

## ***Graphite Bio Launches with \$45 Million Series A Financing***

*-- Next-generation gene editing company launched by Versant Ventures with additional backing by Samsara BioCapital --*

*-- Core technology from Stanford founders Matt Porteus and Maria Grazia Roncarolo --  
-- Josh Lehrer joins as CEO --*

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SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)--Graphite Bio today announced a \$45 million Series A financing led by founding investor Versant Ventures, together with Samsara BioCapital. Graphite Bio is a next-generation gene editing company focused on targeted DNA integration to precisely insert genetic payloads to treat a variety of severe diseases. The company was co-founded by pioneers in the fields of gene editing and gene therapy and has a rapidly maturing pipeline with an upcoming IND and several other candidates advancing toward the clinic in the next 12-18 months.

While many existing gene editing and gene therapy approaches are based on knocking out or randomly integrating genes, Graphite Bio is focused on correcting defective genes by high-efficiency site-specific integration of new genetic sequences. This technology has the potential to precisely repair a damaged portion of a gene, completely replace a malfunctioning gene while retaining normal regulatory control, or to insert a wide range of therapeutic genetic cargoes into precise regions of the genome.

In addition, Graphite Bio's technology has the potential to provide for durable expression while minimizing toxicity from off-target insertions.

"Our flexible, site-specific approach is extremely powerful and could be used to definitively correct the underlying causes of many severe genetic diseases, and also is applicable to broader disease areas," said Josh Lehrer, M.D., CEO of Graphite Bio. "With backing from Versant and Samsara, we look forward to progressing our novel medicines into the clinic for patients with high unmet needs."

Dr. Lehrer joined Graphite Bio after six years at Global Blood Therapeutics (NASDAQ:GBT), where he most recently served as chief medical officer. At GBT, he played a key role in the clinical development and 2019 approval of the company's Oxbryta therapy for sickle cell disease.

### **A next-generation gene editing platform**

CRISPR-Cas9 gene editing has transformed the biotechnology landscape due to its ability to specifically induce double-stranded DNA breaks that can disrupt genes or genetic control regions, leading to novel treatment approaches for a range of diseases. However, it has been challenging to harness CRISPR-Cas9 for the high-efficiency insertion of new genetic cargo.

Graphite Bio's gene editing platform includes several complementary technologies that enable targeted and permanent DNA integration at very high efficiency. The platform builds on seminal work led by Dr. Danny Dever in the laboratory of Dr. Matt Porteus at Stanford University demonstrating an increase in integration efficiency from less than 1% to greater than 50% across diverse genetic lesions in a wide range of cell types.

“Achieving high-efficiency targeted gene integration has been a critical objective of gene editing for more than 15 years, but only now is this technologically possible,” said Jerel Davis, Ph.D., Managing Director at Versant and a Graphite Bio board member. “As the founding investor of CRISPR Therapeutics, Versant has seen first-hand the rapid evolution of the gene editing field. Our collaboration with Matt Porteus on CRISPR Therapeutics was highly productive. We are thrilled to be working with him on Graphite Bio, alongside Maria Grazia, Josh and Danny.”

The efficiency improvements enable clinically meaningful levels of targeted integration for therapeutic applications. The company’s medicines therefore can replace defective genes, insert genetic cargo into specific loci and significantly limit undesirable, indiscriminate transgene expression.

### **Graphite Bio founders and operating plans**

The company’s scientific founders include:

- Matthew Porteus, M.D., Ph.D., Professor of Pediatrics and Stem Cell Transplantation and the Associate Director of Center for Definitive and Curative Medicine at Stanford University. Dr. Porteus is a pioneer in the gene editing field and has been involved in the space for more than 20 years. He is an academic founder of CRISPR Therapeutics, and advisor to a number of companies in the field as well as the FDA. He has advanced several of Graphite Bio’s technologies and programs at his Stanford lab. As a pediatric hematologist, Dr. Porteus treats patients with severe genetic diseases.
- Maria Grazia Roncarolo, M.D., Ph.D., Professor in Stem Cell and Regenerative Medicine, Professor of Pediatrics and of Medicine, Director of the Center for Definitive and Curative Medicine, and Co-Director of the Institute for Stem Cell Biology and Regenerative Medicine at Stanford University. She is a global leader and pioneer in gene therapy, having served as the director of the Telethon Institute for Cell and Gene Therapy at the San Raffaele Scientific Institute in Milan. There, she advanced multiple gene therapy products into the clinic. This work ultimately led to the first approved gene therapy, Strimvelis.

Based on progress to date, Graphite Bio expects to commence Phase 1 studies of its first development candidate in early 2021. The company’s lead program specifically corrects the single nucleotide point mutation in the sickle  $\beta$  globin gene. In cells from patients with the disease, Graphite Bio has shown that its gene correction approach efficiently restores healthy hemoglobin protein and eliminates sickle cell hemoglobin.

In addition, Graphite Bio is advancing several other programs to the clinic, each with the potential to lead to a cure for serious unmet needs.

“It is gratifying to see our work on new gene editing approaches being translated into novel therapies,” said Dr. Porteus. “I’m very excited to be working with Versant again on a start-up and I look forward to collaborating with Samsara and the Graphite Bio team to bring a new generation of genetic treatments to patients.”

In connection with the financing, Abe Bassan, Vice President at Samsara BioCapital, and Carlo Rizzuto, Ph.D., Partner at Versant, joined Graphite Bio’s board.

### **About Graphite Bio**

Graphite Bio is a next-generation gene editing company focused on targeted DNA integration to precisely insert genetic payloads to treat a broad range of severe diseases. The company was co-founded by academic pioneers in the fields of gene editing and gene therapy, and is backed by Versant Ventures and Samsara BioCapital. For more information, please visit [www.graphitebio.com](http://www.graphitebio.com).

### **About Versant Ventures**

Versant Ventures is a leading healthcare venture capital firm committed to helping exceptional entrepreneurs build the next generation of great companies. The firm's emphasis is on biotechnology companies that are discovering and developing novel therapeutics. With \$3.2 billion under management and offices in the U.S., Canada and Europe, Versant has built a team with deep investment, operating and R&D expertise that enables a hands-on approach to company building. Since the firm's founding in 1999, more than 75 Versant companies have achieved successful acquisitions or IPOs. Versant is currently investing out of its seventh fund, Versant Venture Capital VII, a \$600 million global biotech fund closed in December 2018. In parallel the firm co-invests out of its Canadian strategic fund Versant Voyageurs I and its later-stage biotech opportunity fund Versant Vantage I. For more information, please visit [www.versantventures.com](http://www.versantventures.com).

### **About Samsara BioCapital**

Samsara BioCapital is a new breed of biotech investment fund focused on translating cutting-edge biology into new therapies to treat patients with unmet medical needs. Founded in 2016 by Srinivas Akkaraju, MD, PhD, our team of scientists, investors, and entrepreneurs takes a long-term view to value creation across all stages of public and private life science companies. We believe in a collaborative, hands-on approach, working closely with entrepreneurs to harness exciting scientific advances and build leading companies. Samsara actively manages approximately US\$410 million in assets on behalf of endowments, foundations, and family offices. For more information, visit [www.samsaracap.com](http://www.samsaracap.com).

## **Contacts**

Katherine Vega Stultz, COO  
[comms@graphitebio.com](mailto:comms@graphitebio.com)