

**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

Dunkel IJ *et al.*; “Successful treatment of metastatic retinoblastoma”; *Cancer* 89, 2117-2121; Nov 15 2000

**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

- 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
- 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

## 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

## CHRONIC MYELOMONOCYTIC LEUKEMIA

Elliott MA *et al.*, Allogeneic stem cell transplantation and donor lymphocyte infusions for chronic myelomonocytic leukemia, *Bone Marrow Transplantation* 37, 1003-1008, 2006

## ANGIOIMMUNOBLASTIC LYMPHADENOPATHY with DYSPROTEINEMIA

Lindahl J *et al.*; “High-dose chemotherapy and APSCT as a potential cure for relapsing hemolyzing 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.

## MYELOYDYSPLASIA

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

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

## BREAST CANCER

Damon LE *et al.*; “High-dose chemotherapy and hematopoietic stem cell rescue for breast cancer: experience in California”; *Biol. Blood Marrow Transplant* 6, 496-505; 2000

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

Childs R *et al.*, “Regression of Metastatic Renal-Cell Carcinoma after Nonmyeloablative Allogeneic Peripheral-Blood Stem-Cell Transplantation”, *New England Journal of Medicine* 343, 750-758; Sept. 14, 2000

Childs, RW; “Successful Treatment of Metastatic Renal Cell Carcinoma With a Nonmyeloablative Allogeneic Peripheral-Blood Progenitor-Cell Transplant: Evidence for a Graft-Versus-Tumor Effect.”; *J Clin Oncol* 17, 2044-2049; July 1999

## 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

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 thiotepea and hematopoietic stem cell transplantation in pediatric malignant mesenchymal tumors: a phase II study"; *Bone Marrow Transplant* 26, 627-632; Sept. 2000

Michon, J and Schleiermacher, G. "Autologous haematopoietic stem cell transplantation for paediatric solid tumors", *Baillieres Best Practice Research in Clinical Haematology* 12, 247-259, March-June, 1999.

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

## 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

## 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

Rondelli D *et al.*, Allogeneic hematopoietic stem-cell transplantation with reduced-intensity conditioning in intermediate- or high-risk patients with myelofibrosis with myeloid metaplasia, *Blood* 105, 4115-4119, 15 May 2005

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

### 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
- 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 Cells* 17, 366-372; 1999
- Burt RK *et al.*; "Hematopoietic stem cell transplantation of multiple sclerosis, rheumatoid arthritis, and systemic lupus erythematosus"; *Cancer Treat. Res.* 101, 157-184; 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

### 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

### 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

- Passweg, JR *et al.*, Haematopoetic stem cell transplantation for refractory autoimmune cytopenia, *British Journal of Haematology* 125, 749-755, June 2004
- 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

- A.M. Feasel *et al.*, "Complete remission of scleromyxedema following autologous stem cell transplantation," *Archives of Dermatology* 137, 1071-1072; Aug. 2001.

### 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 Cells* 17, 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
- Burt RK *et al.*, "High-dose immune suppression and autologous hematopoietic stem cell transplantation in refractory Crohn disease", *Blood* 101, 2064-2066, March 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

## 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

## 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 Cells* 17, 366-372; 1999
- Burt RK *et al.*; "Hematopoietic stem cell transplantation of multiple sclerosis, rheumatoid arthritis, and systemic lupus erythematosus"; *Cancer Treat. Res.* 101, 157-184; 1999
- Burt, RK *et al.*, "Autologous hematopoietic stem cell transplantation in refractory rheumatoid arthritis: sustained response in two of four patients", *Arthritis & Rheumatology* 42, 2281-2285, November, 1999.

## JUVENILE ARTHRITIS

- I M de Kleer *et al.*, Autologous stem cell transplantation for refractory juvenile idiopathic arthritis: analysis of clinical effects, mortality, and transplant related morbidity, *Ann Rheum Dis* 63, 1318-1326, 2004
- 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 Cells* 17, 366-372; 1999

## MULTIPLE SCLEROSIS

- Saccardi R *et al.*, Autologous HSCT for severe progressive multiple sclerosis in a multicenter trial: impact on disease activity and quality of life, *Blood* 105, 2601-2607, 15 March 2005
- 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
- Mancardi GL *et al.*; "Autologous hematopoietic stem cell transplantation suppresses Gd-enhanced MRI activity in MS"; *Neurology* 57, 62-68; July 10, 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 Cells* 17, 366-372; 1999

Burt RK *et al.*; “Hematopoietic stem cell transplantation of multiple sclerosis, rheumatoid arthritis, and systemic lupus erythematosus”; *Cancer Treat. Res.* 101, 157-184; 1999

#### POLYCHONDRITIS

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

#### ALOPECIA UNIVERSALIS

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.

