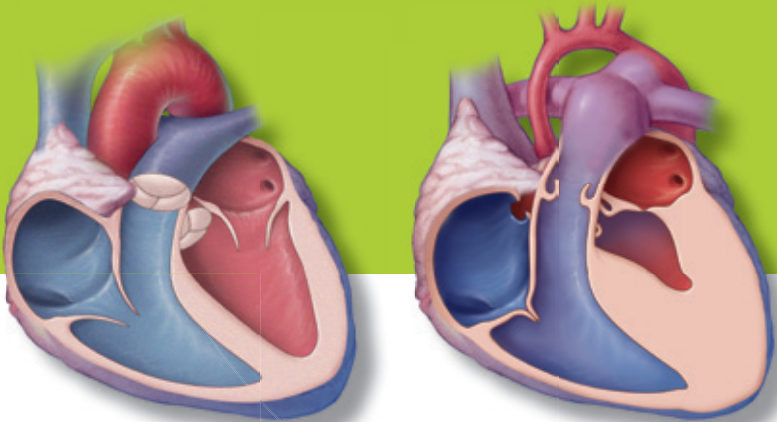
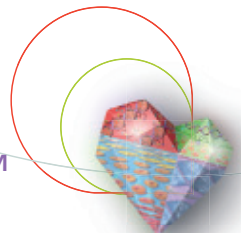




# HELPING CHILDREN AND YOUNG ADULTS WITH HYPOPLASTIC LEFT HEART SYNDROME



TODD AND KAREN WANEK FAMILY PROGRAM  
FOR HYPOPLASTIC LEFT HEART SYNDROME



Todd and Karen Wanek established the Mayo Clinic Todd and Karen Wanek Family Program for Hypoplastic Left Heart Syndrome to accelerate advanced research into the rare congenital heart defect. Their gift was made in honor of their daughter and all children afflicted with HLHS.



**Hypoplastic left heart syndrome (HLHS) is a rare and complex congenital heart disease** in which the left side of a child's heart is severely underdeveloped, leaving only the right side to pump blood to the lungs and the rest of the body. HLHS occurs during fetal growth when the baby's heart is developing. HLHS affects nearly 1,000 newborns per year in the United States alone, and at this time, it cannot be prevented and the cause is unknown.

Today, the only treatment for HLHS is aggressive, early surgical intervention. Even when these surgeries are successful, some children with HLHS still develop heart dysfunction later in life. When severe, this dysfunction often leads to heart transplantation. The Mayo Clinic Todd and Karen Wanek Family Program for Hypoplastic Left Heart Syndrome is working to give children better options.

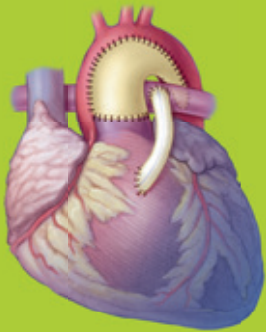
### DIAGNOSIS

Doctors trained in treating children with heart conditions (pediatric cardiologists) diagnose HLHS with an echocardiogram (ultrasound). Doctors often diagnose HLHS through an echocardiogram performed before a baby is born.

### CONVENTIONAL TREATMENT

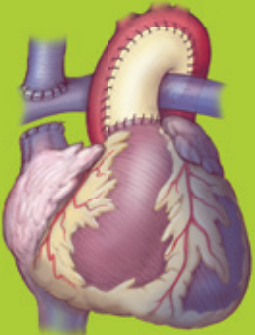
A child with HLHS requires urgent medical evaluation immediately after birth. Treatment of HLHS depends on the severity of the condition and the family's wishes. After diagnosis, a cardiologist helps family members stabilize the child's condition as they consider treatment options. The most common approach involves a three-stage surgical heart procedure (Norwood, Glenn and Fontan operations). These operations are performed sequentially within the first few years of life. This staged treatment plan is designed to create normal blood flow in and out of the functional heart chambers, while also allowing the body to receive the oxygen-rich blood it needs.

Even with this palliative intervention, patients with HLHS may require a heart transplant later in life.



