CURRENT APPLICATIONS OF ADULT STEM CELLS FOR HUMAN PATIENTS (not a complete listing) (sample references)

ADULT STEM CELLS--HEMATOPOIETIC REPLACEMENT

CANCERS

BRAIN TUMORS—medulloblastoma and glioma
Dunkel, IJ; “High-dose chemotherapy with autologous stem cell rescue for malignant brain tumors”; Cancer Invest. 18, 492-493; 2000.
Finlay, JL; “The role of high-dose chemotherapy and stem cell rescue in the treatment of malignant brain tumors: a reappraisal”; Pediatr. Transplant 3 Suppl. 1, 87-95; 1999

RETINOBLASTOMA
Hertzberg H et al.; “Recurrent disseminated retinoblastoma in a 7-year-old girl treated successfully by high-dose chemotherapy and CD34-selected autologous peripheral blood stem cell transplantation”; Bone Marrow Transplant 27(6), 653-655; March 2001
Dunkel IJ et al.; “Successful treatment of metastatic retinoblastoma”; Cancer 89, 2117-2121; Nov 15 2000

OVARIAN CANCER
Schilder, RJ and Shea, TC; “Multiple cycles of high-dose chemotherapy for ovarian cancer”; Semin. Oncol. 25, 349-355; June 1998

MERKEL CELL CARCINOMA

TESTICULAR CANCER
Hanazawa, K et al.; “Collection of peripheral blood stem cells with granulocyte-colony-stimulating factor alone in testicular cancer patients”; Int. J. Urol. 7, 77-82; March 2000.

LYMPHOMA
Tabata M et al.; “Peripheral blood stem cell transplantation in patients over 65 years old with malignant lymphoma--possibility of early completion of chemotherapy and improvement of performance status”; Intern Med 40, 471-474; June 2001
**DoNoHarm: The Coalition of Americans for Research Ethics**


Koizumi M et al.; “Successful treatment of intravascular malignant lymphomatosis with high-dose chemotherapy and autologous peripheral blood stem cell transplantation”; Bone Marrow Transplant 27, 1101-1103; May 2001

**ACUTE LYMPHOBLASTIC LEUKEMIA**

Ohnuma K et al.; “Cord blood transplantation from HLA-mismatched unrelated donors as a treatment for children with haematological malignancies”; Br J Haematol 112(4), 981-987; March 2001

Marco F et al.; “High Survival Rate in Infant Acute Leukemia Treated With Early High-Dose Chemotherapy and Stem-Cell Support”; J Clin Oncol 18, 3256-3261; Sept. 15 2000

**ACUTE MYELOGENOUS LEUKEMIA**

Ohnema K et al.; “Cord blood transplantation from HLA-mismatched unrelated donors as a treatment for children with haematological malignancies”; Br J Haematol 112(4), 981-987; March 2001

Gorin NC et al.; “Feasibility and recent improvement of autologous stem cell transplantation for acute myelocytic leukaemia in patients over 60 years of age: importance of the source of stem cells”; Br. J. Haematol. 110, 887-893; Sept 2000

Bruserud O et al.; “New strategies in the treatment of acute myelogenous leukemia: mobilization and transplantation of autologous peripheral blood stem cells in adult patients”; Stem Cells 18, 343-351; 2000

**CHRONIC MYELOGENOUS LEUKEMIA**

Ohnuna K et al.; “Cord blood transplantation from HLA-mismatched unrelated donors as a treatment for children with haematological malignancies”; Br J Haematol 112(4), 981-987; March 2001

**JUVENILE MYELOMONOCYTIC LEUKEMIA**

Ohnuma K et al.; “Cord blood transplantation from HLA-mismatched unrelated donors as a treatment for children with haematological malignancies”; Br J Haematol 112(4), 981-987; March 2001

**ANGIOIMMUNOBLASTIC LYMPHADENOPATHY with DYSPROTEINEMIA**


**MULTIPLE MYELOMA**


Vesole, DH et al.; “High-Dose Melphalan With Autotransplantation for Refractory Multiple Myeloma: Results of a Southwest Oncology Group Phase II Trial”; J Clin Oncol 17, 2173-2179; July 1999.

