DONALD BARRY KOHN, M.D.

August 28, 2024 **CURRICULUM VITAE**

A. PERSONAL INFORMATION

Business Address	Departments of Microbiology, Immunology & Molecular Genetics (M.I.M.G.); Pediatrics (Division of Hematology/Oncology); and Molecular & Medical Pharmacology The Broad Stem Cell Research Center University of California, Los Angeles David Geffen School of Medicine 3163 TLSB, 610 Charles E. Young Drive East Los Angeles, CA 90095
Business Phone	(310) 794-1964
Fax	(310) 206-0356
E-mail	dkohn1@mednet.ucla.edu
Citizenship	United States

B. EDUCATION

High School	New Trier West-Northfield, IL	1972
B.S. Biology	University of Illinois-Urbana	1976
M.S. Microbiology	University of Illinois-Urbana "Tumor protease-activated pro-drugs"	1978
M.D. Medicine	University of Wisconsin-Madison	1982

Board Certification:	American Board of Pediatric	1988
Medical Licensure:	Wisconsin California	1982 - 2021 1987-present

C. PROFESSIONAL EXPERIENCE

Present Position:

Distinguished Professor, Departments of Microbiology, Immunology & Molecular Genetics (MIMG); Pediatrics (Division of Hematology/Oncology); and Molecular and Medical Pharmacology; David Geffen School of Medicine at UCLA, University of California, Los Angeles (UCLA). 2009–present.

Previous Positions held:

Teaching Assistant, Department of Microbiology, University of Illinois-Urbana, 1976-77.

Research Assistant, Dept. of Microbiology, University of Illinois-Urbana, 1977-78.

Internship, Department of Pediatrics, University of Wisconsin-Madison, 1982-83.

Residency, Department of Pediatrics, University of Wisconsin-Madison, l983-85.

Fellowship, Immunology - Dr. R. Michael Blaese, Metabolism Branch (now Lymphoid Malignancies Branch), National Cancer Institute (NCI), National Institutes of Health (NIH), Bethesda, MD, l985-87.

Attending Physician, Bone Marrow Transplantation, Childrens Hospital Los Angeles 1987 – 2008.

Instructor, Department of Pediatrics, University of Southern California School of Medicine at Division of Research Immunology and Bone Marrow Transplantation, Childrens Hospital Los Angeles, 1987-89.

Assistant Professor, Departments of Pediatrics and Microbiology, USC School of Medicine at Division of Research Immunology and Bone Marrow Transplantation, Childrens Hospital Los Angeles, 1989-1993.

Associate Professor, Departments of Pediatrics and Molecular Microbiology & Immunology, USC School of Medicine at Division of Research Immunology and Bone Marrow Transplantation, Childrens Hospital Los Angeles, 1993-1997.

Professor, Departments of Pediatrics and Molecular Microbiology & Immunology, USC School of Medicine at Division of Research Immunology and Bone Marrow Transplantation, Childrens Hospital Los Angeles, 1997-2008.

Director, The John Connell Pediatric Gene Therapy Program, CHLA, 1994-2008.

Head, Division of Research Immunology and Bone Marrow Transplantation, USC KSOM Department of Pediatrics, Childrens Hospital Los Angeles, 2002-2008.

Director of the Gene, Immune and Stem Cell Therapy Program, The Saban Research Institute of Childrens Hospital Los Angeles, 2002-2008.

Interim Director, The Saban Research Institute, CHLA, 2007.

Director, UCLA Human Gene and Cell Therapy Program, 2009–2022.

D. Professional Activities

SOCIETY MEMBERSHIPS

American Association for the Advancement of Science – 1978.
Alpha Omega Alpha - 1981.
American Society for Microbiology - 1985.
American Society of Hematology – 1989-present.
Society for Pediatric Research - 1991.
American Society for Transplantation and Cellular Therapy- 1994- present.
American Federation for Clinical Research - 1995.
American Society of Gene and Cell Therapy – 1997 - present
Clinical Immunology Society – 1998 - present
American Pediatric Society – 2005.
European Society of Gene and Cell Therapy – 2008-present
European Society of Immunodeficiencies - 2020

1979 Medical student representative to the University of Wisconsin Medical School Educational Policy Committee

1993 Editorial Board: Human Gene Therapy 1993-present

1995-99 Member, Medical Biochemistry Study Section, National Institutes of Health

1995 Chair, Subcommittee on Gene Therapy, American Society for Blood & Marrow Transplantation

1996 Member, Scientific Review Board, National Gene Vector Laboratory

1997 Editorial Board: BLOOD 1997-2003

1997 Member of the Research Council, Childrens Hospital Los Angeles

1998 Patent-5,707,865 “Retroviral Vectors for Expression in Embryonic Cells”

1998 National Gene Vector Laboratories Steering Committee 1998-2008

1997- American Society of Gene and Cell Therapy:

Present Founding member
Chair, Subcommittee on Hematopoietic Stem Cells 1998-2000
Member, Educational Policy Committee 1998-2000
Member, Membership Committee 1998-2000
Member, Board of Directors 1998-2000
Chair, Membership Committee 1999-2001
Chair, Program Committee 2000-2001
Vice President 2001-2002
President-elect 2002-2003
President 2003-2004
Member, Advisory Council, 2004-2009; Chair, 2007-2009
Chair, Nominating Committee 2006
Member, Nominating Committee, 2007
Member, Publications Committee, 2005-2008
Chair, Publications Committee, 2009-2011
Member, Membership Committee, 2009-2011

Co-organizer, Clinical Trials Training Workshop, 2014
Member, Program Committee; Nominating Committee 2015-16
Member, ASGCT/ISCCR Clinical Trial Training Workshop Organizing Committee, 2015-16
Member, Governmental Affairs Committee, 2016-2019
Chair, ASGCT Cell Therapy Abstract review committee, 2017
Co-organizer, Clinical Trials Training Workshop, 2017
ASGCT Representative to ISCT Annual Meeting Program Committee, 2017
Co-Chair, ASGCT/FDA Gene Therapy Liaison Meeting, Sept. 13, 2018
Co-Organizer "Value Summit". Washington DC September 24, 2018
Member, 2019 ASGCT Strategic Planning Workgroup
Abstract Reviewer for Annual Meeting 2019-2024
Member, Nominating and Awards Committee 2020-2024
Member, Education Committee 2020-2024
Recipient, Outstanding Achievement Award, 2022

1999 Member-Institutional Biosafety Committee, CHLA 1999-2004

2000 Editorial Board: Experimental Hematology 2000-2002

2000 Editorial Board: Molecular Therapy 2000-2003

2000 Member-Search Committee for Chair, Department of Pediatrics, Childrens Hospital Los Angeles/Keck School of Medicine USC

2000 Member, Research Institute Pilot and Feasibility Fellowship Program Childrens Hospital Los Angeles -2005

2001 Member-National Institute of Health, National Center for Research Resources – Special Emphasis Panel - NGVL Renewal Contracts

2001 Policy and Admissions Committee – USC KSOM/Caltech MD/PhD and MSTP Program - 2006

2001 Member, Scientific Advisory Committee, NHLBI Center for Fetal Monkey Gene Transfer for Heart, Lung and Blood Diseases. California Regional Primate Research Center, University of California, Davis. 2001-2016.

2002 Editorial Board of Biology of Blood and Marrow Transplantation, 2002-2004

2002 Member, Childrens Hospital Los Angeles, Research Institute Education Committee –2008

2002 Member, Childrens Hospital Los Angeles, Academic Council – 2002-2009

2003 Member, Advisory Panel for the PID Network Consortium, University of Washington School of Medicine -2007

2004 Associate Editor for Molecular Therapy, 2004-06

2005 Member, USC Keck School of Medicine, Faculty Research Council 2005-08

2005-07 Faculty Representative, CHLA Grant Management Oversight Committee

2006 Chair, USC Stem Cell Institute Steering and Search Committee

2006-08 Member: USC Embryonic Stem Cell Research Oversight Committee

2006 US Immunodeficiency Network, member of Advisory Panel

2006 Patent 4-30224/GTI 1021 "Gene Therapy by Administration of Genetically Engineered CD34+ Cells Obtained from Cord Blood"

2006 Member, CHLA Medical Staff Committee

2006 Member, USC University Research Committee (Co-sponsored by Provost and Academic Senate) 2006-2008

2006 Elected as Active Member, American Pediatric Society (APS)

2006 Member, Review Panel for the Doris Duke Charitable Foundation's Clinical Scientist Development Award (CSDA), 2006-201

2006 Co-Chair, Cell and Gene Therapy Committee, Blood and Marrow Transplant Clinical Trials Network.

2007 Invited presenter, "Trans-NIH Workshop on Overcoming Barriers to Clinical Gene Therapy", Bethesda, MD

2008-2012 Member, External Advisory Committee, Northwest Genome Engineering Consortium, Seattle Children's Research Institute/University of Washington

2008-2024 Member, Primary Immune Deficiency Treatment Consortium (PIDTC) Steering Committee, Co-PI

2008- Member, National Gene Vector Biorepository Steering Committee -present

2009- Member, Broad Stem Cell Research Center, UCLA -present

2009- Member, Jonsson Comprehensive Cancer Center, UCLA –present

2009- Member, Molecular Biology Institute, UCLA -present

2009- Member, Advis Comm., Caltech-UCLA Joint Center for Translational Medicine

2010- Co-Organizer, 6th Annual Stem Cell Conference, Broad Center of Regenerative Medicine and Stem Cell Research at UCLA.

2010- Co-Organizer, BMT CTN Cell & Gene Therapy Sub-Committee Workshop:
"Status of Clinical Trial on Immunotherapy of B-Lineage Malignancies with CD19 Chimeric Antigen Receptors." Washington DC May 18, 2010.

2010- Elected Councilor, Clinical Immunology Society - 2013

2010- Member, National Institutes of Health, Office of Biotechnology Assessment,
2015 Recombinant DNA Advisory Committee (RAC). Chair 2013-2015.

2015 Gene Transfer Safety Assessment Board (GTSAB).

2010- Member, Scientific Advisory Committee for PPG P01 CA059350-18,
2012 Michel Sadelain, P.I., Memorial Sloan Kettering Cancer Center.

2010- Member, External Advisory Board for CIRM-funded Disease Team Projects
2014 UCLA (Irvin Chen, Gene Therapy for HIV) and City of Hope (John Zaia, Gene Therapy for HIV) and CIRM Stem Cell Training Program, COH.

2011 Member, UCLA Task Force on Clinical Testing of Intellectual Property

2011- Member, Data Safety Monitoring Board, UCLA Jonsson Comprehensive
2018 Cancer Center

2011-14 Member, Membership Committee, Clinical Immunology Society

2013- President-elect, Clinical Immunology Society. Chair, Publication Sub-Committee

2013- Member, Scientific Advisory Board, HIV Immunotherapeutics Institute,
AIDS Healthcare Foundation

2013- Member, National Institute of Arthritis and Musculoskeletal and Skin
2019 Diseases (NIAMS) Data Safety Monitoring Board for the study, *Vascular Delivery of alpha-Sarcoglycan for LGMD2D*, Jerry Mendell, M.D., PI.

2013- Member, Chair, Data Safety Monitoring Committee; EZN-2279

2020 (Polyethylene glycol recombinant adenosine deaminase [PEG-rADA]), Sigma-Tau/Leadiant Pharmaceuticals, Inc.

2014- President, Clinical Immunology Society

2015- Past President, Clinical Immunology Society
Chair, Publications Committee; Chair, Nominations Committee

2015- UCLA-UCI Alpha Stem Cell Clinic Internal Advisory Committee (IAC)

2015 Member, External Advisory Committee – NHLBI Review of Gene Therapy Resource Programs. Rockville MD December 11, 2105

2016- Member, External Advisory Board, SCID-NET, a European Commission

2018 Research & Innovation multinational cooperative research group

2017- International Society for Cellular Therapy (ISCT):

2019 Member, Immuno- and Gene Therapy Committee
Member 2018 Annual Meeting Organizing Comm. (ASGCT/ISCT session)

2017 Member, Review Panel for the Doris Duke Charitable Foundation's Clinical Scientist Development Award (CSDA).

2017- Member, CIRM Clinical Advisory Panel (CAP) to: Award# DR2-05365. "A monoclonal antibody that depletes stem cells." PI: J. Shizuru, Stanford University.

2018 Member, Bone Marrow Transplant Clinical Trials Network (BMT CTN). Non-Malignant Disease Committee

2018-present Chair, Data Safety Monitoring Board for "Gene Correction of Autologous Hematopoietic Stem Cells in Artemis Deficient SCID" from Mort Cowan, M.D., U.C.S.F.

2018 Coordinating Reviewer, Abstracts on "Gene Therapy and Transfer" for the 60th ASH Annual Meeting

2018 Member, Advisory Board for the Eli and Edythe Broad Center for Regenerative Medicine and Stem Cell Research at USC

2018 Panelist, FDA-ASH Sickle Cell Disease Clinical Endpoints Workshop. "Endpoints for How to Measure Cure" Rockville MD Oct 17-18, 2018.

2019 Member, ad hoc Subcommittee on Genetically-modified HPCs. Foundation for Accreditation of Cellular Therapy (FACT).

2019 Member, Abstract review committees: ISCT, ASGCT, ESGCT.

2019- Member, California NanoSystems Institute, University of California, Los Angeles

2019 Member, Data Safety Monitoring Board for "Haematopoietic Stem Cell Gene Therapy for the Wiskott Aldrich Syndrome (WAS)." Sponsored by Genethon, Evry France.

2019- Chair, Data Safety Monitoring Board for: Protocol LBI-2279-004, "Single Arm, Open-Label, Multicenter, Registry Study of Revcovi (elapegademase-lvlr) Treatment in ADA-SCID Patients Requiring Enzyme Replacement Therapy", Leadiant Biosciences Inc, Gaithersburg MD.

2019 *Ad hoc* reviewer of grant applications to Prinses Beatrix Spierfonds (Netherlands) and Bloodwise (UK).

2020 Member, Search Committee for Director of the UCLA Eli & Edythe Broad Center for Regenerative Medicine and Stem Cell Research

2020 Member, Ad hoc Gene Therapy Task Force, Center for International Blood and Marrow Transplant Research (CIBMTR)

2020 Member, Scientific Advisory Board, Myogene Bio LLC

2020 Member, Scientific Advisory Board, Pluto Immunotherapeutics

2021 Member, Scientific Advisory Board, ImmunoVec

2021 Abstract reviewer, European Society of Gene and Cell Therapy

2021- Member Institute for Genomic Innovation (IGI) Affordability Task Force

2021- Chair, Data Safety Monitoring Board, "Phase I/II, non-randomised, open label study of pCCL-Chim-p47 (Lentiviral vector transduced CD34+ cells) in patients with p47 Autosomal Recessive Chronic Granulomatous Disease (AR-CGD)", Great Ormond Street Hospital for Children NHS Foundation Trust

2021- Member, Data Safety Monitoring Board, "TCRα/β$^+$ T-cell/CD19$^+$ B-cell depleted hematopoietic grafts and a reduced-intensity preparative conditioning regimen containing JSP191 to achieve engraftment and blood reconstitution in patients with Fanconi Anemia; BMT373." Stanford University.

2022 *Ad hoc* reviewer for the Doris Duke Charitable Foundation 2022 Clinical Scientist development Awards

2022 Abstract reviewer. 25th Annual meeting of the American Society of Gene and Cell Therapy (ASGCT). Hematologic and Immunologic Diseases section.

2022 *Ad hoc* reviewer for National Heart, Lung and Blood Institute – Division of Intramural Research -2022 Board of Scientific Counselors Spring Site Visit Virtual Meeting. April 25-26, 2022.

