

CURRICULUM VITAE

Name Stefan Karlsson
Date and Place of Birth: Akureyri, Iceland, 31. May, 1950
Marital Status: Married to Sigurborg Ragnarsdottir, two sons, Ragnar and Jon.

Education:

1970-1976 Cand Med. et Chir. (MD degree), University of Iceland, Medical Faculty.
1976-1977 Rotating Internship, National Hospital and City Hospital, Reykjavik
1977-1979 Residency, Blood Bank, National Hospital, Reykjavik.
1979-1982 PhD student, University College London, PhD, 1982.

Professional Appointments:

1983-1984 Fogarthy Award, Visiting fellow, NHLBI, National Institutes of Health, Bethesda, MD
1984-1986 Visiting associate, NHLBI, National Institutes of Health, Bethesda, MD
1986-1988 Visiting associate (acting Section Chief), NINDS, NIH, Bethesda, MD
1988-1996 Chief, Molecular and Medical Genetics Section, DMNB, NINDS, NIH.
1995- Professor of Molecular Medicine, University of Lund and Senior physician (överläkare) Lund University Hospital.

Medical Certification:

1978 Registered Physician in Iceland
1982 Registered Specialist in Medical Genetics, Iceland
1997 Registered Physician in Sweden

Administrative Responsibilities/Committees/Commissions of Trust:

1991-1995: Chairman, User Committee, Animal Facility 10A, NIH
1994-1995: Member, Gene therapy Committee, Foundation for Strategic Research, Sweden.

- 1996: Organizer (with Prof. Keith Humphries and Dr. Paul Tolstoshev):
Keystone Symposia on "Gene Therapy for Hematopoietic Stem Cells in
Genetic Disease and Cancer", Taos, New Mexico, February 4-10.
- 1997-2002: Member, Developmental Biology Board, Foundation for Strategic Research
- 1998 Organizer (with Prof. Keith Humphries): Keystone Symposia on "Gene
Therapy Strategies for Hematopoietic Stem Cells" in Incline Village,
Nevada, January 10-15.
- 1998-2000: Member, Medical Faculty Council, Lund University
- 1997-2004: Aroseniusfonden, Research Foundation, Board member

Honors/Awards:

- 1983 Fogarty Award
- 1994 Elected member of The American Society for Clinical Investigation
(ASCI)
- 1997 Elected member of The Royal Physiographical Society in Lund
- 2003-2004: Vice President, International Society for Experimental Hematology (ISEH)
- 2004-2005: President-Elect, International Society for Experimental Hematology (ISEH)
- 2005-2006: President, International Society for Experimental Hematology (ISEH)

Pre-doctoral Advisor:

- 1) Pamela H. Correll, PhD, 1988-1993. Retroviral-mediated transfer of the human glucocerebrosidase gene into murine hematopoietic stem cells as a model for gene therapy (1993). George Washington University, Washington DC. Current position: Associate Professor, Pennsylvania State University, University Park, PA, USA.
- 2) Niels Bjarne Woods, 1998-2002. Development of gene therapy for hematopoietic stem cells (2002). Current position: Postdoctoral fellow, Salk Institute.
- 3) Jon Mar Björnsson, 1996-2002. Hox transcription factors in hematopoiesis (2002). Current position: Scientists, DeCode Genetics, Iceland.
- 4) Jonas Larsson MD, 1996-2002. The role of TGF- β in hematopoiesis. Current position: Postdoctoral fellow, Harvard Medical School.

- 5) Thomas Relander MD, 1998-2003 (co-supervisor). Retroviral gene transfer to repopulating hematopoietic cells (2003). Current position: Oncologist, Lund University Hospital.
- 6) Ann Brun, 1997-2004. The role of HoxB4 in hematopoiesis. Current position: Research engineer and postdoctoral scientist, Lund University.

Present PhD students: Johan Flygare (2001-), Ulrika Blank (2001-), Mattias Magnusson (2001-), Göran Karlsson (2002-), Sofie Singbrant (2003-), Ida Berglin (2004-) Co-supervisor : Markus Järås (2002-), Maria Johansson (2003-).

Post-doctoral Advisor:

- 1) John K Fink MD, 1986-1990. Current position: Professor of Neurology, Dept. of Neurology, University of Michigan, Ann Arbor, USA.
- 2) Ashok K Kulkarni PhD, 1987-1993. Current position: Tenured Senior Investigator and Head, Functional Genomics Unit, NIDS, NIH, Bethesda MD
- 3) Boro Dropulic PhD, 1989-1991. Current Position: Founder & CSO, VIRXSYS, Gaithersburg MD, USA and Adjunct Assistant Professor, Johns Hopkins University, MD USA.
- 4) Linda Cairns PhD, 1989-1991. Current Position: Scientists, University of Milano, Italy.
- 5) Licheng Xu MD PhD, 1991-1994. Current Position: Attending Hematologist/Ocologist, Hematology/Oncology, State University of New York Medical Center, Long Island, NY.
- 6) Joseph Higgins MD, 1991-1993. Present Position: Director, Center for Human Genetic Studies & Child Neurology, Mid-Hudson Health Institute, New Paltz, NY, USA.
- 7) Chang-Goo Huh PhD, 1991-1996. Current Position: Staff scientists, Laboratory of Experimental Carcinogenesis, NCI, NIH, Bethesda MD, USA.
- 8) Raphael Schiffmann MD, 1992-1996. Current Position: Chief, Clinical Investigations Section, DMNB, NINDS, NIH, Bethesda MD, USA.
- 9) Makoto Migita MD PhD, 1993-1996. Current Position: Associate Professor of Pediatrics and Biochemistry, Nippon Medical School, Tokyo, Japan.

- 10) Linda Yaswen PhD, 1993-1995. Current Position: Scientist, NIHGR, NIH.
- 11) Jefferey Medin PhD, 1994-1996. Scientist, Division of Experimental Therapeutics, Ontario Cancer Institute and Associate Professor, Department of Medical Biophysics, University of Toronto, Ontario, Canada.
- 12) Cecilia Fahlman MD, PhD, 1995-1997. Current Position: Oncologist, Dept. Of Oncology, Lund University Hospital, Lund, Sweden, Sweden.
- 13) Anders Hall PhD, 1995-1997. Current position: Staff Scientist, AstraZeneca, Lund, Sweden.
- 14) Nina Larsson, MD PhD, 1995-1999. Current Position: Physician, Resident/ Fellow in Clinical Genetics, Lund University Hospital.
- 15) Elisabet Andersson, PhD, 1996-1999. Current Position: Scientist and Laboratory Manager, Karolinska Institute, Stockholm, Sweden.
- 16) Xiaolong Fan, 1997-2001. Current Position: Assistant Professor, Dept. of Immunology, Lund University, Lund, Sweden.
- 17) Hanna Mikkola, MD, PhD, 1999-2000. Current Position: Assistant Professor, University of California, Los Angeles
- 18) Per Levéen PhD, Research Associate 1997-2002. Current position: Scientist, Dept. of Endocrinology, Malmö General Hospital.
- 19) Isao Hamaguchi, MD, PhD, Research Associate 1998-2002. Current position: Senior scientist, National Institutes of Infectious Diseases, Tokyo, Japan.
- 20) Hiroshi Nishiura DDS, PhD, Research Fellow, 2000-2002. Current position: Assistant Professor, Kumamoto University, Japan.
- 21) Thomas Kiefer MD, Hematology Fellow, 2002-2004. Current position: Senior Hematologist/Oncologist, University of Greifswald, Germany.
- 22) Koichi Miyake MD, PhD, 2003-2005, Visiting Scientist. Associate Professor, Nippon Medical School, Tokyo
- 23) Taiju Utsugisawa MD, PhD, 2004-2005, Hematology Fellow. Current position: Hematologist, Tokyo, Japan

Current Postdoctoral Scientist:

Jennifer Moody PhD, 2003-.