**MYELODYSPLASIA**

Ohnuna K et al.; “Cord blood transplantation from HLA-mismatched unrelated donors as a treatment for children with haematological malignancies”; Br J Haematol 112(4), 981-987; March 2001

Bensinger WI et al.; “Transplantation of bone marrow as compared with peripheral-blood cells from HLA-identical relatives in patients with hematologic cancers”; New England Journal of Medicine 344, 175-181; Jan 18 2001

www.stemcellresearch.org
BREAST CANCER
Stiff P et al.; “Autologous transplantation of ex vivo expanded bone marrow cells grown from small aliquots after high-dose chemotherapy for breast cancer”; Blood 95, 2169-2174; March 15, 2000

NEUROBLASTOMA

NON-HODGKIN’S LYMPHOMA
Tabata M et al.; “Peripheral blood stem cell transplantation in patients over 65 years old with malignant lymphoma--possibility of early completion of chemotherapy and improvement of performance status”; Intern Med 40, 471-474; June 2001

HODGKIN’S LYMPHOMA

RENAL CELL CARCINOMA

VARIOUS SOLID TUMORS
Nieboer P et al.; “Long-term haematological recovery following high-dose chemotherapy with autologous bone marrow transplantation or peripheral stem cell transplantation in patients with solid tumours”; Bone Marrow Transplant 27, 959-966; May 2001
Lafay-Cousin L et al.; “High-dose thiotepa and hematopoietic stem cell transplantation in pediatric malignant mesenchymal tumors: a phase II study”; Bone Marrow Transplant 26, 627-632; Sept. 2000


Schilder, RJ et al.; “Phase I trial of multiple cycles of high-dose chemotherapy supported by autologous peripheral-blood stem cells”; J. Clin. Oncol. 17, 2198-2207; July 1999
DoNoHarm: The Coalition of Americans for Research Ethics

SOFT TISSUE SARCOMA

HEMOPHAGOCYTIC LYMPHOHISTIOCYTOSIS
Matthes-Martin S et al.; “Successful stem cell transplantation following orthotopic liver transplantation from the same haploidentical family donor in a girl with hemophagocytic lymphohistiocytosis”; Blood 96, 3997-3999; Dec 1, 2000

WALDENSTROM’S MACROGLOBULINEMIA
Anagnostopoulos A et al.; “High-dose chemotherapy followed by stem cell transplantation in patients with resistant Waldenstrom's macroglobulinemia”; Bone Marrow Transplant 27, 1027-1029; May 2001

POEMS SYNDROME (OSTEOSCLEROTIC MYELOMA)
Dispenzieri A et al., Peripheral blood stem cell transplantation in 16 patients with POEMS syndrome, and a review of the literature, Blood 104, 3400-3407, 15 November 2004

ADULT STEM CELLS— IMMUNE SYSTEM REPLACEMENT

AUTOIMMUNE DISEASES

SCLEROMYXEDEMA

SCLERODERMA
Burt RK et al., “Induction of tolerance in autoimmune diseases by hematopoietic stem cell transplantation: getting closer to a cure?”, Blood 99, 768-784, 1 February 2002
Burt, RK and Traynor, AE; “Hematopoietic Stem Cell Transplantation: A New Therapy for Autoimmune Disease”; Stem Cells17, 366-372; 1999

MULTIPLE SCLEROSIS
Burt RK et al., “Induction of tolerance in autoimmune diseases by hematopoietic stem cell transplantation: getting closer to a cure?”, Blood 99, 768-784, 1 February 2002
Rabusin M et al.; “Immunoaclation followed by autologous hematopoietic stem cell infusion for the treatment of severe autoimmune disease”; Haematologica 85(11 Suppl), 81-85; Nov. 2000
Burt, RK and Traynor, AE; “Hematopoietic Stem Cell Transplantation: A New Therapy for Autoimmune Disease”; Stem Cells17, 366-372; 1999

CROHN’S DISEASE
Kreisel W et al., Complete remission of Crohn’s disease after high-dose cyclophosphamide and autologous stem cell transplantation, Bone Marrow Transplantation 32, 337-340, 2003
Rabusin M et al.; “Immunoablation followed by autologous hematopoietic stem cell infusion for the treatment of severe autoimmune disease”; Haematologica 85(11 Suppl), 81-85; Nov. 2000
Hawkey CJ et al.; “Stem cell transplantation for inflammatory bowel disease: practical and ethical issues”; Gut 46, 869-872; June 2000

