



## Disease: Hypoplastic Left Heart Syndrome

### Hypoplastic Left Heart Syndrome

Hypoplastic left heart syndrome is a rare congenital heart disease with no known cause. It may be diagnosed during pregnancy or soon after the baby is born. Children with Hypoplastic Left Heart Syndrome (HLHS) have an underdeveloped left ventricle which results in low oxygenation of the blood and stress on the heart muscle of the right ventricle. The stress on the heart muscle eventually leads to severe tissue damage and heart failure. Although there are surgeries that can be done to help reconstruct the heart, many patients require a heart transplant.

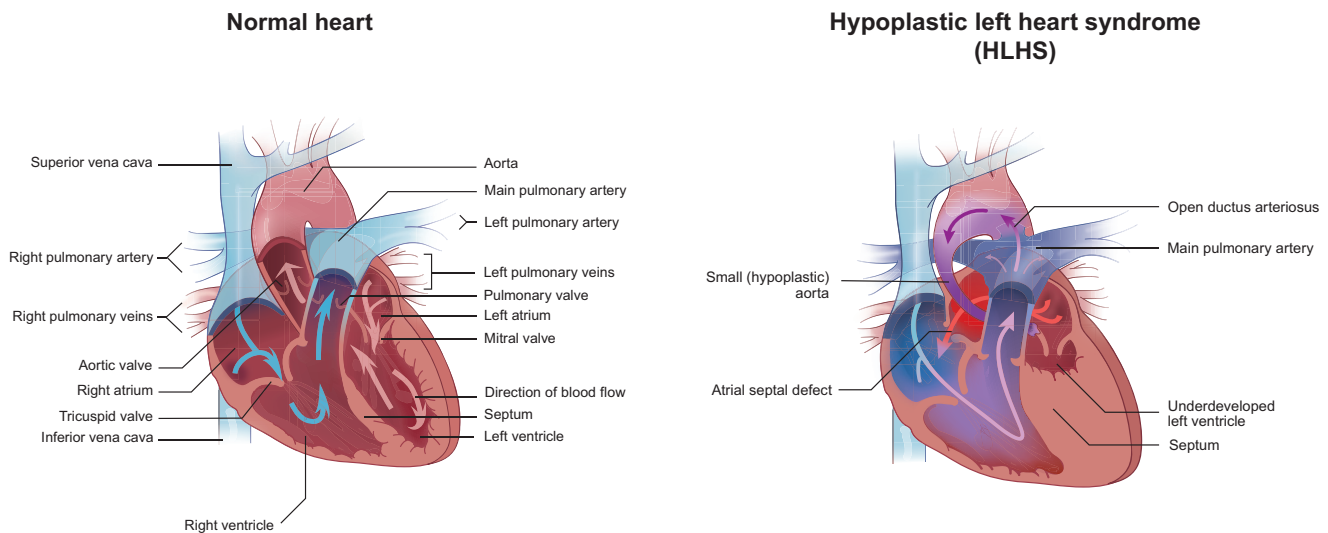


Illustration provided by Gabrielle Portal. [www.gabrielleportal.com](http://www.gabrielleportal.com).

## Incidence

Incidence of HLHS is 2.3 per 10,000 live births in the US. (Reference: Centers for Disease Control and Prevention).

## Impact of Umbilical Cord Blood

Studies have suggested a beneficial effect of umbilical cord blood in cardiac repair either through direct differentiation into heart muscle cells or the secretion of growth factors that stimulate the heart to repair itself<sup>1</sup>. It is hoped that in the context of HLHS, umbilical cord blood may work to strengthen the heart sufficiently to delay a heart transplant. It has been proposed that the child's umbilical cord blood will be suitable for infusion. Since the cells are from the child, there are no issues with rejection.

The Umbilical Cord Blood can be easily collected and stored frozen until needed for the procedure. The Phase 1 safety study proposed by the Mayo Clinic, <http://clinicaltrials.gov/ct2/show/NCT01883076> will determine the feasibility of collection, processing and delivery of autologous cells from the Umbilical Cord Blood for the management of HLHS.

Reference: <sup>1</sup> Acosta et al J Stem Cell Res Ther 2013 Jul 1;( Suppl 4). pii: S4-005.

# Safety Study of Autologous Umbilical Cord Blood Cells for Treatment of Hypoplastic Left Heart Syndrome

## Background

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**Sponsor:** Mayo Clinic

**Condition:** Hypoplastic Left Heart Syndrome

**Intervention:** Biological – Autologous Umbilical Cord Blood

**Stage:** Phase 1

**Study Start Date:** May 2013

**Estimated Study Completion Date:** December 2016

**Source:** <http://www.clinicaltrials.gov/ct2/show/NCT01883076>

## Purpose

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This is a Phase I study to determine the safety and feasibility of injections of autologous umbilical cord blood (UCB) cells into the right ventricle of HLHS children undergoing a scheduled Glenn surgical procedure.

The investigators are doing this research study to find out if autologous stem cells from the individual's own umbilical cord blood can be used to strengthen the muscle of the right side of their heart. This will help determine the safety and feasibility of using cell-based regenerative therapy as an additional treatment for the management of HLHS.

## Detailed Description

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This study is a Phase I trial to determine the safety of autologous mononuclear cells (MNC) derived from umbilical cord blood for intramyocardial delivery into the right ventricle during a planned and non-emergent Stage II surgical palliation in subjects with HLHS. This is the first critical step towards applying autologous MNC therapy as an add-on regenerative intervention for congenital heart disease management.

The choice of HLHS as the target disease for regenerative therapies in congenital heart disease management is multi-factorial and includes the following considerations: **1)** Severity of this incurable disease, **2)** palliative nature and burden of long-term outcomes with a single right ventricular system, **3)** three stages of planned surgical procedures that provide time points to adjunctively intervene, and **4)** prenatal diagnosis enabling planned collection of UCB.

An emerging goal for cardiac regeneration includes the application of cell-based technology to congenital heart disease, which is a favorable substrate due to the lack of fibrotic scarring, and the presence of a microenvironment that is expected to support ongoing cardiac proliferation and growth for functional remuscularization.

This Phase I safety study will determine the feasibility of collection, processing, and delivery of autologous cells as used in adult cardiac regenerative protocols in the setting of HLHS surgical management.

## Related clinical trials of interest

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**Duke University:** Safety and Feasibility Study of Umbilical Cord Blood Cells for Infants With Hypoplastic Left Heart Syndrome (<http://clinicaltrials.gov/ct2/show/NCT01445041>)

**Mayo Clinic:** Umbilical Cord Blood Collection and Processing for Cardiac Regeneration in Hypoplastic Left Heart Syndrome Patients (<http://clinicaltrials.gov/ct2/show/NCT01856049>)