2022 Member, Cellular, Tissue and Gene Therapies Advisory Committee of the Food and Drug Administration (FDA)

2023 Co-Organizer (with Dr. Melissa Spencer) Gene & Cell Therapy Mini-Symposium. UCLA May 15, 2023

E. HONORS AND SPECIAL AWARDS

1971 National Merit Scholarship Semi-Finalist

1972 Edmund J. James Scholar, University of Illinois-Urbana

1980 Advanced with honors, second year of medical school

1981 Advanced with honors, third year of medical school

1981 Elected to Alpha Omega Alpha medical honors society

1981 President of senior medical school class

1982 Harry A. Waisman Award for senior medical student who best demonstrated interest and ability in academic pediatrics

1988 American Board of Pediatrics

1989 Basil O'Conner Starter Fellowship Research Award
March of Dimes Birth Defects Foundation

1991 Junior Faculty Research Award, American Cancer Society

1993 Ross Young Investigator Award, Western Society for Pediatric Research

1993 H. Russell Smith Award for Innovation in Pediatric Biomedical Research

1994 Awarded Endowed Chair in Gene Therapy, Childrens Hospital Los Angeles

1996-2001 Recipient, Elizabeth Glaser Scientist Award, Pediatric AIDS Foundation

2000 Recipient, Doris Duke Distinguished Clinical Scientist Award 2000-2007

2004 USC Mellon Award for Faculty to Faculty Mentoring

2008 Robert M. McAllister Faculty Mentoring Award, from the Department of Pediatrics, Childrens Hospital Los Angeles, USC Keck School of Medicine

2011- University of Wisconsin Medical Alumni Association – Medical Alumni Citation Award, April 29, 2011.

2018 Lifetime Achievement Award, the Pediatric Blood & Marrow Transplant Consortium (PBMTC).

2018 Recipient, 2018 Research Excellence Award from the Inflammation, Immunology, Infectious Diseases or Transplantation (I3T) Research Program, David Geffen School of Medicine at UCLA. Los Angeles CA, October 23, 2018.

2021 International Society for Cell Therapy (ISCT) Career Achievement Award in Cell & Gene Therapy

2022 Outstanding Achievement Award - American Society of Gene and Cell Therapy

2023 Maureen Andrew Mentoring Award - Society for Pediatric Research (SPR)

2024 Founders Medal Recipient – Clinical Immunology Society

2024 2024 Boyle Research Achievement Award, The Immune Deficiency Foundation

E. RESEARCH GRANTS AND FELLOWSHIPS RECEIVED

Present Support

National Institute of Allergy and Infectious Diseases, (R01AI152068) “BCL11B activation as an approach for enhancing the efficacy of immunotherapy”, DBK-Co-I investigator PI C. Parekh, Childrens Hospital Los Angeles., 02/11/2021-01/12/2026, $2,612 direct costs for UCLA

National Heart, Lung and Blood Institute, University of California, San Francisco (R01 HL161291) "Developing gene therapy strategies to treat alpha thalassemia”. DBK Co-i, (T. MacKenzie, P.I.), 01/01/2022-12/31/2026, Total Award: $1,185,160

National Heart, Lung and Blood Institute (prime), Boston Children’s Hospital (OT2HL-154815) “Phase 2 Study of Hematopoietic Stem Cell Gene Transfer Inducing Fetal Hemoglobin in Sickle Cell Disease”, Co-Investigator (D.A. Williams PI), 2/1/2022-12/31/2024, Total Award $1,039,813

California Institute for Regenerative Medicine (CIRM) (DR3-06945) “Clinical Trial of Stem Cell Gene Therapy for Sickle Cell Disease”. DBK PI., 7/1/14-6/30/26, $2,364,631 Annual DC.

California Institute for Regenerative Medicine (CIRM) (CLIN2-09339) “Efficacy and safety of cryopreserved autologous CD34+ HSC transduced with EFS lentiviral vector encoding for human ADA gene in ADA-SCID subjects”. DBK PI, 02/01/22-06/30/26, $1,466,333 Annual DC

California Institute for Regenerative Medicine (CIRM) (TRAN1-11345) “Autologous MPO Knock-Out Hematopoietic Stem and Progenitor Cells for Pulmonary Arterial Hypertension”. DBK PI. . 07/01/2022-12/31/24 Total Award $5,207,433.

California Institute for Regenerative Medicine (CIRM) (DISC2-13405). “Hematopoietic Stem Cell Gene Therapy for α-Thalassemia”. DBK: PI. 09/01/22-08/31/24 Total Award $1,323,007.

California Institute for Regenerative Medicine (CIRM) (TRAN1- 15252) “Hematopoietic Stem Cell Gene Therapy for X-Linked Agammaglobulinemia” DBK PI. 02/01/2024 – 01/31/26, Total Award $4,822,285

California Institute for Regenerative Medicine (CIRM) (DISC0-14458) “Overcoming Barriers for Airway Stem Cell Gene Therapy for Cystic Fibrosis”. Co-Investigator (P.I. B. Gomperts, UCLA), 07/01/2023-12/31/25, Total Award $1,500,000.

California Institute for Regenerative Medicine (CIRM) (DISC2-13415) “Defining the Optimal Gene Therapy Approach of Human Hematopoietic Stem Cells for the Treatment of Dedicator of Cytokinesis 8 (DOCK8) Deficiency.” Co-investigator (P.I. C. Kuo, UCLA). Total award: $1,386,232

California Institute for Regenerative Medicine (CIRM) (CLIN1-14602). “Clinical Translation of Autologous Regenerative Pluripotent Stem Cell Therapy for Blindness”, Co-Investigator (S. Schwartz, P.I., UCLA), 6/1/23-5/31/25, Total Award $6,951,922.

California Institute for Regenerative Medicine (CIRM) (TRAN1-15257), “Adenine Base Editing for Autologous Hematopoietic Stem Cell Gene Therapy of CD3δ SCID” Co-Investigator (PI: Z. Romero Garcia, UCLA), 01/01/2024-06/30/26. Total Award $4,822,285.

California Institute for Regenerative Medicine (CIRM) (CLIN2SACD-1212031, Boston Children’s Hospital “Phase 2 Study of Hematopoietic Stem Cell Gene Transfer Inducing Fetal Hemoglobin in Sickle Cell Disease”. UCLA Site PI (D. Williams, P.I., Boston Children’s Hospital) 2/1/2022-6/30/2026. Total Award: $243,816

California Institute for Regenerative Medicine (C.I.R.M.) (CLIN2SCD-11722) “Transplantation of CRISPR-CAS9 Corrected Hematopoietic Stem Cells (CRISPR_SCD001) in Patients with Severe Cell Disease”. Co-investigator (M. Walters, P.I., UCSF), 2/1/22-1/31/26. Total sub-contract Award: $810,742

California Institute for Regenerative Medicine (CIRM) (CLIN1-13985).“Development of TriLeukeVax, an Engineered Autologous Leukemia Vaccine for Stimulating Cytolytic Immune Responses to Residual Leukemic Stem Cells” Co-Investigator (K Gaensler, PI, UCSF), 3/01/2022-12/31/24, Total Award $6,000,000.

California Institute for Regenerative Medicine (CIRM) (TRAN1-15230) “Treatment of Danon disease using ex vivo gene-modified hematopoietic stem cell transplantation” . Co-Investigator (Eric Adler PI, UCSD) , 01/01/2024-06/30/26, Total award: $5,238,786

California Institute for Regenerative Medicine (CIRM) (DISC2-14130) “A Treatment for Artemis-deficient Severe Combined Immunodeficiency using Non-Viral CRISPR-driven Safe Harbor Transgenesis in Hematopoietic Stem Cells” Co-I. (Fyodor Urnov PI, UCB). 10.01.23-9/31/25.

California Institute for Regenerative Medicine (C.I.R.M.), Rocket Pharma.(2022-1116) “Long term follow-up (LTFU) for gene therapy of leukocyte adhesion deficiency (LAD-1) Phase I/II clinical study to evaluate the safety and efficacy of the infusion of autologous hematopoietic stem cells transduced with a lentiviral vector encoding the *ITGB2* gene.” (Rocket Pharma PI), 9/13/2021- 9/12/2026, Total Award $581,558.

Great Ormond Street Hospital NHS Foundation Trust (United Kingdom) “Clinical Gene Therapy for ADA SCID - Sponsored Research Agreement between UCLA (Kohn Lab) and Great Ormond Street Hospital”. DBK PI, 10/1/2021-9/30/2024, Total Award: $97,542 a.d.c.

Transformatx Biotherapeutics (SRA 2022) “Development of Lentiviral Vectors for Angelman’s Syndrome”. DBK PI. 10/01/2023-09/31/24. Total Award: $1,214,606

Immune Deficiency Foundation (IDF) (2023 IDF Michael Blaese Research Grant Award) “Gene Editing of Hematopoietic Stem Cells to Treat X-Linked Agammaglobulinemia” DBK: P.I.. 06/01/2023-05/31/24, total award: $50,000

Canadian Institute of Health and Research (CIHR) (CLP 184944) “A pilot project to demonstrate feasibility of a Canadian gene therapy clinical trials platform for rare genetic diseases: Gene therapy for CD3δ severe combined immune deficiency.” Role: Co-Investigator (Nicola Wright, PI, University of Alberta), 3/01/23-02/28/25, total award: $1,795,463

Past Support:

National Institutes of Health (NIH) Cellular and Molecular Biology Training Grant, University of Illinois, Urbana, Dept. of Microbiology. 1977-78, $12,000.

Wisconsin Clinical Cancer Center Clinical Assistantship, 1979, $2,000.

American Association of Immunologists (AAI) Travel Grant, VI International Immunology Congress, I986, $500.

Childrens Hospital of Los Angeles, "Retroviral-Mediated Gene Transfer, Expression in Human Hematopoietic Cells," PI, 03/01/89-02/28/91, $40,000 Annual DC.

March of Dimes Birth Defects Foundation Basil O’Conner Starter Fellowship Research Award, #5-735 "Retroviral-Mediated Transfer Of The Human Glucocerebrosidase Gene" PI, 09/01/89-08/31/91, $40,000 Annual DC.

National Institutes of Health (1 RO1 AI29125): "Retroviral Vector-Mediated Transfer of the *nef* Gene to Inhibit HIV," PI, 10/01/89-09/30/92, $94,587 Annual DC.

American Cancer Society-Junior Faculty Research Award (JFRA-62074): "Gene Transduction of Hematopoietic Cells" PI, 01/01/91-12/31/93, $32,000 Annual DC.

March of Dimes, (#1-FY92-0551) "Vectors for Optimal Gene Expression After Hematopoietic Stem Cell Transduction" PI, 07/01/92-06/30/94, $47,273 Annual DC.

National Institutes of Health - RFA Gene Therapy for Cancer, "Cellular Transduction for Drug Delivery in Cancer” Director (Core), 09/30/92-09/29/94, $55,706, Annual DC.

Past Support (Continued):

National Institutes of Health (1 PO1 NS26991): "AIDS-Encephalopathy Multiple Disciplinary Program" (Leslie P. Weiner, Program PI), "Retroviral Vectors to Express HIV Antisense in the CNS," PI, 01/01/89-12/31/94, $104,546, Annual DC.

National Institutes of Health (RFA 89-AI-19 AI25959): "Development and Delivery of Antiviral RNA for AIDS" (John A. Zaia, Program PI, $5,675,340), "Anti-HIV Ribozyme Transduction by Retroviral Vectors," PI, 09/01/90-08/31/95, $90,962 Annual DC.

Pediatric Aids Foundation Stem Cell Gene Therapy with Ribozymes and RRE Decoys PI, 1995-1996, $100,000 Annual DC.

National Institutes of Health (1RO1DK42694) "Glucocerebrosidase Gene Expression in Hematopoietic Cells" P I, 09/01/91-08/31/96 $108,221 Annual DC.

National Institutes of Health (1RO1 DK49000) “Modified Retroviral Vectors for Stable Gene Expression” PI, 10/01/94-09/30/97, $87,974 Annual DC.

National Institutes of Health (CA59318-05) - “Cellular Transduction for Drug Delivery in Cancer” Dr. Kedes - Program Director, “Modified Retroviral Vectors for Persistent Gene Expression” PI, 08/11/95 - 05/31/99, $94,318 Annual DC.

National Institutes of Health (1U19AI36606) ”Molecular Genetic Interventions for Pediatric Aids.” Program Leader Dr. G. Nabel “Bone Marrow Gene Therapy for Pediatric AIDS” PI, 09/01/94 - 08/31/98, $224,800 Annual DC.

National Institutes of Health (CA59318-05) “Cellular Transduction for Drug Delivery in Cancer.” Program Director Dr. Kedes - Vector Production Core, Core Leader, 08/11/95 - 05/31/99, $71,134 Annual DC.

National Institutes of Health (1U19 A138592) “Transduction of Hematopoietic Stem Cells Using Ribozymes for AIDS.” Program Director Dr. Zaia “Transduction of Hematopoietic Stem Cells by Anti-HIV-1 Ribozymes” PI, 09/01/95 - 08/31/99, $116,581 Annual DC.

National Institutes of Health (1 R01 A141959-01) - “Inactivation of Chemokine Co-Receptors for HIV-1 Gene Therapy” Co-PI, 09/01/98-08/31/00 $49,020 Annual DC.

National Institutes of Health (1P50 HL54850) - “Specialized Center for Research on Hematopoietic Stem Cell Biology.”, Director Dr. Parkman, “Stem Isolation and Transduction” Core PI, 09/30/95 - 09/29/00 $119,455 Annual DC.

Pediatric Aids Foundation - Elizabeth Glaser Scientist Award “Stem Cell Gene Therapy with Ribozymes and RRE Decoys”, PI 01/01/96-12/31/2000, $119,955 Annual DC.

Leukemia and Lymphoma Society - “Gene Modification of Leukemia Cells to Induce Anti-Leukemic Immunity” PI, 09/01/97-08/31/01 $97,300 Annual DC.

National Institutes of Health (1 P01 A146030-01) - “Anti-HIV RNA Transduction into Blood Stem Cells in AIDS” Program Director Dr. J. Zaia, “Anti-HIV-1 Gene Therapy Using Hematopoietic Stem Cells” PI, 09/01/99-07/31/03 $152,425 Annual DC.

National Institutes of Health (1RO1 DK56287-01) “Modified Retroviral Vectors for HSC Gene Therapy” PI, 07/01/99-07/20/01 $135,843 Annual DC.

National Institutes of Health (PAR-97-080) “Hematopoietic Stem Cells for Gene Therapy of Pediatric AIDS” Program Leader G. Nabel - “Novel HIV Therapies: Integrated Preclinical/Clinical Program, 06/01/98-05/31/02, $150,000 Annual DC.

National Institutes of Health (1R01 DK54566-01). ”Gene Therapy for Hurler’s Disease” PI, 07/01/98-06/3/02 $200,000 Annual DC.

Past Support (Continued):

National Institutes of Health (P01 NIH HL-96-002B) “Comprehensive Sickle Cell Center: Pathophysiology and Treatment of Vascular Occlusion in Sickle Cell Disease” Program Director Cage S. Johnson - “In Vitro and In Vivo Models of Human Erythropoiesis” PI, 04/01/98-03/31/03 $105,038 Annual DC.

National Institutes of Health (1 R21 DK62649-01) – “Gene Expression in Beta-Cells by Lentiviral Vectors” PI, 07/01/002-06/30/04 $100,000 Annual DC.

TJ Martell Foundation “Gene Therapy for HIV-1”, PI. Seeger 1/1/3-12/31/03 $79,027 Annual DC.

Immune Deficiency Foundation “Lentiviral vectors using the Wiskott-Aldrich Syndrome protein promoter” PI, 01/01/01-12/31/03 $50,000 Annual DC.

US Immunodeficiency Network (N01A1-30070) – “A Site-Specific Gene Therapy Method to Ameliorate Primary Immune Deficiency” PI, 9/1/04 – 12/31/06 $100,000 Annual DC.

National Institutes of Health (1 P01 CA59318). “Cellular Transduction in Cancer” Director, Dr. L. Kedes, PI- Proj “Vector Expression Silencing and Methylation” PI-Core “Vector Development Core” PI, 07/20/01 – 02/28/07 $254,696 Annual DC.

National Institutes of Health (1 RO1 AI52798-01) – “Lentiviral Vector Transfer to Hematopoietic Stem Cells” PI, 06/01/02-05/31/07 $378,035 Annual DC.

