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Graphite Bio Presents Preclinical Data Supporting GPH101 in Sickle Cell Disease at 49th Annual Sickle Cell Disease Association of America National Convention

Data support ability of company's gene editing platform to precisely and efficiently correct the underlying disease-causing mutation and restore adult hemoglobin expression with curative potential

Data show minimal off-target editing using company's exclusively licensed high fidelity Cas9 and robust long-term engraftment

Company on track to enroll first patient in Phase 1/2 CEDAR trial in 2H 2021

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)-- Graphite Bio, Inc. (Nasdaq: GRPH), a clinical-stage, next-generation gene editing company focused on therapies that harness targeted gene integration to treat or cure serious diseases, announced today the presentation of preclinical data for GPH101, an investigational therapy designed to directly correct the genetic mutation responsible for sickle cell disease (SCD). Data were presented at the 49th Annual Sickle Cell Disease Association of America (SCDAA) National Convention in a poster presentation.

"These positive preclinical data are foundational to our sickle cell disease program and support the evaluation of GPH101 in our Phase 1/2 CEDAR trial, for which we are on track to enroll our first patient before the end of the year," said Josh Lehrer, M.Phil., M.D., chief executive officer at Graphite Bio. "These encouraging data reinforce our belief that GPH101 has the potential to directly correct the underlying disease-causing mutation to decrease production of sickle hemoglobin and restore the expression of normal adult hemoglobin with minimal off-target editing. Gene correction has been viewed as the optimal approach to potentially cure sickle cell disease, and the preclinical data we have generated indicate we can do this precisely and efficiently and at rates that are considered potentially curative based on extensive data from patients who have undergone allogeneic stem cell transplant."

Graphite Bio presented data establishing the reproducibility of the company's gene editing platform to generate gene-corrected hematopoietic stem cells (HSCs) for the treatment of SCD. Using Graphite Bio's gene correction platform, which uses an engineered high fidelity Cas9 to reduce off-target cleavage by 30-fold, the company was able to achieve greater than 60% of gene-corrected beta-globin alleles in vitro with minimal off-target activity. After transplant into mice, long-term engraftment (16 weeks) of gene-corrected cells in vivo was achieved, with gene correction frequencies much greater than the predicted curative threshold of 15% gene correction, which is equivalent to 20% cell correction. These

correction frequencies support the potential for this approach to be equivalent or superior to allogeneic hematopoietic stem cell transplant (allo-HSCT) in restoring the expression of normal adult hemoglobin and red blood cell biology.

Additionally, the data showed that gene-corrected red blood cells went from producing 100% sickle hemoglobin to expressing more than 90% normal adult hemoglobin. Long-term preclinical safety data revealed no evidence of abnormal hematopoiesis, genotoxicity or tumorigenicity, including no detectable chromosomal translocations. These preclinical data support Graphite Bio's CEDAR clinical trial, a Phase 1/2 study evaluating the safety, pharmacodynamics, engraftment success, gene correction rates and total hemoglobin, as well as other clinical and exploratory endpoints of GPH101 in patients with severe SCD.

The data presented today were previously published in the June 16 edition of *Science Translational Medicine*. A copy of the poster presentation is available under the Publications section of the Graphite Bio website.

About 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 disease worldwide. SCD is caused by a single mutation in the beta-globin gene that leads to red blood cells that are responsible for delivering oxygen to tissues and organs throughout the body to become misshapen, resulting in anemia, blood flow blockages, intense pain, increased risk of stroke and organ damage, and reduced life span of approximately 30 years. Despite advancements in treatment and care, progressive organ damage continues to cause early mortality and severe morbidity, highlighting the need for curative therapies.

About GPH101

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). GPH101 is the first investigational therapy to utilize 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 normal 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 the safety, engraftment success, gene correction rates, total hemoglobin, as well as other clinical and exploratory endpoints and pharmacodynamics in patients with severe SCD.

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, and the timing for enrollment of the first patient in our Phase 1/2 clinical trial of GPH101 and the availability of initial proof-of-concept data, 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 August 12, 2021. Graphite Bio explicitly disclaims any obligation to update any forward-looking statements except to the extent required by law.

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