Clinical trial uses patients' own cells for personalized treatment after bone marrow transplant

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An innovative clinical trial using the science of "personalized" cellular therapy has begun enrolling children and adults suffering from graft-versus-host-disease (GVHD), a life-threatening complication of bone marrow transplantation in which donor immune lymphocytes attack the organs of the bone marrow transplant recipient.

Bone marrow transplantation is performed in some patients with cancers of the blood or bone marrow, including multiple myeloma and leukemia, as well as in some patients with sickle cell disease, thallesemia, aplastic anemia and inherited immune deficiency.

Physician-researchers at the Aflac Cancer and Blood Disorders Center at Children's Healthcare of Atlanta and Winship Cancer Institute of Emory University will harvest bone marrow cells from children and adults (12 to 65 years) with GVHD. Those cells will be used to manufacture large numbers of personalized autologous marrow mesenchymal stromal cells in the Emory Personalized Immunotherapy Center (EPIC), a dedicated pharmaceutical grade facility located within Emory University Hospital.

By infusing large doses of these personalized bone marrow cells into bone marrow transplant recipients, the physician-researchers aim to target sites of inflammation, potentially reducing GVHD in the intestine, liver and skin and limiting long-term organ damage.

Muna Qayed, MD, MSc. a pediatric hematologist-oncologist at the Aflac Cancer Center at Children's and an assistant professor at Emory School of Medicine, will lead the clinical trial, which is offered only in Atlanta and is supported by CURE Childhood Cancer.

"For patients with GVHD who do not respond to first line therapy, there is no reliable cure, and GVHD can be life threatening or a life-long disabling condition," says Dr. Qayed, "But we hope that through our clinical research, we will be able to significantly impact the course of this disease."

"This trial represents one of the most innovative clinical trials to arise from the growing partnership between the Hematology & Medical Oncology and Pediatrics departments at Emory School of Medicine, Emory Healthcare, and Children's Healthcare of Atlanta," says William (Bill) G. Woods, MD, director of the Aflac Cancer Center.

Blood and bone marrow cells have been used for more than a quarter century to treat life-threatening hematological conditions and are now used in established therapies worldwide. The current clinical trial will use mesenchymal stromal cells from the bone marrow. These cells have been studied more recently for treatment of a wide array of diseases, including autoimmune diseases.

"The beginning of this clinical trial is the culmination of two years' of collaborative effort by a terrific multidisciplinary team at Emory Healthcare, Children's Healthcare of Atlanta and the Aflac Cancer Center," says Edmund Waller, MD, director of Winship's Bone Marrow and Stem Cell Transplant Program and investigator on this trial.

Symptoms of GVHD disease include severe abdominal pain, diarrhea, fever, weight loss, skin rash and liver damage. Additionally, chronic GVHD can affect the joints and lungs, among other organs. Available

therapies designed to suppress the inflammation of GVHD do not work in everyone and can be life-threatening for patients. The occurrence of GVHD after bone marrow transplantation is high, highlighting the need for new therapies.

The Emory and Children's study is technologically innovative and closely regulated. The personalized cells are derived from the patient's own bone marrow rather than using another person's, making the product more likely to be effective. The team manufactures these cells without using animal products, and the cells are delivered fresh and living. The clinical trial is FDA monitored, and patients will not be charged a fee to receive these cells. Patients will undergo close medical follow-up after treatment.

"This represents the first step of a new partnership between Emory and Children's to advance innovative health care for all Georgians - young and old. This is quite literally a new level of care and discovery that is first in Georgia, first in humans and first in children," says Jacques Galipeau, MD, director of EPIC, a professor of pediatric hematology-oncology at Emory School of Medicine and member of Aflac Cancer Center at Children's and Winship Cancer Institute.

"The premise of this new study, and EPIC and its overlying mission, is a focus on cellular and biological therapies that use a patient's own cells to neutralize unwanted cells that make a person sick, and to enhance a patient's ability to recover," says Ian Copland, PhD, EPIC's laboratory director.

Provided by Emory University