Scientific Advisor:

Kimergen, Inc, 1996-1997

Astra Draco AB, 1996-1998

Genethon, Paris, France, Scientific Advisory Board, 2000-2002

Societies:

American Society for Clinical Investigation

American Society of Hematology

International Society for Experimental Hematology

Royal Physiographic Society, Lund

American Society for Gene Therapy

European Society for Gene Therapy

Swedish Society for Gene Therapy

Editorial Board Member:

Experimental Hematology, (Editorial Board, 1997-1999)

Journal of Gene Medicine (Editorial Board, current)

Hematology Journal (Editorial Board, current)

Stem Cells (2003-2005)

European Journal of Hematology (Associate Editor, 2005-)

Journal Reviews:

Blood, Stem Cells, Nature Medicine, Proc Natl Acad Sci USA, Development, Human Gene Therapy, Gene Therapy, Cancer Gene Therapy, Journal of Clinical Investigation, JAMA, Hepatologia, European Journal of Hematology, Leukemia, Biotechniques, J Cellular Physiology, Hematologica

Grant Reviews:

Swedish Cancer Society, Study Section on Hematology, Genetics and Pathology

Swiss National Science Foundation (Gene Therapy Review Board)

Leukemia Research Foundation, UK

Norwegian Research Council

Finnish Academy.

Deutsche Forschungs Gemeinschaft

Ph.D. Thesis Opponent (Examiner):

Sirac Dilber, Doctoral thesis, Karolinska Institute, Stockholm, 1997

Ke-Jun Li, Doctoral thesis, Karolinska Institute, Stockholm, 1998

Menzo Havenga, Doctoral thesis, University of Leiden, 1999

Greger Brodin, Doctoral thesis, Uppsala University, 2002

Axel Schambach, PhD thesis, Hannover Medical School, Germany, 2005

Research Grants:

Medical Research Council, Sweden (1995-)

Cancerfonden, Sweden (1995-)

The Wallenberg Foundation, Sweden (1996-1999)

Astra Draco AB (1996-1999)

Children's Cancer Fund, Sweden (1998-)

Gene Therapy Consortium Grant, Sweden, 1999-2001

Diamond-Blackfan Anemia Foundation, USA, 2001-2002 and 2004-2005.

INHERINET, EC grant with 6 other partners, 2002-2004

The Swedish Stem Cell Program, supported by JDRF, 2003-2005

CONSERT, EC, Integrated project with 20 other partners, 2004-2008.

Invited Speaker (2001-present).

Invited Speaker: "Development of Gene Therapy for Hematopoietic Stem Cells".
Graduate Course in Molecular Medicine, Uppsala University, April 10, 2001.

Seminar: "Gene Therapy of Hematopoietic Stem Cells Using Lentiviral Vectors"
Medical University of Hanover, Germany, June 20, 2001.

Invited Speaker: "Gene Therapy of Hematopoietic Stem Cells Using Lentiviral Vectors".
University Hospital of Freiburg, Medical Faculty, July 6, 2001.

Invited Speaker: "TGF- β Signalling and Hematopoiesis". University of Freiburg,
Medical Faculty, July 5, 2001.

Seminar: "Development of Gene Therapy for Hematopoietic Stem Cells Using Lentiviral Vectors". Institute of Hematology, Erasmus University, Rotterdam, Oct. 15, 2001.

Invited Speaker and Session Chairman: International Symposium on Transplantation and Gene Therapy, Idar-Oberstein, Germany, Oct. 18-20, 2001. Lecture entitled: "Development of Gene Therapy for Hematopoietic Stem Cells Using Lentiviral Vectors".

Invited Speaker: International Postgraduate Course on Lysosomal Storage Disorders, Marstrand Island, near Gothenburg, Sweden, October 29-Nov 1, 2001. Lecture entitled: "Development of Gene Therapy for Gaucher Disease using Lentiviral Vectors".

Invited Speaker: Laboratory of Hematopoiesis, CIEMAT, Madrid, Spain, January 16, 2002. Lecture entitled: "The development of gene therapy for hematopoietic stem cells".

Invited Speaker: The Third Conference on Stem Cell Gene Therapy: Biology and Technology. Lecture entitled: "Regulation of HSC proliferation by Homeobox Transcription Factors in Engineered Model Systems".

Invited Speaker: Diamond-Blackfan Anemia International Consensus Conference 2002. Baltimore, MD, April 30, 2002. Lecture entitled: "The development of gene therapy for RPS19 deficient Diamond-Blackfan Anemia".

Hemophilia 2002: XXV International Congress of the World Federation of Hemophilia, May 19-24, 2002, Seville, Spain. Arosenius Lecture entitled: "Development of Gene Therapy for Blood Disorders by Gene Transfer into Hematopoietic Stem Cells".

Invited Speaker: TGF- β meeting in Uppsala, May 26-27, 2002. Lecture entitled: "The role of TGF- β signaling in inflammation and T cell development".

Invited Speaker: Molecular medicine 2002: Clinical Biochemistry and Coagulation, August 10-13, 2002, Reykjavik, Iceland. Lecture entitled: "Development of Gene Therapy for Hematopoietic Stem Cells"

Invited Speaker: Third National Conference of The Network for Inflammation Research, Sept. 29-Oct. 1, 2002, Lund, Sweden. Lecture entitled: "Hematopoiesis and Inflammation in TGF- β Signaling Deficient Mice".

Invited Speaker: The Roscoe O. Brady, M.D. Symposium, NIH Clinical Center, Lipsetts Auditorium, Oct. 8-9, 2002, Bethesda MD. Lecture entitled "Development of Gene therapy for Hematopoietic Stem Cells".

Invited Speaker: Karolinska Institutet, Huddinge, Nov. 21, 2002. Lecture entitled: "The role of TGF- β signaling and Hox transcription factors in the regulation of hematopoiesis".

Invited Speaker: American Association for Cancer Research Special Conference on: The TGF- β Superfamily: Roles in the Pathogenesis of Cancer and Other Diseases, La Jolla CA, January 15-18, 2003. lecture entitled: Inflammation, Hematopoiesis and T Cell Development in Mice Deficient in TGF- β signaling”.

Invited Speaker: II Meeting of The Spanish Society of Gene Therapy, Barcelona, Spain, March 28-29, 2003. Lecture entitled: Hematopoiesis, Stem Cells and Stem Cell Fate Decisions in Murine Models.

Invited Speaker: Diamond-Blackfan Anemia Conference, New York City, NY, April 5-7, 2003. Lecture entitled: ”Abnormal Function of Hematopoietic Progenitors in DBA: Approaches and Model Systems to Characterize the Hematopoietic Defect.

Invited Speaker and Session Chairman: FASEB Summer Research Conferences: The TGF- β Superfamily: Signaling and Development, Tucson Arizona, July 12-17, 2003. Lecture entitled: The Role of TGF- β in Development and Function of hematopoietic Cells”.

Invited Speaker: Gaucher Network Meeting, Gothenburg, October 23-24, 2003. Lecture entitled: ”Development of gene therapy for Gaucher disease”.

Invited Speaker: Symposium on Gene Therapy for Hematological Disorders, The Annual meeting of The Swedish Medical Association, November 26-28, 2003. Lecture entitled: ”Gene therapy for hematopoietic stem cells”.

Invited Speaker: National Institute of Dental Research, NIH, Bethesda MD, Dec. 2, 2003. lecture entitled: The role of TGF- β in hematopoiesis and T cell development”.

Invited Speaker: National Cancer Institute, NIH, Dec. 2, 2003. Lecture entitled: ”The role of Hox transcription factors in hematopoiesis”.

Invited Lecture: University of Umeå, Sweden, February 27, 2004. Lecture entitled: The role of Smad signaling in hematopoiesis.

Invited Speaker: The Diamond Blackfan anemia Consensus Conference, New York City, April 23-25, 2004: Lecture entitled: Development of cellular models for RPS19 deficient Diamond Blackfan anemia.

Invited Lecture: National University Hospital, Iceland, May 10, 2004: Lecture entitled: Hematopoietic stem cells: Regulatory pathways and development of advanced cell therapies.

Invited Speaker: Molecular Biology of hematopoiesis meeting in Tokyo, August 6, 2004: lecture entitled: The role of Smad signaling in hematopoiesis.

Invited Speaker: The Japanese Society of Gene Therapy annual meeting, Tokyo, August 7, 2004: Lecture entitled: Development of gene therapy for Diamond Blackfan anemia.

Invited Speaker: University of Kyoto, August 3, 2004. Lecture entitled: The role of Smad signaling in hematopoiesis.

Invited Speaker: International Course in Hemophilia, Malmö, 20-24 Sept, 2004. Lecture entitled: Development of gene therapy for Diamond Blackfan anemia.

