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# Graphite Bio Announces U.S. FDA Fast Track Designation Granted to GPH101 for the Treatment of Sickle Cell Disease

*GPH101 is an investigational next-generation gene-edited therapy designed to potentially provide a one-time cure for patients*

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 announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to GPH101 for the treatment of sickle cell disease (SCD). GPH101 is an investigational next-generation gene-edited autologous hematopoietic stem cell (HSC) therapy designed to directly correct the genetic mutation that causes SCD.

“The FDA’s decision to grant Fast Track Designation to GPH101 for sickle cell disease signifies the need for novel medicines for this serious genetic disease and supports the ongoing development of our unique gene correction approach that we believe could offer a definitive cure for sickle cell patients,” said Josh Lehrer, M.D., M.Phil., chief executive officer of Graphite Bio. “This designation has the potential to accelerate the development of GPH101, which we are advancing with the goal of precisely and efficiently correcting the genetic mutation that is the underlying cause of sickle cell disease. We continue to enroll patients in our Phase 1/2 CEDAR trial and expect to dose our first patient later this year, with initial proof-of-concept data anticipated next year.”

The FDA’s Fast Track program 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. GPH101 was previously granted orphan drug designation by the FDA.

**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 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 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 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 UltraHDR™ gene editing platform and our product candidates, the timing for dosing the first patient in our Phase 1/2 CEDAR clinical trial of GPH101 and the availability of initial proof-of-concept data from the trial, and our ability to accelerate the development of GPH101 as a result of the receipt of Fast Track Designation, 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 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. 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.

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