Mayo Clinic News Network

Hypoplastic Left Heart Syndrome

Research collaboration giving hope for future of babies with rare heart defect

Video	Audio
00:09:29 ANDREA SEXTON:	"He's amazing. He is the strongest little boy … If you were just looking at him, you'd never know anything was wrong."
	Andrea and Heath Sexton never imagined that would even be possible for their son Ryals after he was diagnosed with a congenital heart defect while still in the womb.
00:01:44 HEATH SEXTON:	"All of a sudden, to see the ultrasound tech blinking and staring at the monitor and then getting a little frantic and rushing out to get the doctor, I knew something was wrong."
	The diagnosis: Hypoplastic Left Heart Syndrome – or HLHS.
	Essentially, there was no left side of baby Ryals' heart.
	It's a relatively rare condition. Only about a thousand children are born with HLHS each year in the United States.
00:05:35 **HEATH SEXTON** **Ryals' Father**	"Coming home from the hospital we were wondering how is this going to change our lives … Obviously, the care of a child that possibly would need a lot of help, maybe a lot of long-term help. And then, of course, in the back of your mind, obviously, you've read that a lot of children don't make it past three months. And so just thinking about, you know, am I going to be prepared to – to – for a funeral.

	The Sextons packed up and temporarily left their home in Alabama to move to Philadelphia.
	The plan was to place Ryals' hopes of survival in the hands of a team of doctors at CHOP - Childrens Hospital of Philadelphia, one of the leading medical centers for HLHS surgeries and treatment.
	There they met Dr. Joe Rossano, who heads up the Cardiac Center at CHOP.
00:01:11 **JOSEPH ROSSANO, M.D.** **PEDIATRIC CARDIOLOGY** **Children's Hospital of Philadelphia**	"Yeah, they're a very complicated surgery. You know, prior to the, you know, 1980s and '90s, there were, you know, essentially no good surgical options for these patients. But a number of very innovative surgical techniques were developed that have allowed many of these children to survive and thrive."
	This series of surgeries allows children with HLHS to live relatively normal lives, but it isn't perfect.
	Having the right side of the heart perform the tasks normally handled by both sides of the heart puts tremendous stress and pressure on the right side.
	As patients with HLHS are aging, doctors are realizing many of their hearts are unable to continue to function on their own and some patients need a heart transplant.
	But Andrea found out about a clinical trial going on at Mayo Clinic aiming to solve that problem.
00:10:23 **ANDREA SEXTON** **Ryals' Mother**	"Quickly they set me up with an interview over the phone with Dr. Nelson. He was amazing. He gave me hope."
00:06:01 **TIM NELSON, M.D., PH.D.** **DIRECTOR, HLHS PROGRAM** **Mayo Clinic**	"The HLHS Program has a mission to cri – to recreate the right ventricle to make it bigger and stronger … So we're finding ways of inventing new therapies to make that right heart stronger by stimulating growth of the heart muscle and make the five-horsepower engine a ten-horsepower

	engine, a fifty-horsepower engine, and if we make it strong enough, we believe that that has a shot at delaying and preventing transplant for a significant number of these children."
	The key to this groundbreaking regenerative therapy trial is stem cells. But not just any stem cells.
00:07:28 DR. TIMOTHY J. NELSON:	"So the first product that we're testing in our clinical trial is using umbilical cord blood from the baby's own body. So, the child has to be diagnosed in utero, and we have to be able to collect the cord blood at birth. We can collect the cord blood from anywhere in the country and ship it into Rochester, Minnesota to have it processed to have a high concentrated product that gets frozen in low temperatures of liquid nitrogen. That product is frozen for three months until the child has their second surgery, or the Glenn operation, which then we can bring those cells back into the operating room, thaw them, and deliver them directly into the heart muscle.
00:08:07 DR. TIMOTHY J. NELSON:	"The hope of this is that it causes a fertilizer type of an effect where these cells are able to fertilize the right ventricle muscle and allow it to grow bigger and stronger because it's received this cell-based therapy."
	On Thursday, May 4th, 2017, Ryals Sexton became the fifth clinical trial patient in Mayo Clinic's HLHS team's research.
	While surgeons at CHOP performed Ryals' Glenn operation, they injected stem cells from Ryals' umbilical cord blood directly into his tiny heart.
	(counting as cells are injected into the heart: "5, 4, 3, 2, 1, out.")
	After several hours of waiting during Ryals' surgery, his parents are emotional and relieved to hear all went well, and that

	Ryals' cells were injected without any problems.
(10:45 – 10:49)(Nelson and Andrea)	"Thank you. You're welcome. He's a trooper."
(13:12 - 13:39) (Heath and Nelson)	"I felt like we had a superhero come in on this jet plane, and he had this box of super cells. And it felt like you'd come to save my son."
	In an effort to expand the research and help more families, in 2017 the HLHS team at Mayo began creating a Hypoplastic Left Heart Syndrome research consortium.
00:21:41 DR. TIMOTHY J. NELSON:	"So, we've built this infrastructure to be able to do clinical trials and ultimately we need to be able to have a large number of families participating in that. So, the consortium really represents our ability to take the science and technology and teams of people to other centers of excellence that allows us to interact with more families at more locations."
	CHOP joined Mayo Clinic as the first members of the consortium, and Ryals was the first patient to benefit from it.
	Not long after, Children's Hospital of Los Angeles became the third institution to join the consortium, followed soon after by Children's Minnesota.
	Dr. Nelson sees the collaboration eventually leading to breakthroughs that once seemed impossible.
00:32:36 DR. TIMOTHY J. NELSON:	"You can even imagine where the technology is leading us, to be able to recreate patches and constructs and maybe even whole ventricles that can be engineered, that can be transplanted at some point."
	The days after Ryals received his cells were filled with firsts for the Sextons: a first smile and a first bottle.

	They got to bring Ryals back home to Alabama as a growing, relatively healthy baby boy.
	And they've finally gotten what they feared they never would: a sense of normalcy and a chance to dream about Ryals' future.
00:28:00 HEATH SEXTON:	"I guess I see the survivors of the day that are having children and having careers. And that's what I see my son as. And the hope is that he can see his grandchildren."
00:27:23 ANDREA SEXTON:	"I see college. I see happiness and I see my boys playing together. I see a happy family and I believe that this work, this research that these doctors have put hard work into is going to – to let us achieve that."
	(Ryals giggling at balloon)