Invited Speaker: Molecular Hematopoiesis 7, Institute of Child Health, London, October 8, 2004. Lecture entitled: The role of Smad signaling in hematopoiesis

Invited Speaker: Gene vaccination, a meeting organized by The Swedish Gene Technology Advisory Board, Stockholm, October 27, 2004. A lecture entitled: Risks in gene transfer and gene therapy.

Invited Speaker: The Nordic Gaucher Network, organized by Genzyme, November 4-5, 2004. Lecture entitled: A novel mouse model for Gaucher disease.

Invited Speaker: National Cancer Institute, NIH, Bethesda MD, November 30, 2004: Lecture entitled: The Role of Smad signaling in hematopoiesis.

Invited speaker: Genzyme Corporation, Boston, April 12, 2005: Lecture entitled: The Development of Gene therapy for Blood Disorders.

Invited Speaker: The Institute for Stem Cell Research, University of Edinburgh, May 6, 2005: Lecture entitled: "The Role of Smad Signaling in Hematopoiesis"

Invited Speaker: Gordon Research Conference on Red Cells: (Organizer, Dr. Len Zon), Tilton School, Tilton NH, June 12-17, 2005. Lecture: The Development of Gene therapy for Diamond-Blackfan Anemia

Invited Speaker: Long Island Jewish Medical Center, NY, July 11, 2005. Lecture entitled: Diamond Blackfan Anemia, Hematopoiesis, Models and Gene therapy.

Invited Speaker: University of Louisville, KY, August 29-30, 2005. Two lectures entitled: "Diamond Blackfan Anemia, Hematopoiesis, Models and Gene therapy" and "The Role of Smad Signaling in Hematopoiesis".

Invited Speaker: The third annual meeting of the European Stem Cell Excellence Centre (STEC). Cracow, Poland, October 6-9, 2005. A lecture entitled: "The Role of Smad Signaling in Hematopoiesis".

Invited Speaker: Stem Cells: Basic Biology and Biomedical Potential. A meeting organized by the Norwegian Center for Stem Cell Research, Oslo, October 17-18, 2005. A lecture entitled: "The Role of Smad Signaling in Hematopoiesis".

Invited Speaker: Stem cells in basic and medical research. A meeting in Gl Avernoes, Fyn, Denmark, October 24-26, 2005, organized by The Danish Society for Biochemistry and Molecular Biology, The Danish Stem Cell Research Doctoral School and the Danish Society for Flow Cytometry. Two lectures entitled: "The Role of Smad Signaling in Hematopoiesis" and "Development of Gene Therapy for Diamond Blackfan Anemia".

Invited Speaker and Session Chairman: The 7th Annual Diamond Blackfan Anemia International Consensus Conference. New York City, March 11-13, 2006

Invited Speaker: Ernst Moritz Arndt University and Alfred Krupp Wissenschaftskolleg, Greifswald, Germany, March 20, 2006. Lecture entitled: "The Role of Smad Signaling in Hematopoiesis"

Invited Speaker: European Molecular Biology Laboratories, Monterotundo (Rome), Italy, April 3, 2006. Lecture entitled: "The role of Smad signaling in hematopoiesis".

Invited Speaker: The TGF- β meeting, organized by Carl Henrik Heldin, Ludwig Institute for Cancer Research, May 13-14, 2006. "The role of Smad signaling in hematopoiesis".

Invited Speaker: Symposium on Lysosomal Diseases and the Brain. Organizers: A. Futerman, R. Schiffmann, R.O. Brady, K Wong, G. Grabowski. Sacramento, CA, USA, June 23-25, 2006.

Bibliography

1. **Karlsson, S.**, Thorarinsson, H. and Jensson, O. (1978) Syphilis a Islandi 1950-1975, tidni og blodvatnsgreining. The Icelandic Medical Journal. 64: 173-181.
2. **Karlsson, S.**, Arnason, A., Thordarson, G., and Olaisen, B. (1980). Frequency of Gc alleles in Iceland. Human Heredity 30: 119-121.
3. **Karlsson, S.**, Arnason, A. and Jensson, O. (1980) GLO polymorphism in Iceland. Human Heredity 30.
4. **Karlsson, S.**, Skaftadottir, I, Arnason, A., Thordarson and Jensson, O. (1983) Gc subtypes in Icelanders. Human. Hered. 33. 5-8
5. **Karlsson, S.**, Skaftadottir, I, Mackintosh, P., and Jensson, O. (1983) Gc subtypes in Northern Indians. Hum. Hered. 33. 199-200.
6. **Karlsson, S.**, (1983) Erfdætaekni og Laeknisfraedi (Recombinant DNA technology and medicine) Editorial; The Icelandic Medical Journal 69: 191-122.
7. **Karlsson, S.**, Swallow, D.M., Griffiths, B., Corney, G., Hopkinson, D.A., Dawney, A., and Cartron, J.P. (1983). A Genetic polymorphis of human urinary mucin. Ann. Hum. Genet. 47: 263-269.
8. **Karlsson, S.**, Griffith, B., Swallow, D.M., and Hopkinson, D.A. (1984) Glycoproteins in cystic fibrosis: A lectin-binding study. J. Med. Genet.21: 441-446.
9. Griffiths, B., **Karlsson, S.**, Soh, C., Dawney, A., Swallow, D.M. (1984) Tamm-Horsfall glycoprotein: a lectin binding study, Glycoconjugate J. 1: 183-190.
10. **Karlsson, S.**, Humphries, R.K., Gluzman, Y., Nienhuis, A.W. (1985). Transfer of genes into hematopoietic cells using recombinant DNA viruses. Proc. Natl. Acad. Sci. (USA) 82: 158-162.
11. Humphries, R.K., **Karlsson, S.**, Nienhuis, A.W. (1985) Toward's treatment of genetic anemias by gene replacement. In: Principles and Techniques of Human Research and Therapeutics, VL.2, eds. McMahan, F.G., and Ley, J.T., Futura, Mt. Kisco, N.Y.
12. **Karlsson, S.**, Nienhuis, A.W. (1985) Develomental Regulation of Human Globin Genes. Ann. Rev. Biochemistry 54: 1071-1108.

13. Anagnou, N.P., Moulton, A.D., Keller, G., **Karlsson, S.**, Papayannopoulou, T., Stamatoyannopoulos, G., Nienhuis, A.W. (1985). Cis-acting sequences that affect the expression of the human fetal gamma-globin genes. In: *Experimental Approaches for the Study of Hemoglobin Switching*. pp. 163-182, eds. Stamatoyannopoulos, G., Nienhuis, A.W., Alan R. Liss, N.Y.
14. Anagnou, N.P., **Karlsson, S.**, Moulton, A.D., Keller, G., Nienhuis, A.W. (1985). Promoter sequences required for function of human gamma-globin gene. *EMBO J.* 5: 121-126.
15. **Karlsson, S.**, Van Doren, K., Schweiger, S.G., Nienhuis, A.W. Gluzman, Y. (1986). Stable gene transfer and tissue specific expression of a human globin gene using adenoviral vectors. *EMBO J* 5: 2377-2385.
16. Kantoff, P.W., Gillio, A., McLachlin, J., **Karlsson, S.**, Eglitis, M.A., Kohn, D., Karson, E., Zwiebel, J., Bordignon, C., Hutton, J.J., Blaese, R.M., Nienhuis, A., O'Reilly, R., Gilboa, E., Anderson, W.F. (1986). Retroviral mediated gene transfer into hematopoietic cells, *Trans. Assoc. Am. Phys.* 99: 92-102.
17. Anderson, W.F., Kantoff, P., Eglitis, M., McLachlin, J., Karson, E., Zwiebe, J., Nienhuis, A., **Karlsson, S.**, Blaese, R.M., Kohn, D., Gilboa, E., Armentano, D., Zanjani, E.D., Flake, A., Harrison, M.R., Gillio, A., Bordignon, C., O'Reilly, R. (1986). Gene Transfer and Expression in non-human primates using retroviral vectors. *Cold Spring Harbor Symp.* 51: 2327-2341.
18. Karlsson, E., Papayannopoulou, Th., Schweiger, S.G., Stamatoyannopoulos, G., Nienhuis, A.W. (1987) Retroviral mediated transfer of genomic globin genes leads to regulated expression of RNA and protein. *Proc. Natl. Acad. Sci (USA)* 84: 2411-2415.
19. Epstein, N.D., **Karlsson, S.**, O'Brien, S., Modi, W., Nienhuis, A.W. (1987). Discovery of a new moderately repetitive DNA sequence family of novel organization. *Nucl. Acids Res.* 15. 2327-2341.
20. Garver, R.L., Chytil, A., **Karlsson, S.**, Fells, G.G., Brantly, M.L., Courtney, M., Kantoff, P.W., Nienhuis, A.W., Anderson, W.G., Crystal, R.G. (1987). Production of glycosylated, physiologically "normal" human I antitrypsin by mouse fibroblasts modified by insertion of a human I antitrypsin cDNA using a retroviral vector. *Proc. Natl. Acad. Sci. (USA)* 84: 1050-1054.
21. **Karlsson, S.**, Papayannopoulou, Th., Stamatoyannopoulos, G., Nienhuis, A.W. (1987). Regulated expression of human globin genes following

