

The current uses of Stem Cells



netcells

Invest in your future health

There are 2 types of stem cells that can be collected at birth. They are:

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Haematopoietic stem cells -
Contained in the Umbilical Cord Blood.

Mesenchymal stem cells -
Contained in the Umbilical Cord Tissue and in smaller amounts, in the Cord Blood.

Stem cells can be used in standard therapies, clinical trials or in experimental treatments.

STANDARD THERAPIES

Haematopoietic stem cells

Umbilical cord blood is rich in haematopoietic stem cells. "Haematopoietic" means "blood forming" and these stem cells are destined ("programmed") to differentiate into the 3 types of blood cells: red blood cells, white blood cells and platelets. In our bodies, bone marrow is the source of all blood cells. Haematopoietic stem cells are contained in the bone marrow and they continuously make new blood cells to replace old ones. If bone marrow is damaged by disease or medication, it cannot make these essential blood cells, leading to fatal consequences. Therefore, haematopoietic stem cells must be replaced as part of the treatment. This is done via whole bone marrow transplant or stem cell transplant. Cord blood is an alternative source of stem cells for a transplant.

Below is a list of several diseases (but is by no means exhaustive) for which haematopoietic stem cell transplants are standard treatment. For some diseases they are the only therapy and for others, they are employed when front-line therapies have failed or the disease is very aggressive:

Leukaemia

Acute and Chronic (Leukaemia is a cancer of the blood immune system, whose cells are called leukocytes or white blood cells)

Myelodysplastic Syndromes

(Myelodysplasia is sometimes called pre-leukaemia)

Lymphomas

(Lymphoma is a cancer of the white blood cells that circulate in the blood and lymph vessels) such as Hodgkin's lymphoma or Non-Hodgkin's lymphoma

Anaemia

(Deficiencies or malformations of red blood cells that are inherited or acquired diseases)

- Severe aplastic anaemia
- Sickle cell anaemia
- Thalassaemia

- Fanconi's anaemia (Note: the first cord blood transplant in 1988 was for this disease)

- Paroxysmal nocturnal hemoglobinuria (PNH)
- Pancytopenia

Inherited Platelet Abnormalities

(Platelets are small blood cells needed for clotting)

- Amegakaryocytosis / congenital thrombocytopenia

Myeloproliferative Disorders

- Acute myelofibrosis
- Polycythemia vera
- Essential thrombocythemia

Inherited Immune System Disorders

- Severe combined immunodeficiency (SCID)
- Other inherited immune system disorders e.g. Di George syndrome, Kostman syndrome, haemophagocytic lymphohistiocytosis etc.

Metabolic Disorders

- Adrenoleukodystrophy
- Gaucher's disease
- Krabbe disease
- Gunther disease
- Hurler syndrome
- Hunter syndrome
- Neimann-Pick syndrome
- Tay-Sachs disease
- Lesch-Nyhan syndrome

Cancers in the bone marrow

- (Plasma Cell disorders)
- Multiple myeloma
 - Waldenstrom's macroglobulinemia
 - Plasma cell leukaemia

Other cancers

- (Not originating in the bone marrow or blood system)
- Neuroblastoma
 - Retinoblastoma
 - Nephroblastoma

Stem cells can be retrieved from different types of donors:

Allogeneic:

Patient receives stem cells from a matching donor, either a sibling or an unrelated donor



Allogeneic transplantation

Allogeneic transplants (allograft) may be used in the treatment of inherited and non- inherited disorders. Before doing a transplant, the recipient needs to find a matching donor via HLA-type matching tests.

Human leukocyte antigens (HLA) are proteins on the surface of cells that determines the tissue type of a person. The immune system uses the HLA's to differentiate its own cells from that of other (non-self) cells.

To successfully receive tissue from another person, the tissue type has to be the same otherwise the recipients system will see it as foreign and it will reject the donated tissue.

A major advantage of cord blood and cord tissue is that it does not have to be a 100% match for transplant, opening the door to many more useable donors.

HLA typing is done by analysing a sample of blood from the donor and one from the recipient.

Autologous:

Patient receives their own stem cells



Autologous transplantation

This type of transplantation refers to the situation where the child's own stem cells are used for themselves.

It needs to be noted that currently autologous transplantation cannot be used in the treatment of inherited disorders. The reason being that the genes that code for the inherited disorder are carried in every cell i.e. they will also be found in the person's stem cells. In that case, an allogeneic donor will be used.

THERAPIES IN CLINICAL TRIALS

A “clinical trial” is a study in human patients for an emerging therapy that has not been adopted as standard therapy.

Cord Blood

Autologous

Clinical Trials where children use their own (autologous) Cord Blood Stem Cells

- Acquired hearing loss
- Autism
- Cerebral palsy
- Encephalopathy (neonatal)
- Hypoplastic left heart syndrome (HLHS)
- Neonatal oxygen deprivation
- Stroke (pre/ peri-natal)
- Traumatic brain injury
- Type 1 diabetes

Allogeneic

Clinical Trials using donor (allogeneic, includes siblings) Cord Blood Stem Cells

- Alzheimer’s disease
- Autism
- Bronchopulmonary dysplasia
- Cartilage repair
- Cerebral palsy
- Critical limb ischaemia
- Global development delay
- Graft versus host disease (GvHD)
- HIV
- Infertility
- Intraventricular haemorrhage
- Liver cirrhosis
- Neurodegenerative disorders
- Stroke
- Sweat gland regeneration
- Type 1 diabetes

Cord Tissue

Mesenchymal Stem cells

Mesenchymal stem cells are found in abundance in **Umbilical Cord Tissue**. These cells form the various **CONNECTIVE** tissues of the body: bone, cartilage, tendons, muscle and nerves. They are found in nearly all the organs of the body but are difficult to harvest from most sites. Apart from the umbilical cord tissue, mesenchymal stem cells can be harvested from the bone marrow and adult adipose (fat tissue).

Clinical Trials using donor Mesenchymal Stem Cells from Cord Tissue

- Alzheimer’s disease
- Aplastic anaemia
- Cardiomyopathy
- Cartilage repair
- Cerebral palsy
- Connective tissue diseases
- Diabetes (Type 2)
- Erectile dysfunction
- Liver failure
- Lung injury
- Lupus
- Macular degeneration
- Multiple sclerosis
- Muscular dystrophy
- Myocardial infarction
- Osteoarthritis
- Ovarian failure
- Parkinson’s disease
- Psoriasis
- Retinitis Pigmentosa
- Rheumatoid arthritis
- Sepsis
- Spinal cord injury
- Stroke
- Traumatic optic neuropathy
- Ulcerative colitis

As clinical trials are constantly being updated, if you would like to find out more about emerging therapies using cord blood and cord tissue visit the following websites for a comprehensive list: www.clinicaltrials.gov or visit the Parents Guide to Cord Blood Foundation clinical trial search engine: www.parentsguidecordblood.org/en/trials



EXPERIMENTAL TREATMENTS

There are also many experimental therapies that are not yet in human clinical trials for a diverse range of medical conditions. These are being studied either in the laboratory with cell cultures or in animal models similar to the human disease.

