

BroadcastMed | Mayo Clinic First in U.S. Testing Stem Cell Use in Pediatric Congenital Heart Disease Trial

HLHS stands for hypoplastic left heart syndrome. This is a very severe congenital heart defect that's a subject of our research program here at Mayo Clinic. And these children are born without half of their heart. It's actually the strongest part of their heart. And without half a heart, they are at risk of developing heart failure. And so our strategy at Mayo Clinic is to try to develop new innovative treatments to try to strengthen the weak heart that they're born with.

The goal of this study is to see if taking cells at the time of birth from the umbilical cord and then making or culturing these cells. Is there a role for them in injecting them back into that right ventricle at the time of the second operation? And see if having those cells in there will stimulate growth of more heart cells in that pumping chamber. The hope would be that that would make the ventricle stronger. So potentially it could do the work of both ventricles for a very long time. Or at the very least, in a ventricle that's not that strong, it may delay the time to transplant.

So this study is really designed for 10 patients to undergo this initial testing of safety and feasibility. And with that experience, we hope that we learn how to proceed and how to scale this and make it more available, more broadly, as we go forward.

This is the first time that the FDA has approved any kind of stem-cell therapy for the pediatric population. So it hasn't been seen in other anomalies-- it hasn't been shown in other anomalies-- because this is truly a breakthrough study in which, for the first time, we're giving children with this anomaly an opportunity to potentially improve their long-term outcome and their cardiac function.

We really dream about the day where we can use this type of technology to regenerate the heart. And what I mean by that is by planting the right seed, or stem cells, in the right patient at the right time, we dream about being able to make the heart bigger, better, and stronger so that we can delay or prevent transplant down the road.