- transfer with retroviral vectors. In: *Developmental Control of Globin Gene Expression*, pp. 595-603, Eds. Stamatoyannopoulos, G. and Nienhuis, A.W. Allan R. Liss, N.Y.
22. Kantoff, P.W., Gillio, A.P., McLachlin, J.R., Bordignon, C., Eglitis, M.A., Kernan, N.A., Moen, R.C., Kohn, D.B., Yu, S.F., Karson E., **Karlsson, S.**, Zwiebel, J.A., Gilboa, E., Blaese, M., Nienhuis, A., O'Reilly, R.J., Anderson, W.F. (1987). Expression of Human Adenosine Deaminase in Nonhuman Primates after Retrovirus-mediated Gene Transfer. *J. Exp. Med.*, 166: 219-234.
 23. Nienhuis, A. W., Donahue, R.E., **Karlsson, S.**, Clark, S.C., Agricola, B., Antinoff, N., Pierce, J.E., Turner, P. Anderson, A.W., Nathan, D.G. (1987). Recombinant Human Granulocyte-Macrophage Colony-Stimulating Factor (GM-CSF) Shortens the Period of Neutropenia after Autologous Bone Marrow Transplantation in a Primate Model. *J. Clin. Invest.* 80: 5783-5777.
 24. Wong, P.M.C., **Karlsson, S.**, Chung, S. W., Holt, J., Redner, R., Turner, P., Nienhuis, A.W. (1987). Transfer of Genes into Hematopoietic cells using Retroviral Vectors. In: *Recent Advances in Leukemia and Lymphoma*, pp. 553-566. Alan R. Liss, Inc.
 25. Kohn, D.B., Kantoff, P.W., Eglitis, M.A., McLachlin, J.R., Moen, R.C., Karson, E., Zwiebel, J.A. Nienhuis, A., **Karlsson, S.**, O'Reilly, R., Gillio, A., Bordignon, C., Gilboa, E., Zanjani, E.D., Blaese, R.M. and Anderson, W.F.: Retroviral-Mediated Gene Transfer into Mammalian Cells. *Blood Cells* 13: 285-298, 1987.
 26. Nienhuis, A., Bodine, D., Browder, T., Chung, S.W., Dunbar, C., **Karlsson, S.**, Wong, P. (1988). Transfer of Genes into Hematopoietic cells with Retroviral Vectors. In: *Cell Physiology of Blood* pp. 79-89, Eds, R.B. Gunn & J.C. Parker. Rockefeller University Press, N.Y.
 27. **Karlsson, S.**, Bodine, D.M., Perry, L., Papyannopoulou, T., and Nienhuis, A.W.: (1988). Expression of the human beta-globin gene following retroviral-mediated transfer into multipotential hematopoietic progenitors of mice. *Proc. Natl. Acad. Sci. (USA)* 85: 6026-6066.
 28. Bodine, D.M., **Karlsson, S.**, Papayannopoulou, T. and Nienhuis, A.W. (1989). Expression of human beta globin genes introduced into primitive murine hematopoietic progenitor cells by retrovirus mediated gene transfer. In: *Hemoglobin Switching, Part B* pp 219-233, Eds, G. Stamatoyannopoulos and A.W. Nienhuis. Alan R. Liss, N.Y.
 29. Bodine, D.M., **Karlsson, S.** and Nienhuis, A.W. (1989). The combination of IL-3 and IL-6 preserves stem cell function in culture and enhances

retrovirus mediated gene transfer into hematopoietic stem cells. Proc. Natl. Acad. Sci., USA 86: 8897-8901.

30. Correll, P.H., Fink, J.K., Brady, R.O., Perry, L.K., **Karlsson, S.** (1989) Production of human glucocerebrosidase in mice following retroviral gene transfer into multipotential hematopoietic progenitor cells. Proc. Natl. Acad. Sci., (USA) 86: 8912-8916.
31. Fink, J.K., Correll, P.H., Perry, L.K., Brady, R.O., **Karlsson, S.** (1990). Correction of glucocerebrosidase deficiency following retroviral-mediated gene transfer into hematopoietic progenitor cells from patients with Gaucher disease. Proc. Natl. Acad. Sci., (USA) 87: 2334-2338.
32. Correll, P.H., Kew, Y., Perry, L.K., Brady, R.O., Fink, J.K., **Karlsson, S.** (1990). Expression of human glucocerebrosidase in long-term reconstituted mice following retroviral-mediated gene transfer into hematopoietic stem cells. Human Gene Therapy 1: 277-287.
33. **Karlsson, S.** (1991). Treatment of genetic defects in hematopoietic cell function by gene transfer. Blood 78: 2481-2492.
34. Abraham, N.G., Benz, E.J., **Karlsson, S.**, Clark, S.C. (1991). The Stem Cell Mavens had a Blast. New Biologist 4: 42-47.
35. Correll, P.H., Colilla, S., Brady, R.O., **Karlsson, S.** (1992). Expression of the glucocerebrosidase gene following transfer into primitive hematopoietic cells of mice. In: Molecular Biology of Hematopoiesis, Eds, Abraham, N.G., Marks, P. Levine, A.S., Konwalinka. G. Intercept Ltd., Andover, England, 335-343.
36. Correll, P.H., Colilla, S., Dave, H.P.G., Brady, R.O., **Karlsson, S.** (1992). Gene therapy of inherited disorders with special reference to Gaucher disease. In: Correction of certain genetic disorders by transplantation, Eds. J.R. Hobbs, P.G. Riches, The COGENT Trust, Ruislip, Middlesex, U.K., pp.142-155.
37. Correll, P.H., Colilla, S., Dave, H.P.G., **Karlsson, S.** (1992). High levels of human glucocerebrosidase activity in macrophages of long-term reconstituted mice after retroviral infection of hematopoietic stem cells. Blood 80: 331-336.
38. Kulkarni, A.B., Huh, C., Becker, D., Lyght, M., Geiser, A., Roberts, A.B., Sporn, M.B., Ward, J.M., **Karlsson, S.** (1993). Transforming growth factor-beta 1 null mutation in mice causes excessive inflammatory response and early death. Proc. Natl. Acad. Sci. USA 90: 770-774.

