APPENDIX I

Diseases Treated by Blood Stem Cells

Hematopoietic stem cells are capable of evolving into all the specific cell types in the blood and immune system. They can be found in people of all ages. The three sources of hematopoietic stem cells which are routinely used for medical treatments are:

1. the bone marrow of an adult person
2. the peripheral blood of an adult person
3. the umbilical cord blood of a newborn baby

When a patient requires a Hematopoietic Stem Cell Transplant (HSCT), the treating physician will decide which source of stem cells to use. This will depend on several factors, including but not limited to: the degree of match between donor and patient (sometimes the donor and patient are one and the same person), the expected speed of engraftment, and the amount of time available to search for a perfectly matching donor.

Standard Therapies

These are diseases for which Hematopoietic 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. Most of the diseases for which HSCT is a standard treatment are disorders of blood cell lineage, ranging from the stem cells in the bone marrow down to specific cell types in the blood.

Leukemias

(Leukemia is a cancer of the blood immune system, whose cells are called leukocytes or white cells)

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)

Myelodysplastic Syndromes
(Myelodysplasia is sometimes called pre-leukemia)

- 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
(Lymphoma is a cancer of the leukocytes that circulate in the blood and lymph vessels)

- Hodgkin's Lymphoma
- Non-Hodgkin's Lymphoma Burkitt's Lymphoma

Inherited Red Cell (Erythrocyte) Abnormalities
(Red cells contain hemoglobin and carry oxygen to the body)

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

Other Disorders of Blood Cell Proliferation

Anemias (Anemias are deficiencies or malformations of red cells)

- severe Aplastic Anemia
- Congenital Dyserythropoietic Anemia
- Fanconi Anemia (Note: the first cord blood transplant in 1988 was for this disease)
- Paroxysmal Nocturnal Hemoglobinuria (PNH)
- Pure Red Cell Aplasia

Inherited Platelet Abnormalities (Platelets are small blood cells needed for clotting)

- 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 which is 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

Inherited Immune System Disorders - Other

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

Phagocyte Disorders (Phagocytes are immune system cells that can engulf and kill foreign organisms)

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

Cancers in the bone marrow (Plasma Cell Disorders)

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

Other cancers (Not originating in the blood system)

- Neuroblastoma

Therapies in Clinical Trials

These are diseases for which stem cell treatments have been shown 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 be curative, but the optimum dosage and usage of the stem cells is still under investigation.

Transplants for Cancerous Tumors

- Breast Cancer
- Other Gynecological Cancers
- Ewing's sarcoma
- Renal cell carcinoma

Transplants for Inherited Disorders affecting the Immune System & 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

Mucopolysaccharidoses (MPS) Storage Diseases

- Mucopolysaccharidoses (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)/Adrenomyeloneuropathy (AMN)
- Krabbe Disease (Globoid Cell Leukodystrophy)
- Metachromatic Leukodystrophy

Lysosomal Storage Diseases

- Gaucher Disease
- Niemann-Pick Disease
- Sandhoff Disease
- Tay-Sachs Disease
- Wolman Disease

Inherited Disorders - Other

- Lesch-Nyhan Syndrome
- Osteopetrosis

Transplants for Disorders of Cell Proliferation

Histiocytic Disorders

- Familial Erythrophagocytic Lymphohistiocytosis
- Hemophagocytosis
- Langerhans Cell Histiocytosis (LCH; formerly called Histiocytosis-X)

Transplants for diseases of the Central Nervous System

- Multiple Sclerosis (MS)
- Krabbe's Disease

Gene Therapy (ie: Transplanting genetically altered stem cells)

- Glanzmann Thrombasthenia
- Severe Combined Immunodeficiency (SCID)
  SCID with Adenosine Deaminase Deficiency (ADA-SCID)
  SCID which is X-linked

Diabetic and Atherosclerotic Ulcers (ie: Chronic Limb Ischemia of diverse etiology-Atherosclerosis, Diabetes Mellitus, Burger's disease)

Cellular Cardiomyoplasty (ie: Strengthening damaged heart muscle by infusing stem cells or promoting their growth)

- autologous stem cell transplantation
- drug-enhanced stem cell proliferation

Experimental Treatments
Auto-Immune Diseases

- Arthritis, Juvenile
- Arthritis, Rheumatoid
- Crohn's Disease
- Diabetes, Type I, II
- Evan Syndrome

- Juvenile Dermatomyositis
- Systemic Lupus Erythematosus
- Alopecia Universalis

Gene Therapy (ie: Transplanting genetically altered stem cells)

- Fanconi's Anemia
- Metabolic Disorders (Leukodystrophy Diseases, Storage Disorders, etc.)
- Parkinson's Disease

Nerve cell repair

Diseases of the Central Nervous System

- Amyotrophic Lateral Sclerosis (ALS, or "Lou Gehrig's disease")
- Alzheimer's Disease
- Huntington's Disease
- Parkinson's Disease

Traumatic injury

- Spinal cord injury
- Stroke recovery

Organ repair

Eyes

- Limbal corneal transplantation
- Retina regeneration

Skin

- Diabetic and Atherosclerotic ulcers

Kidney

- Combined transplant of kidney plus hematopoietic stem cells
- Growth of renal cells from hematopoietic stem cells

Liver

- Growth of liver cells from hematopoietic stem cells
- Cirrhosis

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