SICKLE CELL DISEASE
What is Sickle Cell Disease (SCD)?

- *Sickle Cell Disease* (SCD) is a disease passed down through families in which red blood cells (oxygen carrying cells) form an abnormal crescent shape.

- Children with SCD suffer from severe infections and damage to the organs in the body.

- Some are even frequently hospitalized.
What is Sickle Cell Disease (SCD)?

- It is an inherited blood disorder characterized primarily by chronic anaemia and periodic episodes of pain.

- Haemoglobin molecules, in each red blood cell, carry oxygen from the lungs to body organs and tissues and bring carbon dioxide back to the lungs.

- In sickle cell disease, the haemoglobin is defective.

- After haemoglobin molecules give up their oxygen, some may cluster together and form long, rod-like structures. These structures cause red blood cells to become stiff and sickle shaped.
Types of SCD

- There are several types of SCD
- The most common type is **Sickle Cell Anaemia** where a child has inherited two genes that produce an abnormal haemoglobin called "S" haemoglobin ("SS" disease)
- There are several other, less common types of sickle cell disease where one gene produces "S" haemoglobin and the other gene produces "C" haemoglobin (**SC disease**)
- **S-Beta Thalassemia** is caused when a child inherits one gene producing a "beta-thalassemia" type of haemoglobin and the other gene produces “S" hemoglobin ("S-beta thalassemia")
- Your child could have inherited only one of these types. It is important for you to know which one
Symptoms of SCD

- Attacks of abdominal pain
-Bone pain
- Breathlessness
-Delayed growth and puberty
-Fatigue
-Fever
-Paleness
-Rapid heart rate
-Ulcers on the lower legs (in adolescents and adults)
- Yellowing of the eyes and skin (jaundice)
How does a child get SCD?

- The presence of two defective genes is needed for sickle cell disease

If each parent carries one sickle haemoglobin gene (S) and one normal gene (A), each child has

- a 25% chance of inheriting two defective genes and having sickle cell anaemia;
- a 25% chance of inheriting two normal genes and not having the disease;
- and a 50% chance of being an unaffected carrier like the parents.
Incidence of SCD in India

- Highest incidence in Central and South India
- 10% of Indian population affected
- 40% are CARRIERS
- Main ethnicities affected include Punjabis, Parsis, Biharis and tribal populations across the country
Cord Blood Treatment

Successful Cord Blood Transplantation for Sickle Cell Anemia From a Sibling Who Is Human Leukocyte Antigen-Identical: Implications for Comprehensive Care


We report the successful transplantation of umbilical cord blood stem cells from a sibling who is human leukocyte antigen-matched to a child with sickle cell anemia. Conditioning was with busulfan, cyclophosphamide, and antithymocyte globulin. Time to neutrophil count >500/μL was 23 days and to platelet count >50,000/μL was 49 days. Full donor engraftment was achieved without graft-versus-host disease. This case demonstrates the potential usefulness of harvesting cord blood from full siblings of patients with sickle cell disease. Routine collection of umbilical cord blood from siblings should be considered for patients with sickle cell disease, and may increase acceptance and use of transplantation by families.
Matched-related donor transplantation for sickle cell disease: report from the Center for International Blood and Transplant Research


Summary
We report outcomes after myeloablative haematopoietic cell transplantation (HCT) from human leucocyte antigen (HLA)-matched sibling donors in 67 patients with sickle cell disease transplanted between 1989 and 2002. The median age at transplantation was 10 years and 67% of patients had received >10 red blood cell transfusions before HCT. Most patients achieved haematopoietic recovery and no deaths occurred during the early post-transplant period. Sixty-four of 67 patients are alive with 5-year probabilities of disease-free and overall survival of 85% and 97% respectively. This report confirms and extends earlier reports that HCT from HLA-matched related donors offers a very high survival rate, with few transplant-related complications and the elimination of sickle-related complications in the majority of patients who undergo this therapy.
Advantages of Cord Blood Banking

- Unless your baby has a sibling, SCD can only be treated via BONE MARROW TRANSPLANT.

- This transplant requires a **100% matched donor**.

- Probability of finding a perfectly matched donor = **1 in 30,000**.
Advantages of Cord Blood Banking

- Banking cord blood can provide a source of treatment of SCD for your baby
- Cord Blood Banking is a painless, simple procedure
- It provides 80% chance of potentially curing your baby of SCD and 80 other blood-related disorders
THANK YOU