39. Freas, D., Correll, P.H., **Karlsson, S.**, Pluznik, D. (1993). Evaluation of expression of transferred genes in differentiating myeloid cells: Expression of human glucocerebrosidase in murine macrophages. *Human Gene Therapy*. 4: 283-290.
40. **Karlsson, S.**, Correll, P.H., Xu, L. (1993) Gene transfer and bone marrow transplantation with special reference to Gaucher's disease. *Bone Marrow Transplantation Suppl 1*, 11: 124-127.
41. Kulkarni, A., **Karlsson, S.** (1993). Transforming growth factor-beta 1 (TGF- β 1) knockout mice: A mutation in one cytokine gene causes a dramatic inflammatory disease. *Am. J. Pathology*. 143: 3-9.
42. Glick, A.B., Kulkarni, A., Tennenbaum, T., Hennings, H., Flanders, K.C., O'Reilly, M., Sporn, M.B., **Karlsson, S.**, Yuspa, S.H. (1993) Loss of TGF-beta expression in skin and skin tumors is associated with hyperproliferation and a high risk for malignant conversion. *Proc. Natl. Acad. Sci. USA*. 90: 6076-6080.
43. Geiser, A.G., Letterio, J.J., Kulkarni A.B., **Karlsson, S.**, Roberts, A.B., Sporn, M.B. (1993). TGF- β 1 controls expression of major histocompatibility genes in the post-natal mouse: Aberrant histocompatibility antigen expression in the pathogenesis of the TGF- β 1 null mouse phenotype. *Proc. Natl. Acad. Sci. USA*. 90:9944-9948.
44. **Karlsson, S.**, Xu, L., Correll, P., Kluepfel-Stahl, S. (1994). Towards gene therapy of Gaucher disease: Transfer of the glucocerebrosidase gene into primitive hematopoietic cells. In: *Molecular Biology of Hematopoiesis*, Vol 3, pp. 635-644, Eds. Abraham, N.G., Shadduck, R.K., Levine, A.S., Takaku, F. Intercept Ltd, Andover, Hampshire, U.K.
45. Kulkarni, A., Ward, J.M., Geiser, A.G., Letterio, J.J., Hines, K., Christ, M., D'Souza, R.N., Huh, C-G., Roberts, A.B., Sporn, M.B., McCartney-Francis, N., Wahl, S., Glick, A.B., Yuspa, S.H., Mackall, C., Gress, R., **Karlsson, S.** (1994). TGF- β 1 knockout mice: Immune dysregulation and pathology. In: *Molecular Biology of Hematopoiesis*, Vol 3, pp.749-757, Eds. Abraham, N.G., Shadduck, R.K., Levine, A.S., Takaku, F. Intercept Ltd, Andover, Hampshire, U.K.
46. Xu, L., Stahl, S. K., Dave, H.P.G., Schiffmann, R., Correll, P.H., Kessler, S., **Karlsson** (1994). Correction of the enzyme deficiency in hematopoietic cells of Gaucher patients using a clinically acceptable retroviral transduction protocol. *Experimental Hematology*. 22: 223-230.

47. Correll, P.H. and **Karlsson, S.** (1994). Towards therapy of Gaucher's disease by gene transfer into hematopoietic cells. *European J. Hematology* 253-264.
48. Hines, K. L., Kulkarni, A.K., McCarthy, J.B., Tian, H., Ward, J.M., Christ, M., McCartney-Francis, N.L., Furcht, L. T., Karlsson S., Wahl, S. (1994) Synthetic fibronectin peptides interrupt inflammatory cell infiltration in TGF- β 1 knockout mice. *Proc. Natl. Acad. Sci. USA.* 91: 5187-5191.
49. Freas-Lutz, D.L., Correll, P.H., Dougherty, S., Xu. L., Pluznik, D.H., **Karlsson, S.** (1994). Expression of human glucocerebrosidase in murine macrophages: Identification of efficient retroviral vectors. *Experimental Hematology* 22: 613-621.
50. Shafer, F. E., Dropulic, B., Ely, C., Schafer, C.A., Freas, D., Witkowska, H. E., Schechter, A. N., Noguchi, C.T., Dewey, M., **Karlsson, S.** (1994) Locus control region coupled β S-Antilles- and alpha2-hemoglobin genes selects for high alpha2-Hemoglobin expression in adult transgenic mice. *J. Biomed Sci* 1: 147-153.
51. Correll, P. H. Colilla, S., **Karlsson, S.** (1994) Retroviral vector design for long-term expression in murine hematopoietic cells in vivo. *Blood* 84: 1812-1822.
52. Christ, M., McCartney-Francis, N.L., Kulkarni, A.B., Ward, J. M., Mackall, C.L., Gress, R.E., Hines, K.L., Tian, H., **Karlsson, S.**, Wahl, S.M. (1994). Immune dysregulation in TGF- β 1 deficient mice. *J Immunol* 153: 1936-1946
53. Huh, C., Nagle, J.M., Kozak, C.A., Abrahamson, M., **Karlsson, S.** (1995). Structural organization, expression and chromosomal mapping of the mouse Cystatin C gene (Cst3). *Gene* 152: 221-226.
54. Glick, A.B., Lee, M.M., Darwiche, D., Kulkarni, A.B., **Karlsson, S.**, Yuspa, S.H. (1994). Targeted deletion of the TGF- β 1 gene causes rapid progression to squamous cell carcinoma. *Genes & Development* 8: 2429-2440.
55. Kulkarni, A.B., Ward, J.M., Yaswen, L., Mackall, C.L., Bauer, S.R., Huh, C., Gress, R. E., **Karlsson, S.** (1995). TGF- β 1 null mice: An animal model for inflammatory disorders. *Am J Pathol* 146: 1-12.
56. Martin, J., Dickson, M.C., Cousens, F., Kulkarni, A., **Karlsson, S.**, Akhurst, R.J. (1994). Analysis of homozygous TGF- β 1 null mouse embryos demonstrates defects in yolk sac vasculogenesis and haematopoiesis. *Ann. N.Y. Acad. Sci.* 172: 300-308.

57. Xu, L.C., Young, H. A., Blanco, M., Kessler, S., Roberts, A.B., **Karlsson, S.** (1994). Poor transduction efficiency of human hematopoietic progenitor cells by a high titer amphotropic retroviral producer cell clone. *J. Virology* 68:7634-7636.
58. Xu, L.C., Stahl, S., Blanco, M., Dunbar, C., **Karlsson, S.** (1995). Growth factors and stromal support generate very efficient retroviral transduction of peripheral blood CD34+ cells from Gaucher patients. *Blood* 86: 141-146 .
59. Medin, J.A., Migita, M., **Karlsson, S.** (1995). Gene therapy of enzyme and immune deficiencies in the hemopoietic system. In: *Cell Therapy*, eds Morstyn, G. and Sheridan, W. Cambridge University Press, New York, pp. 385-412.
60. Xu, L.C., **Karlsson, S.**, Byrne, E.R., Kluepfel-Stahl, S., Kessler, S.W., Agricola, B.A., Sellers, S., Kirby, M., Dunbar, C., Brady, R.O., Nienhuis, A.W., Donahue, R.E. (1995). Long-term in vivo expression of the human glucocerebrosidase gene in non-human primates using retroviral supernatant transduction protocols. *Proc Natl Acad Sci USA* 92: 4372-4376. .
61. Schiffmann, R., Medin, J.A., Ward, J.M., Stahl, S., Cottler-Fox, M., **Karlsson, S.** (1995). Transfer of the human glucocerebrosidase gene into hematopoietic stem cells of nonablated recipients: Successful engraftment and long-term expression. *Blood* 86:1218-1227.
62. Dickson, M.C., Martin, J.S., Cousins, F.M., Kulkarni, A.B., **Karlsson, S.**, Akhurst, R.J. (1995). Defective haematopoiesis and vasculogenesis in transforming growth factor β 1 knock out mice *Development* 121: 1845-1854.
63. **Karlsson, S.** (1994). Gene therapy of hematopoietic cells. In: Bok Davids, ed. O.G. Björnsson, Haskolautgafan, Haskoli Islands, Reykjavik, pp. 709-728.
64. Hakansson K., Huh, C., Grubb, A., **Karlsson, S.**, Abrahamsson, M. (1996). Mouse and rat Cystatin C. *Escherichia Coli* production, characterization and tissue distribution. *Comp Biochem Physiol* 114: 303-311.
65. Yaswen, L., Kulkarni, A.B., Frederickson, T., Mittelman B., Schiffmann, R., Payne, S., Longenecker, G., Mozes, E., **Karlsson, S.** (1996). Autoimmune manifestations in the TGF- β 1 knockout mouse. *Blood* 87: 1439-1445.

