

**PEER-REVIEWED REFERENCES SHOWING APPLICATIONS OF  
ADULT STEM CELLS THAT PRODUCE  
THERAPEUTIC BENEFIT 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.

Abrey, LE *et al.*; “High dose chemotherapy with autologous stem cell rescue in adults with malignant  
primary brain tumors”; J. Neurooncol. 44, 147-153; Sept., 1999

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

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**OVARIAN CANCER**

Stiff PJ *et al.*; “High-dose chemotherapy and autologous stem-cell transplantation for ovarian cancer: An  
autologous blood and marrow transplant registry report”; Ann. Intern. Med. 133, 504-515; Oct. 3,  
2000

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

**MERKEL CELL CARCINOMA**

Waldmann V *et al.*; “Transient complete remission of metastasized merkel cell carcinoma by high-dose  
polychemotherapy and autologous peripheral blood stem cell transplantation”; Br. J. Dermatol. 143,  
837-839; Oct 2000

**TESTICULAR CANCER**

Bhatia S *et al.*; “High-dose chemotherapy as initial salvage chemotherapy in patients with relapsed  
testicular cancer”; J. Clin. Oncol. 18, 3346-3351; Oct. 19, 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

Josting, A; “Treatment of Primary Progressive Hodgkin’s and Aggressive Non-Hodgkin’s Lymphoma: Is  
There a Chance for Cure?”; J Clin Oncol 18, 332-339; 2000

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

## NON-HODGKIN'S LYMPHOMA

- Buadi FK *et al.*, Autologous hematopoietic stem cell transplantation for older patients with relapsed non-Hodgkin's lymphoma, *Bone Marrow Transplant* 37, 1017-1022, June 2006
- 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
- Josting, A; "Treatment of Primary Progressive Hodgkin's and Aggressive Non-Hodgkin's Lymphoma: Is There a Chance for Cure?"; *J Clin Oncol* 18, 332-339; 2000
- Kirita T *et al.*; "Primary non-Hodgkin's lymphoma of the mandible treated with radiotherapy, chemotherapy, and autologous peripheral blood stem cell transplantation"; *Oral Surg Oral Med Oral Pathol Oral Radiol Endod.* 90, 450-455; Oct. 2000

## HODGKIN'S LYMPHOMA

- Peggs KS *et al.*, "Clinical evidence of a graft-versus-Hodgkin's-lymphoma effect after reduced-intensity allogeneic transplantation", *Lancet* 365, 1934-1941, 4 June 2005
- Josting, A; "Treatment of Primary Progressive Hodgkin's and Aggressive Non-Hodgkin's Lymphoma: Is There a Chance for Cure?"; *J Clin Oncol* 18, 332-339; 2000

## ACUTE LYMPHOBLASTIC LEUKEMIA

- Laughlin MJ *et al.*; "Hematopoietic engraftment and survival in adult recipients of umbilical-cord blood from unrelated donors", *New England Journal of Medicine* 344, 1815-1822; June 14, 2001
- 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

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- 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
- 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

- Laughlin MJ *et al.*; "Hematopoietic engraftment and survival in adult recipients of umbilical-cord blood from unrelated donors", *New England Journal of Medicine* 344, 1815-1822; June 14, 2001
- 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

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## CHRONIC MYELOMONOCYTIC LEUKEMIA

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## ANGIOIMMUNOBLASTIC LYMPHADENOPATHY with DYSPROTEINEMIA

Lindahl J *et al.*; “High-dose chemotherapy and APSCT as a potential cure for relapsing hemolysing AILD”; *Leuk Res* 25(3), 267-270; March 2001

## MULTIPLE MYELOMA

Aviles A *et al.*, Biological modifiers as cytoreductive therapy before stem cell transplant in previously untreated patients with multiple myeloma, *Annals of Oncology* 16, 219-221, 2005

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.

