Jasper Therapeutics Announces Positive Clinical Data from a Phase I/II Trial of Briquilimab as a Conditioning Treatment in Sickle Cell Disease and Beta Thalassemia

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- *All three sickle cell disease participants treated with briquilimab successfully engrafted with neutrophil engraftment within 12-16 days*
- *First two participants with peripheral blood chimerism at 60 days after allogeneic stem cell transplant achieved 100% donor myeloid chimerism*
- *First participant treated has a total hemoglobin level of 13.3 g/dL at five months follow up, increased from 8-9 g/dL at baseline*

REDWOOD CITY, Calif., Jan. 03, 2023 (GLOBE NEWSWIRE) -- Jasper Therapeutics, Inc. (NASDAQ: JSPR), a biotechnology company focused on transforming the field of hematopoietic stem cell therapies, today announced positive clinical data from the first three participants in an investigator-initiated Phase 1/2 clinical trial (NCT05357482) evaluating the addition of briquilimab (formerly known as JSP191), Jasper's anti-c-KIT monoclonal antibody, to an existing bone marrow transplantation regimen (NCT00061568) in individuals with sickle cell disease (SCD) and beta thalassemia considered at high risk for complications from or ineligible for standard myeloablative hematopoietic stem cell transplant. The addition of briquilimab is being studied as a potential way to achieve a higher percentage of healthy donor stem cell engraftment (donor chimerism) without increased toxicity. The Phase 1/2 clinical study is led by Dr. John F. Tisdale, Director of the Cellular and Molecular Therapeutics Laboratory, NHLBI.

"While stem cell infusion with healthy donor stem cells or gene-corrected cells are potentially curative options for SCD and beta thalassemia, they are both limited by the toxicity of current conditioning regimens using busulfan or melphalan, which are often cited as the most concerning safety risks for transplant patients and physicians," said Ronald Martell, President and CEO of Jasper Therapeutics. "With briquilimab, we hope to offer a highly targeted conditioning regimen to directly address conditioning toxicity as a barrier limiting the ability of patients to access curative hematopoietic stem cell therapies."

For SCD and beta-thalassemia, transplantation of healthy donor stem cells is a multi-step process. After donor cells are collected, a human subject's existing stem cells must be cleared from the bone marrow to make space for the transplanted cells, which is known as bone marrow conditioning. Next, the newly transplanted cells must survive and replicate within the bone marrow, which is known as bone marrow engraftment. The extent of engraftment is measured by the proportion of the donor cells and the human subject's own cells, which is known as donor chimerism. As has been shown, improving chimerism is crucial to lead to a sufficient proportion of healthy donor stem cells that produce healthy red blood cells and reverse the sickle phenotype after the stem cell transplant.

The primary objective of the study is to determine if the addition of briquilimab would increase the proportion of patients with donor myeloid chimerism ≥98% at 1-year post-transplant. Briquilimab has the potential to improve disease-free survival in combination with low-dose irradiation as part of a transplant conditioning regimen. The study is currently actively enrolling at NHLBI.

In this study, briquilimab was added to the regimen used at NHLBI consisting of alemtuzumab, low-dose irradiation, and sirolimus prior to infusion of mobilized peripheral blood cells from human leukocyte-antigen matched related donors. All three sickle cell study participants treated with briquilimab have successfully engrafted with no briquilimab-related severe adverse events observed. Participant 1 achieved neutrophil engraftment at 12 days after transplant and platelet engraftment at 17 days after transplant. Participant 2 achieved neutrophil engraftment at 12 days and platelet engraftment at 10 days. Participant 3 achieved neutrophil engraftment at 16 days and platelet engraftment at 8 days. Both of the first two participants with peripheral blood chimerism achieved 100% donor myeloid chimerism at 60 days post-transplant. At five months post-transplant, the first participant treated with briquilimab has a total hemoglobin of 13.3 g/dL, increased from 8-9 g/dL at baseline.

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 to 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 cell engraftment following targeted briquilimab conditioning in the treatment of sickle cell disease and beta thalassemia and Jasper's ability to potentially deliver a targeted non-genotoxic conditioning agent to patients with these indications. 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. 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.

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