



Jasper Therapeutics Announces Data from First Transplant-naïve Patient in Phase 1 Clinical Trial of JSP191 as Conditioning Agent in Patients with SCID Undergoing Hematopoietic Cell Transplantation

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JSP191, a targeted antibody, is the first biologic agent to be evaluated in multiple trials as a stem cell depleting conditioning agent prior to blood stem cell transplant in patients with SCID or hematologic disorders –

Data presented at 62nd American Society of Hematology (ASH) Annual Meeting & Exposition

REDWOOD CITY, Calif.--([BUSINESS WIRE](#))--Jasper Therapeutics, Inc., a biotechnology company focused on hematopoietic cell transplant therapies, today announced clinical data from its ongoing multicenter Phase 1 clinical trial of JSP191, a first-in-class anti-CD117 monoclonal antibody, in patients with severe combined immune deficiency (SCID). The trial is evaluating JSP191 as a conditioning agent to enable stem cell transplantation in patients with SCID who are either transplant-naïve or who received a prior stem cell transplant with a poor outcome.

Data from the first transplant-naïve SCID patient in the Phase 1 trial, a 6-month-old infant, showed that a single dose of JSP191 administered prior to stem cell transplant was effective in establishing sustained donor chimerism followed by development of B, T and NK immune cells. No treatment-related adverse events were reported. The data were presented by primary investigator Rajni Agrawal-Hashmi, M.D., of Stanford University, at the 62nd American Society of Hematology (ASH) Annual Meeting & Exposition.

"We have previously shown that JSP191 can be successfully used as a single conditioning agent in SCID patients who had failed a previous transplant," said Kevin N. Heller, M.D., Executive Vice President, Research and Development, of Jasper Therapeutics. "This new data presented at ASH 2020 showing success in an infant with SCID undergoing a first transplant provides proof of concept of the safety and efficacy of the use of JSP191 as an alternative to genotoxic chemotherapies currently used to deplete stem cells prior to transplant."

Hematopoietic cell transplantation offers the only curative therapy for SCID, a severe genetic immune disorder that leaves patients without a functioning immune system. With this approach, standard-of-care chemotherapeutic conditioning regimens are given prior to transplant to reduce the number of blood stem cells in the bone marrow to make space for donor blood stem cells to engraft and cure the patient. JSP191 is designed to replace the need for chemotherapeutic conditioning agents, which are DNA-damaging and highly toxic.

Dr. Heller added, "With our Phase 1 trials in SCID and hematologic disorders underway, we are planning to expand the development of JSP191 into additional indications, such as gene therapies, autoimmune diseases, Fanconi's anemia and other rare disorders that can be cured by stem cell transplant."

The open-label, multicenter Phase 1 study is evaluating the safety, tolerability and efficacy of JSP191 as a conditioning agent in patients with SCID undergoing first or repeat hematopoietic cell transplantation. Up to three different doses of JSP191 are being assessed for dose-limiting toxicities. The trial is currently open for enrollment at Stanford University, the University of California, San Francisco, Memorial Sloan Kettering Cancer Center, the University of California, Los Angeles, and Cincinnati Children's Hospital. Additional clinical trial sites in the United States will initiate enrollment in the coming weeks.

About SCID

Severe combined immune deficiency (SCID) is a group of rare disorders caused by mutations in genes involved in the development and function of infection-fighting immune cells. Infants with SCID appear healthy at birth but are highly susceptible to severe infections. The condition is fatal, usually within the first year or two of life, unless infants receive immune-restoring treatments, such as transplants of blood-forming stem cells, gene therapy or enzyme therapy.

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 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 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 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.



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