## MYELOUDYSPLASIA

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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

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Paquette, RL *et al.*, “Ex vivo expanded unselected peripheral blood: progenitor cells reduce posttransplantation neutropenia, thrombocytopenia, and anemia in patients with breast cancer”, *Blood* 96, 2385-2390; October, 2000.

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

Koc, ON *et al.*; “Rapid Hematopoietic Recovery After Coinfusion of Autologous-Blood Stem Cells and Culture-Expanded Marrow Mesenchymal Stem Cells in Advanced Breast Cancer Patients Receiving High-Dose Chemotherapy”; *J Clin Oncol* 18, 307-316; January 2000

## NEUROBLASTOMA

Kawa, K *et al.*; “Long-Term Survivors of Advanced Neuroblastoma With MYCN Amplification: A Report of 19 Patients Surviving Disease-Free for More Than 66 Months”; *J Clin Oncol* 17:3216-3220; October 1999

## RENAL CELL CARCINOMA

Barkholt L *et al.*, Allogeneic haematopoietic stem cell transplantation for metastatic renal carcinoma in Europe, *Annals of Oncology* published online 28 April 2006

Arya M *et al.*, Allogeneic hematopoietic stem-cell transplantation: the next generation of therapy for metastatic renal cell cancer, *Nat Clin Pract Oncol.* 1, 32-38, Nov 2004

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## SOFT TISSUE SARCOMA

Blay JY *et al.*; “High-dose chemotherapy with autologous hematopoietic stem-cell transplantation for advanced soft tissue sarcoma in adults”; *J. Clin. Oncol.* 18, 3643-3650; Nov 1 2000

## EWING'S SARCOMA

Drabko K *et al.*, Megachemotherapy followed by autologous stem cell transplantation in children with Ewing's sarcoma, *Pediatric Transplantation* 9, 618-621, 2005

## VARIOUS SOLID TUMORS

Pedrazzoli P *et al.*, High dose chemotherapy with autologous hematopoietic stem cell support for solid tumors other than breast cancer in adults, *Annals of Oncology* published online 17 March 2006

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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

## 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

## 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

## MYELOFIBROSIS

Cornetta K *et al.*, Umbilical cord blood transplantation in adults: results of the prospective Cord Blood Transplantation (COBLT), *Biol Blood Marrow Transplant* 11, 149-160, February 2005

Cervantes F, Modern management of myelofibrosis, *Br J Haematol* 128, 583-592, March 2005

Kroger N *et al.*, Pilot study of reduced-intensity conditioning followed by allogeneic stem cell transplantation from related and unrelated donors in patients with myelofibrosis, *Br J Haematol* 128, 690-697, March 2005

Thiele J *et al.*, Dynamics of bone marrow changes in patients with chronic idiopathic myelofibrosis following allogeneic stem cell transplantation, *Histol Histopathol* 20, 87-89, July 2005

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Benesova P *et al.*, [Complete regression of bone marrow fibrosis following allogeneic peripheral blood stem cell transplantation in a patient with idiopathic myelofibrosis] [Article in Czech], *Cesk Patol* 40, 167-171, October 2004

# ADULT STEM CELLS—IMMUNE SYSTEM REPLACEMENT

## AUTOIMMUNE DISEASES

### DIABETES TYPE I (JUVENILE DIABETES)

Voltarelli JC *et al.*, Autologous Nonmyeloablative Hematopoietic Stem Cell Transplantation in Newly Diagnosed Type 1 Diabetes Mellitus, *Journal of the American Medical Association* 297, 1568-1576, 11 April 2007

### SYSTEMIC LUPUS

- Burt RK *et al.*, Nonmyeloablative hematopoietic stem cell transplantation for systemic lupus erythematosus, *Journal of the American Medical Association* 295, 527-535, February 1, 2006
- 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
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- 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
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- Burt, RK and Traynor, AE; "Hematopoietic Stem Cell Transplantation: A New Therapy for Autoimmune Disease"; *Stem Cells* 17, 366-372; 1999
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### SJOGREN'S SYNDROME

