Advantages of haploidentical transplantation:

Almost all people have at least one potential haploidentical match in their family. Hence, children need not wait eternally for an unrelated donor, when there is half matched family member available.

Haploidentical transplants are becoming more common because they can help find a donor quickly and doctors researching this type of transplant have found that they can be a good alternative to other types. Studies worldwide have shown the outcomes with haploidentical BMT for Thalassaemia to be around 70-80%, which is as good as a fully matched unrelated donor and is only slightly less compared to matched sibling donor transplantation (80-90%).

Q5 Are there any specific side effects I should expect?

Side effects of a haploidentical transplant, similar to any transplantation, vary from person to person and can be short term or long term. They are often caused by the conditioning therapy you have before the transplant, as well as the effect of the transplant itself.

Short term side effects include need for blood and platelet transfusions, increased risk of infections, sore mouth (mucositis), liver and kidney problems, tiredness, diarrhoea and feeling or being sick and loss of appetite.

Long term side effects include - Increased risk of infection, fatigue, Graft versus host disease (GvHD).

References:

1. Hematopoietic Stem Cell Transplantation in Thalassemia and Sickle Cell Anemia. Guido Lucarelli et al. Cold Spring HarbPerspect Med. 2012 May; 2(5): a011825.
2. Hematopoietic Stem Cell Transplantation for Homozygous β Thalassemia and β Thalassemia/Hemoglobin E Patients from Haploidentical Donors. U Anurathapan et al. Bone Marrow Transplant. 2016 June ; 51(6): 813–818. doi:10.1038/bmt.2016.7.

Dr. Rela Institute & Medical Centre is a multi-specialty quaternary care hospital located in Chennai, India.

The Institute is within the campus of Sri Balaji Medical College and University, which is spread across 30 acres. It has 13 operating theatres with 450 beds, inclusive of 150 critical care beds.

The Institute is conveniently located 10 minutes from the Domestic and International Airport.

The hospital is designed to provide highly specialized care in various departments with a focus on multi-organ transplantation. Prof. Dr. Mohammed Rela, a world renowned surgeon in the field of Liver surgery and transplantation is the Chairman and Managing Director of the Institute.

In addition to quaternary & quality care, is also committed to provide day to day primary and secondary care to the local population, with facilities of international standards.

The Institute would provide comprehensive support to international patients travelling for medical treatment such as language assistance, stay, visa and travel.

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Department of
Paediatric Haematology, Oncology,
& Stem Cell Transplantation

Thalassaemia

Thalassaemia

Q1 What is thalassaemia?

Thalassaemia is a disorder of the body's red blood cells that isgenetically inherited and lifelong. It is caused by an abnormality in thegenes that control haemoglobin — the part of red blood cells thatcarry oxygen around the body. This results in the reduced amount ofnormal haemoglobin.

When the haemoglobin gets low, the body will try even harder toproduce the red blood cells. However, as they do not contain muchhaemoglobin, the child becomes anaemic. This effort can makesome of the organs too big, for example the liver and spleen, andchange the shape of the face.

Q2 What kind of problems can happen in thalassaemia?

Problems related to anaemia:

Anaemia makes people feel tired and can cause a decrease ingrowth. babies may feed poorly. Older children may lose their appetite and their tummy mays well with the large liver and spleen. The bone marrow can also expand to give characteristic bone thinning with swelling of facial bones, which is typical of thalassaemia.

Problems related to transfusion:

Each time blood is given the patient will also receive iron which is an essential part of haemoglobin. If the extra iron is not removed, it deposits around the body, mainly in the liver but also in vital organs such as the heart, pancreas and hormone producing glands. This results in serious problems, for example diabetes, failure to grow and go through puberty, infertility, low thyroid function and liver disease.

Other problems:

Infection, bone thinning, Infertility, side effects of chelation and kidney stones are some of the other problems encountered in a thalassaemic child.

Treatment:

Blood transfusion:

Good management will involve regular blood transfusions, usually every three to four weeks. This is to keep the haemoglobin high enough (> 9g/dl) so that these symptoms are reduced to an absolute minimum or are not present at all.

Iron chelation:

There is no naturalway to get rid of iron from the body, therefore we must usemedications to help remove the extra iron. These medications arecalled iron chelators and the treatment is called iron chelationtherapy.

This may be:

- Deferasirox which is one dissolvable tablet a day (taken as a drink)
- Desferriox amine which is given through an injection under the skin over several hours, a few days a week
- Deferiprone which is one tablet taken three times a day
- Combination of these.

Haematopoietic stem cell transplantation (HSCT/BMT):

The only permanent cure for Thalassaemia is Stem cell transplantation. Here, the bone marrow of your child is replaced by a healthy bone marrow from the donor. These healthy stem cells grow in thebone marrow and restore your child's body's ability to make blood cells, specifically healthy redblood cells and haemoglobin without thalassemia.

Q3 What are stem cells?

Stem cells are the very young cells that mature and develop into red blood cells, white blood cellsand platelets. Red cells (erythrocytes) carry oxygen to other cells in your body. White blood cells(leukocytes) fight infection. Platelets (thrombocytes) help blood to clot. All of these cells developfrom the stem cells. Stem cells are produced in the bone marrow. Very small numbers of stem cells also circulate inthe blood stream. These are called peripheral blood stem cells (PBSC's). Stem cells are alsopresent in the blood of the umbilical cord of a baby. Therefore, there are three places to obtainstem cells for transplantation: the bone marrow, the blood stream, or from the umbilical cordimmediately after birth.

HLA typing of stem cells:

An allogeneic transplant is one in which your child has a brother or sister who donates their stemcells for transplant. Your child's brother or sister must be a tissue match, also called an HLAmatch. To determine if one of your children is a match for another, a blood test (called HLAtyping) is performed. The chances of a sibling fully HLA matched is 25-30%. This is called a matched sibling donor (MSD) transplantation. If the siblings are not matched, next step is to determine if either of the parents are HLA matched. The chances of either parent to be fully HLA matched is 5-10%.

Q4 What to do if there is no matched family donor?

In situations, where there is no matched family donor, search is done for a fully HLA matched unrelated donor (MUD) through Indian and World Stem cell registries. If a matched donor is found in any of the registries, the registry is contacted to contact the donor for stem cell donation. The additional expenditure incurred towards procurement of stem cells from the donor registry is approximately USD 10,000 to 15,000.

Haploidentical transplantation:

If there is no fully HLA matched donor found in the family or any of the registries, the next best option is using half matched parent or sibling or a first-cousin as donor. This is called haploidentical transplantation. Biological parents are always a half-match for their children, and vice versa.