	16,711	8,857
Total operating expenses	<u>26,776</u>	<u>17,533</u>	<u>52,734</u>	<u>26,901</u>
Loss from operations	(26,776)	(17,533)	(52,734)	(26,901)
Other income (expense), net:				
Other income, net	840	4	963	4
Change in fair value of the Series A redeemable convertible preferred stock tranche liability	—	—	—	(10,341)
Total other income (expense), net	<u>840</u>	<u>4</u>	<u>963</u>	<u>(10,337)</u>
Net loss	<u>\$ (25,936)</u>	<u>\$ (17,529)</u>	<u>\$ (51,771)</u>	<u>\$ (37,238)</u>
Unrealized loss on investments	<u>(724)</u>	<u>—</u>	<u>(1,033)</u>	<u>—</u>
Comprehensive loss	<u>\$ (26,660)</u>	<u>\$ (17,529)</u>	<u>\$ (52,804)</u>	<u>\$ (37,238)</u>
Net loss per share attributable to common stockholders—basic and diluted	<u>\$ (0.48)</u>	<u>\$ (3.45)</u>	<u>\$ (0.95)</u>	<u>\$ (8.45)</u>
Weighted-average shares used in computing net loss per share—basic and diluted	<u>54,572,866</u>	<u>5,087,008</u>	<u>54,284,836</u>	<u>4,405,357</u>
* Includes stock-based compensation as follows:				
Research and development	\$ 1,259	\$ 614	\$ 2,632	\$ 811
General and administrative	2,101	1,404	4,070	2,240
	<u>\$ 3,360</u>	<u>\$ 2,018</u>	<u>\$ 6,702</u>	<u>\$ 3,051</u>

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

	June 30, 2022	December 31,
	(unaudited)	2021
Assets		
Current assets:		
Cash and cash equivalents	\$ 75,171	\$ 376,976
Investments in marketable securities, current	247,121	—
Prepaid expenses and other current assets	4,352	4,760
Total current assets	326,644	381,736
Restricted cash, non-current	1,716	1,716
Investments in marketable securities, non-current	5,988	—
Property and equipment, net	10,467	6,507
Operating lease right-of-use assets	8,636	11,574
Other assets	335	454
Total assets	\$ 353,786	\$ 401,987
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 3,619	\$ 2,453
Accrued compensation	2,181	2,689
Accrued research costs	460	633
Accrued expenses and other current liabilities	882	886
Operating lease liabilities, current	4,926	5,482
Total current liabilities	12,068	12,143
Operating lease liabilities, non-current	3,262	5,794
Total liabilities	15,330	17,937
Stockholders' equity:		
Common stock	1	1
Additional paid-in-capital	532,610	525,400
Accumulated other comprehensive loss	(1,033)	—
Accumulated deficit	(193,122)	(141,351)
Total stockholders' equity	338,456	384,050
Total liabilities and stockholders' equity	\$ 353,786	\$ 401,987

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