

# Diseases Treated with Stem Cells and its Potential Applications

## Diseases Treated with Stem Cells

The following is a list of some of the diseases that have been treated with cord blood and other sources of similar type of stem cells (Haematopoietic Stem Cell), such as bone marrow and peripheral blood.

STANDARD THERAPIES	Allogeneic	Autologous
<b>BLOOD CANCERS</b>		
<b>LEUKAEMIA</b>		
Acute Biphenotypic Leukaemia*	x	
Acute Lymphoblastic Leukaemia*	x	x
Acute Myelogenous Leukaemia*	x	
Acute Undifferentiated Leukaemia*	x	
Chronic Myelogenous Leukaemia*	x	
Chronic Lymphocytic Leukaemia*	x	
Juvenile Myelomonocytic Leukaemia*	x	
Juvenile Chronic Myelogenous Leukaemia	x	
<b>MYELOPROLIFERATIVE NEOPLASMS</b>		
Acute Myelofibrosis	x	
Agnogenic Myeloid Metaplasia	x	
Essential Thrombocythemia	x	
Polycythemia Vera	x	
<b>MYELOYDYSPLASTIC SYNDROMES</b>		
Refractory Anaemia*	x	
Refractory Anaemia with Excess Blasts*	x	
Refractory Anaemia with Excess Blasts in Transformation*	x	
Refractory Anaemia with Ringed Sideroblasts (Sideroblastic Anaemia)*	x	
Chronic Myelomonocytic Leukaemia*	x	
<b>OTHER BLOOD CANCERS</b>		
Multiple Myeloma	x	x
Plasma Cell Leukaemia	x	x
Waldenstrom's Macroglobulinemia	x	x
Histiocytic Neoplasms		x
<b>SOLID TUMORS</b>		
Hodgkin Lymphoma*	x	
Langerhans' Cell Histiocytosis*	x	
Neuroblastoma*	x	x
Non Hodgkin Lymphoma (Burkitt's Lymphoma)*	x	
Retinoblastoma		x
Medulloblastoma		x
Wilms Tumor		x

STANDARD THERAPIES	Allogeneic	Autologous
<b>NON MALIGNANT BLOOD DISORDERS</b>		
<b>ANAEMIAS (DEFICIENCIES OR MALFORMATIONS OF RED CELLS)</b>		
Aplastic Anaemia*	x	
Congenital Dyserythropoietic Anaemia	x	
Fanconi's Anaemia*	x	
Paroxysmal Nocturnal Hemoglobinuria	x	
<b>HEREDITARY BONE MARROW FAILURE SYNDROMES</b>		
Diamond Blackfan Syndrome*	x	x
Dyskeratosis Congenita*	x	x
Pearson's Syndrome	x	
Shwachman Diamond Syndrome*	x	x
<b>INHERITED RED CELL ABNORMALITIES</b>		
Pure Red Cell Aplasia	x	
Sickle Cell Anaemia*	x	
Beta Thalassemia Major / Cooley's Anaemia	x	
<b>INHERITED PLATELET ABNORMALITIES</b>		
Congenital Amegakaryocytosis Thrombocytopenia	x	
Glanzmann's Thrombasthenia*	x	
<b>IMMUNE DISORDERS</b>		
<b>SEVERE COMBINED IMMUNE DEFICIENCY (SCID)</b>		
Bare Lymphocyte Syndrome*	x	
Omenn Syndrome*	x	
Reticular Dysgenesis	x	
Neutrophil Actin Deficiency	x	
SCID with Adenosine Deaminase Deficiency (ADA SCID)*	x	
SCID which is X linked*	x	
SCID with absence of T & B Cells*	x	
SCID with absence of T Cells, Normal B Cells*	x	

*Diseases Treated with Stem Cells and its Potential Applications (cont'd)*

STANDARD THERAPIES	Allogeneic	Autologous
<b>NEUTROPENIAS</b>		
Kostmann Syndrome (Infantile Genetic Agranulocytosis)*	x	
Myelokathexis*	x	
<b>PHAGOCYTE DISORDERS</b>		
Chediak Higashi Syndrome*	x	
Chronic Granulomatous Disease*	x	
<b>INHERITED DISORDERS OF THE IMMUNE SYSTEM &amp; OTHER ORGANS</b>		
Cartilage Hair Hypoplasia	x	
Gunther's Disease (Congenital Erythropoietic Protoporphyrinuria)*	x	
Systemic Mastocytosis*	x	
<b>OTHER INHERITED IMMUNE SYSTEM DISORDERS</b>		
Common Variable Immunodeficiency*	x	
DiGeorge Syndrome*	x	
Evans Syndrome*	x	
Hemophagocytic Lymphohistiocytosis	x	
IKK Gamma Deficiency (NEMO Deficiency)*	x	
IPEX Syndrome*	x	
Leukocyte Adhesion Deficiency*	x	
Wiskott Aldrich Syndrome*	x	
X linked Lymphoproliferative Disease (Duncan's Syndrome)*	x	
X linked Hyper IgM Syndrome	x	
Ataxia-Telangiectasia	x	

