Jasper Therapeutics Announces European Union Orphan Drug Designation for Briquilimab as a Conditioning Treatment for Patients Prior to Receiving a Stem Cell Transplant

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REDWOOD CITY, Calif., Jan. 04, 2023 (GLOBE NEWSWIRE) -- Jasper Therapeutics, Inc. (NASDAQ: JSPR), a biotechnology company focused on transforming the field of hematopoietic cell transplant (HCT) therapies, today announced that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) has granted orphan drug designation to briquilimab (formerly known as JSP191), a monoclonal antibody targeting the CD117 (stem cell factor) receptor, for conditioning treatment prior to HCT. Previously, the U.S. Food and Drug Administration granted orphan drug designation to briquilimab in HCT, as well as rare pediatric disease designation for the treatment of severe combined immunodeficiency (SCID).

"The EMA's decision to grant orphan drug designation to briquilimab highlights the clear need for non-genotoxic, targeted conditioning for patients receiving hematopoietic stem cell transplant," said Ronald Martell, President and Chief Executive Officer of Jasper Therapeutics. "Transplants have the potential to cure several hematologic cancers and genetically inherited diseases. However, the toxicities associated with genotoxic conditioning needed to prepare patients for these procedures often limit their use. We believe that briquilimab has the potential to fill this gap, effectively expanding access to curative stem cell transplant across a range of indications. With the orphan designation, the EMA has demonstrated support of briquilimab in Europe and Jasper is committed to advancing this therapy globally."

Jasper is currently conducting clinical studies of briquilimab as a conditioning agent prior to hematopoietic stem cell transplant in patients with SCID, a rare, life-threatening, pediatric disorder affecting an estimated 1/58,000 births in the general population, and separately in patients with acute myeloid leukemia (AML) or myelodysplastic syndromes (MDS). In these diseases, prognosis is poor for patients and transplant rates are low due to the highly toxic conditioning required.

Jasper's ongoing clinical trial in SCID is evaluating briquilimab 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 presented data from this study at multiple scientific conferences, which demonstrated that briquilimab has been well tolerated with no treatment-related adverse events across multiple patients ranging from 3 months to 38 years old. Successful stem cell engraftment and immune reconstitution have also been observed.

Jasper has also presented data from a study that has demonstrated a tolerable safety profile and full donor chimerism in 24 of 24 patients with AML or MDS. To date, there have been no reports of briquilimab-related significant adverse events, classical acute grade II-IV graft versus host disease, or transplant related mortality within 100 days. MDS are a group of disorders in which immature blood-forming cells in the bone marrow become abnormal and do not make new blood cells or make defective blood cells, leading to low numbers of normal blood cells, especially red blood cells. In about one in three patients, MDS can progress to AML, a rapidly progressing cancer of the bone marrow cells. Both are diseases of the elderly with high mortality.

About Orphan Drug Designation in Europe

Orphan drug designation in the European Union (EU) is granted by the European Commission based on a positive opinion adopted by the EMA Committee for Orphan Medicinal Products (COMP). The EMA's orphan designation is given to drugs and biologics in the European Union for conditions defined as rare diseases, which affect no more than 5 in 10,000 people in the EU, with most drugs awarded this status in the EU being for pediatric use. Companies that meet the EMA's orphan designation criteria are eligible for incentives that include protocol assistance from the EMA, potential fee reductions, and EU marketing exclusivity for 10 years after approval.

About Briquilimab (formerly known as JSP191)

Briquilimab is a targeted, monoclonal antibody that inhibits the cell-surface receptor c-KIT, also known as CD117. It is currently being evaluated as a conditioning agent for cell and gene therapies, as well as a standalone therapy. To date, briquilimab has a demonstrated efficacy and safety profile in 130 dosed subjects and healthy volunteers, with clinical outcomes as a conditioning agent in severe combined immunodeficiency (SCID), acute myeloid leukemia (AML), myelodysplastic syndromes (MDS), Fanconi anemia (FA), and sickle cell disease (SCD). In addition, briquilimab is being advanced as a transformational non-genotoxic conditioning agent for gene therapy and as a primary therapeutic in low-risk MDS patients. Clinical studies also suggest briquilimab can be used as a primary therapeutic to treat mast cell diseases such as chronic spontaneous urticaria (CSU), chronic inducible urticaria (CIndU), and allergic asthma.

