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MKT-016-013-00

# What is Cord Blood Used for Today?

## Cord Blood Stem Cells: Current Uses

Cord blood stem cells can be used in the treatment of over 80 diseases. This doesn't mean they will be used and using them doesn't guarantee success. Each case is unique and only a doctor can determine eligibility.

#### Cancers

- Acute lymphoblastic leukemia (ALL)
- Acute myeloid leukemia (AML)
- Burkitt's lymphoma
- Chronic myeloid leukemia (CML)
- Juvenile myelomonocytic leukemia (JMML)
- Non-Hodgkin's lymphoma
- Hodgkin's lymphoma
- Lymphomatoid granulomatosis
- Myelodysplastic syndrome (MDS)
- Chronic myelomonocytic leukemia (CMML)

#### **Immunodeficiencies**

- Ataxia telangiectasia
- Chronic granulomatous disease
- DiGeorge syndrome
- IKK gamma deficiency
- Immune dysregulation polyendocrineopathy
- X-linked Mucolipidosis, Type II
- Myelokathexis X-linked immunodeficiency
- Severe combined immunodeficiency
- Adenosine deaminase deficiency
- Wiskott-Aldrich syndrome
- X-linked agammaglobulinemia
- X-linked lymphoproliferative disease
- Omenn's syndrome
- Reticular dysplasia
- Thymic dysplasia
- Leukocyte adhesion deficiency

#### **Blood Disorders**

- Sickle-cell anemia (hemoglobin SS)
- HbSC disease
- Sickle βo Thalassemia
- α-thalassemia major (hydrops fetalis)
- β-thalassemia major (Cooley's anemia)
- β-thalassemia intermedia
- E-βo thalassemia
- E-β+ thalassemia

#### Metabolic Disorders

- Adrenoleukodystrophy Gaucher's disease (infantile)
- Metachromatic leukodystrophy
- Krabbe disease (globoid cell leukodystrophy)
- Gunther disease
- Hermansky-Pudlak syndrome
- Hurler syndrome
- Hurler-Scheie syndrome
- Hunter syndrome
- Sanfilippo syndrome
- Maroteaux-Lamy syndrome
- Mucolipidosis Type II, III
- Alpha mannosidosis
- Niemann Pick Syndrome, type A and B
- Sandhoff Syndrome
- Tay-Sachs Disease
- Lesch-Nyhan disease

### Bone Marrow Failure Syndromes

- Amegakaryocytic thrombocytopenia
- Autoimmune neutropenia (severe)
- Congenital dyserythropoietic anemia
- Cyclic neutropenia
- Diamond-Blackfan anemia
- Evan's syndrome
- Fanconi anemia
- Glanzmann's disease
- Juvenile dermatomyositis
- Kostmann's syndrome
- Red cell aplasia
- Shwachman syndrome
- Severe aplastic anemia
- Congenital sideroblastic anemia
- Thrombocytopenia with absent radius (TAR syndrome)
- Dyskeratosis congenita

#### Other

- Osteopetrosis
- Langerhans cell histiocytosis
- Hemophagocytic lymphohistiocytosis

At CReATe Cord Blood we have been collecting and storing MSCs since 2006 using our exclusive Peristem™ technology.

# Scientific progress. Growing potential.

The therapeutic value of cord blood transplants today is clear, approximately 35,000 cord blood transplants have been performed worldwide.¹ Scientists around the world continue to look for new possibilities for cord blood stem cells; many are involved in an exciting area of research known as regenerative medicine. In recent years a special type of stem cell called Mesenchymal Stem Cells (MSCs), found in cord tissue, have been identified as highly capable cells with immense therapeutic potential.².³ Stem cells from cord tissue have the power to regenerate other cells, as well as, structural and connective tissue. Research is ongoing in many areas, some of which include:

- Lung cancer<sup>4</sup>
- Parkinson's disease<sup>5</sup>
- Rheumatoid arthritis<sup>6</sup>
- Sports injuries (cartilage)<sup>7</sup>
- Type 1 diabetes<sup>8</sup>
- Stroke<sup>9</sup>



