

Diseases Being Treated with Stem Cells

The information contained in the preceding charts was adapted from: Parent's Guide to Cord Blood. Diseases treated. <http://parentsguidecordblood.org/en/diseases>. Updated November 1st, 2020.

APPROVED THERAPIES

These are diseases for which hematopoietic (blood-forming) stem cell transplants (HSCT) are a medically accepted treatment. For some diseases, they are the primary form of therapy. For other diseases, they are only employed when other therapies have failed or the disease is very aggressive.

LEUKEMIAS

Acute Leukemia

- Acute Lymphoblastic Leukemia (ALL)
- Acute Myelogenous Leukemia (AML)
- Acute Biphenotypic Leukemia
- Acute Undifferentiated Leukemia

Chronic Leukemia

- Chronic Myelogenous Leukemia (CML)
- Chronic Lymphocytic Leukemia (CLL)
- Juvenile Chronic Myelogenous Leukemia (JCML)
- Juvenile Myelomonocytic Leukemia (JMML)

MYELODYPLASTIC SYNDROMES

- Refractory Anemia (RA)
- Refractory Anemia with Ringed Sideroblasts (RARS)
- Refractory Anemia with Excess Blasts (RAEB)
- Refractory Anemia with Excess Blasts in Transformation (RAEB-T)
- Chronic Myelomonocytic Leukemia (CMML)

LYMPHOMAS

- Hodgkin Lymphoma
- Non-Hodgkin Lymphoma (Burkitt's Lymphoma)

INHERITED RED CELL (ERYTHROCYTE) ABNORMALITIES

- Beta Thalassemia Major (Cooley's Anemia)
- Blackfan-Diamond Anemia
- Pure Red Cell Aplasia
- Sickle Cell Disease

OTHER DISORDERS OF BLOOD CELL PROLIFERATION

Inherited Platelet Abnormalities

- Amegakaryocytosis/Congenital Thrombocytopenia
- Glanzmann Thrombasthenia

Myeloproliferative Disorders

- Acute Myelofibrosis

- Agnogenic Myeloid Metaplasia (Myelofibrosis)
- Polycythemia Vera
- Essential Thrombocythemia

Inherited Immune System Disorders— Severe Combined Immunodeficiency (SCID)

- SCID with Adenosine Deaminase Deficiency (ADA-SCID)
- SCID, X-linked
- SCID with Absence of T & B Cells
- SCID with Absence of T Cells, Normal B Cells
- Omenn Syndrome

Inherited Immune System Disorders— Neutropenias

- Kostmann Syndrome
- Myelokathexis

Other Inherited Immune System Disorders

- Ataxia-Telangiectasia
- Bare Lymphocyte Syndrome
- Common Variable Immunodeficiency
- DiGeorge Syndrome
- Leukocyte Adhesion Deficiency
- Lymphoproliferative Disorders (LPD)
- Lymphoproliferative Disorder, X-linked (Epstein-Barr Virus Susceptibility)
- Wiskott-Aldrich Syndrome

Phagocyte Disorders

- Chediak-Higashi Syndrome
- Chronic Granulomatous Disease
- Neutrophil Actin Deficiency

OTHER CANCERS

NOT ORIGINATING IN THE BLOOD STREAM

- Neuroblastoma
- Retinoblastoma
- Medulloblastoma

TRANSPLANTS FOR INHERITED DISORDERS AFFECTING THE IMMUNE SYSTEM AND OTHER ORGANS

- Cartilage-Hair Hypoplasia
- Gunther's Disease (Erythropoietic Porphyria)
- Hermansky-Pudlak Syndrome
- Pearson's Syndrome
- Shwachman-Diamond Syndrome

- Systemic Mastocytosis

TRANSPLANTS FOR INHERITED METABOLIC DISORDERS

Mucopolysaccharide (MPS) Storage Diseases

- Mucopolysaccharidosis (MPS)
- Hurler's Syndrome (MPS-IH)
- Scheie Syndrome (MPS-IS)
- Hunter's Syndrome (MPS-II)
- Sanfilippo Syndrome (MPS-III)
- Morquio Syndrome (MPS-IV)
- Maroteaux-Lamy Syndrome (MPS-VI)
- Sly Syndrome, Beta-Glucuronidase Deficiency (MPS-VII)
- Mucopolipidosis II (I-Cell Disease)

Leukodystrophy Disorders

- Adrenoleukodystrophy (ALD) / Adrenomyeloneuropathy (AMN)
- Krabbe Disease (Globoid Cell Leukodystrophy)
- Metachromatic Leukodystrophy
- Pelizaeus-Merzbacher Disease

Lysosomal Storage Diseases

- Niemann-Pick Disease
- Sandhoff Disease

Anemias

- Severe Aplastic Anemia
- Congenital Dyserythropoietic Anemia
- Fanconi Anemia
- Paroxysmal Nocturnal Hemoglobinuria (PNH)
- Pure Red Cell Aplasia
- Reticular Dysgenesis

PLASMA CELL NEOPLASMS

- Multiple Myeloma
- Plasma Cell Leukemia
- Waldenstrom's Macroglobulinemia
- Wolman Disease

Other Inherited Disorders

- Lesch-Nyhan Syndrome
- Osteopetrosis



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INVESTIGATIONAL THERAPIES

These are diseases for which stem cell treatments are being investigated by clinicians as potential therapies but have not been approved by any regulatory body such as the FDA. Clinical trials are evaluating the potential of these therapies.

AUTOIMMUNE DISEASES

- Lupus
- Crohn's Disease
- Rheumatoid Arthritis
- Multiple Sclerosis (MS)
- GVHD (Graft vs. Host Disease)
- Scleroderma
- Systemic Sclerosis
- Ulcerative Colitis
- Alopecia Areata
- Eczema
- Psoriasis
- Amyotrophic Lateral Sclerosis (ALS)

NEUROLOGICAL CONDITIONS AND INJURY

- Cerebral Palsy
- Autism
- Spinal Cord Injury
- Encephalopathy
- Acquired Hearing Loss
- Stroke Recovery
- Traumatic Brain Injury
- Alzheimer's Disease
- Global Developmental Delay
- Intraventricular Hemorrhage
- Parkinson's Disease

DIABETES AND COMPLICATIONS

- Diabetes Mellitus Type I
- Diabetes Mellitus Type II
- Diabetic Foot Ulcer
- Diabetic Peripheral Neuropathy

GENETIC RELATED AND METABOLIC DISEASE

- Aging Frailty
- Severe Combined Immunodeficiency (SCID)
- Duchenne Muscular Dystrophy
- Epidermolysis Bullosa
- Hereditary Ataxia
- Lysosomal Storage Diseases
- Metabolic Syndrome
- Spinal Muscular Atrophy
- Tay-Sachs Disease

VARIOUS DISEASES AND CONDITIONS

- Acute Respiratory Distress Syndrome
- Bronchopulmonary Dysplasia
- Erectile Dysfunction
- Eye Diseases
- Fistula
- HIV
- Kidney Failure
- Liver Cirrhosis/Failure
- Peyronie's Disease
- Premature Ovarian Failure
- Uterine Scars
- Wounds

CARDIOVASCULAR INDICATIONS

- Acute Myocardial Infarction
- Heart Failure
- Peripheral Artery Disease
- Critical Limb Ischemia
- Cardiomyopathy
- Surgery for Congenital Heart Defects

ORTHOPEDIC AND SURGICAL INDICATIONS

- Anklosing Spodylitis
- Cartilage Injuries
- Cleft Palate Repair
- Non-Union Fracture
- Osteoarthritis
- Osteochondral Lesions
- Spinal Fusion Surgery

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