National Institutes of Health (1R34 A1069947-01) “Lentiviral Vector for HIV-1”, PI, 05/01/06 – 04/30/07 $75,000 Annual DC.

Juvenile Diabetes Research Foundation (17-2006-1137) “Lentiviral Vectors to Characterize Beta Cell Differentiation, PI 01/01/07 – 12/31/07 $50,000 Annual DC.

Doris Duke Charitable Foundation, Distinguished Clinical Scientist Award – “Gene Therapy for Blood Diseases Using Hematopoietic Stem Cells” PI, 12/15/00 – 12/14/07 $274,576 Annual DC.

National Institutes of Health (3 MO1 RR0043-35S1) “General Clinical Research Center” Director Dr. Weinberg, Clinical Gene Therapy Core, Core Director, 12/01/97-11/30/11 $125,000 ADC.

National Institutes of Health (1P50 HL54850) “Specialized Center for Research on Hematopoietic Stem Cell Biology” Director Dr. Parkman, Core B “Stem Isolation and Transduction” Core PI, 09/30/95 - 09/29/09 $147,351 Annual DC.

California Institute for Regenerative Medicine (CIRM). “Stem Cell Training Grant” PI, 05/01/06-04/30/09 $724,493 Annual DC.

California Institute for Regenerative Medicine. Disease Team Planning Grant: “Stem Cell Gene Therapy for Sickle Cell Disease”. PI, 08/01/08-01/31/09 $31,110.00Annual DC.

National Institutes of Health (1P50 HL085036) Center for Pediatric Stem/Progenitor Cell Research” (Director Dr. A Tarantal- UCD); DBK role: Vector Core Leader, 09/29/05 – 08/31/09 $139,733 Annual DC.

Juvenile Diabetes Research Foundation (JDRF #35-2008-622). “Modeling Pancreas from Human Embryonic Cells”. (PI: A.Hayek, UCSD). DBK role: Director, Vector Core B. 9/1/08-8/31/11. $65,000 Annual DC.

California Institute for Regenerative Medicine – Early Translational Award, (TR1-01269) “In Utero Model to Assess the Fate of Transplanted Cells for Translational Research and Pediatric Therapies.” (PI: A.Tarantal UC Davis). DBK role: Director, Vector Core. 9/01/09-08/31/12. $60,000 Annual DC.

Past Support (Continued):

Doris Duke Charitable Foundation. 2009 Innovations in Clinical Research Award. "β-globin Gene Correction in Hematopoietic Stem Cells for Sickle Cell Disease." PI. 01/01/2010-12/31/2012. $149,824 Annual DC.

National Institutes of Health-FDA (1 R01 FD003005-01) – "MND-ADA Transduced CD34+ Cells for ADA-SCID" PI. 9/30/05 – 2/29/13 $300,000 Annual DC.

National Institutes of Health-NIAID (1 R01 AI074043) "In Vivo ADA Gene Delivery for the Treatment of SCID" PI. 06/01/07-05/31/13 $318,011 Annual DC.

California Institute for Regenerative Medicine- Disease Team Award (#DR1-01452) "Stem Cell Gene Therapy for Sickle Cell Disease" DBK PI. 02/01/10-06/30/14, $1,567,038 Annual DC.

National Institutes of Health-NCI (1 PO1 CA132681 (PI: D.Baltimore – California Institute of Technology) "Stem Cell Engineered Tumor Immunity in Man." DBK role: Project 3 PI:

"Transduction of hematopoietic stem cells for enhanced immunotherapy of melanoma" 04/15/10-2/28/15, $197,214 Annual DC.

National Institutes of Health-NHLBI (2P01 HL073104-01) "Gene Therapy Using Hematopoietic Stem Cells" Program Director- Donald B. Kohn, 04/01/03-06/31/15, $1,231,885 Annual DC.

National Institutes of Health-NIAID (1 U01 AI087628 (PI: D.Williams – Boston Children's Hospital) "Gene Therapy for SCID-X1 using a Self-Inactivating Gammaretroviral Vector." DBK role: Clinical Site PI. 5/09/10-8/08/15, $124,219 Annual DC.

Doris Duke Charitable Foundation – 2013 Innovations in Clinical Research Award "Site-Specific Gene Modification in Hematopoietic Stem Cells for Sickle Cell Disease." DBK – PI. 09/01/13-0831/16, $150,000 Annual DC

California Institute for Regenerative Medicine (CIRM) (TR4-06823). "Beta-Globin Gene Correction of Sickle Cell Disease in Hematopoietic Stem Cells." DBK PI. 12/01/2013 - 11/30/2016, $398,551 Annual DC.

Social and Scientific Systems, Inc./NIH-NHLBI (CRB-SSS-S-15-004351 1840). "A Phase I/II, Non-Randomized, Multicenter, Open-Label Study of G1XCGD (Lentiviral Vector Transduced CD34+ Cells) in Patients with X-linked Chronic Granulomatous Disease." DBK PI. 05/01/15-05/31/17.

Biogen Idec – Sponsored Research Agreement. "Improving Homology Directed Repair (HDR) In Hematopoietic Stem Cells (HSC)." DBK - PI, 12/15/15-12/14/18.

Doris Duke Charitable Foundation - Collaborations in Sickle Cell Disease "Direct Comparison of Gene Therapy Approaches to Treating Sickle Cell Disease." DBK PI, 11/1/16-10/31/17. $33,333

National Institutes of Health-NIAID (U01 AI100801) "EFS-ADA Lentiviral Vector Transduction of Bone Marrow CD34+ Cells for ADA-SCID." DBK PI. 8/1/12- 7/31/18, $491,762 Annual DC.

California Institute for Regenerative Medicine (CIRM)/ UCSD (CLIN1-09230). "Ex vivo transduced autologous human CD34+ hematopoietic stem cells for treatment of cystinosis." S.Cherqui PI, Kohn, Co-I. 11/1/16-10/31/18. $275,118 Annual DC.

The Broad Foundation - Innovative Pilot Stem Cell Research Award – "Gene Editing for X-linked Agammaglobulinemia" DBK – PI, 8/1/17 - 10/31/18 $125,000 direct costs.

California Institute for Regenerative Medicine (CIRM) (CLIN1-08686). "Regeneration of a Normal Corneal Surface by Limbal Stem Cell Therapy." S. Deng PI, Kohn Co-I. 8/1/16-11/30/18. $7,849 Annual DC.

Past Support (Continued):

California Institute for Regenerative Medicine (CIRM) (TRAN1-08533). "Stem Cell-Based iNKT Cell Therapy for Cancer." L.Yang PI, Kohn Co-I. 7/1/16-12/31/18. $348,600 Annual DC.

BioMarin - Sponsored Research Agreement. "Novel Approaches to Gene Therapy for Sickle Cell." DBK - PI 12/19/14-01/31/19 $1,557,092 Annual DC

California Institute for Regenerative Medicine. (CIRM) (DRA2-05309) "Genetic Re-Programming of Stem Cells to Fight Cancer." A.Ribas PI, Kohn Co-I. 04/01/14-03/31/20. $721,246 ADC.

California Institute for Regenerative Medicine (CIRM) /UCSF-CHRCO (TRAN1-09292). "Curing Sickle Cell Disease with CRISPR-Cas9 Genome Editing." M.Walters PI, Kohn, Co-I. 4/1/17-07/31/19. $303,451 Annual DC.

National Institutes of Health-NIAID, University of California, San Francisco (PI: M. Cowan) (U54 AI 082973) "Primary Immunodeficiency Treatment Consortium (PIDTC)" DBK: Co-PI. 9/1/14 – 8/31/19 $ 1,599,566

National Institutes of Health-NIAID, University of California, San Francisco (PI: M. Cowan) PIDTC Pilot Grant "Hematopoietic Stem Cell Gene Therapy for IPEX" DBK: PI. 9/1/2018 – 8/31/2019 $25,000

U54 AI082973 (Multi-PI: Puck/Kohn) 9/1/2019–8/31/2020 NIH/UCSF $25,268 Primary Immune Deficiency Treatment Consortium (PIDTC)

National Institutes of Health-NHLBI (SCL0029) "Pre-Clinical Assessment of a Refined β-Globin Lentiviral Vector to Improve Access to Efficacious Gene Therapy for Hemoglobinopathies" DBK PI 11/1/20-7/23/21 $426,771 annual direct costs

National Institutes of Health- NCATS Boston Children's Hospital (PI: D.A.Williams) (1U01TR001814) "Disseminating Curative Biological Therapies for Rare Pediatric Diseases" DBK Site PI. 9/1/2016-8/31/2021. $154,165 Annual DC.

Avrobio Inc (SRA 2020-0565) "cGMP EFS-CTSN Cell Product for Toxicology Study" DBK PI 9/01/2020-7/29/2021 $190,804 DC

CIRM CLIN1-11497 (PI M. Walters) 06/01/19-05/31/21 - UCSF/Oakland Childrens. "Curing Sickle Cell Disease with CRISPR-Cas9 genome editing." Role: Director, GMP cell processing.

Wiskott Aldrich Foundation (20214167 Research Grant) "Novel Lentiviral Vector for Gene Therapy for Wiskott Aldrich Syndrome". DBK PI, 3/15/2021-3/14/2022, $57,995 a.d.c.

ImmunoVec, Inc. (SRA 2021-0024) "Pre-Clinical Studies for Gene Therapy for with Regulated Lentiviral Vectors". DBK PI, 5/24/2021-5/24/2022, $201,962 annual direct costs

California Institute for Regenerative Medicine (CIRM) (DISC2-12111) "Hematopoietic Stem Cell Gene Therapy for X-linked Agammaglobulinemia (XLA)" DBK PI 01/01/2021-12/31/2021 $182,583 DC

Broad Stem Cell Research Center (BSCRC Innovation Award) "Improving Site-Specific Gene Insertion in HSC". DBK PI, 7/1/2021-6/30/2022, Total Award (for all years): $150,000 a,d,c,

California Institute for Regenerative Medicine (C.I.R.M.) (TRAN1-11536) "Ex Vivo Gene Editing of Autologous Hematopoietic Stem Cells for the Treatment of X-Linked Hyper IgM Syndrome". Co-investigator (C. Kuo, P.I.), 02/01/2020 - 07/31/2022, $564,102 annual direct costs

National Institutes of Health- NIAMS, University of Southern California (PI: J Lieberman) (2R01AR057076) "The Use of Regional Gene Delivery to Heal Critical Sized Bone Defects", DBK: Co-I., 4/1/17-3/31/22, $5,434 Annual DC.

Past Support (Continued):

UCSF/Oakland Children's, CIRM CLIN1-11497 (PI M. Walters) "Curing Sickle Cell Disease with CRISPR-Cas9 genome editing". Role: Director, GMP cell processing. 06/01/19-05/31/21

California Institute for Regenerative Medicine (CIRM), University of California, San Francisco (TRAN1-11259) "Developing Engineered Autologous Leukemia Vaccines to Target Residual Leukemic Stem Cells. Co-investigator" (K Gaensler, P.I.). Total Award $909,107

SCID Angels for Life "IL7Rα Gene Editing for IL7Rα SCID". DBK PI, 04/01/2022-3/31/2023, $25,000 annual direct costs

National Institute of Allergy and Infectious Diseases, Boston Children's Hospital (PI: SY Pai) (5U01AI125051-02) "Gene therapy for SCID-X1 with low dose busulfan and a SIN-lentiviral vector", DBK Site PI., 9/1/16-6/30/23 $181,590 annual direct costs to UCLA

California Institute for Regenerative Medicine (CIRM) CLIN2 (PI: S. Cherqui) "A Phase 1/2 Study to Determine the Safety and Efficacy of Transplantation with Autologous Human CD34+ Hematopoietic Stem Cells {HSC} from Mobilized Peripheral Blood Stem Cells {PBSC} of Patients with Cystinosis Modified by Ex Vi*v* o Transduction using the pCCL-CTNS Lentiviral Vector". UCLA Role: GMP cell processing, 06/01/2019 - 05/31/23, UCSD $509,029 annual dc

California Institute for Regenerative Medicine (CIRM) (CLIN2-08231). "A Phase I/II, Non-Randomized, Multicenter, Open-Label Study of G1XCGD (Lentiviral Vector Transduced CD34+ Cells) in Patients with X-Linked Chronic Granulomatous Disease." DBK PI. 09/01/15 – 12/31/23. $7,083,364 total costs.

Jeffrey Modell Foundation "Gene Therapy for CD3delta Severe Combined Immune Deficiency". DBK PI, 01/01/2022-12/31/2023, $125,000 annual direct costs

Rocket Pharma (CIRM, prime) (RP-L401-0120) "Gene Therapy for Infantile Malignant Osteopetrosis (IMO): A Phase I Clinical Trial to Evaluate the Safety and Preliminary Efficacy of the Infusion of Autologous CD34+ Enriched Cells Transduced with a Lentiviral Vector Encoding the *TCIRG1* Gene". DBK PI, 11/13/2020-11/12/2024, $254,906 annual direct costs

National Institutes of Health-NHLBI (T32HL086345-11) "Training in Developmental Hematology," DBK PI. 4/1/18-5/31/24. $189,933 annual direct costs

F. SOCIETY MEMBERSHIPS

American Association for the Advancement of Science – 1978.
Alpha Omega Alpha - 1981.
American Society for Microbiology - 1985.
American Society of Hematology - 1989.
Society for Pediatric Research - 1991.
American Society for Transplantation and cellular Therapy- 1994.
American Federation for Clinical Research - 1995.
American Society of Gene and Cell Therapy – 1997.
Clinical Immunology Society - 1998
American Pediatric Society – 2005.
European Society of Gene and Cell Therapy – 2008-present
European Society of Immunodeficiencies - 2020

G. RESEARCH INTERESTS

Gene therapy of genetic diseases of blood cells

Gene transfer and expression in human hematopoietic stem cells

Gene editing in human hematopoietic stem cells

Hematopoietic stem cell transplantation for primary immune deficiencies

H. Sponsor for FDA INVESTIGATIONAL NEW DRUG (IND) PERMITS:

1. **BB IND 6753** (Transduction of CD34+ cells from the bone marrow of HIV-1 infected children: Comparative marking by an RRE decoy gene and a neutral gene). (Closed)

2. **BB IND 8556** (Transduction of CD34+ cells from the umbilical cord blood of infants or the bone marrow of children with adenosine deaminase (ADA)-deficient Severe Combined Immunodeficiency (SCID). (Accrual completed, in long-term follow-up through 2027)

3. **BB IND 8929** (Retroviral-mediated transfer of the REVM10 and FX genes into CD34+ cells from the bone marrow of HIV-1 infected children). (Closed)

4. **BB IND 15440** (Autologous Transplantation of Bone Marrow CD34+ Stem/Progenitor Cells after Addition of a Normal Human ADA cDNA by the EFS-ADA Lentiviral Vector for Adenosine Deaminase (ADA)-Deficient Severe Combined Immunodeficiency (SCID). (Open May, 2013) (Transferred to Orchard Therapeutics, 2018, returned to UCLA 2021).