66. Migita, M., Medin, J.A., Pawliuk, R., Jacobson, S., Amiri, M., Humphries, R.K., **Karlsson, S.** (1995). Selection of transduced CD34+ progenitors and enzymatic correction of cells from Gaucher patients, using bicistronic vectors. *Proc Natl Acad Sci USA* 92: 12075-12079.
67. Medin, J., Migita, M., Jacobson, S., Pawliuk, R., Amiri, M. Humphries, R.K., **Karlsson, S.** (1996). A bicistronic therapeutic retroviral vector enables sorting of transduced CD34+ cells and corrects the enzyme deficiency in cells from Gaucher patients. *Blood* 87: 1754-1762.
68. **Karlsson, S.**, Medin, J.A., Migita, M., Stahl, S., Schiffmann, R. (1996). Towards gene therapy of Gaucher's disease. In: *Molecular Biology of Hematopoiesis*, Vol 4, ed. Abraham, NG.
69. Donahue, R.E., Byrne, E.R., Thomas, T.E., Kirby, M.R., Agricola, B.A., Sellers, S.E., Gaudernack, G., **Karlsson, S.**, Lansdorp, P.M. (1996). Transplantation and gene transfer of the human glucocerebrosidase gene into immunoselected primate CD34+Thy-1+ cells. *Blood* 88: 4166-4172.
70. Dunbar C, Kohn D, Karlsson S, Barton N, Brady R, Cottler-Fox M, Crooks G, Emmons R, Esplin J, Leitman S, Lenarsky C, Nolta J, Parkman R, Pensiero M, Schiffmann R, Tolstoshev P, Weinberg K (1996). Retroviral mediated transfer of the cDNA for human glucocerebrosidase into hematopoietic stem cells of patients with Gaucher Disease: a phase 1 study. *Hum Gene Ther* 7: 231-241.
71. Medin, J.A., **Karlsson, S.** (1997). Viral vectors for gene therapy of hematopoietic cells. *Immunotechnology* 3: 3-19.
72. Reiser, J., Harmison, G., Kluepfel-Stahl, S., Brady, R.O., **Karlsson, S.**, Schubert, M. (1996). Transduction of nondividing cells using pseudotyped defective high-titer human immunodeficiency virus type 1 particles. *Proc. Natl. Acad. Sci. USA* 93: 15266-15271.
73. Medin JA; Karlsson S (1997). Selection of retrovirally transduced cells to enhance the efficiency of gene therapy. *Proc Assoc Am Physicians* 109:111-119.
74. Kohn, D.B., Schuening, F., Dunbar, C., Pensiero, M., Kiem, H-P., Beach, K., Scott, R., Zaboikin, M., Miller, D., Storb, R., Darovsky, B., Wells, S., Nolta, J., Esplin, J., Barton, N., Brady, R.O., **Karlsson, S.** (1997). Early trials of gene transfer for Gaucher disease. In: *Jewish Genetic Diseases*, ed. R.J. Desnick. (in press).
75. **Karlsson, S.** (1997). Gene Therapy of Hematopoietic Cells. *J Int Med* 242: 95-99, (Suppl 740).

76. Kulkarni, AB, **Karlsson, S** (1997). Inflammation and TGF- β 1: Lessons from the TGF- β 1 null mouse. *Res in Immunol* 148: 453-456.
77. Dunbar, C.E., Kohn, D.B., Schiffman, R., Barton, N.W., Nolta, J., Esplin, J., Pensiero, M., Long, Z., Lockey, C., Emmons, R.V.B., Leitman, S., Krebs, C. B., Carter, C., Brady, R. O., **Karlsson, S.** (1998). Retroviral transfer of the glucocerebrosidase gene into CD34+ cells from patients with Gaucher disease: In vivo detection of transduced cells without myeloablation. *Human Gene Therapy* 9:2629-2640
78. Medin, J.M., Richter, J., **Karlsson, S.** (1998). Clinical Applications of gene therapy: Correction of genetic disease affecting hematopoietic cells. In: *Stem Cell Biology and Gene Therapy*, eds. PJ Quesenberry, GS Stein, BG Forget, SM Weissman, pp. 363-384, Wiley-Liss, New York
79. **Karlsson, S.**, Medin, J.A., Migita, M., Schiffmann, R., Stahl, S.K. (1998). Gene therapy of Gaucher disease. In: *Gene Therapy*, Fundacion BBV. Documenta, pp. 315-324, Fundacion BBV, Bilbao.
80. Sellers, S., Tisdale, J. F., Bodine, D.M., Williams, D.A., Karlsson, K., Meztger, M., Donahue, R.E., Dunbar, C.E. (1999). No discrepancy between in vivo gene marking efficiency assessed in peripheral blood populations compared to bone marrow progenitors or CD34+ cells. *Human Gene Ther* 10: 633-640.
81. Huh, C.-G., Håkansson, K., Nathanson, C-M, Thorgeirsson, U., Jonsson, N., Grubb, A., Abrahamsson, M., **Karlsson, S.** (1999). Decreased metastatic spread in mice homozygous for a null allele of the cystatin C protease inhibitor gene. *Molecular Pathology* 52: 332-340.
82. Kobayashi, S., Yoshida, K., Ward, J.M., Letterio, J.J., Longenecker, G., Yaswen, L., Mittelman, B., Mozes, E., Roberts, A.B., **Karlsson, S.**, Kulkarni, A. B. (1999). β 2-Microglobulin Deficient Background Ameliorates Lethal Phenotype of the TGF- β 1Null Mouse. *J Immunol* 163: 4013-4019.
83. Laine, M., Richter, J., Fahlman, C., Rapola, J., Renlund, M., Peltonen, L., **Karlsson, S.**, Jalanko, A. (1999). Correction of peripheral lysosomal accumulation in mice with aspartylglucosaminuria by bone marrow transplantation. *Exp. Hematol.* 27: 1467-1474.
84. Relander, T., Fahlman, C., **Karlsson, S.**, Richter, J. (2000). Low level of gene transfer to and engraftment of murine bone marrow cells from long term bone marrow cultures. *Exp Hematol* 28:373-381.

85. Rosenberg SA, Blaese RM, Brenner MK, Deisseroth AB, Ledley FD, Lotze MT, Wilson JM, Nabel GJ, Cornetta K, Economou JS, Freeman SM, Riddell SR, Brenner M, Oldfield E, Gansbacher B, Dunbar C, Walker RE, Schuening FG, Roth JA, Crystal RG, Welsh MJ, Culver K, Heslop HE, Simons J, Wilmott RW, Boucher RC, Siegler HF, Barranger JA, **Karlsson S**, Kohn D, Galpin JE, Raffel C, Hesdorffer C, Ilan J, Cassileth P, O'Shaughnessy J, Kun LE, Das TK, Wong-Staal F, Sobol RE, Haubrich R, Sznol M, Rubin J, Sorcher EJ, Rosenblatt J, Walker R, Brigham K, Vogelzang N, Hersh E, Eck SL. Human gene marker/therapy clinical protocols. *Hum Gene Ther.* 2000 ;11(6):919-79.
86. Fan, X., Brun A., Segrén, S, Jacobsen, S.E.W., **Karlsson, S.** (2000). Efficient adenoviral vector transduction of human hematopoietic SCID-repopulating and long-term culture-initiating cells. *Hum Gene Ther* 11:1313-1327.
87. Li, S-L, Chen, S, Xu, X, Sundstedt, A, Paulsson, KM, **Karlsson, S**, Sjögren, H-O, Wang, P. Cytokine-induced SH2 protein-1 (CIS 1) promotes T cell receptor mediated proliferation and prolongs survival of activated T cells (2000). *J Exp Med* 191: 985-994.
88. **Karlsson, S.** (2000). The first steps on the gene therapy pathway to anti-sickling success. *Nature Medicine* 6: 139-140.
89. Woods, N-B, Fahlman, C, Mikkola, H, Hamaguchi, I, Olsson, K, Zufferey R, Jacobsen, SEW, Trono, D, **Karlsson, S** (2000). Lentiviral gene transfer into primary and secondary NOD/SCID repopulating cells. *Blood* 96:3725-3733.
90. Hamaguchi, I., Woods, N-B, Panagopoulos, I., Andersson, E., Mikkola, H., Fahlman, C., Zufferey, R., Carlsson, L, Trono, D., **Karlsson, S.** (2000). Lentiviral vector gene expression during ES cell derived hematopoietic development in vitro. *J Virol* 74: 10778-10784.
91. Mikkola, H, Woods, N-B, Sjögren, M, Helgadottir, H, Hamaguchi, I, Jacobsen, SE, Trono, D, **Karlsson, S** (2000). Lentiviral gene transfer in murine hematopoietic stem cells is compromised by latency of proviral integration and results in vector mosaicism and heterogenous gene expression in the progeny cells. *J Virol* 74: 11911-11918.
92. Fan, X, Brun, A, **Karlsson, S** (2000) Adenoviral vector design for high-level transgene expression in primitive human hematopoietic progenitors. *Gene Therapy* 7:2132-2138.
93. Richter, J, **Karlsson, S.** (2001). Clinical gene therapy in hematology, the past and the future. *Int J Hematol* 73: 162-169.

