



Jasper Therapeutics and Graphite Bio Announce Collaboration to Evaluate JSP191 as Conditioning Regimen for Novel Gene Replacement Therapy in Patients with XSCID

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REDWOOD CITY, Calif. & SOUTH SAN FRANCISCO, Calif.--([BUSINESS WIRE](#))--Jasper Therapeutics, Inc., a biotechnology company focused on hematopoietic cell transplant therapies, and Graphite Bio, Inc., a next-generation gene editing company focused on therapies that harness targeted gene integration to treat or cure serious diseases, today announced a research and clinical collaboration agreement to evaluate JSP191, Jasper's first-in-class anti-CD117 monoclonal antibody, as a targeted, non-toxic conditioning regimen for Graphite Bio's investigational GPH201 gene replacement therapy for severe combined immune deficiency (SCID) in patients with IL2RG deficiency, known as x-linked SCID (XSCID).

XSCID is a severe, inherited disorder of the immune system with symptoms often presenting in early infancy, including persistent infections and failure to thrive. Without treatment, XSCID is typically fatal to patients in the first two years of life.

Graphite Bio is focused on the development of potentially curative therapies for patients suffering from serious diseases, using its targeted gene integration platform to harness the natural cellular process of homology directed repair (HDR) in order to efficiently repair genetic defects at their source, deliver genetic cargo with precision and engineer new cellular effector functions. Jasper Therapeutics' JSP191 is a first-in-class humanized monoclonal antibody that depletes hematopoietic stem cells from bone marrow and acts as a conditioning agent in patients prior to receiving a hematopoietic stem cell transplant. JSP191 is currently being evaluated in multiple trials as a stem cell depleting conditioning agent, including a Phase 1/2 trial to achieve donor stem cell engraftment in SCID patients undergoing hematopoietic cell transplant and a separate Phase 1/2 trial in AML/MDS patients undergoing hematopoietic cell transplant.

"This collaboration with Jasper demonstrates our shared commitment to pioneering novel therapeutic approaches with the potential to significantly improve the treatment experiences of individuals with devastating conditions who stand to benefit from gene replacement therapies, initially for patients with XSCID," said Josh Lehrer, M.Phil., M.D., chief executive officer at Graphite Bio. "GPH201 harnesses our targeted gene integration platform to precisely target the defective gene that causes XSCID and replace it with a normal copy. We are impressed by the initial positive clinical results demonstrated by JSP191 when used as a conditioning regimen, and look forward to collaborating with the Jasper team to explore how our novel technologies can be brought to more patients with XSCID and other indications."

"Our collaboration with Graphite Bio is an exciting opportunity to further advance the field of curative gene correction by combining a targeted gene integration platform with our first-in-class targeted CD117 antibody, JSP191, that has already demonstrated preliminary clinical efficacy and safety as a conditioning agent in XSCID patients and those with blood cancers undergoing allogeneic hematopoietic stem cell transplant," said Bill Lis, executive chairman and CEO, Jasper Therapeutics.

Graphite Bio and Jasper will collaborate on research, and potentially a clinical study, evaluating JSP191 as a conditioning agent for GPH201. Each company will retain commercial rights to their respective technologies.

About JSP191

JSP191 (formerly AMG 191) is a first-in-class humanized monoclonal antibody in clinical development as a conditioning agent that clears hematopoietic stem cells from bone marrow. JSP191 binds to human CD117, a receptor for stem cell factor (SCF) that is expressed on the surface of hematopoietic stem and progenitor cells. The interaction of SCF and CD117 is required for stem cells to survive. JSP191 blocks SCF from binding to CD117 and disrupts critical survival signals, causing the stem cells to undergo cell death and creating an empty space in the bone marrow for donor or gene-corrected transplanted stem cells to engraft.

Preclinical studies have shown that JSP191 as a single agent safely depletes normal and diseased hematopoietic stem cells, including in animal models of SCID, myelodysplastic syndromes (MDS) and sickle cell disease (SCD). Treatment with JSP191 creates the space needed for transplanted normal donor or gene-corrected hematopoietic stem cells to successfully engraft in the host bone marrow. To date, JSP191 has been evaluated in more than 90 healthy volunteers and patients.

JSP191 is currently being evaluated in two separate clinical studies in hematopoietic cell transplant. The first clinical study is evaluating JSP191 as a sole conditioning agent in a Phase 1/2 dose-escalation and expansion trial to achieve donor stem cell engraftment in patients undergoing hematopoietic cell transplant for severe combined immunodeficiency (SCID), which is potentially curable only by this type of treatment. JSP191 is also being evaluated in combination with another conditioning regimen in a Phase 1 study in patients with MDS or acute myeloid leukemia (AML) who are receiving hematopoietic cell transplant. For more information about the design of these clinical trials, visit www.clinicaltrials.gov (NCT02963064 and NCT04429191).

Additional studies are planned to advance JSP191 as a conditioning agent for patients with other rare and ultra-rare monogenic disorders and autoimmune diseases.

About GPH201

GPH201 is a first-in-human investigational hematopoietic stem cell treatment that will be evaluated as a potentially curative therapy for patients suffering from XSCID. GPH201 is generated using Graphite Bio's precise and efficient targeted gene integration platform technology to directly replace the defective IL2RG gene, maintain normal IL2RG regulation and expression, and ultimately lead to the production of fully functional adaptive immune

cells.

About Jasper Therapeutics

Jasper Therapeutics is a biotechnology company focused on the development of novel curative therapies based on the biology of the hematopoietic stem cell. The company's lead compound, JSP191, is in clinical development as a conditioning antibody that clears hematopoietic stem cells from bone marrow in patients undergoing a hematopoietic cell transplant. This first-in-class conditioning antibody is designed to enable safer and more effective curative hematopoietic cell transplants and gene therapies. For more information, please visit us at jaspertherapeutics.com.

About Graphite Bio, Inc.

Graphite Bio is a next-generation gene editing company focused on the development of potentially curative therapies for patients suffering from serious diseases. The company's targeted gene integration platform harnesses the natural cellular process of homology directed repair (HDR) to efficiently repair genetic defects at their source, deliver genetic cargo with precision and engineer new cellular effector functions. Graphite Bio is leveraging its differentiated platform, initially focused on ex vivo engineering of hematopoietic stem cells, to advance a portfolio of transformative treatments with potential for saving and dramatically improving patients' lives. The company was co-founded by academic pioneers in the fields of gene editing and gene therapy, including Maria Grazia Roncarolo, MD, and Matthew Porteus, MD, PhD, and is backed by Versant Ventures and Samsara BioCapital. For more information, please visit graphitebio.com.

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