5. **BB IND 16028** (Autologous Bone Marrow Stem Cells (CD34+) Cultured W/ Cytokines; Transduced W/ Self-inactivating (SIN) Lentiviral Vector Expressing Human β-globin (LENTI/BetaAS3-FB); Following Busulfan). (Open June, 2014)

6. **BB IND 16141** (Autologous Bone Marrow Stem Cells (CD34+ Miltenyi CliniMacs) Cultivated W/ Cytokines; Transduced W/ Self-Inactivating (SIN) Lentiviral Vector Expressing Human gp91phox (G1XCGD); Following Busulfan) (Open Oct. 2014)

I. POST-DOCTORAL FELLOWS (current - 1):

Aurelian Colamartino, Ph.D. 2022 – *BTK* Gene Editing for XLA

Previous fellows (37):

Leonard Sender (MD) 1988-1989 Glucocerebrosidase (GC) gene expression
Joel Weinthal (MD) 1989-1991 GC gene expression in murine bone marrow
Gay M. Crooks (MD) 1991-1993 Gene transfer to human hematopoietic stem cells.
Wanda J. Krall (PhD) 1993-1995 Regulation of gene from retroviral vectors
Jan A. Nolta (PhD) 1994-1996 Gene transfer into human stem cells
Punam Malik (MD) 1995-1997 Lineage-directed gene expression, AAV
Mei-Mei Huang (PhD) 1995-1997 Gene therapy for Hurler disease,Wiskott-Aldrich
Robert Cooper (MD) 1995-1997 Role of methylation in vector silencing
Leo Mascarenhas (MD) 1996-1998 Gene transfer into human leukemia cells
Renata Stripecke (PhD) 1995-1999 Immunotherapy of childhood leukemia
Stephanie Halene (MD) 1997–2000 Vector expression/silencing
Scott Case (PhD) 1997–2000 Lentiviral vectors for gene therapy
Steve Rawlings (PhD) 1999–2000 Vectors encoding HIV inducible apoptosis
Diana Fan (MD) 2000 2001-2002 Immunotherapy for childhood leukemia
Noriko Satake (MD) 1998–2001 Immune responses and tolerance to transgenes
Barbara Engel (MD) 1998–2001 HIV gene therapy
Carolyn Lutzko (PhD) 1999–2004 Lineage specific gene expression
Hiroshi Kobayashi (MD) 2002–2004 Gene therapy for lysosomal storage disease
Kit Shaw (PhD) 2002–2007 Lineage specific gene expression
Roger Hollis (PhD) 2003–2006 Gene transfer to human hematopoietic stem cells
Christof Kahl (PhD) 2003–2007 Gene transfer to rhesus CD34+ cells
Noriko Satake (MD) 2005–2007 Immunotherapy of leukemia
Ken Sakurai (MD/PhD) 2007–2010 Gene expression in human ESC
Satiro De Oliveira (MD) 2008-2010 Immunotherapy of leukemia by myeloid effectors
Francesca Giannoni (PhD) 2008-2010 Immunotherapy for cancer and leukemia
Rafael Ruiz de Assini (PhD) 2010-2011β-globin gene correction for sickle cell disease
Fabrizia Urbanati (PhD) 2010-12 β-globin gene correction for sickle cell disease
Sarah Larson (MD) 2011-2013 - Immunotherapy for Leukemia
Zulema Romero-Garcia (PhD) 2010 -2014 Gene therapy for sickle cell disease
Kismet Baldwin (MD) 2011 - 2014 Gene transfer to human HSC

Caroline Kuo (MD)	2012 – 2014 Correction of the CD154 gene for X-HIM
Gavin Roach (MD)	2012 – 2014 Clinical trial of sickle cell gene therapy
Carmen Bjurstroem (PhD)	2013-2015 Gene editing in hematopoietic stem cells
Alok Joglekar (PhD)	2013-2014 ZFN editing in HSC
Robert Drummond (MD/PhD)	2015-2017 Correction of the sickle cell mutation
Alexandra Miggelbrink, (MD)	2016-2017 CRISPR-mediated gene correction in HSC
Kathryn Bradford (MD)	2018-2020 Gene editing for Common Variable Immune Deficiency
Shanna White, M.D.	2020-2022 Gene Therapy for Sickle Cell Disease

J. GRADUATE STUDENTS FOR THE Ph.D. (current - 6)

Mr. Ralph V. Crisostomo	(2018-) Cell cycle and gene editing
Ms. Kevyn Hart	(2019-) Gene therapy for sickle cell disease
Ms. Eva Segura	(2019-) Gene therapy for alpha-thalassemia
Mr. Christopher Luthers	(2020-) Gene therapy for X-linked agammaglobulinemia
Ms. Vrishti Sinha*	(2023-) Gene Therapy for DOCK8 Deficiency
Mr. Dylan Smock	(2023-) Novel methods for gene editing in HSC

*Jointly with Caroline Kuo

K. Previous graduate students (24) (degree earned –Institution, Department)

Jan A. Nolta (PhD – USC, Microbiology) - 1994 - Growth of human hematopoietic stem cells in immune deficient mice

Pia M. Challita (PhD – USC, Microbiology) 1994 - DNA methylation in gene therapy

Ingrid Bahner (PhD – USC, Microbiology) 1996 - Gene therapy for AIDS

Paul Robbins (PhD – USC, Microbiology) 1997 - Expression by modified retroviral vectors

Sunita Coutinho (MD/PhD – USC, Microbiology/Medicine) 2000 - Lineage-directed expression by retroviral vectors

Tanja Gruber (MD/PhD – USC, Microbiology/Medicine) 2001 - Dendritic cells for gene therapy of childhood ALL

Dennis Haas (PhD – USC, Microbiology) 2003- Lentiviral vectors for transduction of hematopoietic stem cells

Aaron Logan (MD/PhD – USC, Microbiology) 2004 - Immunotherapy for leukemia

Sarah Nightingale (PhD – USC, Microbiology) 2006 – Stable non-viral and viral, non-stable gene transfer to hematopoietic stem cells

Martina Blumenthal (PhD – USC, Microbiology) 2007 – Leukemia Immunotherapy

Teiko Sumiyoshi (PhD – USC, Microbiology) 2009 –Gene transfer to human HSC by the *Sleeping Beauty* transposon.

Christopher Choi (PhD – USC, Microbiology) 2009 – HSC expansion by manipulation of the beta-catenin pathway.

Eszter Pais, MD (PhD – USC, System Biology & Disease) 2009– Pancreatic beta cell production and expansion

Denise Sarracino (PhD – USC, Systems Biology & Disease) 2009 – Gene therapy for ADA-deficient SCID.

Alok Joglekar – (PhD – UCLA, Dept. of MIMG), 2013 - Zinc Finger Nucleases for ADA Gene Correction

Jennifer Wherley – (M.S., UCLA, Dept. of MIMG) 2013 - Gene transfer for sickle cell disease

Eric Gschweng – (PhD – UCLA, Dept. of MIMG), 2015 - Immunotherapy for cancer and leukemia

Megan Hoban – (PhD – UCLA, Dept. of MIMG), 2015 - Site-specific nucleases for sickle cell gene therapy

Aaron Cooper – (PhD – UCLA, Molecular Biology Institute Interdepartmental Program), 2016 - Vector integromics.

Richard Morgan (MSTP) - (PhD – UCLA, Molecular & Medical Pharmacology), 2019 Lentiviral vectors for gene therapy for hemoglobinopathies

Katelyn Masiuk (MSTP) - (PhD - UCLA, Molecular Biology Institute Interdepartmental Program), 2019 Hematopoietic stem cell gene therapy

Anastasia Lomova (PhD – UCLA, Molecular & Medical Pharmacology), 2019 Homology-directed gene repair in HSC

David Gray (PhD – UCLA), Immunity, Microbes and Molecular Pathogenesis), 2020 Gene correction for X-linked agammaglobulinemia

Nebula Han (PhD – UCLA, Molecular & Medical Pharmacology), 2021 Improving Titer and Infectivity of Lentiviral Vectors for Gene and Cell Therapy

Ryan Wong (PhD – UCLA, Molecular & Medical Pharmacology), 2021 Bioinformatics-Guided Design of Endogenously Regulated Lentiviral Vectors for Hematopoietic Stem Cell Gene Therapy

Paul G. Ayoub ((PhD – UCLA, Molecular & Medical Pharmacology) 2023 Lentiviral vectors for gene therapy

12 Visiting students from California State University, Northridge under the California Institute for Regenerative Medicine (CIRM) **CSUN-UCLA Bridges to Stem Cell Research Program** (*CIRM TB1-01183, EDUC2-08411*):

Behrod Katebian	2010-2011
Anthony Cuccia	2011-2012
Michelle Mojadidi	2013-2014
Joseph Long	2014-2015
Marie Parma	2015-2016
Brian Tulloh	2016-2017
Isaac Villegas	2017-2018
Jason Quintos	2018-2019
Portia Elms	2019-2020
Katherine Orr	2021-2022
Delaram Zare	2022-2023
Andrea Gutierrez	2023-present

M. PUBLICATIONS

Peer Reviewed

1. **Kohn DB**, Weber MJ, Carl PL, Katzenellenbogen JA, Chakravarty PK. Peptidyl derivatives of (^{3}H)-aniline as sensitive, stable protease substrates. Anal Biochem 97:269-276, 1979.

2. Ecanow B, Gold BH, **Kohn DB**, Ecanow C. The role of inert particles in malignant transformations: a hypothesis of carcinogenesis. Physiol Chem Phys 11:97-107, 1979.

3. Trigg ME, **Kohn DB**, Sondel PM, Chesney PJ. Tracheal aspirate examination for *Pneumocystis carinii* cysts as a guide to therapy in Pneumocystis pneumonia. J Pediatr l02:88l-883, l983.

4. Kantoff PW, **Kohn DB**, Mitsuya H, Armentano D, Sieberg M, Zweibel JA, Eglitis MA, McLachlin JR, Wiginton DA, Hutton JJ, Horowitz SD, Gilboa E, Blaese RM, Anderson WF. Correction of adenosine deaminase deficiency in human T and B cells using retroviral gene transfer. Proc Natl Acad Sci (USA) 83:6563-6567, 1986.

5. Anderson WF, Kantoff P, Eglitis M, McLachlin J, Karson E, Zwiebel J, Nienhuis A, Karlsson S, Blaese RM, **Kohn D**, et al. Gene transfer and expression in nonhuman primates using retorviral vectors. Cold Spring Sym Quant Biol 51:1073-1081, 1986.

6. Kantoff PW, Billio A, McLachlin JR, Flake AW, Eglitis MA, Moen R, Karlsson S, **Kohn DB**, Karson E, Zwiebel JA, et al. Retroviral-mediated gene transfer into hematopoietic cells. Trans Assoc Am Physicians 99:92-102, 1986.

7. **Kohn DB**, Uehling DT, Peters ME, Fellows KW, Chesney PJ. Short-course amphotericin B therapy for isolated candiduria in children. J of Pediatr ll0:3l0-3l3, l987.

8. **Kohn DB**, Trigg ME, Borcherding W, Hong R. Immunologic studies of lymph node lymphocytes in the generalized lymphadenopathy syndrome. Am J Pediatr Hem Onc 9:l-7, 1987.

9. Kantoff PW, Gillio A, McLachlin JR, Bordignon C, Eglitis MA, Kernan NA, Moen RC, **Kohn DB**, Yu S, Karson E, Karlsson S, Zweibel JA, Gilboa E, Blaese RM, Nienhuis A, O'Reilly RJ, Anderson WF. Expression of human adenosine deaminase in non-human primates after retroviral mediated gene transfer. J Exp Med l66:2l9-234, 1987.

10. **Kohn DB**, Kantoff PW, Eglitis M, McLachlin JR, Moen RC, Karson E, Zweibel JA, Nienhuis A, Karlsson S, O'Reilly R, Gillio A, Bordignon C, Gilboa E, Zanjani ID, Blaese RM, Anderson WF. Retrovirus-mediated gene transfer into mammalian cells. Blood Cells l3:285-296, 1987.

PUBLICATIONS (Cont'd)

11. Eglitis MA, Kantoff PW, McLachlin JR, Gillio A, Flake AW, Bordignon C, Moen RC, Karson EM, Zwiegel JA, **Kohn DB**, et al. Gene therapy: efforts at developing large animal models for autologous bone marrow transplant and gene transfer with retroviral vectors. Ciba Found Symp 130:229-246, 1987.

12. Eglitis MA, **Kohn DB**, Moen RC, Blaese RM, Anderson WF. Infection of human hematopoietic progenitor cells using a retroviral vector with a xenotropic pseudotype. Biochem Biophys Res Comm 151:201-206, 1988.

13. Fearon ER, **Kohn DB**, Winkelstein JA, Vogelstein B, Blaese RM. Carrier detection of Wiskott-Aldrich Syndrome. Blood 72:1735-39, 1988.

14. **Kohn DB**, Mitsuya H, Ballow M, Selegue JE, Barankiewicz J, Cohen A, Gelfand E, Anderson WF, Blaese RM. Establishment and characterization of adenosine deaminase (ADA)-deficient human T cell lines. J of Immunol 142:3971-77, 1989.

15. **Kohn DB**, Kantoff PW. Potential applications of gene therapy. Transfusion 29:812-820, 1989.

16. Nolta JA, **Kohn DB**. Comparison of the effects of growth factors on retroviral vector-mediated gene transfer and the proliferative status of human hematopoietic progenitor cells. Hum Gene Ther 3:257-268, 1990.

17. Nolta JA, Sender LS, Barranger JA, **Kohn DB**. Expression of human glucocerebrosidase in murine long-term bone marrow cultures after retroviral vector-mediated transfer. Blood 75:787-797, 1990.

18. Lenarsky C, Weinberg K, Petersen J, Nolta J, Brooks G, Annett G, **Kohn D**, Parkman R. Autologous bone marrow transplantation with 4-hydroperoxycyclophosphamide purged marrows for children with acute non-lymphoblastic leukemia in second remission. Bone Marrow Transplant 6:425-429, 1990.

19. Ozkaynak MF, Lenarsky C, **Kohn D**, Weinberg K, Parkman R. *Mycobacterium avium*-intracellulare infections after allogeneic bone marrow transplantation in children. Am J Pediatr Hematol-Oncol 12:220-224, 1990.

20. Ozkaynak MF, Weinberg K, **Kohn D**, Sender L Parkman R, Lenarsky C. Hepatic veno-occlusive disease post-bone marrow transplantation in children conditioned with busulfan and cyclophosphamide: incidence, risk factors, and clinical outcome. Bone Marrow Transplant 7:467-474, 1991.

21. Weinthal J, Nolta JA, Yu X-J, Lilley J, Uribe L, **Kohn D**. Expression of human glucocerebrosidase following retroviral vector-mediated transduction of murine hematopoietic stem cells. Bone Marrow Transplant 8:403-412, 1991.

PUBLICATIONS (Cont'd)

22. **Kohn DB**, Nolta JA, Weinthal J, Bahner I, Yu XJ, Lilley J, Crooks GM. Toward gene therapy for Gaucher disease. Hum Gene Ther 2:101-105, 1991.

23. Bedgood RM, Bahner I, **Kohn DB**, Stallcup MR. Two different genes coding for processable and nonprocessable forms of a viral envelope protein can account for the hormonal stimulation of protein processing in W7MGI lymphoma cells. Mol Endo 6:459-467, 1992.

24. Nolta JA, Yu XJ, Bahner I, **Kohn DB**. Retroviral-mediated transfer of the human glucocerebrosidase gene into cultured Gaucher bone marrow. J Clin Invest 90:342-348, 1992.

25. Nolta JA, Crooks GM, Overell RW, Williams DE, **Kohn DB**. Retroviral vector-mediated gene transfer into primitive human hematopoietic progenitor cells: effects of mast cell growth factor (MGF) combined with other cytokines. Exp Hematol 20:1065-1071, 1992.

26. Miller AR, Skotzko MJ, Rhoades K, Belldegrun AS, Tso C-L, Kaboo R, McBride WH, Jacobs E, **Kohn D**, Moen R, Economou JS. Simultaneous use of two retroviral vectors in human gene marking trials: feasibility and potential applications. Hum Gene Ther 3:619-624, 1992.

27. Bahner I, Zhou C, Yu X-J, Hao Q-L, Guatelli JC, **Kohn DB**. Comparison of *Trans-Dominant* Inhibitory mutant human immunodeficiency virus Type 1 genes expressed by retroviral vectors in human T lymphocytes. J of Virol 67:3199-3207, 1993.

28. Gordon EM, Tang H, Salazar R, **Kohn DB**. Expression of coagulation factor IX in human hepatoma (HepG2) cell cultures after retroviral vector-mediated transfer. Am J Pediatr Hematol/Oncol 15:196-203, 1993.

29. Roberts WD, Weinberg KI, **Kohn DB**, Sender L, Parkman R, Lenarsky C. Granulocyte recovery in pediatric marrow transplant recipients treated with granciclovir for cytomegalovirus infection. Am J Pediatric Hematol/Oncol 15:320-323, 1993.