94. Woods, N-B, Mikkola, H, Nilsson, E, Olsson, K, Trono, D, **Karlsson, S**. (2001). Lentiviral-mediated gene transfer into hematopoietic cells. *J Int Med* 249: 339-343.
95. Relander, T, Hawley, R, **Karlsson, S**, Richter, J (2001). Retroviral transduction of human CD34+ cells on fibronectin fragment CH-296 is inhibited by high concentrations of virus particles present in the medium-implications for design of gene transfer protocols for repopulating hematopoietic cells. *J Gene Med* 3: 207-218.
96. Larsson, J, Goumans M-J, Jansson-Sjöstrand L, van Rooijen M, Ward, D, Levéen, P, Xu, X, ten Dijke, P, Mummery, C L, **Karlsson, S** (2001). Abnormal vascular development, but intact hematopoietic potential in transforming growth factor- β receptor I deficient mice. *EMBO J* 20: 1663-1673.
97. Björnsson, JM, Andersson, E, Lundström, P, Larsson, N, Repetowska, E, Humphries, RK, **Karlsson, S** (2001). Proliferation of primitive myeloid progenitors can be reversibly induced by HoxA10. *Blood* 98: 3301-3308.
98. Fan, X, Valdimarsdottir, G, Larsson J, Brun, A, Magnusson, M, Jacobsen SE, ten Dijke, P, **Karlsson, S** (2002). Transient disruption of autocrine transforming growth factor- β signalling leads to enhanced survival and proliferation potential in single primitive human hematopoietic progenitor cells. *J Immunol* 168: 755-762.
99. Relander, T, **Karlsson, S**, Richter, J (2002). Oncoretroviral gene transfer of NOD/SCID repopulating cells using three different viral envelopes. *J Gene Med* 4: 122-132.
100. Woods, N-B, Ooka, A, **Karlsson, S** (2002). Development of gene therapy for hematopoietic stem cells using lentiviral vectors. *Leukemia* 16: 563-569.
101. Levéen, P, Larsson, J, Ehinger, M, Mikkola, H, Jansson, L, Xu, X, **Karlsson, S** (2002). Induced disruption of the transforming growth factor- β type II receptor gene in mice leads to a lethal inflammatory disease that is transplantable. *Blood* 100:560-568.
102. Karlsson S, Ooka A, Woods, N-B (2002). Development of gene therapy for blood disorders by gene transfer into haematopoietic stem cells. *Haemophilia* 8: 255-260.
103. Bjorgvinsdottir H, Bryder D, Sitnicka E, Ramsfjell V, De Jong I, Olsson K, Rusterholz C, **Karlsson S**, Jacobsen SE. Efficient Oncoretroviral Transduction of Extended Long-Term Culture-Initiating Cells and

- NOD/SCID Repopulating Cells: Enhanced Reconstitution with Gene-Marked Cells Through an Ex Vivo Expansion Approach. *Hum Gene Ther.* 2002 13(9):1061-73.
104. Hamaguchi, I, Ooka, A, Richter, J, Dahl, N, **Karlsson, S** (2002). Gene transfer improves erythroid development in ribosomal protein S19 deficient Diamond-Blackfan Anemia. *Blood* 100: 2724-2731
105. Fasth, A, Mellgren, K, **Karlsson, S**, Richter, J. (2002). Framgångsrik genterapi av barn med svår immunodefekt (in Swedish, transl. Successful gene therapy of children with severe immunodeficiency). *Läkartidningen* 99: 3010-3012.
106. Woods, N-B, Muessig, A, Schmidt M, Flygare, J, , Olsson, K, Salmon, P, Trono, D, von Kalle, C, **Karlsson, S** (2003). Lentiviral vector transduction of NOD/SCID repopulating cells results in multiple vector integrations per transduced cell: risk of insertional mutagenesis. *Blood* 101:1284-9
107. Richter, J, Relander T, Fasth A, **Karlsson S** (2003). Bakslag för geneterapi: Två fall av leukemi i fransk studie. *Läkartidningen* 100: 498-500
108. Hamaguchi, I, Nishiura, H, Flygare, J, Brun, A, Dahl, N, Richter, J, **Karlsson, S**. (2003). Multipotent hematopoietic progenitors from ribosomal protein S19 (RPS19) deficient Diamond-Blackfan anemia patients exhibit a proliferation deficiency which improves upon enforced expression of RPS19. *Molecular Therapy* 7: 613-622.
109. Björnsson JM, Larsson N, Brun, A, Magnusson, M Andersson, E, Lundström, P, , Larsson J, Repetowska E, Ehinger, M, Humphries, RK, **Karlsson S** (2003). Reduced proliferative capacity of hematopoietic stem cells deficient in Hoxb3 and HoxB4. *Mol. Cellular Biol.* 23: 3872-3883.
110. Schmidt M, Glimm H, Wissler M, Hoffmann G, Olsson K, Sellers S, Carbonaro D, Tisdale JF, Leurs C, Hanenberg H, Dunbar CE, Kiem HP, **Karlsson S**, Kohn DB, Williams D, Von Kalle C. Efficient characterization of retro-, lenti-, and foamyvector-transduced cell populations by high-accuracy insertion site sequencing. *Ann N Y Acad Sci.* 2003 May;996:112-21.
111. Larsson J, Blank, U, Helgadottir, H, Levéen P, Björnsson JM, Ehinger, M, Goumans, M-J, Fan X, **Karlsson, S** (2003). TGF- β signaling-deficient hematopoietic stem cells have normal self-renewal and regenerative ability *in vivo* despite increased proliferative capacity *in vitro*. *Blood* 102: 3129-3135.

112. Brun A, Fan X, Björnsson, JM, Humphries, RK, **Karlsson S** (2003). Enforced adenoviral vector-mediated expression of HOXB4 in human umbilical cord blood CD34+ cells promotes myeloid differentiation but not proliferation. *Molecular Therapy* 8: 618-628.
113. Goumans, M-J, Valdimarsdottir, G, Itoh, S, Larsson, J, Mummery, C, **Karlsson, S**, ten Dijke, P. (2003). Activin receptor-like kinase (ALK)-1 is an opposing transducer of lateral TGF- β /ALK-5 signaling. *Molecular Cell* 12:817-828.
114. Baum C, von Kalle C, Staal FJT, Li Z, Feshe, B, Schmidt M, Weerkamp F, **Karlsson, S**, Wagemaker, G, Williams, DA (2003). Chance and necessity: Insertional mutagenesis in gene therapy and its consequences. *Molecular Therapy* 9:5-13.
115. Kahn J, Byk, T, Jansson-Sjöstrand L, Petit, I, Shivtiel S, Nagler A, Hardan I, Deutch V, Gazit Z,, Gazit D, **Karlsson S**, Lapidot T (2003). Overexpression of CXCR4 on human CD34+ progenitors increases their proliferation, migration and NOD/SCID repopulation. *Blood* 103:2942-2949.
116. Nilsson M, **Karlsson S**, Fan, X (2004). Functionally distinct subpopulations of cord blood CD34+ cells are transduced by adenoviral vectors with serotype 5 or 35 tropism. *Molecular Therapy* 9:377-388.
117. Matsson H, Davey EJ, Drapchinskaia N, Hamaguchi I, Ooka, A, Levéen P, Forsberg E, **Karlsson S**, Dahl N (2003). Targeted disruption of the ribosomal protein S19 is lethal prior to implantation. *Mol. Cellular. Biol.* 24:4032-4037.
118. Nilsson M, Ljungberg, J, Richter, Kiefer, T, Magnusson, M, Lieber, A, Widegren, B, **Karlsson, S**, Fan, X (2004). A versatile system for generation of fiber retargeted adenoviral vectors: Efficient gene delivery into malignant hematopoietic cells. *J Gene Med* 6:631-641.
119. Brun A C M, Björnsson JM, Magnusson M, Larsson N, Ehinger M, Nilsson E, **Karlsson S** (2004). Hoxb4 deficient mice have normal hematopoietic development but exhibit a mild proliferation defect in hematopoietic stem cells. *Blood* 103: 4126-4133.
120. Sasaki, Y, Jensen, C, **Karlsson S**, Jacobsen, SEW (2004). Enforced expression of cyclin D2 enhances the proliferative potential of myeloid progenitors, accelerates in vivo myeloid reconstitution and promotes rescue of mice from lethal myeloablation. *Blood* 104:986-992.