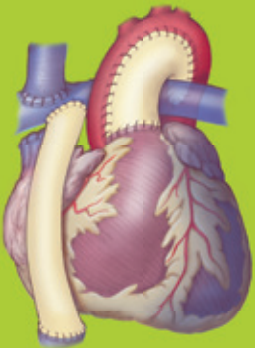
### **Stage 1 (Norwood Procedure):**

Usually performed within the first week of life, the Norwood procedure allows the heart's lower right chamber (right ventricle) to pump blood to the lungs and body (similar to what it did before the baby was born).



### **Stage 2 (Glenn Procedure):**

The Glenn procedure is typically performed between four and six months of age. It reduces the work of the right ventricle by allowing it to pump blood mainly to the aorta and routing blood returning from the upper body to flow directly into the lungs.



### **Stage 3 (Fontan Procedure):**

Performed between 18 months and three years of age, the Fontan procedure connects the remaining blood vessels carrying blood from the lower body directly to the lungs. This surgery allows the rest of the blood coming back from the body to flow to the lungs, normalizes the oxygen level, and reduces the workload facing the right ventricle to a nearly normal level.

## **FOLLOW-UP CARE**

Following surgery, children with HLHS need long-term monitoring of the reconstructed heart and blood vessels and often need to take heart medications. Follow-up appointments with a cardiologist include echocardiograms and other procedures (magnetic resonance imaging [MRI], blood tests, heart catheterizations) to test heart function as the child grows.

## **HOW IS MAYO CLINIC IMPROVING TREATMENT OPTIONS FOR HLHS?**

While advances in research and medicine have unlocked new opportunities for babies born with HLHS, outcomes are still far from ideal. Children and young adults with HLHS live with the risk of heart transplant every day.

Regenerative medicine strategies for HLHS have the potential to provide an alternative to heart transplantation. Using stem cells of different types and from various sources — including cells from the patient's own body — regenerative therapies for HLHS could replace, rejuvenate or regenerate defective tissues, leaving new, healthy tissues in their place.

To better understand and treat HLHS, researchers and physicians in the Mayo Clinic Todd and Karen Wanek Family Program for Hypoplastic Left Heart Syndrome are taking a multifaceted approach that includes research into cell-based strategies, imaging and outcomes, human genetics, and the creation of a biorepository.

### **Cell-Based Treatments**

Team members are developing cell-based therapies to make the heart muscle stronger. Researchers believe stem cells will increase the volume and strength of the heart muscle to give it greater durability and power to pump blood throughout the body. Such approaches could restore the pumping ability of the right ventricle once it begins to decline or perhaps prevent the decline altogether, eliminating the need for a future heart transplant.

The Todd and Karen Wanek Family Program for HLHS is launching clinical trials that offer the latest advances in cell therapy to people with HLHS. These trials aim to determine how stem cells derived from different sources in the body, delivered at different times, can help children with HLHS. For example, one trial collects stem cells from umbilical cord blood following birth and delivers the cells directly into the heart muscle or intravenously during the Glenn procedure.

Other research applies state-of-the-art technology to reprogram stem cells from an ordinary skin sample. Once reprogrammed, the stem cells can be converted into heart-like cells.

Researchers are also using such cells to map out how HLHS develops in the first place. For instance, after gathering surgically discarded tissues from children with HLHS and skin biopsies from their unaffected parents, researchers are creating reprogrammed stem cells to compare the two groups. By finding molecular differences, researchers will be able to understand how HLHS develops.

### Imaging and Outcomes

Physicians and scientists are working with families to better understand the natural history and predictors of long-term right-ventricular performance in people with HLHS. They are having early success in establishing how long an HLHS heart will perform by comparing the medical history of patients with HLHS with ultrasound and MRI images of their hearts.

To provide better answers, researchers are improving how cardiac performance is assessed. A primary goal is improving the ability to noninvasively assess right-ventricular function in HLHS, as well as in other complex forms of congenital heart disease, where the right ventricle is required to support systemic (aortic) circulation.

The result is that declines in cardiac function can be detected — and therefore managed — as early as possible.

### Human Genetics

Researchers believe HLHS can likely be traced to abnormal genes that cause underdevelopment of the left ventricle in the heart. But today, the identity of those genes remains elusive.