#### **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**

Amrolia, P. *et al.*, “Nonmyeloablative stem cell transplantation for congenital immunodeficiencies”, *Blood* 96, 1239-1246, Aug. 15, 2000.



# ANEMIAS and OTHER BLOOD CONDITIONS

## SICKLE CELL ANEMIA

Klein A *et al.*, Hematopoietic stem cell transplantation for severe sickle cell disease, *Rev Med Brux.* 2005;26 Spec no:Sp23-5

Adamkiewicz TV *et al.*, Transplantation of unrelated placental blood cells in children with high-risk sickle cell disease, *Bone Marrow Transplant.* 34, 405-411, Sept 2004

Wu CJ *et al.*, Molecular assessment of erythroid lineage chimerism following nonmyeloablative allogeneic stem cell transplantation, *Exp Hematol.* 31, 924-933, Oct 2003

Gore L. *et al.*; "Successful cord blood transplantation for sickle cell anemia from a sibling who is human leukocyte antigen-identical: implications for comprehensive care", *J Pediatr Hematol Oncol* 22(5):437-440; Sep-Oct 2000

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

Wethers DL; "Sickle cell disease in childhood: Part II. Diagnosis and treatment of major complications and recent advances in treatment"; *Am. Fam. Physician* 62, 1309-1314; Sept. 15, 2000

## SIDEROBLASTIC ANEMIA

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

Gonzalez MI *et al.*; "Allogeneic peripheral stem cell transplantation in a case of hereditary sideroblastic anaemia"; *British Journal of Haematology* 109, 658-660; 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

## 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

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

## 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



## FANCONI'S ANEMIA

Bitan M *et al.*, Fludarabine-based reduced intensity conditioning for stem cell transplantation of fanconi anemia patients from fully matched related and unrelated donors, *Biol Blood Marrow Transplant.* 12, 712-718, July 2006

Tan PL *et al.*, Successful engraftment without radiation after fludarabine-based regimen in Fanconi anemia patients undergoing genotypically identical donor hematopoietic cell transplantation, *Pediatr Blood Cancer*, 46, 630-636, May 1, 2006

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

## 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

# **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**

- Horwitz EM *et al.*, "Isolated allogeneic bone marrow-derived mesenchymal cells engraft and stimulate growth in children with osteogenesis imperfecta: Implications for cell therapy of bone", *Proceedings of the National Academy of Sciences USA* 99, 8932-8937; 25 June 2002.
- Horwitz EM *et al.*, "Clinical responses to bone marrow transplantation in children with severe osteogenesis imperfecta", *Blood* 97, 1227-1231; 1 March 2001.
- Horwitz, EM *et al.*; "Transplantability and therapeutic effects of bone marrow-derived mesenchymal cells in children with osteogenesis imperfecta"; *Nat. Med.* 5, 309-313; March 1999.

### **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
- Henderson TR *et al.*; “The long term outcome of limbal allografts: the search for surviving cells”; *Br J Ophthalmol* 85(5), 604-609; May 2001
- Daya SM, Ilari FA; “Living related conjunctival limbal allograft for the treatment of stem cell deficiency”; *Ophthalmology* 180, 126-133; January 2001
- Schwab IR *et al.*; “Successful transplantation of bioengineered tissue replacements in patients with ocular surface disease”; *Cornea* 19, 421-426; July 2000.
- Tsai *et al.*; “Reconstruction of damaged corneas by transplantation of autologous limbal epithelial cells.”; *New England Journal of Medicine* 343, 86-93, 2000.
- Tsubota K *et al.*; “Treatment of severe ocular-surface disorders with corneal epithelial stem-cell transplantation”; *New England Journal of Medicine* 340, 1697-1703; June 3, 1999

## **WOUNDS & INJURIES**

### **LIMB GANGRENE**

- Tateishi-Yuyama E *et al.*; “Therapeutic angiogenesis for patients with limb ischaemia by autologous transplantation of bone-marrow cells: a pilot study and a randomised controlled trial”; *Lancet* 360, 427-435; 10 August 2002.