30. Lenarsky C, Weinberg K, **Kohn DB**, Parkman R. Unrelated donor BMT for Wiskott-Aldrich syndrome. Bone Marrow Transplant 12:145-147, 1993.

31. Weinberg K, Hershfield MS, Bastian J, **Kohn D**, Sender L, Parkman R, Lenarsky C. T lymphocyte ontogeny in adenosine deaminase deficient severe combined immune deficiency following treatment with polyethylene glycol modified adenosine deaminase. J Clin Invest 92:596-602, 1993.

PUBLICATIONS (Cont'd)

32. Crooks GM, **Kohn, DB**. Growth factors increase amphotropic retrovirus binding to human CD34 positive bone marrow progenitor cells. Blood 82:3290-3297, 1993

33. Miller AR, McBride WH, Dubinett SM, Dougherty GJ, Thacker JD, Shau H, **Kohn DB**, Moen RC, Walker MJ, Chiu R, Schuck BL, Rosenblatt JA, Huang M, Dhanani S, Rhoades K, Economou JS. Transduction of human melanoma cell lines with human interleukin-7 gene using retroviral-mediated gene transfer: Comparison of immunologic properties with interleukin-2. Blood 82:3686-3694, 1993

34. Challita PM, **Kohn DB**. Lack of expression from a retroviral vector after transduction of murine hematopoietic stem cells is associated with methylation. Proc Natl Acad Sci (USA). 91:2567-2571, 1994.

35. Nolta JA, Hanley MB, **Kohn DB**. Sustained human hematopoiesis in immunodeficient mice by co-transplantation of marrow stroma expressing human IL-3: Analysis of gene transduction of long-lived progenitors. Blood 83:3041-3051, 1994.

36. Krall WJ, Challita PM, Perlmutter L, Skelton D, **Kohn DB**. Cells expressing human glucocerebrosidase from a retroviral vector repopulate tissue macrophages and the CNS microglia after bone marrow transplantation. Blood 83:2737-2748, 1994.

37. Zhou C, Bahner IC, Larson GP, Zaia JA, Rossi JJ, **Kohn DB**. Inhibition of HIV-1 in human T-lymphocytes by retrovirally transduced anti-*tat* and *rev* hammerhead ribozymes. Gene 149:33-39, 1994.

38. Hanley ME, Parkman R, **Kohn DB**. Umbilical cord blood cell transduction by retroviral vectors: pre-clinical studies to optimize gene transfer. Blood Cells, 20:539-546, 1994.

39. Gordon EM, D'Alisa R, Tang H. Salazar R, Sabatino RD, Dorio R, **Kohn DB**, Holt J. Characterization of a monoclonal antibody-purified recombinant factor IX produced in human hepatoma (HepG2) cell cultures after retroviral vector-mediated transfer. International J of Pediatr Hematol Oncol. 1: 1-7, 1994.

40. Raffel C, Culver K, **Kohn D**, Nelson M, Siegel S, Gillis F, Link CJ, Villablanca JG, Anderson WF. Gene therapy for the treatment of recurrent pediatric malignant astrocytomas with in vivo tumor transduction with the herpes simplex thymidine kinase gene/ganciclovir system. Hum Gene Ther 5:863-890, 1994.

41. Challita PM, Skelton D, El-Khoueiry Anthony, Yu X-J, Weinberg K, **Kohn DB**. Multiple modifications in cis-elements of the long terminal repeat of retroviral vectors lead to increased expression and decreased DNA methylation in embryonic carcinoma cells. J of Virol 69:748-755, 1995.

PUBLICATIONS (Cont'd)

42. Nolta JA, Smogorzewska EM, **Kohn DB**. Analysis of optimal conditions for retroviral-mediated transduction of primitive human hematopoietic cells. Blood 86:101-110, 1995.

43. Hao QL, Malik P, Salazar R, Tang H, Gordon EM, **Kohn DB**. Expression of biologically active human factor IX in human hematopoietic cells after retroviral vector-mediated gene transduction. Hum Gene Ther 6:873-880, 1995.

44. **Kohn DB**, Weinberg KI, Nolta, JA, Heiss L, Lenarsky C, Crooks GM, Hanley ME, Annett G, Brooks JS, El-Khoureiy, Lawrence K, Wells S, Moen RC, Bastian J, Williams-Herman DE, Elder M, Wara D, Bowen T, Hershfield MS, Mullen CA, Blaese RM, Parkman R. Engraftment of gene-modified umbilical cord blood cells in neonates with adenosine deaminase deficiency. Nat Med 1:1017-1023, 1995.

45. Malik P, Krall WJ, Yu XJ, Zhou C, **Kohn DB**. Retroviral-mediated gene expression in human myelomonocytic cells: A comparison of hematopoietic cell promoters to viral promoters. Blood 86:2993-3005, 1995.

46. Wells S, Malik P, Pensiero M, **Kohn DB**, Nolta JA. The presence of an autologous marrow stromal cell layer increases glucocerebrosidase gene transduction of long-term culture initiating cells (LTCICs) from the bone marrow of a patient with Gaucher disease. Gene Ther 2:512-520, 1995.

47. Parkman R, Crooks G, **Kohn DB**, Lenarsky C, Weinberg K. Bone marrow transplantation for metabolic disease. Cancer Treat Res 76:87-96, 1995.

48. Krall WJ, Skelton DC, Yu X-J, Riviera I, I Lehn P, Mulligan RC, **Kohn DB**. Increased splicing efficiency accounts for augmented expression from the MFG vector in hematopoietic cells. Gene Ther 3:37-48, 1996.

49. Zhou C, Bahner I, Rossi JJ, **Kohn DB**. Expression of hammerhead ribozymes by retroviral vectors to inhibit HIV-1 replication: comparison of RNA levels of viral inhibition. Antisense Nucleic Acid Drug Dev 6:17-24, 1996.

50. Nolta JA, Dao MA, Wells S, Smogorzewska EM, **Kohn DB**. Transduction of pluripotent human hematopoietic stem cells demonstrated by clonal analysis after engraftment in immune deficient mice. Proc Natl Acad Sci (USA) 93:2414-2419, 1996.

51. Taylor N, Smith S, Uribe L, **Kohn D**, Weinberg K. Correction of IL-2 receptor function in X-SCID lymphoblastoid cells by retrovirally mediated transfer of the GammaC gene. Blood 87:3103-3107, 1996.

52. Jadus MR, Irwin MCN, Irwin MR, Horansky RD, Pepper KP, **Kohn DB**, Wepsic HT. Macrophages can recognize and kill tumor cells bearing the membrane isoform of macrophage colony stimulating factor. Blood 87:5232-5241, 1996.

PUBLICATIONS (Cont'd)

53. Bahner I, Kearns K,Hao Q-L, Zhou C, Smogorzewska EM, **Kohn DB**. Transduction of human CD34+ hematopoietic progenitor cells by a retroviral vector expressing an RRE decoy inhibits HIV-1 replication in the myelomonocytic cells produced in long-term culture. J of Virol 70:4352-4360, 1996.

54. Imren S, **Kohn DB**, Shimada H, Blavier L, DeClerck Y. Overexpression of tissue inhibitor of metalloproteinases-2 by retroviral mediated gene transfer in vivo inhibits tumor growth and invasion. Cancer Res 56: 2891-2895, 1996.

55. Shull RM, Lu X, McEntee MF, Bright RM, Pepper K. **Kohn DB**. Myloblast gene therapy in canine mucopolysaccharidosis I: Abrogation by an immune response to α-L-Iduronidase. Hum Gene Ther 7:1595-1603, 1996.

56. Kido M, Rich KA, Yang G, Barron E, **Kohn DB**, Al-Ubaidi MR, Blanks JC. Use of a retroviral vector with an internal opsin promoter to direct gene expression to retinal photoreceptor cells. Current Eye Res:15:833-844, 1996.

57. **Kohn DB**. Gene therapy for hematopoietic and immune disorders. Bone Marrow Transplant 18:S55, 1996.

58. Dunbar C, **Kohn D**. Retroviral mediated transfer of the cDNA for human glucocerebrosidase into hematopoietic stem cells of patients with Gaucher disease. A phase I study. Hum Gene Ther 7:231-253, 1996.

59. Taylor N, Bacon KB, Smith S, Jahn T, Kadlecek TA, Uribe L, **Kohn DB**. Reconstitution of T cell receptor signaling in ZAP-70-deficient cells by retroviral transduction of the ZAP-70 gene. J of Exp Med 184:2031-2036, 1996.

60. Kearns K, Bahner I, Bauer G, Wen SD, Wheeler S, Woods L, Miller R, Church J and **Kohn DB**. Suitability of bone marrow from HIV-1 infected donors for retroviral-mediated gene transfer. Hum Gene Ther 8:301-311, 1997.

61. Bertrands E, Castanotto D, Zhou C, Carbonnelle C, Lee NS, Good P, Chatterjee S, Grange T, Pictet R, **Kohn D**, Engelke D, Rossi, JJ. The Expression cassette determines the functional activity of ribozymes in mallalian cells by controlling their intracellular localization. RNA 3:75-88, 1997.

62. Dao M, Hannum CH, **Kohn DB**, Nolta JA. Flt-3 ligand preserves the clonogenic capacity of primitive human hematopoietic cells during ex vivo retroviral-mediated transduction. Blood 89:446-456, 1997.

63. Malik P, McQuiston SA, Yu X-J, Pepper KA, Krall WJ, **Kohn DB**. Recombinant Adeno-associated Virus (rAAV) Mediates a High Level of Gene Transfer but a Low Efficiency of Integration in Human Hematopoietic Cells. J of Virol 71:1776-1783, 1997.

PUBLICATIONS (Cont'd)

64. Bauer G, Valdez P, Kearns K, Bahner I, Wen S-F, Zaia J, **Kohn DB**. Inhibition of HIV-1 replication after transduction of G-CSF-mobilized CD34+ cells from HIV-1-infected donors using retroviral vectors containing anti-HIV-1 genes. Blood 89:2259-2267,1997.

65. Lu Y, Planelles V, Li X, Palaniappan C, Day B, Challita-Eid P, Amado R, Stephens D, **Kohn D**, Baker A, Fay P, Bambara RA, Rosenblatt JD. Inhibition of HIV-I replication using a mutated $tRNA^{Lys3}$ primer. J of Biological Chem 272:14523-14531, 1997.

66. Bahner I, Kearns K, Coutinho S, Leonard EH, **Kohn DB**. Infection of human marrow stroma by HIV-1 is both required and sufficient for HIV-1-induced hematopoietic suppression in vitro: demonstration by gene-modification of primary human stroma. Blood 90:1787-1798, 1997.

67. Huang MM, Wong A, Yu X-J, Kakkis E, **Kohn DB**. Retroviral-mediated transfer of the human alpha-L-induronidase cDNA into human hematopoietic progenitor cells leads to correction in trans of Hurler fibroblasts. Gene Ther 4:1150-1159, 1997.

68. Robbins PB, Yu X-J, Skelton DM, Pepper KA, Wasserman RM, Zhu L, **Kohn DB**. Increased probability of expression from modified retroviral vectors in embryonal stem cells and embryonal carcinoma cells. J of Virol 9466-9474, 1997.

69. Wang H, Jenkins R, Kaido T, Kan-Mitchell J, **Kohn DB**, Mitchell MS. B7-1 constimulation facilitates generation of human tumor-specific cytotoxic T lymphocytes for adoptive cellular immunotherapy. Vaccine Res 6:75-89, 1997.

70. Malik P, Fisher TC, Barsky LW, Zeng L, Izadi P, Hiti AL, Weinberg KI, Coates TD, Meiselman HJ, **Kohn DB**. An in vitro model of human red blood cell production for hematopoietic progenitor cells. Blood 15:2664-2671, 1998.

71. deVos S, **Kohn DB**, Cho SK, McBride WH, Said JW, Koeffler. Immunotherapy against murine leukemia. Leukemia 12:401-405, 1998.

72. Verma S, Woffendin C, Bahner I, Ranga U, Xu L, Yang Z-Y, King SR, **Kohn DB**, Nabel GJ. Gene transfer into human umbilical cord blood-derived CD34+ cells by particle-mediated gene transfer. Gene Ther 5:692-699, 1998.

73. **Kohn DB**, Hershfield MS, Carbonaro D, Shigeoka A, Brooks J, Smogorzewska EM, Barsky LW, Chan R, Burotto F, Annett G, Nolta JA, Crooks G, Kapoor N, Elder M, Wara D, Bowen T, Madsen E, Snyder FF, Bastian J, Muul L, Blaese RM, Weinberg K, Parkman R. T lymphocytes with a normal ADA gene accumulate after transplantation of transduced autologous umbilical cord blood CD34+ cells in ADA-deficient SCID neonates. Nat Med 4:775-780, 1998.

PUBLICATIONS (Cont'd)

74. Robbins PB, Skelton DM, Yu J-J, Halene SA, Leonard EH, **Kohn DB**. Consistent, persistent expression from modified retroviral vectors in murine hematopoietic stem cells. Proc Natl Acad Sci (USA) 95:10182-10187, 1998.

75. Stripecke R, Skelton DC, Gruber T, Afar D, Pattengale PK, Witte O, **Kohn DB**. Immune response to Philadelphia chromosome-positive acute lymphoblastic leukemia induced by expression of CD80, Interleukin 2, and Granulocyte-macrophage colony-stimulating factor. Hum Gene Ther 9:2049-2062, 1998.

76. Wang L, Robbins PB, **Kohn DB**. High resolution analysis of cytosine methylation in 5' long terminal repeat of retroviral vectors. Hum Gene Ther 9:2321-2330, 1998.

77. Lutzko C, Kruth S, Abrams-Ogg ACG, Ruedy C, Nanji S, Liheng L, Lau K, Clark BR, Foster R, **Kohn DB**, Shull RM, Dube ID. Genetically corrected autologous stem cells engraft but host immune responses limit their utility in canine alpha-L-iduronidase deficiency. Blood 93:1895-1905, 1999.

78. Dunbar CE, **Kohn DB**, Schiffmann R, Barton NW, Nolta J, Explin J, Pensiero M, Long Z, Lockey C, Emmons RVB, Leitman Sc, Krebs CB, Carter C, Brady RO, Karlsson S. Retroviral transfer of the glucocerebrosidase gene into CD34+ cells from patients with Gaucher disease: In vivo detection of transduced cells without myeloablation. Hum Gene Ther 9:2629-2640, 1998.

79. Mascarenhas L, Stripecke, R, Case SS, Xu D, Weinberg KI, **Kohn DB**. Gene delivery to human B-precursor acute lymphoblastic leukemia cells. Blood 92:3537-3545, 1998.

80. Bauer G, Sauter S, Ibanez C, Rice CR, Valdez P, Jolly D, **Kohn DB**. Increased gene transfer into human CD34+ progenitor cells using retroviral vectors produced by a canine packaging cell line. Biol Blood Marrow Transplant 4:119-127, 1998.

81. Dunbar C, Chang L, Mullen C, Ramsey WJ, Carter C, **Kohn D**, Parkman R, Lenarsky C, Weinberg K, Wara D, Culver KW, Anderson WF, Leitman S, Fleisher T, Klein H, Shearer G, Clerici M, McGarrity G, Bastian J, Hershfield MS. Amendment to clinical research project Proj 90-C-195. April 1, 1993. Treatment of severe combined immunodeficiency disease (SCID) due to adenosine deaminase deficiency with autologous lymphocytes transduced with a human ADA gene. Human Gene Therapy 10:477-488, 1999.

82. Case SS, Price MA, Jordan CT, Yu XJ, Wang L, Bauer G, Haas DL, Xu D, Stripecke R, Naldini L, **Kohn DB**, Crooks GM. Stable and efficient transduction of CD34+/CD38- human hematopoietic cells by HIV-1 based lentiviral vectors. Proc Natl Acad Sci (USA) 96:2988-2993, 1999.

