

Diseases Being Treated with Stem Cells*

Standard Therapies—These are diseases for which hematopoietic (blood-forming) stem cell transplants (HSCT) are a standard treatment. For some diseases, they are the only therapy, and in other diseases, they are only employed when front-line therapies have failed or the disease is very aggressive.

<p>Leukemias</p> <p>Acute Leukemia</p> <ul style="list-style-type: none"> Acute Lymphoblastic Leukemia (ALL) Acute Myelogenous Leukemia (AML) Acute Biphenotypic Leukemia Acute Undifferentiated Leukemia <p>Chronic Leukemia</p> <ul style="list-style-type: none"> Chronic Myelogenous Leukemia (CML) Chronic Lymphocytic Leukemia (CLL) Juvenile Chronic Myelogenous Leukemia (JCML) Juvenile Myelomonocytic Leukemia (JMML) <p>Myelodysplastic Syndromes</p> <ul style="list-style-type: none"> 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) <p>Lymphomas</p> <ul style="list-style-type: none"> Hodgkin's Lymphoma Non-Hodgkin's Lymphoma (Burkitt's Lymphoma) <p>Inherited Red Cell (Erythrocyte) Abnormalities</p> <ul style="list-style-type: none"> Beta Thalassemia Major (Cooley's Anemia) Blackfan-Diamond Anemia Pure Red Cell Aplasia Sickle Cell Disease <p>Other Disorders of Blood Cell Proliferation</p> <p>Anemias</p> <ul style="list-style-type: none"> Severe Aplastic Anemia Congenital Dyserythropoietic Anemia Fanconi Anemia Paroxysmal Nocturnal Hemoglobinuria (PNH) Pure Red Cell Aplasia <p>Inherited Platelet Abnormalities</p> <ul style="list-style-type: none"> Amegakaryocytosis/Congenital Thrombocytopenia Glanzmann Thrombasthenia 	<p>Myeloproliferative Disorders</p> <ul style="list-style-type: none"> Acute Myelofibrosis Agnogenic Myeloid Metaplasia (Myelofibrosis) Polycythemia Vera Essential Thrombocythemia <p>Inherited Immune System Disorders—Severe Combined Immunodeficiency (SCID)</p> <ul style="list-style-type: none"> 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 <p>Inherited Immune System Disorders—Neutropenias</p> <ul style="list-style-type: none"> Kostmann Syndrome Myelokathexis <p>Other Inherited Immune System Disorders</p> <ul style="list-style-type: none"> 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 <p>Phagocyte Disorders</p> <ul style="list-style-type: none"> Chediak-Higashi Syndrome Chronic Granulomatous Disease Neutrophil Actin Deficiency Reticular Dysgenesis <p>Cancers in the Bone Marrow (Plasma Cell Disorders)</p> <ul style="list-style-type: none"> Multiple Myeloma Plasma Cell Leukemia Waldenstrom Macroglobulinemia 	<p>Other Cancers (not originating in the blood stream)</p> <ul style="list-style-type: none"> Neuroblastoma Retinoblastoma <p>Transplants for Inherited Disorders Affecting the Immune System and Other Organs</p> <ul style="list-style-type: none"> Cartilage-Hair Hypoplasia Gunther's Disease (Erythropoietic Porphyrin) Hermansky-Pudlak Syndrome Pearson's Syndrome Shwachman-Diamond Syndrome Systemic Mastocytosis <p>Transplants for Inherited Metabolic Disorders</p> <p>Mucopolysaccharide (MPS) Storage Diseases</p> <ul style="list-style-type: none"> 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) <p>Leukodystrophy Disorders</p> <ul style="list-style-type: none"> Adrenoleukodystrophy (ALD)/Adrenomyeloneuropathy (AMN) Krabbe Disease (Globoid Cell Leukodystrophy) Metachromatic Leukodystrophy <p>Lysosomal Storage Diseases</p> <ul style="list-style-type: none"> Gaucher Disease Niemann-Pick Disease Sandhoff Disease Tay-Sachs Disease Wolman Disease <p>Other Inherited Disorders</p> <ul style="list-style-type: none"> Lesch-Nyhan Syndrome Osteopetrosis
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Therapies in Clinical Trials†—These are diseases for which stem cell treatments have been shown to be potentially beneficial, but additional studies are required before they can be used as standard therapy.

<p>Autoimmune Diseases</p> <ul style="list-style-type: none"> Diabetes, Type 1 Lupus Crohn's Disease <p>Transplants for Diseases of the Central Nervous System</p> <ul style="list-style-type: none"> Multiple Sclerosis (MS) Cerebral Palsy 	<p>Transplants for Disorders of Cell Proliferation</p> <p>Histiocytic Disorders</p> <ul style="list-style-type: none"> Familial Erythrophagocytic Lymphohistiocytosis Hemophagocytosis Langerhans Cell Histiocytosis (LCH; formerly called Histiocytosis-X) <p>Epidermolysis Bullosa</p>	<p>Gene Therapy</p> <ul style="list-style-type: none"> Glanzmann Thrombasthenia Severe Combined Immunodeficiency (SCID) SCID with Adenosine Deaminase Deficiency (ADA-SCID) SCID, X-linked <p>Cellular Cardiomyoplasty</p> <ul style="list-style-type: none"> This has been tested for various cardiac conditions <p>Transplants for Cancerous Tumors</p> <ul style="list-style-type: none"> Breast Cancer Ewing Sarcoma Renal Cell Carcinoma
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Experimental Treatments‡—These are diseases for which stem cell treatments are being actively investigated.

<p>Autoimmune Diseases</p> <ul style="list-style-type: none"> Arthritis, Juvenile Arthritis, Rheumatoid Crohn's Disease Evans Syndrome Juvenile Dermatomyositis Scleroderma <p>Gene Therapy (ie, transplanting genetically altered stem cells)</p> <ul style="list-style-type: none"> Fanconi Anemia Metabolic Disorders (eg, Leukodystrophy Diseases, Storage Disorders) Parkinson's Disease 	<p>Nerve Cell Repair</p> <p>Diseases of the Central Nervous System</p> <ul style="list-style-type: none"> Amyotrophic Lateral Sclerosis (ALS or Lou Gehrig's Disease) Alzheimer's Disease Huntington's Disease Parkinson's Disease <p>Traumatic Injury</p> <ul style="list-style-type: none"> Spinal cord injury Stroke recovery 	<p>Organ Repair</p> <p>Kidney</p> <ul style="list-style-type: none"> Combined transplant of kidney plus hematopoietic stem cells Growth of renal cells from adult stem cells <p>Liver</p> <ul style="list-style-type: none"> Growth of liver cells from hematopoietic stem cells <p>Lungs</p> <ul style="list-style-type: none"> Growth of airway epithelia from stem cells in bone marrow
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*The information contained in the preceding charts was adapted from: Parent's Guide to Cord Blood. Diseases treated by blood stem cells. <http://parentsguidecordblood.org/content/usa/medical/diseases.shtml?navid=22>. Updated November 27, 2009. Accessed September 20, 2013.

†Uses in "Therapies in Clinical Trials" and "Experimental Treatments" sections are not consistent with current labeling, are investigational and may never become standard therapy.



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