# Clinical Trials

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

Diagnosis	Trial stage	Trial registry
Acquired Hearing Loss	phase 1	NCT01343394 NCT02038972
Autism	phase 2	NCT01638819
	phase 1	NCT02176317
		India
Cerebral Palsy	phase 2	NCT01147653
		NCT01072370
		NCT01988584
6 1 1 1 1 1 1		Japan
Cerebral Palsy	phase 1	Romania
		Slovakia
5		Spain
Encephalopathy (neonatal)	phase 1	UMIN000014903
Hypoplastic Left Heart Syndrome (HLHS)	phase 1	NCT01445041
		NCT01856049
		NCT01883076
Neonatal Oxygen Deprivation	phase 1	NCT00593242
		NCT01506258
		NCT01649648
		Japan
(pre-/peri-natal) Stroke	phase 1	NCT02460484
Traumatic Brain Injury	phase 1	NCT01251003
		NCT01700166
Type 1 Diabetes	phase 1 & 2	NCT00989547
		NCT00873925
		CoRD
	phase 0	ACTRN12613000186752

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

Diagnosis	Trial stage	Trial registry
Alzheimer's Disease	phase 1/2	NCT02054208
Bronchopulmonary Dysplasia	phase 1/2	NCT02381366
	phase 2	NCT01897987
Cartilage Repair	Approved by	NCT01733186
	Korean FDA	
	(Cartistem)	
Cerebral Palsy	phase 2	NCT01193660
		NCT01528436
		NCT01639404
		NCT01991145
		NCT02025972
Cerebral Palsy	phase 1	NCT02599207
		Russia
Critical Limb Ischemia	phase 1	NCT01019681
		KCT0000194
Global development delay	phase 1	NCT01769716
both autologous and allogeneic)		
Graft versus Host Disease (GvHD)	phase 1/2	KCT0000389
HIV	phase 1	NCT02140944
		Spain
nfertility	phase 1	ChiCTR-OPC-14005553
		NCT02313415
ntraventricular hemorrhage	phase 1	NCT02274428
_iver Cirrhosis	unknown	ChiCTR-TRC-14004411
Neurodegenerative disorders	phase 1	NCT02236065
Stroke	phase 1	NCT01673932
		NCT02397018
		NCT02433509
Sweat gland regeneration	unknown	NCT02304562
Type 1 Diabetes	phase 2	NCT01350219
		NCT01996228

#### Clinical Trials using donor Mesenchymal Stem Cells (MSC) from Cord Tissue

Diagnosis	Trial registry		
Alzheimer's disease	NCT01547689		
Aplastic anemia	NCT02218437		
Cardiomyopathy	NCT02635464		
	NCT02439541		
Cartilage repair	NCT02291926		
Cerebral Palsy	NCT01929434		
	ChiCTR-0NC-12003124		
Connective tissue diseases	ChiCTR-0PB-15005956		
Diabetes (Type 2)	NCT02302599		
	ChiCTR-TRC-12002868		
Erectile Dysfunction	NCT02579148		
	ChiCTR-OCN-15007041		
Liver failure	NCT01844063		
	ChiCTR-ONRC-13004581		
	NCT02223897		
Lung injury	NCT02444455		
	NCT02444858		
Lupus	ChiCTR-OPC-15006322		
Multiple Sclerosis	NCT01883661		
	NCT02587715		
	NCT02237547		
Muscular Dystrophy	NCT02285673		
Myocardial Infarction	NCT02323477		
Osteoarthritis	NCT02237846		
	NCT02580695		
Ovarian failure	NCT02644447		
Parkinson's disease	ChiCTR-0NRC-12002937		
Psoriasis	NCT02491658		
Retinitis pigmentosa	NCT01914913		
Rheumatoid Arthritis	NCT01985464		
Sepsis	ChiCTR-TRC-14005094		
Spinal Cord Injury	NCT02481440		
Spiriat cord injury	NCT02237547		
Stroke	NCT02378974		
	ChiCTR-ONRC-12002929		
Traumatic optic neuropathy	ChiCTR-TRC-14005093		
Ulcerative colitis	NCT02442037		

Tables researched by Frances Verter, PhD, Alexey Bersenev, MD PhD, and Pedro Silva Couto, MSc ©2014-2016. Sources of trial data are ClinicalTrials.gov and WHO International Clinical Trials Registry Platform (ICTRP) including: Chinese Clinical Trial Registry (ChiCTR), Korea's Clinical Research Information Service (CRIS), Japan's University hospital Medical Information Network (UMIN), Australian New Zealand Clinical Trials Registry (ANZCTR), etc., plus private communication from lead investigators.

For a link to any of these clinical trials, please visit http://parentsguidecordblood.org/en/trials.