PUBLICATIONS (Cont'd)

83. **Kohn DB**, Bauer GH, Valdez P, Rice CR, Rothschild JC, Carbonaro D,Brody K, Hao QL, Zhou C, Bahner I, Kearns K, Wheeler S, Haden E, Wilson K, Salata C, Dolan C, Wetter C, Aguilar-Cordova E, Church J. A clinical trial of retroviral-mediated transfer of an RRE decoy gene into CD34+ cells from the bone marrow of HIV-1 infected children*.* Blood 94:368-371, 1999.

84. Crooks GM, Fuller J, Petersen D, Izadi P, Barsky L, Malik P, Pattengale PK, **Kohn DB**, Gasson JC. Constitutive HOXA5 expression inhibits erythropoiesis and increases myelopioesis from human hematopoietic progenitors. Blood 94:519-528, 1999.

85. Stripecke R, Villacres MDC, Skelton D, Halene, S, **Kohn DB**. Immune response to green fluorescent protein: Implications for gene therapy of hematologic malignancies. Gene Ther 6:1305-1312, 1999.

86. Stripecke R, Skelton DC, Pattengale PK, Shimada H, **Kohn DB**. Combination of CD80 and GM-CSF co-expression by a leukemia cell vaccine: pre-clinical studies in a murine model recapitulating Philadelphia chromosome positive acute lymphoblastic leukemia. Hum Gene Ther 10:2109-2122, 1999.

87. Halene S, Wang L, Cooper R, Rockstoce DC, Robbine PM, **Kohn DB**. Improved expression in murine hematopoietic and lymphoid cells after transplantation of bone marrow transduced with a modified retroviral vector. Blood 94:3349-57, 1999.

88. Huang MM, Tsuboi S, Wong A, Yu XJ, Oh-Eda M, Derry JM, Francke U, Fukuda M, Weinberg KI, **Kohn DB**. Expression of human Wiskott-Aldrich syndrome protein in patients' cells leads to partial correction of a phenotypic abnormality of cell surface glycoproteins. Gene Ther 7:314-320, 2000

89. Engel BC, Bauer G, Pepper KA, Bockstoce DC, Yu XY, Chen SY, **Kohn DB**. Intrakines - evidence for a trans-cellular mechanism of action. Mol Ther 1:165-170, 2000.

90. Smogorzewska EM, Brooks J, Annett G, Kapoor N, Crooks GM, **Kohn DB**, Parkman R, Weinberg KI. T-cell depleted haploidentical bone marrow transplantation for the treatment of children with severe combined immune-deficiency. Archivum Immunol et Therapiae Experimentalis, 48:111-118, 2000.

91. Halene S, **Kohn DB**. Gene therapy using hematopoietic stem cells: Sisyphus approaches the crest. Hum Gene Ther 11:1259-1267, 2000.

92. Islam TC, Branden LJ, **Kohn DB**, Islam KB, Smith CIE. Btk mediated apoptosis, a possible mechanism for failure to generate high titer retroviral producer clones. J Gene Med 2:204-209, 2000.

PUBLICATIONS (Cont'd)

93. Haas DL, Case SS, Crooks GM, Kohn DB. Critical factors influencing stable transduction of human CD34(+) cells with HIV-1-derived lentiviral vectors. Mol Ther. 2000 Jul;2(1):71-80.

94. Stripecke R, Cardoso A, Pepper K, Skelton D, Xu XY, Mascarenhas L, Weinberg K, Nadler L, **Kohn DB**. Lentiviral vectors for efficient delivery of CD80 and granulocyte-macrophage-colony-stimulating factor in human acute lymphoblastic leukemia and acute myeloid leukemia cells to induce antileukemic immune response. Blood 96:1317-1326, 2000.

95. 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**, et al. Human gene marker/therapy clinical protocols. Hum Gene Ther 11:919-979, 2000.

96. Tarantal AF, O'Rourke JP, Case SS, Newbound GC, Li J, Lee CI, Baskin CR, **Kohn DB**, Bunnell BA. Rhesus monkey model for fetal gene transfer: studies with retroviral based vector system. Mol Ther 3: 128-138, 2001.

97. Tarantal AF, Lee CI, Ekert JE, McDonald R, **Kohn DB**, Plopper CG, Case SS, Bunnell BA. Lentiviral vector gene transfer into fetal rhesus monkeys (Macaca mulatta): Lung- targeting approaches. Mol Ther 4:614-621, 2001.

98. Skelton D, Satake N, **Kohn DB**. The enhanced green fluorescent protein (eGFP) ism minimally immunogenic in C57BL/6 mice. Gene Ther 8:1813-1814, 2001.

99. Gruber TA, Skelton DC, **Kohn DB**. Requirement for natural killer cells in CD40 ligand mediated rejection of Philadelphia chromosome positive acute lymphoblastic leukemia cells. J Immunol 168:73-80, 2002.

100. Shah AJ, Kapoor N, Weinberg KI, Crooks GM, **Kohn DB**, Lenarsky C, Kaufman F, Epport K, Wilson K, Parkman R. Second hematopoietic stem cell transplantation in pediatric patients: overall survival and long-term follow-up. Biol Blood Marrow Transplant 8:221-228, 2002.

101. O'Rourke JP, Newbound GC, **Kohn DB**, Olsen JC, Bunnell BA. Comparison of gene transfer efficiency and gene expression with HIV-1 and EIAV derived lentivirus vectors. J Virol 76:1510-1515, 2002.

102. Kurre P, Morris J, Andrews RG, **Kohn DB**, Kiem H-P. Kinetics of fluorescence expression in non-human primates transplanted with GFP retrovirus-modified CD34 cells. Mol Ther 6:83-90, 2002.

PUBLICATIONS (Cont'd)

103. Yates F, Malassis-S･ ris M, Stockholm D, Bouneaud C, Larousserie F, Noguiez-Hellin P, Danos O, **Kohn DB**, Fischer A,de Villartay J-P, Cavazzana-Calvo M. Gene therapy of RAG-2-/-mice: sustained correction of the immunodeficiency. Blood 100:3942-3949, 2002.

104. Logan AC, Lutzko C, **Kohn DB**. Advances in lentiviral vector design for gene-modification of hematopoietic stem cells. Curr Opinion in Biotechnology 13:429-436, 2002.

105. Grove JE, Lutzko C, Priller J, Henegariu O, Theise ND, **Kohn DB**, Krause DS. Marrow-derived cells as vehicles for delivery of gene therapy to pulmonary epithelium. Am J Resp Cell and Mol Biology, 27:645-651, 2002.

106. Price MA, Case SS, Carbonaro DA, Yu X-J, Petersen D, Sabo KM, Curran MA, Engel BC, Margarian H, Abnkowitz JL, Nolan GP, **Kohn DB**, Crooks GM. Expression from second generation feline immunodeficiency virus vectors is impaired in human hematopoietic cells. Mol Ther 6:645-652, 2002.

107. Burgos JS, Rosol M, Moats RA, Khankaldyyan V, **Kohn DB**, Nelson, Jr. MD, Laug WE. Time course of bioluminescent signal in orthotopic and heterotopic brain tumors in nude mice. BioTechniques 34: 1184-1188, 2003.

108. **Kohn, DB**, Sadelain M, Glorioso JC. Occurrence of leukemia following gene therapy of X-linked SCID. Nat Rev Cancer 3:477-488, 2003.

109. Schmidt M, Carbonaro DA, Speckmann C, Wissler M, Bohnsack J, Elder M, Aronow BJ, Nolta JA, **Kohn DB**, Von Kalle C. Clonality analysis after retroviral-mediated gene transfer to CD34+ cells from the cord blood of ADA-deficient SCID neonates. Nat Med 9:463-468, 2003.

110. Goto H, Yang B, Peterson D, Pepper K, **Kohn DB**, Reynolds CP. Transduction of green fluorescence protein increases oxidative stress and enhances sensitivity to cytotoxic drugs in neuroblastoma cell lines. Mol Cancer Ther 2:911-917, 2003.

111. Kurre P, Morris J, Thomasson B, **Kohn DB**, Kiem H-P. Scaffold attachment region containing retrovirus vectors improve long-term proviral expression after transplantation of GFP-modified CD34+ baboon repopulating cells. Blood 102:3117-3119, 2003.

112. Lutzko C, Logan A, Senadheera D, Petersen D, **Kohn DB**. Lentivirus vectors incorporating the immunoglobulin heavy chain enhancer and matrix attachment regions provide position-independent expression in a B lymphocytes. J Virol 77:7341-7351, 2003.

PUBLICATIONS (Cont'd)

113. Schmidt M, Glimm H, Wissler M, Hoffman 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 foamy vector-transduced cell populations by high-accuracy insertion site sequencing. Ann NY Acad Sci 996:112-121, 2003.

114. Wang L, Haas D, Halene S, **Kohn DB**. Effects of the negative control region on expression from retroviral LTR. Mol Ther 7:438-440, 2003

115. Wang X, Rosol M, Ge S, Petersen D, McNamara G, Pollack H, **Kohn DB**, Nelson MD, Crooks GM. Dynamic tracking of human hematopoietic stem cell engraftment using in vivo bioluminescense imaging. Blood 102:3478-3482, 2003.

116. Haas DL, Lutzko C, Logan AC, Cho GJ, Skelton D, YU X-J, Pepper KA, **Kohn DB**. The moloney murine leukemia virus repressor binding site represses expression in murine and human hematopoietic stem cells. J Virol 77:9439-9450, 2003.

117. Zheng Y, Rozengurt N, Ryazantsev S, **Kohn DB**, Satake N, Neufeld EF. Treatment of the mouse model of mucopolysaccharidosis I with retrovirally transduced bone marrow. Mol Genetics and Metabolism 79:233-244, 2003.

118. Logan AC, Nightingale S, Haas DL, Cho GJ, Pepper KA, **Kohn DB**. Factors influencing the titer and infectivity of lentiviral vectors. Hum Gene Ther 15:976-988, 2004.

119. Zheng Y, Ryazantsev S, Ohmi K, Zhao HZ, Rozengurt N, **Kohn DB**, Newfeld EF. Retrovirally transduced bone marrow has a therapeutic effect on brain in the mouse model of mucopolysaccharidosis IIIB. Mol Genet Metab 82:286-295, 2004.

120. Kearns-Jonker M, Fisher-Lougheed J, Shulkin I, Dleihauer A, Mitsuhashi N, **Kohn DB**, Weinberg K, D'Apice AJ, Starnes VA, Cramer DU. Use of lentiviral vectors to induce long-term tolerance to gal(+) heart grants. Transplant 77:1748-1754, 2004.

121. Lee CI, Cowan MJ, **Kohn DB**, Tarantal AF. Simian immunodeficiency virus infection of hematopoietic stem cells and bone marrow stromal cells. J acquir Immune Defic Syndr. 36:553-561, 2004.

122. Logan AC, Haas DL, Kadri T, **Kohn DB**. Integrated self-inactivating lentiviral vectors produce full-length genomic transcripts competent for encapsidation and integration. J of Virol 78:8421-8436, 2004.

123. Shah AJ, Lenarsky C, Kapoor N, Crooks GM, **Kohn DB**, Parkman R, Epport K, Wilson K, Weinberg K. Busulfan and cyclophosphamide as a conditioning regimen for pediatric acute lymphoblastic leukemia patients undergoing bone marrow transplantation. J Pediatr Hematol Oncol 26:91-97, 2004.

PUBLICATIONS (Cont'd)

124.Lee CI, **Kohn DB**, Ekert JE, Tarantal AF. Morphological analysis and lentiviral transduction of fetal monkey bone marrow-derived mesenchymal stem cells. Mol Ther 9:112-123, 2004.

125. Gruber TA, Skelton DC, Pepper K, Petersen D, **Kohn DB**. Recombinant murine Ilterleukin-12 elicits potent anti-leukemic immune responses in a murine model of Philadelphia chromosome positive acute lymphoblastic leukemia. Cancer Gene Ther 12:818-824, 2005.

126.Kobayashi H, Carbonaro D, Pepper K, Petersen D, Ge S, Jackson HA, Shimada H, Moats R, **Kohn DB**. Neonatal gene therapy of MPS I mice by intravenous injection of a lentiviral vector. Mol Ther 11:776-789, 2005.

127.Podsakoff GM, Engel BC, Carbonaro DA, Choi C, Smogorzewska EM, Bauer G, Selander D, Csik S, Wilson K, Betts MR, Koup RA, Nabel GJ, Bishop K, King S, Schmidt M, von Kalle C, Church JA, **Kohn DB**. Selective survival of peripheral blood lymphocytes in children with HIV-1 following delivery of an anti-HIV gene to bone marrow CD34 (+) cells. Mol Ther 12:77-86, 2005.

128. Podsakoff GM, Engel BC, **Kohn DB**. Perspectives on gene therapy for immune deficiencies. Biol Blood Marrow Transplant 11:972-976, 2005.

129.Lee CC, Jimenez DG, **Kohn DB**, Tarantal AF. Fetal gene transfer using lentiviral vectors and the potential for germ cell transduction in rhesus monkeys (Macaca mulatta). Hum Gene Ther 16:417-425, 2005.

130.Tarantal AF, McDonald RJ, Jimenez DF, Lee CC, O'Shea CE, Leapley AC, Won RH, Plopper CG, Lutzko C, **Kohn DB**. Intrapulmonary and intramyocardial gene transfer in rhesus monkeys (Macaca mulatta): safety and efficiency of HIV-1-derived lentiviral vectors for fetal gene delivery. Mol Ther 12:87-98, 2005.

131.Chan B, Wara D, Bastian J, Hershfield MS, Bohnsack J, Azen CG, Parkman R, Weinberg K, **Kohn DB**. Long-term efficacy of enzyme replacement therapy for adeno deaminase (ADA)-deficient severe combined immunodeficiency (SCID). Clin Immunol117:133-143, 2005.

132.Shah AJ, Kapoor N, Crooks GM, Parkman R, Weinberg KI, Wilson K, **Kohn DB**. Successful hematopoietic stem cell transplantation for Niema Pick disease type B. Pediatrics 116:1022-1025, 2005.

133.Jimenez DF, Lee CI, O'Shea C, **Kohn DB**, Ekert JE, Tarantal AF. HIV-1 derived lentiviral vector and fetal route of administration on transgene biodistribution and expression in rhesus monkeys (Macaca mulatto). Gene Ther 12:821-830, 2005.

PUBLICATIONS (Cont'd)

134. Mitsuhashi N, Fischer-Lougheed J, Shulkin I, Kleihauer A, **Kohn DB**, Weinberg KI, Starnes VA, Kearns-Jonker M. Tolerance induction by lentiviral gene therapy with a non-myeloablative regimen. Blood 107:2286-2293, 2006.

135. Kahl CA, Tarantal AF, Lee CI, Jimenez DF, Choi C, Pepper K, Petersen D, Fletcher MD, Leapley AC, Burns TS, Ultsch MN, **Kohn DB**. Effects of Busulfan dose escalation on gene marking of hematopoietic stem cells by a SIV lentiviral vector in infant rhesus monkeys. Exp Hematol, 34:369-381, 2006.

136. Jang JE, Shaw K, Yu X-J, Petersen D, Pepper K, Lutzko C, **Kohn DB**. Specific gene transfer to human embryonic stem cells using pseudotyped lentiviral vectors. Stem Cells and Development, 15:109-117, 2006.

137. Unwalla HJ, Li HT, Bahner I, Li MJ, **Kohn DB**, Rossi JJ. Novel Pol II fusion promoter directs human immunodeficiency virus type 1-inducible co-expression of a short hairpin RNA and protein. J Virol 80:1863-1873, 2006.

138. Carbonaro DA, Jin X, Petersen D, Wang X, Dorey F, Kil KS, Aldrich M, Blackburn MR, Kellems RE, **Kohn DB**. In vivo transduction by intravenous injection of a lentiviral vector expressing human ADA into neonatal ADA gene Knock-out mice. A Novel form of enzyme replacement therapy for ADA-deficiency. Mol Ther 3:1121-1132, 2006.

