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Graphite Bio Announces Formation of Scientific Advisory Board

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)-- Graphite Bio, Inc. (Nasdaq: GRPH), a clinical-stage, next-generation gene editing company focused on developing therapies that harness targeted gene integration to treat or cure serious diseases, today announced the formation of its Scientific Advisory Board (SAB), which is initially comprised of four experts in stem cell and cancer biology, cell and gene therapy, hematology and immunology. The SAB will provide strategic and scientific counsel as the company advances its next-generation gene editing platform technology and research and development programs.

“We are honored and excited to have this group of esteemed scientists and clinicians join us as we work to leverage our platform technology, which harnesses the power of high-efficiency homology directed repair, to develop potential one-time curative therapies for genetic and serious diseases,” said Jane Grogan, Ph.D., chief scientific officer of Graphite Bio. “These scientific thought leaders have decades of experience in their respective fields, and their input and feedback will be invaluable as we work to advance our goal of transforming critical components of the gene therapy treatment paradigm – from discovery to development and manufacturing to delivery – so that as many patients as possible can benefit from these individualized therapies.”

The founding members of Graphite Bio’s SAB include:

- **Maria Grazia Roncarolo, M.D., SAB Chair** – Dr. Roncarolo is one of the world’s foremost experts in gene therapy and one of Graphite Bio’s academic founders. She is recognized globally for her leadership in translating scientific discoveries in genetic diseases and regenerative medicine into novel patient therapies, including the world’s first ex vivo gene therapy. The George D. Smith Professor in Stem Cell and Regenerative Medicine, Professor of Pediatrics and of Medicine at Stanford University, Dr. Roncarolo established the Stanford Center for Definitive and Curative Medicine to cure patients with currently incurable diseases through the development of innovative stem cell and gene-based therapies. During her earlier tenure as director of the Telethon Institute for Gene Therapy at the San Raffaele Scientific Institute in Milan, Dr. Roncarolo developed novel approaches in gene therapy. Her work at the Institute led to the discovery of ex vivo gene therapies for genetic diseases of the immune system, including ADA-SCID and WASP, and metabolic diseases such as metachromatic leukodystrophy. The landmark stem cell gene therapy treatment for ADA-SCID was the world’s first to be approved by the European Medicines Agency (EMA) under the brand name Strimvelis® in May 2016.

- **John E. Dick, Ph.D.** – Professor Dick is a Canadian researcher in stem cell biology and senior scientist at the Princess Margaret Cancer Centre and McEwen Stem Cell Institute, University Health Network; and professor of molecular genetics at the University of Toronto. He is recognized for identifying and characterizing normal and leukemia human hematopoietic stem cells. His lab provided direct evidence for the

cancer stem cell hypothesis, transforming views of the origin and nature of cancer and laying the foundation for new approaches to cancer therapy. In recognition of his seminal contributions to the fields of molecular hematology, stem cell biology and oncology, he was elected as a foreign member of the National Academy of Medicine (USA), a fellow of the Royal Society of Canada, the Royal Society of London, UK, and the AACR Academy. He has received numerous prestigious awards, including the Dameshek Prize, Thomas Prize and Mentor Award from the American Society of Hematology; the Clowes Memorial Award and the AACR-Pezcoller Prize from the American Association for Cancer Research; and the Keio Medical Science Prize.

- **Natalia Gomez-Ospina, M.D., Ph.D.** – Dr. Gomez-Ospina has dual appointments in the Divisions of Medical Genetics and Stem Cell Transplantation and is a faculty member in the Institute for Stem Cell Biology and Regenerative Medicine at Stanford University School of Medicine. She also leads the Program for Inherited Metabolic Disorders at Stanford, which brings together basic scientists and clinicians to facilitate the development of therapies for metabolic diseases. A physician-scientist and medical geneticist, Dr. Gomez-Ospina is conducting research to develop safer, more effective therapies for lysosomal storage disorders. Her clinical and research interests bridge genetics and transplantation. She established an adaptable platform for treating lysosomal enzyme deficiencies and performed first-of-its-kind preclinical studies to support the clinical development of autologous transplantation of genome-edited cells to treat patients with mucopolysaccharidosis type I (Hurler syndrome) and Gaucher disease. Dr. Gomez-Ospina led the discovery and characterization of genetic syndromes, including the infantile cholestasis syndrome caused by mutations in the bile acid receptor. She is the lead author of research published in The New England Journal of Medicine*,* Cell, Nature Communications and the American Journal of Medical Genetics. She earned an M.D. and a Ph.D. in chemical and systems biology from Stanford University School of Medicine.

- **Scot A. Wolfe, Ph.D.** – Dr. Wolfe is a professor in the Department of Molecular, Cell and Cancer Biology (MCCB) at the University of Massachusetts Chan Medical School. His research is focused on genome editing systems and protein-DNA interactions. His group is developing improved tools for targeted genome modification and gene regulation, with the goal of increasing their precision and effectiveness for therapeutic translation. This research has spanned developing a variety of improved CRISPR-based genome editing systems and new methods for engineering the DNA-binding specificity of zinc finger proteins. His team is focused on the therapeutic translation of their improved genome editing and gene regulation systems to specific disease applications, such as sickle cell disease, beta-thalassemia, various forms of muscular dystrophy and Hermansky-Pudlak syndrome. He has published more than 65 research articles in peer-reviewed journals. He earned a Ph.D. at Harvard University and completed his postdoctoral research at the Massachusetts Institute of Technology.

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.

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