Investigators are applying comprehensive whole-genome sequencing and bioinformatics analyses in the search for HLHS-related genetic variations. They are also trying to detect gains or losses in chromosomal material in people with HLHS.

### Biorepository

To study HLHS, researchers are working with families to assemble family histories, gather tissue and cell samples, collect genomic information, and obtain heart images from people with HLHS and their relatives. This vast amount of information and material is combined in one place to maximize its usefulness — a biorepository.

A key piece of infrastructure, a biorepository holds clinical data, genetic data, tissue and other physical specimens. Physicians and researchers can draw on it in the future to continue to study HLHS and explore new treatment options.



#### For more information on the Hypoplastic Left Heart Syndrome Program at Mayo Clinic, visit

[www.mayoclinic.org/hlhs](http://www.mayoclinic.org/hlhs) or <http://www.mayo.edu/research/centers-programs/todd-karen-wanek-family-program-hypoplastic-left-heart-syndrome/overview>.

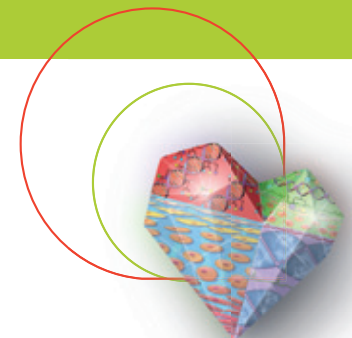
#### For more information about regenerative medicine at Mayo Clinic, visit

<http://mayoresearch.mayo.edu/regenerative-medicine/>.

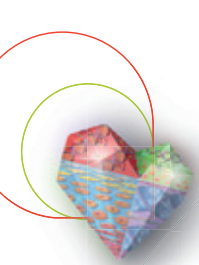
Mayo Clinic is an in-network insurance provider for millions of people and works with hundreds of health insurance companies. In most cases, Mayo Clinic doesn't require a physician referral. Some insurers require referrals or may have additional requirements for certain medical care.



For current information,  
please visit  
[HLHSblog.mayoclinic.org](http://HLHSblog.mayoclinic.org)  
or scan the QR Code.







The Todd and Karen Wanek Family Program for HLHS collaborates across disciplinary, departmental and organizational lines to better understand and treat HLHS. The Center for Regenerative Medicine, Children's Center, Transplant Center, Divisions of Pediatrics Cardiology and Cardiovascular Surgery, and others work together, building on Mayo Clinic's extensive research resources and clinical practice.

### **Center for Regenerative Medicine**

The Center for Regenerative Medicine harnesses Mayo Clinic's collective knowledge, resources, and skills to teach the body to heal from within. Driven by patient needs, the unprecedented advances in science, technology and medicine offer patients hope and definitive solutions for conditions beyond repair by conventional medicine. Regenerative Medicine techniques are charting a new course in health care by using native and bioengineered cells, assistive devices, material science and tissue engineering platforms to develop tomorrow's curative therapies and groundbreaking surgical procedures for a wide range of chronic diseases, disabilities, injuries, and congenital conditions.

### **Mayo Clinic Children's Center, Divisions of Pediatric Cardiology and Cardiovascular Surgery**

Mayo Clinic's pediatric cardiology and cardiovascular surgery programs have an excellent reputation for managing complex heart disease cases. For more than 50 years, the program's renowned team of experts has been providing comprehensive clinical services to diagnose and treat the most difficult congenital and acquired heart disease cases in infants, children and young adults. Each year, Mayo Clinic records 5,000 to 6,000 visits by children and adolescents for congenital heart problems. Mayo heart surgeons perform 350 to 400 operations each year to repair congenital heart defects.

### **Mayo Clinic Transplant Center**

Mayo Clinic is one of the largest and most experienced surgical practices in the world. Mayo has more than 300 surgeons; 122 operating rooms among its three locations in Arizona, Florida and Minnesota; and performs more transplants than any other medical center in the world. Mayo Clinic has preeminent adult and pediatric transplant programs that offer heart, liver, kidney, pancreas, limb and bone marrow transplant services.

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