

Stem cell therapy furthers research for infants with hypoplastic left heart syndrome

July 23 2019, by Terri Malloy

A phase I clinical trial is the first research monitored by the Food and Drug Administration that demonstrates the potential of regenerative therapy for hypoplastic left heart syndrome (HLHS) through collecting, processing and injecting an infant's own stem cells directly into the heart at the time of surgery. A paper detailing the clinical trial was published in *The Journal of Thoracic and Cardiovascular Surgery*.

The study focused on the safety and feasibility of stem cell treatments designed to strengthen the heart muscle of children with [hypoplastic left heart syndrome](#), a severe congenital heart disease. The research is funded by the Todd and Karen Wanek Family Program for Hypoplastic Left Heart Syndrome at Mayo Clinic and Mayo Clinic.

Hypoplastic left heart syndrome affects approximately 1,000 infants in the U.S. each year. In these babies, the left side of the heart is critically underdeveloped, requiring surgical intervention to support remaining function in the right side of the heart. Patients with hypoplastic left heart syndrome undergo three staged reconstructive surgeries. The Norwood surgery is typically performed within the first days of life. The Glenn surgery takes place within the first few months of age. And the Fontan operation is performed at 2 to 4 years of age.

Since HLHS is rare, no single hospital can treat enough of these patients to advance research with the scope, scale and pace provided by Mayo Clinic's HLHS Consortium, says Tim Nelson, M.D., Ph.D., senior author and director of the Todd and Karen Wanek Family Program for

Hypoplastic Left Heart Syndrome. Within this growing collaboration, seven hospitals and an advocacy group work together to provide care for HLHS patients across the U.S. The combined efforts of treatment teams facilitate the sharing of care options and information that benefit patients, families and research.

The phase I clinical trial studied 10 babies diagnosed with hypoplastic left heart syndrome before birth, each enrolled in the ongoing Mayo Clinic Umbilical Cord Blood Collection Study. A minimum of 35 milliliters of cord blood was collected at the time of birth using a specialized collection kit. Then this blood was sent to Mayo Clinic for processing and storage. Mayo Clinic's Human Cellular Therapy Laboratories and ReGen Theranostics Inc. manufactured a highly concentrated specialized stem cell product that could be stored and then shipped back to the hospital to be directly thawed and injected into the heart muscle at the time of the baby's second surgery.

"The infrastructure is now in place to collect and process stem [cells](#) with this method for any HLHS baby born in the U.S.," Dr. Nelson says.

Each patient underwent the first surgery and then received his or her processed stem cells during the second operation. This study was a first for using a cell-based therapy by direct injection during heart surgery in children. The study team was required to gather three months of follow-up data from the first child before administering the injection to the second child, and likewise for the third.

All 10 patients successfully underwent the second surgery with injection of stem cells directly into the heart. There were no deaths, and none of the children had any significant safety concerns over six months following [surgery](#).

"We now have a reproducible protocol to utilize stem cell therapy in

babies with HLHS. Our hopes are that this groundbreaking research will lead to stem cell therapy strengthening these babies' hearts while delaying or even preventing the need for a heart transplant in some," says Harold Burkhardt, M.D., a pediatric cardiothoracic surgeon at OU Medicine in Oklahoma and first author on the paper.

The HLHS Consortium is now conducting a larger phase IIb study with 50 infants. This study focuses on testing the ability of the stem cells to improve heart function. This study also is enrolling control patients who do not receive cell delivery because hypoplastic left [heart syndrome](#) was not diagnosed before birth or cord blood was not collected.

Provided by Mayo Clinic

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