RHEUMATOID ARTHRITIS
Burt RK et al., “Induction of tolerance in autoimmune diseases by hematopoietic stem cell transplantation: getting closer to a cure?”, Blood 99, 768-784, 1 February 2002
Burt RK et al., “Induction of remission of severe and refractory rheumatoid arthritis by allogeneic mixed chimerism”, Arthritis & Rheumatism 50, 2466-2470, August 2004
Verburg RJ et al.; “High-dose chemotherapy and autologous hematopoietic stem cell transplantation in patients with rheumatoid arthritis: results of an open study to assess feasibility, safety, and efficacy”; Arthritis Rheum 44(4), 754-760; April 2001
Rabusin M et al.; “Immunoablation followed by autologous hematopoietic stem cell infusion for the treatment of severe autoimmune disease”; Haematologica 85(11 Suppl), 81-85; Nov. 2000
Burt, RK and Traynor, AE; “Hematopoietic Stem Cell Transplantation: A New Therapy for Autoimmune Disease”; Stem Cells17, 366-372; 1999

JUVENILE ARTHRITIS
Rabusin M et al.; “Immunoablation followed by autologous hematopoietic stem cell infusion for the treatment of severe autoimmune disease”; Haematologica 85(11 Suppl), 81-85; Nov. 2000
Burt, RK and Traynor, AE; “Hematopoietic Stem Cell Transplantation: A New Therapy for Autoimmune Disease”; Stem Cells17, 366-372; 1999

SYSTEMIC LUPUS
Burt RK et al., “Induction of tolerance in autoimmune diseases by hematopoietic stem cell transplantation: getting closer to a cure?”, Blood 99, 768-784, 1 February 2002
Wulffraat NM et al.; “Prolonged remission without treatment after autologous stem cell transplantation for refractory childhood systemic lupus erythematosus”; Arthritis Rheum 44(3), 728-731; March 2001
Rosen O et al.; “Autologous stem-cell transplantation in refractory autoimmune diseases after in vivo immunoablation and ex vivo depletion of mononuclear cells”; Arthritis res. 2, 327-336; 2000
Traynor AE et al.; “Treatment of severe systemic lupus erythematosus with high-dose chemotherapy and haemopoietic stem-cell transplantation: a phase I study”; Lancet 356, 701-707; August 26, 2000
Burt, RK and Traynor, AE; “Hematopoietic Stem Cell Transplantation: A New Therapy for Autoimmune Disease”; Stem Cells17, 366-372; 1999
Traynor A and Burt RK; “Haematopoietic stem cell transplantation for active systemic lupus erythematosus”; Rheumatology 38, 767-772; August 1999
Martini A et al.; “Marked and sustained improvement 2 years after autologous stem cell transplant in a girl with system sclerosis”; Rheumatology 38, 773; August 1999

POLYCHONDRTIS

www.stemcellresearch.org
DoNoHarm: The Coalition of Americans for Research Ethics
Rosen O et al.; “Autologous stem-cell transplantation in refractory autoimmune diseases after in vivo immunoablation and ex vivo depletion of mononuclear cells”; Arthritis res. 2, 327-336; 2000

SYSTEMIC VASCULITIS
Rabusin M et al.; “Immunoablation followed by autologous hematopoietic stem cell infusion for the treatment of severe autoimmune disease”; Haematologica 85(11 Suppl), 81-85; Nov. 2000

SJOGREN’S SYNDROME
Rabusin M et al.; “Immunoablation followed by autologous hematopoietic stem cell infusion for the treatment of severe autoimmune disease”; Haematologica 85(11 Suppl), 81-85; Nov. 2000

BEHCET’S DISEASE
Rabusin M et al.; “Immunoablation followed by autologous hematopoietic stem cell infusion for the treatment of severe autoimmune disease”; Haematologica 85(11 Suppl), 81-85; Nov. 2000

MYASTHENIA
Rabusin M et al.; “Immunoablation followed by autologous hematopoietic stem cell infusion for the treatment of severe autoimmune disease”; Haematologica 85(11 Suppl), 81-85; Nov. 2000

AUTOIMMUNE CYTOPENIA
Rabusin M et al.; “Immunoablation followed by autologous hematopoietic stem cell infusion for the treatment of severe autoimmune disease”; Haematologica 85(11 Suppl), 81-85; Nov. 2000
Papadaki HA et al.; “Assessment of bone marrow stem cell reserve and function and stromal cell function in patients with autoimmune cytopenias”; Blood 96, 3272-3275; Nov 1 2000

ALOPECIA UNIVERSAL
Seifert B et al., Complete remission of alopecia universalis after allogeneic hematopoietic stem cell transplantation, Blood 105, 426-427, 1 January 2005

IMMUNODEFICIENCIES

X-LINKED LYMPHOPROLIFERATIVE SYNDROME and X-LINKED HYPERIMMUNOGLOBULIN M SYNDROME
Banked unrelated umbilical cord blood was used to reconstitute the immune system in 2 brothers with X-linked lymphoproliferative syndrome and 1 boy with X-linked hyperimmunoglobulin-M syndrome. Two years after transplantation, all 3 patients have normal immune systems. These reports support the wider use of banked partially matched cord blood for transplantation in primary immunodeficiencies.