<b>METABOLIC DISORDERS</b>		
<b>LEUKODYSTROPHY DISORDERS</b>		
Adrenoleukodystrophy*	x	
Krabbe Disease (Globoid Cell Leukodystrophy)*	x	
Metachromatic leukodystrophy*	x	
Pelizaeus-Merzbacher Disease	x	

STANDARD THERAPIES	Allogeneic	Autologous
<b>LYSOSOMAL STORAGE DISEASES</b>		
Alpha Mannosidosis*	x	
Gaucher's Disease	x	
Niemann Pick Disease	x	
Sandhoff Disease	x	
Wolman Disease*	x	
<b>MUCOPOLYSACCHARIDOSIS (MPS) STORAGE DISEASES</b>		
Hunter Syndrome*	x	
Hurler Syndrome*	x	
Maroteaux Lamy Syndrome*	x	
Mucopolidosis II (I-cell Disease)*	x	
Morquio Syndrome	x	
Sanfilippo Syndrome*	x	
Scheie Syndrome*	x	
Sly Syndrome (beta glucuronidase deficiency)	x	
<b>OTHER METABOLIC DISORDERS</b>		
Lesch-Nyhan Syndrome*	x	
Osteopetrosis*	x	
Hermansky-Pudlak Syndrome	x	

Banking cord blood does not guarantee that the cells will provide a cure or be applicable in every situation. The use of stem cells will ultimately be determined by the treating physician.

# Clinical Trials

With the advancement of stem cell<sup>+</sup> research, the potential for future use of stem cell grows. Below is a list of diseases currently under Clinical Trials. These are diseases for which stem cell<sup>+</sup> treatments appear to be beneficial, but have not been adopted as standard therapy. For some of these diseases, stem cell transplants only slow the progression of the disease, but do not produce a cure. For other diseases, stem cell treatments may help effect a cure, but further research is needed to determine the best candidate patients for stem cell therapy, the optimum stem cell dosage, the optimum method of cell delivery, etc.

For some patients, clinical research trials represent an avenue for receiving promising new therapies that would not otherwise be available. Patients with difficult to treat or currently “incurable” diseases, such as AIDS or certain types of cancer, may want to pursue participation in clinical research trials if standard therapies are not effective. Clinical research trials are sometimes lifesaving.

CLINICAL TRIALS	Allogeneic	Autologous
Alzheimer's Disease*	X	
Amyotrophic Lateral Sclerosis	X	X
Autism*		X
Brain Tumour		X
Cardiomyopathy	X	X
Cartilage repair	X	X
Cerebral palsy*		X
Cleft Palate Repair (Alveolar)		X
Compartment Syndrome (Battlefield Trauma)		X
Critical Limb Ischemia	X	X
Crohn's disease	X	X
Diabetes Type 1*	X	X
Epidermolysis Bullosa*	X	
Ewing Sarcoma	X	
Graft versus Host Disease (GvHD)	X	
Hearing Loss (acquired sensorineural)*		X
HIV*		X
Huntington's Disease		X
Hypoplastic Left Heart Syndrome*		X
Hypoxic Ischemic Encephalopathy (HIE)*		X
Ischemic Heart Disease	X	X
Ischemic Stroke		X
Kidney plus stem cell transplant	X	
Liver cirrhosis*	X	
Lupus		X
Multiple Sclerosis		X
Myocardial Infarction		X
Open cardiac surgery for congenital heart diseases		X
Ovarian Cancer (Link to clinical trials)		X
Parkinson's Disease		X
Rhabdomyosarcoma	X	
Rheumatoid Arthritis		X
Scleroderma	X	X
Spinal cord injury*	X	X
Testicular Tumour		X
Tissue Engineered Vascular Grafts for cardiac defects		X
Traumatic Brain Injury		X

\*Treated with Cord Blood

\*Stem cells mentioned here comprises of other cell lines such as Mesenchymal stem cells. The clinical trials and experimental treatments listed above may be using other lines of stem cells, and not only haematopoietic stem cells.

**References:**

- Diseases treated page. Parent's Guide to Cord Blood Foundation. <http://parentsguidecordblood.org/diseases.php>. Accessed Dec 2015.
- Stem cell treatment page. Cord Blood Registry Website. <http://www.cordblood.com/benefits-cord-blood/umbilical-cord-stem-cells/stem-cell-treatments>. Accessed Dec 2015.
- Clinical trial registry page. ClinicalTrials.gov. <http://clinicaltrials.gov>. Accessed Dec 2015.