About Jasper Therapeutics

Jasper Therapeutics is a clinical-stage biotechnology company focused on unlocking access to curative therapies by targeting and eliminating diseased stem cells. Jasper's lead program is briquilimab, a first-in-class monoclonal antibody targeting c-KIT (CD117), an important receptor found on stem cells and mast cells. In parallel, Jasper is advancing its mRNA cellular programming platform which is designed to overcome key limitations of allogeneic and autologous gene-edited stem cell grafts by transiently modifying stem cells with mRNA, augmenting them to treat several diseases of the blood and bone marrow. Both innovative programs have the potential to enable curative therapies for a greater number of patients with life-threatening cancers, genetic disorders, and inflammatory diseases. For more information, please visit us at jaspertherapeutics.com.

Forward-Looking Statements

Certain statements included in this press release that are not historical facts are forward-looking statements for purposes of the safe harbor provisions

under the United States Private Securities Litigation Reform Act of 1995. Forward-looking statements are sometimes accompanied by words such as "believe," "may," "will," "estimate," "continue," "anticipate," "intend," "expect," "should," "would," "plan," "predict," "potential," "seem," "seek," "future," "outlook" and similar expressions that predict or indicate future events or trends or that are not statements of historical matters. These forward-looking statements include, but are not limited to, statements regarding the potential long-term benefits of hematopoietic stem cells engraftment following targeted briquilimab conditioning. These statements are based on various assumptions, whether or not identified in this press release, and on the current expectations of Jasper and are not predictions of actual performance. These forward-looking statements are provided for illustrative purposes only and are not intended to serve as, and must not be relied on by an investor as, a guarantee, an assurance, a prediction or a definitive statement of fact or probability. In addition, a positive opinion from the EMA on Jasper's application for orphan drug designation for briquilimab is not a guarantee of trial success or approval. Actual events and circumstances are difficult or impossible to predict and will differ from assumptions. Many actual events and circumstances are beyond the control of Jasper. These forward-looking statements are subject to a number of risks and uncertainties, including general economic, political and business conditions; the risk that the potential product candidates that Jasper develops may not progress through clinical development or receive required regulatory approvals within expected timelines or at all; risks relating to uncertainty regarding the regulatory pathway for Jasper's product candidates; the risk that clinical trials may not confirm any safety, potency or other product characteristics described or assumed in this press release; the risk that Jasper will be unable to successfully market or gain market acceptance of its product candidates; the risk that Jasper's product candidates may not be beneficial to patients or successfully commercialized; patients' willingness to try new therapies and the willingness of physicians to prescribe these therapies; the effects of competition on Jasper's business; the risk that third parties on which Jasper depends for laboratory, clinical development, manufacturing and other critical services will fail to perform satisfactorily; the risk that Jasper's business, operations, clinical development plans and timelines, and supply chain could be adversely affected by the effects of health epidemics, including the ongoing COVID-19 pandemic; the risk that Jasper will be unable to obtain and maintain sufficient intellectual property protection for its investigational products or will infringe the intellectual property protection of others; and other risks and uncertainties indicated from time to time in Jasper's filings with the SEC. If any of these risks materialize or Jasper's assumptions prove incorrect, actual results could differ materially from the results implied by these forward-looking statements. While Jasper may elect to update these forward-looking statements at some point in the future, Jasper specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Jasper's assessments of any date subsequent to the date of this press release. Accordingly, undue reliance should not be placed upon the forward-looking statements.

Contacts:
John Mullaly (investors)
LifeSci Advisors
617-429-3548
jmullaly@lifesciadvisors.com

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

Lauren Barbiero (media)
Real Chemistry
646-564-2156
lbarbiero@realchemistry.com

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