Reference:
Ziegner UH et al.; “Unrelated umbilical cord stem cell transplantation for X-linked immunodeficiencies”; J Pediatr 138(4), 570-573; April 2001

Eight children with severe immunodeficiencies treated by adult bone marrow stem cell transplants. Six of 8 showed relatively normal immune systems after 1 year.

Reference
SEVERE COMBINED IMMUNODEFICIENCY SYNDROME-X1 (ASC gene therapy)
Cavazzana-Calvo M et al.; “Gene therapy of human severe combined immunodeficiency (SCID)-X1 disease”; Science 288, 669-672; April 28, 2000

ANEMIAS

SICKLE CELL ANEMIA
Steen RG et al.; “Improved cerebrovascular patency following therapy in patients with sickle cell disease: initial results in 4 patients who received HLA-identical hematopoietic stem cell allografts”; Ann Neurol 49(2), 222-229; Feb. 2001

SIDEROBLASTIC ANEMIA
Gonzalez MI et al.; “Allogeneic peripheral stem cell transplantation in a case of hereditary sideroblastic anaemia”; British Journal of Haematology 109, 658-660; 2000

RED CELL APLASIA
Rabusin M et al.; “Immunoablation followed by autologous hematopoietic stem cell infusion for the treatment of severe autoimmune disease”; Haematologica 85(11 Suppl), 81-85; Nov. 2000

APLASTIC ANEMIA
Gurman G et al.; “Allogeneic peripheral blood stem cell transplantation for severe aplastic anemia”; Ther Apher 5(1), 54-57; Feb. 2001
Kook H et al.; “Rubella-associated aplastic anemia treated by syngeneic stem cell transplantations”; Am. J. Hematol. 64, 303-305; August 2000

AMEGAKARYOCYTIC THROMBOCYTOPENIA
Yesilipek et al.; “Peripheral stem cell transplantation in a child with amegakaryocytic thrombocytopenia”; Bone Marrow Transplant 26, 571-572; Sept. 2000

CHRONIC EPSTEIN-BARR INFECTION
Fujii N et al.; “Allogeneic peripheral blood stem cell transplantation for the treatment of chronic active epstein-barr virus infection”; Bone Marrow Transplant 26, 805-808; Oct. 2000
Okamura T et al.; “Blood stem-cell transplantation for chronic active Epstein-Barr virus with lymphoproliferation”; Lancet 356, 223-224; July 2000

FANCONI’S ANEMIA
Kohli-Kumar M et al., “Haemopoietic stem/progenitor cell transplant in Fanconi anaemia using HLA-matched sibling umbilical cord blood cells”, British Journal of Haematology 85, 419-422, October 1993
DIAMOND BLACKFAN ANEMIA
Ostronoff M et al., “Successful nonmyeloablative bone marrow transplantation in a corticosteroid-resistant infant with Diamond-Blackfan anemia”, Bone Marrow Transplant. 34, 371-372, August 2004

THALASSEMIA
Tan PH et al., “Unrelated peripheral blood and cord blood hematopoietic stem cell transplants for thalassemia major”, Am J Hematol 75, 209-212, April 2004

PRIMARY AMYLOIDOSIS
Sezer O et al.; “Novel approaches to the treatment of primary amyloidosis”; Exper Opin. Invest. Drugs 9, 2343-2350; Oct 2000
ADULT STEM CELLS—REPLACEMENT OF SOLID TISSUES

OTHER METABOLIC DISORDERS

OSTEOGENESIS IMPERFECTA

OSTEOPETROSIS
Driessen GJ et al., Long-term outcome of haematopoietic stem cell transplantation in autosomal recessive osteopetrosis: an EBMT report, Bone Marrow Transplantation 32, 657-663, October 2003
Schulzetal, HLA-haploidentical blood progenitor cell transplantation in osteopetrosis, Blood 99, 3458-3460, 1 May 2002

CEREBRAL X-LINKED ADRENOLEUKODYSTROPHY
Peters C et al., Cerebral X-linked adrenoleukodystrophy: the international hematopoietic cell transplantation experience from 1982 to 1999, Blood 104, 881-888, 1 August 2004

SANDHOFF DISEASE
Karen Augé, “Stem cells infuse kin with hope”, Denver Post Tuesday, August 24, 2004

HURLER’S SYNDROME
Koc ON et al., Allogeneic mesenchymal stem cell infusion for treatment of metachromatic leukodystrophy (MLD) and Hurler syndrome (MPS-IH), Bone Marrow Transplant 215-222; Aug 2002.

