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MACC Fund Center for Cancer and Blood Disorders brings life-changing gene therapy to kids with severe sickle cell disease and transfusion dependent Thalassemia

Children's Wisconsin will be only provider of new Casgevy therapy in Badger State

MILWAUKEE, Sept. 17, 2024 — The MACC Fund Center for Cancer and Blood Disorders at Children's Wisconsin is now offering the newly FDA-approved Casgevy therapy to children 12 years and older who have severe sickle cell disease or transfusion-dependent thalassemia. Casgevy works by adding a gene that makes fetal hemoglobin, which reduces the clinical effects of sickle cell disease or thalassemia.

"Sickle cell disease is a painful, debilitating blood disorder that is inherited, and patients are diagnosed at birth on the newborn screen," said Julie-An Talano, MD, medical director of blood and marrow transplant at Children's Wisconsin. "Given what we've seen so far of this therapy's results, we're optimistic that this therapy can decrease the severe acute events associated with the disease, especially acute painful events."

Currently, about 1,200 people live with sickle cell disease in the state of Wisconsin (with 85 percent of these individuals living in the southeastern region) and 117 infants were born in Wisconsin with sickle cell disease between the years 2016 and 2020. The Children's Wisconsin sickle cell disease program currently provides care for about 400 children living with sickle cell disease.

Children's Wisconsin will be offering gene therapy in children with severe symptoms of sickle cell disease and children receiving blood transfusions to manage their thalassemia. Symptoms of sickle cell disease are broad and can range from severe acute intermittent pain, chronic daily pain, lung problems, stroke, enlarged spleens, kidney problems and bone necrosis in hips and shoulders.

Thalassemia are a group of lifelong inherited blood disorders caused by genetic changes of hemoglobin, the protein in red blood cells that carries oxygen throughout the body. These disorders cause the red blood cells to become fragile, and the body is not able to make enough red blood cells and hemoglobin. Children with severe forms of thalassemia require lifelong blood transfusions, with a hallmark complication of too much iron in the body. Iron deposition subsequently leads to life-threatening complications of the liver, heart and endocrine organs.

Prior to this new gene therapy being approved, typical treatment for sickle cell disease could include a daily oral medication called hydroxyurea, regular blood transfusions that lead to the need for iron chelation (removing excess iron from the blood) and possibly a bone marrow transplant as a curative therapy. Children with thalassemia need blood transfusions every few weeks and would require a bone marrow transplant as curative therapy.

"The big difference between Casgevy and traditional approaches is that, in the clinical trials that led up to FDA approval, this new gene therapy resulted in more than 93 percent of patients having at least 12 consecutive months without a severe vaso-occlusive crisis in the two-year study time after administration," said Dr. Talano. "Additionally, 92 percent of patients with thalassemia were transfusion-free for more than 12 months after receiving Casgevy, What's more, this therapy is able to achieve these results with significantly fewer serious side effects than existing therapies."

Casgevy is made from the patient's own blood stem cells, which are modified using CRISPR technology, and returned to the patient in a single-dose infusion as part of a stem cell transplant. Prior to the infusion, the patient's stem cells are collected for the modification, and then the patient goes through a round of high-dose chemotherapy intended to destroy the existing cells from the patient's bone marrow that make the mal-formed blood cells. The gene-modified cells that are infused produce fetal hemoglobin that can overcome the need for blood transfusions in most patients and improve severe pain crises in patients with sickle cell disease.

MACC Fund Center physicians estimate the total treatment will involve an approximate two-month hospital stay.

About Children's Wisconsin

Children's Wisconsin is the region's only independent health care system dedicated solely to the health and well-being of children. The hospital, with locations in Milwaukee and Neenah, Wisconsin, is recognized as one of the leading pediatric health care centers in the United States. It is ranked among the top pediatric hospitals in the country by *U.S. News & World Report*'s 2023-24 Best Children's Hospitals report. Children's provides primary care, specialty care, urgent care, emergency care, mental and behavioral health care, community health services, foster and adoption services, child and family counseling, child advocacy services and family resource centers. In 2022, Children's invested more than $171 million in the community to improve the health status of children through medical care, advocacy, education and pediatric medical research. Children's achieves its mission in part through donations from individuals, corporations and foundations and is proud to be a member of Children's Miracle Network Hospitals.

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