### **SURFACE WOUND HEALING**

- Badiavas EV and Falanga V, “Treatment of chronic wounds with bone marrow-derived cells”, *Archives of Dermatology* 139, 510-516, 2003

### **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**

### **ACUTE HEART DAMAGE**

- Joseph J *et al.*, Safety and effectiveness of granulocyte-colony stimulating factor in mobilizing stem cells and improving cytokine profile in advanced chronic heart failure, *American Journal of Cardiology* 97, 681-684, 1 March 2006
- Blocklet D *et al.*, Myocardial homing of nonmobilized peripheral-blood CD34+ cells after intracoronary injection, *Stem Cells* 24, 333-336, February 2006
- Janssens S *et al.*, Autologous bone marrow-derived stem-cell transfer in patients with ST-segment elevation myocardial infarction: double-blind, randomised controlled trial, *Lancet* 367, 113-121, 14 January 2006
- Patel AN *et al.*, Surgical treatment for congestive heart failure with autologous adult stem cell transplantation: a prospective randomized study, *Journal Thoracic Cardiovascular Surgery* 130, 1631-1638, December 2005

- Ince H *et al.*, Preservation from left ventricular remodeling by front-integrated revascularization and stem cell liberation in evolving acute myocardial infarction by use of granulocyte-colony-stimulating factor (FIRSTLINE-AMI), *Circulation* 112, 3097-3106, 15 November 2005
- Ince H *et al.*, Prevention of left ventricular remodeling with granulocyte colony-stimulating after acute myocardial infarction, *Circulation* 112, I-73-I-80, 30 August 2005
- Bartunek J *et al.*, Intracoronary injection of CD133-positive enriched bone marrow progenitor cells promotes cardiac recovery after recent myocardial infarction, *Circulation* 112, I-178-I-183, 30 August 2005
- Dohmann HFR *et al.*, Transendocardial autologous bone marrow mononuclear cell injection in ischemic heart failure, *Circulation* 112, 121-126, 26 July 2005
- 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
- Britten MB *et al.*, "Infarct remodeling after intracoronary progenitor cell treatment in patients with acute myocardial infarction"; *Circulation* 108, 2212-2218; Nov 2003
- 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
- Menasché P *et al.* "Myoblast transplantation for heart failure." *Lancet* 357, 279-280; Jan 27, 2001
- Menasché P *et al.* ["Autologous skeletal myoblast transplantation for cardiac insufficiency. First clinical case."] [article in French] *Arch Mal Coeur Vaiss* 94(3), 180-182; March 2001

## **CHRONIC CORONARY ARTERY DISEASE**

- Strauer BE *et al.*, Regeneration of human infarcted heart muscle by intracoronary autologous bone marrow cell transplantation in chronic coronary artery disease, *Journal of the American College of Cardiology* 46, 1651-1658, 1 November 2005

## **NEURAL DEGENERATIVE DISEASES & INJURIES**

### **STROKE**

- Shyu W-C *et al.*, Granulocyte colony-stimulating factor for acute ischemic stroke: a randomized controlled trial, *Canadian Medical Association Journal* 174, 927-933, 28 March 2006
- Stilley CS *et al.*, Changes in cognitive function after neuronal cell transplantation for basal ganglia stroke, *Neurology* 63, 1320-1322, October 2004
- Meltzer CC *et al.*; "Serial [18F]Fluorodeoxyglucose Positron Emission Tomography after Human Neuronal Implantation for Stroke"; *Neurosurgery* 49, 586-592; 2001.
- Kondziolka D *et al.*; "Transplantation of cultured human neuronal cells for patients with stroke"; *Neurology* 55, 565-569; August 2000

## **PARKINSON'S DISEASE**

### **USING DIRECT STIMULATION OF PATIENTS' ENDOGENOUS ADULT NEURAL STEM CELLS:**

Love S *et al.*, Glial cell line-derived neurotrophic factor induces neuronal sprouting in human brain, *Nature Medicine* 11, 703-704, July 2005

Slevin JT *et al.*, Improvement of bilateral motor functions in patients with Parkinson disease through the unilateral intraputaminial infusion of glial cell line-derived neurotrophic factor, *Journal of Neurosurgery* 102, 216-222, February 2005

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)

## **SPINAL CORD INJURY**

Lima C *et al.*, Olfactory mucosa autografts in human spinal cord injury: A pilot clinical study, *Journal of Spinal Cord Medicine* 29, 191-203, July 2006

## **LIVER DISEASE**

### **CHRONIC LIVER DISEASE**

Gordon MY *et al.*, Characterisation and clinical application of human CD34+ stem/progenitor cell populations mobilised into the blood by G-CSF, *Stem Cells* 24, 1822-1830, July 2006; published online March 30, 2006

### **LIVER CIRRHOSIS**

Terai S *et al.*, Improved liver function in liver cirrhosis patients after autologous bone marrow cell fusion therapy, *Stem Cells* published online 15 June 2006; DOI: 10.1634/stemcells.2005-0542

## **BLADDER DISEASE**

### **END-STAGE BLADDER DISEASE**

Atala A *et al.*, Tissue-engineered autologous bladders for patients needing cytoplasty, *The Lancet* 367, 1241-1246, 15 April 2006