121. Oe S, Lemmer ER, Conner EA, Factor VM, Levéen P, Larsson J, **Karlsson S**, Thorgeirsson SS (2004). Intact signaling by transforming growth factor beta is not required for termination of liver regeneration in mice. *Hepatology* 40:1098-105.
122. **Karlsson S**. Stem cell expansion: Success and complexities. *Blood* 104:2210-2211.
123. Chuva de Sousa Lopes S, Feijen A, Korving J, Korchynsky O, Larsson J, **Karlsson S**, tenDijke P, Lyons K, Goldschmeding R, Doevendaans P, Mummery C (2004). Connective tissue growth factor expression and Smad signaling during mouse heart development and myocardial infarction. *Dev Dyn* 231: 542-550.
124. Carvalho RLC, Jonker C, Goumans M-J, Larsson J, Bouwman P, **Karlsson S**, ten Dijke P, Arthur HM, Mummery CL (2004). Defective paracrine signaling by TGF- β in yolk sac vasculature of endoglin mutant mice: a paradigm for Hereditary Hemorrhagic Telangiectasia. *Development* 131:6237-47.
125. Flygare J, Kiefer T, Miyake K, Utsugisawa T, Hamaguchi I, Da Costa L, Richter J, Davey E, Mattsson H, Dahl N, Wizerowich M, Trono D, **Karlsson S** (2005) Deficiency of Ribosomal Protein S19 in CD34⁺ cells generated by siRNA blocks erythroid development and mimics defects seen in Diamond Blackfan anemia. *Blood* 105: 4627-4634.
126. Miyake K, Flygare J, Kiefer T, Utsugisawa T, Richter J, Ma Z, Wizerowicz M, Trono D, **Karlsson S** (2005). Development of Cellular Models for Ribosomal Protein S19 (RPS19) Deficient Diamond-Blackfan Anemia Using Inducible Expression of siRNA against RPS19. *Molecular Therapy* 11:627-637
127. Wurdak H, Ittner LM, Lang KS, Levéen P, Suter U, Fischer JA, **Karlsson S**, Born W, Sommer L (2005) Inactivation of TGF- β signaling in neural crest stem cells leads to multiple defects reminiscent of DiGeorge syndrome. *Genes and Development* 19:530-535.
128. Malm TM, Koistinaho M, Pärepallo M, Vatanen T, Ooka, A, **Karlsson S**, Koistinaho J (2005) Bone-marrow-derived cells contribute to the recruitment of microglial cells in response to β -amyloid deposition in APP/PS1 double transgenic Alzheimer mice. *Neurobiology of Disease* 18:134-142.
129. Larsson J, Blank U, Klintman, Magnusson M, **Karlsson S** (2005) Quiescence of hematopoietic stem cells and maintenance of the stem cell

pool is not dependent on TGF- β signaling in vivo. *Experimental Hematology* 33:592-596.

130. Karlsson G, Liu Y, Goumans M-J, Larsson J, Lee J-S, Thorgeirsson SS, Rignér M, **Karlsson S** (2005). TGF- β signals exclusively through receptor complexes involving ALK-5 in fibroblasts. *Physiological Genomics* 21: 396-403.
131. Larsson J, **Karlsson S** (2005). The role of Smad signaling in hematopoiesis. *Oncogene* 24:5676-5692.
132. Levéen P, Carlsén M, White-Makowska, A, Oddson S, Larsson J, Goumans MJ, Cilio CM, **Karlsson S** (2005). TGF- β type II receptor deficient thymocytes develop normally but demonstrate increased CD8+ proliferation in vivo. *Blood* 106: 4234-4240.
133. **Karlsson S** (2005). Gene therapy: Efficient targeting of hematopoietic stem cells. *Blood* 106:3333.
134. Johansson M, Jansson L, Ehinger M, Fasth A, **Karlsson S**, Richter J (2006). Neonatal hematopoietic stem cell transplantation cures oc/oc mice from osteopetrosis. *Exp Hematol* 34:242-249.
135. Miyake N, Brun ACM, Magnusson M, Miyake K, Scadden DT, **Karlsson S** (2005) HOXB4-induced self-renewal of hematopoietic stem cells is significantly enhanced by 21 deficiency. *Stem Cells* 24:653-661.
136. Ittner LM, Wurdak H, Schwerdtfeger K, Kunz T, Ille F, Leveen P, Hjalt T, Suter U, **Karlsson S**, Hafezi F, Born W, Sommer L (2005). Compound developmental eye disorders following inactivation of TGF- β signaling in neural-crest stem cells. *Journal of Biology* (in press), E-published 2005
137. Utsugisawa, T, Moody JL, Aspling M, Nilsson E, Carlsson L, **Karlsson S** (2005). A road map towards defining the role of Smad signaling in hematopoietic stem cells. *Stem Cells* 24: 1128-1136.
138. Mattsson H, Davey EJ, Fröjmark AS, Miyake K, Utsugisawa T, Flygare J, Zahou E, Byman I, Landin B, Ronquist G, **Karlsson S**, Dahl N (2006). Erythropoiesis in the Rps19 disrupted mouse: Analysis of erythropoietin response and biochemical markers for Diamond-Blackfan anemia. *Blood Cells, Molecules and Diseases* 36:259-264.
139. Dudas, M, Kim J, Li W-Y, Nagy A, Larsson J, **Karlsson S**, Chai Y, Kaartinen V (2006). Epithelial and ectomesenchymal role of the type I TGF- β receptor ALK5 during facial morphogenesis and palatal fusion. *Developmental Biology* 296:298-314.

140. Frutkin AD, Shi H, Levéen P, **Karlsson S**, Dichek D (2006). Use of SMMHC-Cre versus SM22-Cre mice. A critical developmental role for *tgfr2* in myogenic cell lineages is revealed in mice expressing SM22-Cre, not SMMHC-Cre. *Journal of Molecular and Cellular Cardiology* (in press).
141. Singbrant S, Moody J, Blank U, Karlsson G, Zwijsen A, **Karlsson S** (2006). *Smad5* is dispensable for adult murine hematopoiesis. *Blood* (in press).
142. Berglin-Enquist I, Nilsson E, Ooka A, Månsson J-E, Olsson K, Ehinger M, Brady RO, Richter J, **Karlsson S** (2006). Effective cell and gene therapy in a murine model of Gaucher disease. *Proc. Natl. Acad. Sci. USA* 103:13819-13824. E-publ Sept 5, 2006.
143. Blank U, Karlsson G, Moody J, Utsugisawa T, Magnusson M, Singbrant S, Larsson J, **Karlsson S** (2006). *Smad7* promotes self-renewal of hematopoietic stem cells in vivo. *Blood* (in press).
144. Flygare J, Aspesi A, Bailey JC, Miyake K, Caffrey JM, **Karlsson S**, Ellis SR (2006). Human RPS19, the gene mutated in Diamond Blackfan Anemia, encodes a ribosomal protein required for the maturation of 40S ribosomal subunits. *Blood* (in press).
145. Magnusson M, Brun ACM, Miyake N, Larsson J, Ehinger M, Björnsson JM, Wutz A, Sigvardsson M, **Karlsson S** (2006). Distinct hematopoietic cell fates are regulated by graded expression of *HoxA10*. Submitted for publication.
146. Karlsson G, Blank U, Moody JL, Ehinger M, Singbrant S, Deng C-X, **Karlsson S** (2006). *Smad4* is critical for self-renewal of hematopoietic stem cells. Submitted for publication.
147. Moody JL, Singbrant S, Karlsson G, Blank U, Aspling M, Flygare J, Bryder D, **Karlsson S** (2006). Endoglin is not critical for hematopoietic stem cell function but modulates progenitor sensitivity to TGF- β and impacts erythroid development. Submitted for publication.
148. Honjo Y, Nagieni CN, Larsson J, Rao NS, Hooks JJ, Chan C-C, **Karlsson S**, Kulkarni AB (2006). Conditional knockout of the TGF- β receptor 1 in the neuronal cells results in retinal detachment and cataract. Submitted for publication.