139. Nightingale SJ, Hollis RP, Pepper KA, Petersen D, Yu X-J, Yang C, Bahner IC**,** **Kohn DB**. Transient gene expression by non-integrating lentiviral (NIL) vectors. Mol Ther 13:1121-1132, 2006.

140. Hollis RP, Nightingale SJ, Pepper KA, Yu X-J, Barsky L, Crooks GM, **Kohn DB**. Stable gene transfer to human CD34+ hematopoietic progenitor cells using the sleeping beauty transposon. Exp Hematol 34:1333-1343, 2006.

141. Anilkumar G, Barwe SP, Christiansen JJ, Rajasekaran SA, **Kohn DB**, Rajasekaran AK. Association of prostate-specific membrane antigen with caveolin-1 and its caveolae-dependent internalization in microvascular endothelial cells: Implications for targeting to tumor vasculature. Microvasc Res 2006.

142. Bahner I, Sumiyoshi T, Kagoda M, Swartout R, Peterson D, Pepper K, Dorey F, Reiser J, and **Kohn DB**. Lentiviral vector transduction of a dominant-negative rev gene into human CD34+ hematopoietic progenitor cells potently inhibits HIV-1 replication. Mol Ther 15:76-85, 2007

PUBLICATIONS (Cont'd)

143. Engel BC, Podsakoff GM, Ireland J, Smogorzewska M, Carbonaro DA, Wilson K, Shah A, Kapoor N, Sweeney M, Borchert M, Crooks G, Weinberg K, Parkman R, Rosenblatt H, Wu SQ, Hershfield M, Candotti F, **Kohn DB**. Prolonged pancytopenia in a gene therapy patient with ADA- deficient SCID and trisomy 8 mosaicism. Blood 109:503-506, 2007.

144. Blumenthal M, Skelton D, Pepper KA,, Jahn T, Methangkool K, **Kohn DB**. Effective suicide gene therapy for leukemia in a novel model of insertional oncogenesis in mice. Mol Ther 15:183-192, 2007.

145. Fischer-Lougheed JY, Tarantal AF, Shulkin I, Mitsuhashi N, **Kohn BD**, Lee CC,, Kearns-Jonker M. Gene therapy to inhibit xenoantibody production using lentiviral vectors in non-human primates. Gene Ther 14:49-57, 2007.

146. Barwe SP, Maul RS, Christiansen JJ, Anilkumar G, Cooper CR, **Kohn DB**, Rajasekaran AK. Preferential association of prostate cancer cells expressing prostate specific membrane antigen to bone marrow matrix. Int J Oncol 4:899-904, 2007.

147. Shah AJ, Kapoor N, Crooks GM, Weinberg KI, Azim HA, Killen R, Kuo L, Rushing T, **Kohn DB**, and Parkman R. The Effects of Campath 1H upon Graft-Versus-Host Disease, Infection, Relapse, and Immune Reconstitution in Recipients of Pediatric Unrelated Transplants. Biol Blood Marrow Transplant 13:584-593, 2007.

148. Higashimoto T, Urbinati F, Perumbeti A, Jiang G, Zarzuela A, Chang LJ, **Kohn DB**, Malik P. The woodchuck hepatitis virus post-transcriptional regulatory element reduces read through transcription from retroviral vectors. Gene Ther 14:1298-1304, 2007.

149. Taylor JA, Vojtech L, Bahner I, **Kohn DB**, Laer DV, Russell DW, Richard RE. Foamy virus vectors expressing anti-HIV transgenes efficiently block HIV-1 replication. Mol Ther 14(17):1298-304, 2007.

150. Rodriguez RT, Velkey JM, Lutzko C, Seerke R, **Kohn DB**, Oshea KS, Firpo MT. Manipulation of OCT4 levels in human embryonic stem cells results in induction of differential cell types. Exp Biol Med 232:1368-1380, 2007.

151. Abdel-Azim H, Zhu Y, Hollis R, Wang X, Ge S, Hao QL, Smbatyan G, **Kohn DB**, Rosol M, Crooks GM. Expansion of multipotent and lymphoid-committed human progenitors through intracellular dimerization of Mpl. Blood 111:4064-74, 2008.

152. Kahl CA, Cannon PM, Oldenburg J, Tarantal AF, **KohnDB**. Tissue-specific restriction of cyclophilin A-independent HIV-1 and SIV-derived lentiviral vectors. Gene Ther 15:1079-89, 2008

PUBLICATIONS (Cont'd)

153. Carbonaro DA, Jin X, YuX-J, Skelton D, Mi T, Dorey F, Kellem RE, Blackburn M, **Kohn DB**. Neonatal bone marrow transplantation of ADA-deficient SCID mice results in immunological reconstitution despite low levels of engraftment and an absence of selective donor T lymphoid expansion. Blood 111:5745-5754, 2008.

154. Singh H, Manuri PR, Olivares S, Dara N, Dawson MJ, Huls H, Hackett PB, **Kohn DB**, Shpall EJ, Champlin R, Cooper LJN. Redirecting specificity of T cells for CD19 using Sleeping Beauty System. Cancer Res 68:2961-2971, 2008

155. Bauer G, Dao MA, Case SS, Meyerrose T, Wirthlin L, Zhou P, Wang X, Herrbrich P, Arevalo J, Csik S, Skelton DC, Walker J, Pepper K, **Kohn DB**, Nolta JA. In Vivo biosafety model to assess the risk of adverse events from retroviral and lentiviral vectors. Mol Ther 16(7):1308-15, 2008.

156. Chhabra A, Yang L, Wang P, Das R, Chakraborty NG, Dorsky D, **Kohn DB,** Hardy C, Ribas A, Economou J, Baltimore D, Mukherji B. CD34+CD25- T cells transduced to express MHC class I-restricted epitope specific TCR synthesize Th1 cytokines and exhibit MHC class I-restricted cytolytic effector function in a human melanoma model. J Immunol. 181(2):1063-70, 2008

157. Shah AJ, Epport K, Azen C, Killen R, Wilson K, De Clerck D, Crooks G, Kapoor N, **Kohn DB**, Parkman R, Weinberg KI. Progressive declines in neurocognitive function among survivors of hematopoietic stem cell transplantation for pediatric hematologic malignancies. J Pediatr Hematol Oncol. 30:411-8, 2008.

158. Griffith LM, Cowan MJ, Kohn DB, Notarangelo LD, Puck JM, Schultz KR, Buckley RH, Eapen M, Kamani NR, O'Reilly RJ, Parkman R, Roifman CM, Sullivan KE, Filipovich AH, Fleisher TA, Shearer WT. Allogeneic hematopoietic cell transplantation for primary immune deficiency diseases: current status and critical needs. J Allergy Clin Immunol 122:1087-96, 2008.

159. Gruber TA, Shah AJ, Hernandez M, Crooks GM, Abdel-Azim H, Gupta S, McKnight S, White D, Kapoor N, **Kohn DB**. Clinical and genetic heterogeneity in Omenn syndrome and severe combined immune deficiency. Pediatr Transplant. 13:244-50, 2009.

160. Shah AJ, Kapoor N, Cooper RM, Crooks GM, Lenarsky C, Abdel-Azim H, Wu SQ, Wilson K, Weinberg KI, Parkman R, **Kohn DB**. Pre- and post-natal treatment of hemophagocytic lymphohistiocytosis. Pediatr Blood Cancer. 2009 52(1):139-142.

161. Lin M, Epport K, Azen C, Parkman R, **Kohn DB**, Shah AJ. Long-Term Neurocognitive Function of Pediatric Patients with Severe Combined Immune Deficiency (SCID): Pre- and Post-Hematopoietic Stem Cell Transplant (HSCT). J Clin Immunol. 29:231-7, 2009.

PUBLICATIONS (Cont'd)

162. Sondergaard CS, Haldrup C, Beer C, Andersen B, **Kohn DB**, and Pedersen L. Preloading Potential of Retroviral Vectors is Packaging Cell Clone Dependent and Centrifugation onto CH-296 Ensures Highest Transduction Efficiency. Hum Gene Ther. 20:337-349, 2009

163. Shaw KL, Pais E, Ge S, Hardee C, Skelton D, Hollis RP, Crooks GM, **Kohn DB**. Lentiviral vectors with amplified beta cell-specific gene expression. Gene Ther. 16:998-1008, 2009.

164. Sumiyoshi T, Holt NG, Hollis RP, Ge S, Cannon PM, Crooks GM, and **Kohn DB**. Stable transgene expression in primitive human CD34+ hematopoietic stem/ progenitor cells using the *Sleeping Beauty* transposon system. Human Gene Therapy, 20:1607-1626, 2009.

165. **Kohn, DB**. Update on gene therapy for primary immune deficiencies. Clin Immunol, 135:247-54. 2010.

166. Pais E, Park J, Alexy T, Nikolian V, Ge S, Shaw K, Senadheera S, Hardee CL, Skelton D, Hollis R, Crooks GM and **Kohn DB**. Regulated expansion of human pancreatic beta cells. Molecular Therapy, 18:1389-96, 2010.

167. Becker PS, Taylor JA, Trobridge GD, Zhao X, Beard BC, Chien S, Adair J, and **Kohn DB**, Wagner JE, Shimamura A, Kiem HP. Preclinical correction of human Fanconi anemia complementation group A bone marrow cells using a safety-modified lentiviral vector. Gene Ther 17:1244-1252, 2010.

168. Holt N, Wang J, Kim K, Friedman G, Wang X, Taupin V, Crooks GM, **Kohn DB**, Gregory PD, Holmes MC, and Cannon PM. Human hematopoietic stem/progenitor cells modified by zinc-finger nucleases targeted to CCR5 control HIV-1 in vivo. Nat Biotechnol 28:839-847, 2010.

169. **Kohn DB**, Dotti G, Brentjens R, Savoldo B, Jensen M, Cooper LJN, June CH, Rosenberg S, Sadelain M and Heslop H. CARS on Track in the Clinic: Report of a Meeting Organized by the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) Sub-Committee on Cell and Gene Therapy. - Washington D.C., May 18, 2010. Molecular Therapy, Mol Ther 19:432-438, 2011.

170. Horwitz EM, Horowitz MM, Difronzo NL, **Kohn DB,** Heslop HE; for the BMT CTN State of the Science Cell and Gene Therapy Committee. Guidance for Developing Phase II Cell Therapy Trial Proposals for Consideration by the Blood and Marrow Transplant Clinical Trials Network. Biol Blood Marrow Transplant 17:192-106, 2011.

PUBLICATIONS (Cont'd)

171. Sokolic R, Maric I, Kesserwan C, Garabedian E, Hanson IC, Bali P, Hershfield MS, **Kohn DB**, Wayne AS, and Candotti F. Myeloid dysplasia and bone marrow hypocellularity in adenosine deaminase-deficient severe combined immune deficiency. Blood 118(10):2688-94, 2011.

172. Cooper AR, Patel S, Senadheera S, Plath K, **Kohn DB** and Hollis RP. Highly Efficient Large-Scale Lentiviral Vector Concentration by Tandem Tangential Flow Filtration. Journal of Virological Methods 177:1-9, 2011. PMC4276401

173. Vatakis DN. Koya RC, Nixon CC, Wei L, Kim SG, Avancena P, Bristol G, Baltimore D, **Kohn DB**, Ribas A, Radu CG, Galic Z, and Zack JA. Anti-tumor activity from antigen specific CD8 T cells generated in vivo from genetically engineered human hematopoietic stem cells. Proc Natl Acad Sci (USA) 108(51):E1408-16, 2011.

174. Karumbayaram S, Lee P, Azghadi S, Cooper AR, **Kohn DB**, Pyle A, Clark A, Byrne J, Zack J, Plath K, and Lowry WE. From Skin Punch Biopsy to Neurons and Glia through a Pluripotent Cell Intermediate Under Xenogenic-free Good Manufacturing Practice (GMP) Conditions. Stem Cells Transl Med 1:36–43, 2012.

175. Tarantal AF, Giannoni F, Sumiyoshi T, Lee CI, Hollis RH, Wherley J, Martinez M, Leapley A, Kahl C, Louie SG, and **Kohn DB**. Non-Myeloablative Conditioning Regimen to Increase Engraftment of Gene-Modified Hematopoietic Stem Cells. Mol Ther 20:1033-45, 2012.

176. Corrigan-Curay J, Cohen-Haguenauer O, O'Reilly M, Ross SR, Fan H, Rosenberg N, Somia N , King N, Friedmann T, Dunbar C, Aiuti A, Naldini L, Baum C, von Kalle C, Kiem H-P, Montini E, Bushman F, Sorrentino BP, Carrondo M, Malech H, Gahrton G, Shapiro R, Wolff L, Rosenthal E, Jambou R, Zaia J, and **Kohn DB**. Challenges in Vector and Trial Design using Retroviral vectors for Long Term Gene Correction in Hematopoietic Stem Cell Gene Therapy: Summary of a Symposium Sponsored by the NIH Office of Biotechnology Activities and the EC DG-research NoE for the Advancement of Clinical Gene Transfer and Therapy. Mol Ther 20:1084-94, 2012.

177. Parekh C, Sahaghian A, Kim W, Scholes J, Ge S, Zhu Y, Asgharzadeh S, Hollis R, **Kohn D**, Ji L, Malvar J, Wang X, Crooks G. Novel pathways to erythropoiesis induced by dimerization of intracellular C-Mpl in human hematopoietic progenitors. Stem Cells. 2012 Apr;30(4):697-708. PMID: 22290824

178. Carbonaro DA, Jin X, Wang X, Rozengurt N, Blackburn MR and **Kohn DB.** Gene therapy *ex vivo* in ADA-deficient mice: the role of enzyme replacement therapy and cytoreduction. Blood 120:3677-3687, 2012.

PUBLICATIONS (Cont'd)

179. Candotti F, Shaw K, Muul L, Carbonaro D, Sokolic R, Choi C, Shurman S, Garabedian E, Kesserwan C, Jagadessh GJ, Fu PY, Gschweng E, Cooper A, Tisdale JF, Weinberg KI, Crooks GM, Kapoor N, Shah A, Abdel-Azim H, Yu XJ, Smogorzewska M, Wayne AS, Rosenblatt HM, Davis CM, Hanson C, Rishi RG, Wang X, Gjertson D, Yang OO, Balamurugan A, Bauer G, Ireland J, Engel BC, Podsakoff GM, Hershfield M, Blaese RM, Parkman R, and **Kohn DB**. Gene Therapy for Adenosine Deaminase-Deficient Severe Combined Immune Deficiency: Clinical Comparison of Retroviral Vectors and Treatment Plans. Blood, 120: 3635-3646, 2012. **Plenary Paper**.

180. Harrison F, Yeagy BA, Rocca CJ, **Kohn DB**, Salomon DR, and Cherqui S. Hematopoietic stem cell gene therapy for the multi-systemic lysosomal storage disorder cystinosis. Mol Ther 21:433-44, 2013. PMID: 23089735

181. De Oliveira SN, Wang J, Ryan C, Morrison SL, **Kohn DB**, Hollis RP. A CD19/Fc fusion protein for detection of anti-CD19 chimeric antigen receptors. J Transl Med 11:23, 2013.

182. O'Reilly M, **Kohn DB**, Bartlett J, Benson JM, Brooks P, Byrne B, Cammozi C, Cornetta K, Crystal R, Fong Y, Gargiulo L, Gopal-Srivastava R, High KA, Jacobson SG, Jambou R, Montgomery M, Rosenthal E, Samulski RJ, Skarlatos SI, Sorrentino B, Wilson JM, Xie Y, Corrigan-Curay J. Gene Therapy for Rare Diseases: Summary of a National Institutes of Health Workshop, September 13, 2012. Hum Gene Ther 24:355-62, 2013

183. McCracken M, Gschweng EH, Nair-Gill E, McLaughlin J, Cooper A, Riedinger M, Cheng D, Nosala C, **Kohn DB**, and Witte ON. Long-term In vivo monitoring of mouse and human hematopoietic stem cell engraftment with a human Positron Emission Tomography reporter. PNAS (USA), 110:1857-62, 2103.

