### 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.

**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 (JMMML)

**Myelodysplastic 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’s Lymphoma
- Non-Hodgkin’s 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**
- Severe Aplastic Anemia
- Congenital Dyserythropoietic Anemia
- Fanconi Anemia
- Paroxysmal Nocturnal Hemoglobinuria (PNH)
- Pure Red Cell Aplasia

**Inherited Platelet Abnormalities**
- Amegakaryocytosis/Congenital Thrombocytopenia
- Glanzmann Thrombasthenia

**Myeloproliferative Disorders**
- Acute Myelofibrosis
- Agnogenic Myeloid Metaplasia (Myeloidfibrosis)
- Polycythemia Vera
- Essential Thrombocytosis

**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 (CVID)
- DiGeorge Syndrome
- Leukocyte Adhesion Deficiency
- Lymphoproliferative Disorders, X-linked
- Lymphoproliferative Disorder, X-linked (Epstein-Barr Virus Susceptibility)

**Phagocyte Disorders**
- Chediak-Higashi Syndrome
- Chronic Granulomatous Disease
- Chediak-Higashi Syndrome
- DiGeorge Syndrome
- Leukocyte Adhesion Deficiency
- Lymphoproliferative Disorder, X-linked

**Cancers in the Bone Marrow (Plasma Cell Disorders)**
- Multiple Myeloma
- Plasma Cell Leukemia
- Waldenstrom Macroglobulinemia

**Other Cancers (not originating in the blood stream)**
- Neuroblastoma
- Retinoblastoma

**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)
  - Mucolipidosis II (I-Cell Disease)

**Leukodystrophy Disorders**
- Adrenoleukodystrophy (ALD/Adenomyeloneuropathy (AMN))
- Krabbe Disease (Globoid Cell Leukodystrophy)
- Metachromatic Leukodystrophy

**Lysosomal Storage Diseases**
- Gaucher Disease
- Niemann-Pick Disease
- Sandhoff Disease
- Tay-Sachs Disease
- Wolman Disease

**Other Inherited Disorders**
- Lesch-Nyhan Syndrome
- Osteopetrosis

### Therapies in Clinical Trials—These are diseases for which stem cell treatments are being actively investigated.

**Autoimmune Diseases**
- Diabetes, Type 1
- Lupus
- Crohn’s Disease

**Transplants for Diseases of the Central Nervous System**
- Multiple Sclerosis (MS)
- Cerebral Palsy

**Transplants for Disorders of Cell Proliferation**
- Familial Erythrophagocytic Lymphohistiocytosis
- Hemophagocytosis
- Langerhans Cell Histiocytosis (LCH; formerly called Histiocytosis-X)

**Epidermolysis Bullosa**
- Multiple Myeloma
- Plasma Cell Leukemia
- Waldenstrom Macroglobulinemia

**Organ Repair**
- Kidney
  - Combined transplant of kidney plus hematopoietic stem cells
  - Growth of renal cells from adult stem cells
- Liver
  - Growth of liver cells from hematopoietic stem cells
- Lungs
  - Growth of airway epithelia from stem cells in bone marrow

### Experimental Treatments—These are diseases for which stem cell treatments are being actively investigated.

**Autoimmune Diseases**
- Arthritis, Juvenile
- Arthritis, Rheumatoid
- Crohn’s Disease
- Evans Syndrome
- Juvenile Dermatomyositis
- Scleroderma

**Gene Therapy**
- Fanconi Anemia
- Metabolic Disorders (eg, Leukodystrophy Diseases, Storage Disorders)
- Parkinson’s Disease

**Nerve Cell Repair**
- Amyotrophic Lateral Sclerosis (ALS or Lou Gehrig’s Disease)
- Alzheimer’s Disease
- Huntington’s Disease
- Parkinson’s Disease

**Gene Therapy**
- Glanzmann Thrombasthenia
- Severe Combined Immunodeficiency (SCID)
- SCID with Adenosine Deaminase Deficiency (ADA-SCID)
- SCID, X-linked

**Cellular Cardiomyoplasty**
- This has been tested for various cardiac conditions

**Transplants for Cancerous Tumors**
- Breast Cancer
- Ewing Sarcoma
- Renal Cell Carcinoma

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2Uses in “Therapies in Clinical Trials” and “Experimental Treatments” sections are not consistent with current labeling, are investigational and may never become standard therapy.