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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

### SCLEROMYXEDEMA

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### SCLERODERMA

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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  
Burt RK *et al.*, “High-dose immune suppression and autologous hematopoietic stem cell transplantation in refractory Crohn disease”, *Blood* 101, 2064-2066, March 2003  
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#### BEHCET’S DISEASE

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#### 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  
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#### MULTIPLE SCLEROSIS

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

#### BUERGER’S DISEASE

Kim D-I *et al.*, Angiogenesis facilitated by autologous whole bone marrow stem cell transplantation for Buerger’s disease, *Stem Cells* 24, 1194-1200, 2006

## IMMUNODEFICIENCIES

#### SEVERE COMBINED IMMUNODEFICIENCY SYNDROME

Grunebaum E *et al.*, Bone marrow transplantation for severe combined immune deficiency, *Journal of the American Medical Association* 295, 508-518, 1 February 2006

Cavazzana-Calvo M *et al.*; “Gene therapy of human severe combined immunodeficiency (SCID)-X1 disease”; *Science* 288, 669-672; April 28, 2000

(NOTE: gene therapy using bone marrow adult stem cells as gene vehicle)

#### 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.

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**Reference**

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# ANEMIAS and OTHER BLOOD CONDITIONS

## SICKLE CELL ANEMIA

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## SIDEROBLASTIC ANEMIA

Ayas M *et al.*; "Congenital sideroblastic anaemia successfully treated using allogeneic stem cell transplantation"; *Br J Haematol* 113, 938-939; June 2001

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## APLASTIC ANEMIA

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Kook H *et al.*; "Rubella-associated aplastic anemia treated by syngeneic stem cell transplantations"; *Am. J. Hematol.* 64, 303-305; August 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

## AMEGAKARYOCYTIC THROMBOCYTOPENIA

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## 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. Investig. Drugs* 9, 2343-2350; Oct 2000

## 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



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# **ADULT STEM CELLS—REPAIR/REPLACEMENT OF SOLID TISSUES**

## **METABOLIC DISORDERS**

### **HURLER'S SYNDROME**

- Cox-Brinkman J *et al.*, Haematopoietic cell transplantation (HCT) in combination with enzyme replacement therapy (ERT) in patients with Hurler syndrome, *Bone Marrow Transplantation* 38, 17-21, 2006
- Staba SL *et al.*, Cord-blood transplants from unrelated donors in patients with Hurler's syndrome", *New England Journal of Medicine* 350, 1960-1969, 6 May 2004
- 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.

### **OSTEOGENESIS IMPERFECTA**

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### **KRABBE LEUKODYSTROPHY**

- Escolar ML *et al.*, "Transplantation of umbilical cord-blood in babies with infantile Krabbe's disease", *New England Journal of Medicine* 352, 2069-2081, 19 May 2005
- Krivit W *et al.*, "Hematopoietic Stem-Cell Transplantation in Globoid-Cell Leukodystrophy", *New England Journal of Medicine* 338, 1119-1127, Apr 16, 1998

### **OSTEOPETROSIS**

- Tsuji Y *et al.*, Successful nonmyeloablative cord blood transplantation for an infant with malignant infantile osteopetrosis, *J Pediatr Hematol Oncol.* 27, 495-498, Sept 2005
- 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
- Schulz *et al.*, 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

## **OCULAR**

### **CORNEAL REGENERATION**

- Inatomi T *et al.*, Midterm results on ocular surface reconstruction using cultivated autologous oral mucosal epithelial transplantation, *American Journal of Ophthalmology* 141, 267-275, February 2006
- 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
- Anderson DF *et al.*; “Amniotic membrane transplantation for partial limbal stem cell deficiency”; *Br J Ophthalmol* 85(5), 567-575; May 2001
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