May 12, 2022

# Graphite Bio Reports Recent Business Progress and First Quarter 2022 Financial Results

*GPH101 for sickle cell disease granted U.S. FDA Fast Track Designation; dosing of first patient in Phase 1/2 CEDAR clinical trial on track for second half of 2022, with initial proof-of-concept data anticipated in 2023*

*Oral presentation highlighting preclinical data for GPH102 for beta-thalassemia at upcoming ASGCT 25th Annual Meeting*

*$352.1 million in cash, cash equivalents and investments in marketable securities as of March 31, 2022; cash runway into fourth quarter of 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 first quarter 2022 financial results.

“In the first quarter of 2022, we announced our updated research and development priorities to focus on programs that maximize the capabilities of our next-generation gene editing platform and can significantly impact patient outcomes. We continue to advance our research and development efforts across our pipeline, particularly the execution of our Phase 1/2 CEDAR clinical trial of GPH101 for sickle cell disease. We remain on track to dose our first patient in the second half of this year, and we look forward to generating data that demonstrates GPH101’s curative potential using our unique gene correction approach,” said Josh Lehrer, M.D., M.Phil., chief executive officer of Graphite Bio. “In addition, we are excited to share at the ASGCT 25th Annual Meeting next week more information about our GPH102 program for beta-thalassemia, which demonstrates our platform’s gene replacement capabilities. Similar to our gene correction approach in sickle cell disease, we believe our gene replacement approach in beta-thalassemia could be the optimal way to treat the disease and provide a definitive cure to patients.”

**Program Updates**

*GPH101 for Sickle Cell Disease*

- Granted 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. In November 2021, GPH101, an investigational therapy designed to directly correct the genetic mutation responsible for sickle cell disease (SCD), was granted orphan drug designation by the FDA.

- Continued patient enrollment in the Phase 1/2 CEDAR clinical trial of GPH101 at multiple sites across the United States. The company remains on track to dose its first patient in the second half of 2022, with initial proof-of-concept data anticipated in 2023.

*GPH102 for Beta-Thalassemia*

- Received acceptance of an oral presentation highlighting the discovery and preclinical development of GPH102 at the American Society of Gene and Cell Therapy (ASGCT) 25th Annual Meeting. The company will also present an encore trial-in-progress poster about the Phase 1/2 CEDAR trial of GPH101 for SCD. The hybrid meeting will take place virtually and at the Walter E. Washington Convention Center in Washington, D.C., from May 16-19.
- Continued to advance the preclinical development of GPH102. Using the company's gene replacement approach, GPH102 is designed to directly replace and normalize the entire mutated beta-globin gene with a functional gene and restore adult hemoglobin expression to levels similar to individuals who do not have the disease. The company expects to submit an IND for this program by mid-2024, pending feedback from regulatory authorities

**First Quarter Financial Highlights**

- **Cash Position:** As of March 31, 2022, cash, cash equivalents and investments in marketable securities totaled $352.1 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 $18.2 million for the first quarter of 2022, which includes $1.4 million in stock-based compensation expense.
- **G&A Expenses:** General and administrative expenses were $7.7 million for the first quarter of 2022, which includes $2.0 million in stock-based compensation expense.
- **Net Loss:** Net loss was $25.8 million, or $0.48 per basic and diluted share, for the quarter ended March 31, 2022.

**About GPH101 for Sickle Cell Disease**

GPH101 is an investigational next-generation gene-edited autologous hematopoietic stem cell (HSC) therapy designed to directly correct the genetic mutation that causes sickle cell disease (SCD). SCD is a serious, life-threatening inherited blood disorder that 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. GPH101 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.

Graphite Bio is evaluating GPH101 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 for Beta-Thalassemia**

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 harnessing the power of high-efficiency precision gene repair 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 UltraHDR™ gene editing platform is designed 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.

**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, 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 views about our plans, intentions, expectations, strategies, prospects, 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 hurdles or delays in patient enrollment and dosing, and in the initiation, progress, conduct and completion of our 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 March 31, | |
|---|---|---|
| | **2022** | **2021** |
| **Operating expenses*:** | | |
| Research and development | $ 18,246 | $ 5,377 |
| General and administrative | 7,712 | 3,991 |
| Total operating expenses | 25,958 | 9,368 |
| Loss from operations | (25,958) | (9,368) |
| Other income (expense), net: | | |
| Other income, net | 123 | — |
| Change in fair value of the Series A redeemable convertible preferred stock tranche liability | — | (10,341) |
| Total other income (expense), net | 123 | (10,341) |
| Net loss attributable to common stockholders | $ (25,835) | $ (19,709) |
| Unrealized loss on investments | (309) | — |
| Net loss and comprehensive loss | (26,144) | (19,709) |
| Net loss per common share – basic and diluted | $ (0.48) | $ (5.75) |
| Weighted-average number of common shares outstanding – basic and diluted | 54,005,299 | 3,425,089 |
| *** Includes stock-based compensation as follows:** | | |
| Research and development | $ 1,373 | $ 196 |
| General and administrative | 1,969 | 837 |
| **Total stock-based compensation expense** | $ 3,342 | $ 1,033 |

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

| | March 31, 2022 | December 31, 2021 |
|---|---|---|
| **Assets** | (unaudited) | |
| Current assets: | | |
| Cash and cash equivalents | $ 185,070 | $ 376,976 |
| Investments in marketable securities, current | 154,112 | — |
| Prepaid expenses and other current assets | 4,574 | 4,760 |
| Total current assets | 343,756 | 381,736 |
| Restricted cash, non-current | 1,716 | 1,716 |
| Investments in marketable securities, non-current | 12,927 | — |
| Property and equipment, net | 9,374 | 6,507 |
| Operating lease right-of-use assets | 10,122 | 11,574 |
| Other assets | 711 | 454 |
| Total assets | $ 378,606 | $ 401,987 |
| **Liabilities and stockholders' equity** | | |
| Current liabilities: | | |
| Accounts payable | $ 3,069 | $ 2,453 |
| Accrued compensation | 1,023 | 2,689 |
| Accrued research costs | 1,879 | 633 |
| Accrued expenses and other current liabilities | 1,158 | 886 |
| Operating lease liabilities, current | 5,636 | 5,482 |
| Total current liabilities | 12,765 | 12,143 |
| Operating lease liabilities, non-current | 4,542 | 5,794 |
| Total liabilities | 17,307 | 17,937 |
| Stockholders' equity: | | |
| Common stock | 1 | 1 |
| Additional paid-in capital | 528,793 | 525,400 |
| Accumulated other comprehensive loss | (309) | — |
| Accumulated deficit | (167,186) | (141,351) |
| Total stockholders' equity | 361,299 | 384,050 |
| Total liabilities and stockholders' equity | $ 378,606 | $ 401,987 |

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