KRABBE LEUKODYSTROPHY

OCULAR

CORNEAL REGENERATION
Nishida K et al., Corneal reconstruction with tissue-engineered cell sheets composed of autologous oral mucosal epithelium, New England Journal of Medicine 351, 1187-1196, 16 September 2004
Anderson DF et al.; “Amniotic Membrane Transplantation After the Primary Surgical Management of Band Keratopathy”; Cornea 20(4), 354-361; May 2001
DoNoHarm: The Coalition of Americans for Research Ethics

Daya SM, Ilari FA; “Living related conjunctival limbal allograft for the treatment of stem cell deficiency”; Ophthalmology 180, 126-133; January 2001


Ocular corneal regeneration

WOUNDS & INJURIES

LIMB GANGRENE

SURFACE WOUND HEALING

JAWBONE REPLACEMENT
Warnke PH et al., Growth and transplantation of a custom vascularised bone graft in a man, Lancet 364, 766-770, 28 August 2004

SKULL BONE REPAIR
Lendeckel S et al., Autologous stem cells (adipose) and fibrin glue used to treat widespread traumatic calvarial defects: case report, Journal of Cranio-Maxillofacial Surgery 32, 370-373, 2004

HEART DAMAGE
Wollert KC et al., “Intracoronary autologous bone-marrow cell transfer after myocardial infarction: the BOOST randomised controlled clinical trial”, Lancet 364, 141-148, 10 July 2004


Perin EC et al.; “Transendocardial, autologous bone marrow cell transplantation for severe, chronic ischemic heart failure”; Circulation 107, r75-r83; published online May 2003

Stamm C et al.; “Autologous bone-marrow stem-cell transplantation for myocardial regeneration”; The Lancet 361, 45-46; 4 January 2003

Tse H-F et al.; “Angiogenesis in ischaemic myocardium by intramyocardial autologous bone marrow mononuclear cell implantation”; The Lancet 361, 47-49; 4 January 2003

Strauer BE et al.; “Repair of infarcted myocardium by autologous intracoronary mononuclear bone marrow cell transplantation in humans”; Circulation 106, 1913-1918; 8 October 2002

Strauer BE et al.; “Myocardial regeneration after intracoronary transplantation of human autologous stem cells following acute myocardial infarction”; Dtsch Med Wochenschr 126, 932-938; Aug 24, 2001


NEURAL DEGENERATIVE DISEASES & INJURIES

www.stemcellresearch.org
**STROKE**

Stilley CS et al., Changes in cognitive function after neuronal cell transplantation for basal ganglia stroke, *Neurology* 63, 1320-1322, October 2004


**PARKINSON’S DISEASE**

Lévesque M and Neuman T, “Autologous transplantation of adult human neural stem cells and differentiated dopaminergic neurons for Parkinson disease: 1-year postoperative clinical and functional metabolic result”, American Association of Neurological Surgeons annual meeting, Abstract #702; 8 April 2002


Gill SS et al.; “Direct brain infusion of glial cell line-derived neurotrophic factor in Parkinson disease”; *Nature Medicine* 9, 589-595; May 2003 (published online 31 March 2003)

See also July 14, 2004 Senate testimony by Dr. Michel Lévesque:
   http://commerce.senate.gov/hearings/testimony.cfm?id=1268&wit_id=3670

and Mr. Dennis Turner:
   http://commerce.senate.gov/hearings/testimony.cfm?id=1268&wit_id=3676

**SPINAL CORD INJURY**

See July 14, 2004 Senate testimony by Dr. Jean Peduzzi-Nelson:
   http://commerce.senate.gov/hearings/testimony.cfm?id=1268&wit_id=3671

and a more extensive testimony at:
   http://www.stemcellresearch.org/testimony/peduzzi-nelson.htm

and Ms. Laura Dominguez:
   http://commerce.senate.gov/hearings/testimony.cfm?id=1268&wit_id=3673

and Ms. Susan Fajt:
   http://commerce.senate.gov/hearings/testimony.cfm?id=1268&wit_id=3674