Jasper Therapeutics Announces Orphan Drug and Rare Pediatric Disease Designations for JSP191 for Conditioning Treatment Prior to Stem Cell Transplant

June 14, 2021

REDWOOD CITY, Calif.--(BUSINESS WIRE)--Jasper Therapeutics, Inc., a biotechnology company focused on hematopoietic cell transplant therapies, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to JSP191, a monoclonal antibody targeting the CD117 (stem cell factor) receptor, for conditioning treatment prior to hematopoietic stem cell transplantation. In addition, the FDA granted rare pediatric disease designation to JSP191 as a conditioning treatment for patients with severe combined immunodeficiency (SCID), a life-threatening genetic illness that is typically fatal within two years without hematopoietic cell transplantation.

“The FDA’s decision to grant orphan drug designation to JSP191, and rare pediatric disease designation in SCID, underscores the critical need for innovative conditioning agents for patients undergoing hematopoietic cell transplant, in an indication where currently there is no FDA approved or consensus guidelines recommended conditioning agent,” said William Lis, Executive Chairman and CEO of Jasper Therapeutics. “Hematopoietic cell transplant is a potentially life-saving cure for multiple diseases such as SCID, acute myeloid leukemia, myelodysplastic syndromes, sickle cell disease, auto immune disease and others. However, the genotoxic mechanism of action of current conditioning agents limits the use of curative transplants due to severe adverse safety events or lack of efficacy due to the use of a reduced dose or no conditioning prior to transplant. We believe JSP191 has the potential to safely and effectively expand lifesaving, curative stem cell transplant across multiple diseases. The orphan drug and rare pediatric designations from the FDA provide additional momentum and validation for JSP191, and we are committed to advancing its use across multiple indications to improve patient care.”

Jasper is currently conducting clinical studies of JSP191 as a conditioning agent prior to hematopoietic stem cell transplant in patients with SCID and separately in patients with acute myeloid leukemia (AML) or myelodysplastic syndromes (MDS). SCID is a rare, life-threatening, pediatric disorder affecting an estimated 1/58,000 births in the general population. The ongoing SCID clinical trial is evaluating JSP191 as a conditioning agent to enable stem cell transplantation in patients who are either transplant-naive or who received a prior stem cell transplant with a poor outcome. Jasper has presented data from this study at multiple scientific conferences, which demonstrated that JSP191 has been well tolerated with no treatment-related adverse events across multiple patients ranging from 3 months to 38 years old. Successful engraftment and immune reconstitution has also been observed. Given the preliminary safety, tolerability and evidence of efficacy, Jasper intends to continue enrolling evaluating previously transplanted and newly diagnosed SCID patients to support additional filings with FDA.

About Orphan Drug Designation and Rare Pediatric Disease Designation
The FDA Orphan Drug Designation program provides orphan status to drugs and biologics that are intended for the safe and effective treatment, diagnosis or prevention of rare diseases that affect fewer than 200,000 people in the United States. Among the benefits of orphan designation are seven years of market exclusivity following FDA approval, waiver or partial payment of application fees, and tax credits for clinical testing expenses conducted after orphan designation is received.

The FDA defines a rare pediatric disease as a serious or life-threatening disease primarily affecting individuals age 18 years or younger that impacts fewer than 200,000 people in the United States. The FDA Rare Pediatric Disease designation and voucher program is intended to facilitate development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. Under this program, the FDA awards priority review vouchers to sponsors of product applications that meet certain criteria. The voucher can be used to receive a priority review of a subsequent marketing application for a different product or sold or transferred to another company.

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 is a humanized monoclonal antibody in clinical development as a conditioning agent that blocks stem cell factor receptor signaling leading to clearance of hematopoietic stem cells from bone marrow, creating an empty space for donor or gene-corrected transplanted stem cells to engraft. To date, JSP191 has been evaluated in more than 90 healthy volunteers and patients. It is currently enrolling in two clinical trials for myelodysplastic syndromes (MDS)/acute myeloid leukemia (AML) and severe combined immunodeficiency (SCID) and expects to begin enrollment in four additional studies in 2021 for severe autoimmune disease, sickle cell disease, chronic granulomatous disease and Fanconi anemia patients undergoing hematopoietic cell transplantation.

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 is advancing two potentially groundbreaking programs. JSP191, an anti-CD117 monoclonal antibody, is in clinical development as a conditioning agent that clears hematopoietic stem cells from bone marrow in patients undergoing a hematopoietic cell transplantation. It is designed to enable safer and more effective curative allogeneic and autologous hematopoietic cell transplants and gene therapies. In parallel, Jasper Therapeutics is advancing its preclinical engineered hematopoietic stem cell (eHSC) platform, which is designed to overcome key limitations of allogeneic and autologous gene-edited stem cell grafts. Both innovative programs have the potential to transform the field and expand hematopoietic stem cell therapy cures to a greater number of patients with life-threatening cancers, genetic diseases and autoimmune

diseases.

Contacts

Lily Eng
Real Chemistry
206-661-8627
leng@realchemistry.com

Jeet Mahal
Jasper Therapeutics
650-549-1403
jmahal@jaspertherapeutics.com