184. Giannoni F, Hardee CL, Wherley J, Gschweng E, Senadheera S, Kaufman ML, Chan R, Bahner I, Gersuk V, Wang X, Gjertson D, Baltimore D, Witte ON, Economou JS, Ribas A, and **Kohn DB**. Allelic Exclusion and Peripheral Reconstitution by TCR Transgenic T Cells Arising from Transduced Human Hematopoietic Stem/Progenitor Cells. Mol Ther 21:1044-54.2013.

185. Dvorak CC, Cowan MJ, Logan BR, Notarangelo LD, Griffith LM, Puck JM, **Kohn DB**, Shearer WT, O'Reilly RJ, Fleisher TA, Pai SY, Hanson IC, Pulsipher MA, Fuleihan R, Filipovich A, Goldman F, Kapoor N, Small T, Smith A, Chan KW, Cuvelier G, Heimall J, Knutsen A, Loechelt B, Moore T, Buckley RH. The Natural History of Children with Severe Combined Immunodeficiency: Baseline Features of the First Fifty Patients of the Primary Immune Deficiency Treatment Consortium Prospective Study 6901. J Clin Immunol. 2013. PMID: 23818196

PUBLICATIONS (Cont'd)

186. Kwan A, Church JA, Cowan MJ, Agarwal R, Kapoor N, **Kohn DB**, Lewis DB, McGhee SA, Moore TB, Stiehm ER, Porteus M, Aznar CP, Currier R, Lorey F, Puck JM. Newborn screening for severe combined immunodeficiency and T-cell lymphopenia in California: Results of the first 2 years. J Allergy Clin Immunol. 2013 Jul;132(1):140-150.e7. PMID: 23810098

187. Romero Z*, Urbinati F*, Geiger S, Cooper A, Wherley J, Kaufman ML, Hollis R, Senadheera S, Ruiz de Assin R, Sahagian A, Wang X, Gjertson D, DeOliveira S, Kempert P, Shupien S, Abdel-Azim H, Walters M, Meiselman HJ, Marder V, Coates TD, and **Kohn DB**. Gene transfer to human bone marrow for sickle cell disease. J Clin Invest, 123:3317–3330, 2013. PMID: 23863630
*Equal contributions.

188. Joglekar A, Hollis RP, Kuftinec G, Shenadeera S, Awe JP, Chan R, Reik A, Flinders C, Byrne JA, Holmes MC, Gregory PD, and **Kohn DB**. Integrase-Defective Lentiviral Vectors for Delivery of Zinc Finger Nucleases and Donor Templates for Site-Specific Gene Modification. Mol Ther.21:1705-1717, 2013. PMID: 23857176

189. De Oliveira SN, Ryan C, Giannoni F, Hardee C, Tremcinska I, Katebian B, Wherley J, Sahaghian A, Grogan T, Elashoff D, Cooper L, Hollis R, and **Kohn DB**. Modification of Hematopoietic Stem/Progenitor Cells with CD19-specific Chimeric Antigen Receptors as a Novel Approach for Cancer Immunotherapy. Human Gene Ther. 24:824-39, 2013. PMID: 23978226

190. Awe JP, Lee PC, Ramathal C, Vega-Crespo , Durruthy-Durruthy J, Cooper A, Karumbayaram S, Lowry W, Clark A, Zack J, Sebastiano V, **Kohn DB**, Pyle A, Martin M, Lipshutz GS, Phelps PE, Reijo-Pera R and Byrne JA. Generation and characterization of transgene-free human induced pluripotent stem cells and conversion to putative clinical-grade status. Stem Cell Res & Ther 4:87, 2013.

191. Shearer WT, Dunn, E, Notarangelo LD, Dvorak C, Puck J, Logan B, Griffith LM, **Kohn DB**, O'Reilly RJ, Fleisher TA, Pai S-Y, Martinez CA, Buckley RH, and Cowan MJ. Establishing diagnostic criteria for severe combined immune deficiency (SCID), leaky SCID, and Omenn Syndrome: The primary immune deficiency treatment consortium experience. J All Clin Immunol, 2013 133(4):1092-8. PMID: 24290292 PMCID: PMC3972266

192. Stoyanova TI, Cooper AR, Drake JM, Liu X, Armstrong AJ, Zhang H, **Kohn DB**, Huang J, Witte ON, and Goldstein AS. Prostate cancer originating in basal cells progresses to adenocarcinoma maintained by luminal-like tumor-propagating cells. PNAS, Proc Natl Acad Sci (U S A). 2013 Nov 26. 110(50):20111-6. PMID: 24282295 PMCID: PMC3864278

PUBLICATIONS (Cont'd)

193. Carbonaro DA, Zhang L, Jin X, Montiel-Equihua C, Geiger S, Carmo M, Cooper A, Fairbanks L, Kaufman ML, Sebire NJ, Hollis RP, Blundell MP, Senadheera S, Fu PY, Sahaghian A, Chan RY, Wang X, Cornetta K, Thrasher AJ, **Kohn DB**, Gaspar HB. Pre-clinical demonstration of lentiviral vector mediated correction of immunological and metabolic abnormalities in models of adenosine deaminase deficiency. Mol Ther 22:607-22, 2014. PMID: 24256635 PMCID: PMC3944341

194. Griffith LM, Cowan MJ, Notarangelo LD, **Kohn DB**, Puck JM, Pai SY, Ballard B, Bauer SC, Bleesing JJ, Boyle M, Brower A, Buckley RH, van der Burg M, Burroughs LM, Candotti F, Cant AJ, Chatila T, Cunningham-Rundles C, Dinauer MC, Dvorak CC, Filipovich AH, Fleisher TA, Bobby Gaspar H, Gungor T, Haddad E, Hovermale E, Huang F, Hurley A, Hurley M, Iyengar S, Kang EM, Logan BR, Long-Boyle JR, Malech HL, McGhee SA, Modell F, Modell V, Ochs HD, O'Reilly RJ, Parkman R, Rawlings DJ, Routes JM, Shearer WT, Small TN, Smith H, Sullivan KE, Szabolcs P, Thrasher A, Torgerson TR, Veys P, Weinberg K, Zuniga-Pflucker JC; on behalf of the workshop participants. Primary Immune Deficiency Treatment Consortium (PIDTC) report. J Allergy Clin Immunol. 2013, 133(2):335-47. PMID: 24139498

195. Chodon T, Comin-Anduix B, Chmielowski B, Koya RC, Wu Z, Ng C, Avramis E, Seja E, Villanueva A, McCannel TA, Ishiyama A, Czernin J, Auerbach M, Wang X, Gjertson DW, Cochran A, Cornetta KG, Wong DL, Hamid O, Samlowski W, Cohen PA, Daniels GA, Mukherji B, Zack JA, **Kohn DB**, Heath JR, Glaspy JA, Witte ON, Baltimore D, Economou JS, Ribas A. Adoptive cell transfer of MART-1 TCR transgenic lymphocytes generated in a one-week culture with dendritic cell vaccination in patients with metastatic melanoma. Clinical Cancer Research, 2014 1;20(9):2457-65. PMID:24677652

196. Joglekar AV, Stein L, Ho M, Hoban M, Hollis RP, and **Kohn DB**. Dissecting the mechanism of histone deacetylase inhibitors to enhance the activity of zinc finger nucleases delivered by integrase-defective lentiviral vectors. Hum Gene Ther. 2014 Jul;25(7):599-608. PMID: 24568341 PMCID: PMC4098962

197. O'Reilly M, Federoff HJ, Fong Y, **Kohn DB**, Patterson AP, Ahmed N, Aravind A, Boye SE, Crystal R, De Oliveira S, Gargiulo L, Harper SQ, Ikeda Y, Jambou R, Montgomery M, Prograis L, Rosenthal E, Sterman DH, Vandenberghe LH, Zoloth L, Abedi M, Adair J, Adusumilli PS, Goins WF, Gray J, Monahan P, Popplewell L, Sena-Esteves M, Tannous B, Weber T, Wierda W, Gopal-Srivastava R, McDonald CL, Rosenblum D, Corrigan-Curay J. Gene Therapy: Charting a Future Course Summary of a National Institutes of Health Workshop, April 12, 2013. Hum Gene Ther. 2014 Jun;25(6):488-97. PMID: 24773122.

PUBLICATIONS (Cont'd)

198. Kim WS, Zhu Y, Deng Q, Chin CJ, He CB, Grieco AJ, Dravid GG, Parekh C, Hollis RP, Lane TF, Bouhassira EE, **Kohn DB**, and Crooks GM. Erythropoiesis from human embryonic stem cells through erythropoietin-independent AKT signaling. Stem Cells. 2014 32(6):1503-14. PMID: 24677652 PMC403736

199. Kohn LA, Seet CS, Scholes J, Codrea F, Chan R, Zaidi-Merchant S, Zhu Y, De Oliveira S, Kapoor N, Shah A, Abdel-Azim H, **Kohn DB**, Crooks GM. Human Lymphoid Development in the Absence of Common γ-Chain Receptor Signaling. J Immunol. 2014 192(11):5050-8. PMID: 24771849. PMCID: PMC4052377

200. Carbonaro Sarracino DA, Tarantal AF, Lee CI, Martinez M, Jin X, Wang X, Hardee CL, Geiger S, Kahl CA and **Kohn DB**. Effects of Vector Backbone and Pseudotype on *In Vivo* Lentiviral Vector-Mediated Gene Transfer for ADA-deficiency: Studies in Mice and Rhesus Monkeys. Mol Ther 15:1079-89, 2014. PMID: 18385767.

201. Gschweng EH, McCracken MN, Koya R, Chodon T, Kaufman ML, Hollis RP, Saini N, Ribas A, Witte ON, and **Kohn DB**. PET Imaging and Suicide Gene Function of HSV-sr39TK in a Humanized Mouse Model of Hematopoietic Stem Cell Based Immunotherapy. Cancer Res. 74:5173-83, 2014. PMID: 25038231

202. Corrigan-Curay J, Kiem HP, Baltimore D, O'Reilly M, Brentjens RJ, Cooper L, Forman S, Gottschalk S, Greenberg P, Junghans R, Heslop H, Jensen M, Mackall C, June C, Press O, Powell D, Ribas A, Rosenberg S, Sadelain M, Till B, Patterson AP, Jambou RC, Rosenthal E, Gargiulo L, Montgomery M, **Kohn DB**. T-cell immunotherapy: looking forward. Mol Ther. 2014 Sep;22(9):1564-74. PMID: 25186558

203. Pai SY, Logan BR, Griffith LM, Buckley RH, Parrott RE, Dvorak CC, Kapoor N, Hanson IC, Filipovich AH, Jyonouchi S, Sullivan KE, Small TN, Burroughs L, Skoda-Smith S, Haight AE, Grizzle A, Pulsipher MA, Chan KW, Fuleihan RL, Haddad E, Loechelt B, Aquino VM, Gillio A, Davis J, Knutsen A, Smith AR, Moore TB, Schroeder ML, Goldman FD, Connelly JA, Porteus MH, Xiang Q, Shearer WT, Fleisher TA, **Kohn DB**, Puck JM, Notarangelo LD, Cowan MJ, O'Reilly RJ. Transplantation outcomes for severe combined immunodeficiency, 2000–2009. N Engl J Med, 371:434-46, 2014. PMID:25075835.

204. Kwan A, Abraham RS, Currier R, Brower A, Andruszewski K, Abbott JK, Baker M, Ballow M, Bartoshesky LE, Bonilla FA, Brokopp C, Brooks E, Caggana M, Celestin J, Church JA, Comeau AM, Connelly JA, Cowan MJ, Cunningham-Rundles C, Dasu T, Dave N, De La Morena MT, Duffner U, Fong CT, Forbes L, Freedenberg D, Gelfand EW, Hale JE, Hanson IC, Hay BN, Hu D, Infante A, Johnson D, Kapoor N, Kay DM, **Kohn DB**, Lee R, Lehman H, Lin Z, Lorey F, Abdel-Mageed A, Manning A, McGhee S, Moore TB, Naides SJ, Notarangelo LD, Orange JS, Pai SY, Porteus M, Rodriguez R, Romberg N, Routes J, Ruehle M,

Rubenstein A, Saavedra-Matiz CA, Scott G, Scott PM, Secord E, Seroogy C, Shearer WT, Siegel S, Silvers SK, Stiehm ER, Sugerman RW, Sullivan JL, Tanksley S, Tierce ML 4th, Verbsky J, Vogel B, Walker R, Walkovich K, Walter JE, Wasserman RL, Watson MS, Weinberg GA, Weiner LB, Wood H, Yates AB, Puck JM. Newborn screening for severe combined immunodeficiency in 11 screening programs in the United States. JAMA, 312(7):729-38, 2014. PMID: 25138334

205. Hacein-Bey-Abina S, Pai S-Y, Gaspar HB, Armant M, Bayford J, Berry C, Blanche S, Blondeau J, de Boer H, Buckland K, Caccavelli L, Cros G, De Oliveira S, Fischer A, Guo D, Harris C, Hopkins G, Lehmann LE, London W, van der Loo JCM, Malani N, Male F, Malik P, Marinovic A, McNichol AM, Moshous D, Neven B, Oleastro N, Picard C, Ritz J, Rivat C, Schambach A, Sherman E, Silberstein L, Touzot F, Tsytsykova A, Baum C, Bushman FD, **Kohn DB**, Filipovich AH, Notarangelo LD, Cavazzana M, Williams DA, Thrasher AJ. A modified γ-retrovirus vector for X-linked severe combined immunodeficiency. N Engl J Med, 2014 371(15):1407-17. PMID: 25295500

206. **Kohn DB**. Eliminating SCID Row: new approaches to SCID. Hematology 2014 2014:475-480.

207. Cooper AR, Lill GR, Gschweng EH, and **Kohn DB**. Rescue of splicing-mediated intron loss maximizes expression in lentiviral vectors containing the human Ubiquitin C promoter. *Nucleic Acids Res*. 43(1): 682–690, 2014. PMC4288199

208. Baldwin KM, Urbinati F, Romero Z, Campo-Fernandez C, Kaufman ML, Cooper AR, Hollis RP and **Kohn DB**. Enrichment of human hematopoietic stem/progenitor cells facilitates transduction for stem cell gene therapy. *Stem Cells*, 2015 May;33(5):1532-42. PMID: 25588820.

209. Awe JP, Gschweng EH, Vega-Crespo A, Voutila J, Williamson MH, Truong B, **Kohn DB**, Kasahara N, Byrne JA. Putative Immunogenicity Expression Profiling Using Human Pluripotent Stem Cells and Derivatives. Stem Cells Transl Med. 2015 Feb;4(2):136-45. PMID: 25575527

210. Hoban MD, Cost GJ, Mendel MC, Romero Z, Kaufman ML, Joglekar AV, Ho M, Lumaquin D, Gray D, Lill GR, Cooper AR, Urbinati F, Senadheera S, Zhu A, Liu PQ, Paschon DE, Zhang L, Rebar EJ, Wilber A, Wang, X, Gregory PD, Holmes MC, Reik A, Hollis RP, and **Kohn DB**. Correction of the sickle-cell disease mutation in human hematopoietic stem/progenitor cells. *Blood,* 125:2597-2604, 2015. **Plenary Paper** PMC4408287

211. Urbinati F, Hargrove PW, Geiger S, Romero Z, Wherley J, Kaufman ML, Hollis RP, Chambers CB, Persons DA, **Kohn DB**, and Wilber W. Potentially therapeutic levels of anti-sickling globin gene expression following lentivirus-mediated gene

transfer in sickle cell disease bone marrow CD34^{+} cells. *Expt. Hematol*, 2015 May;43(5):346-51. PMID:25681747 PMC4428920

212. Romero Z, Campo B, Wherley J, Kaufman ML, Cooper AR, Urbinati F, Hoban MD, Baldwin KM, Lumaquin D, Wilson A, Senadheera S, Hollis RP, **and Kohn DB**. The human ankyrin 1 promoter insulator sustains gene expression in a beta-globin lentiviral vector in hematopoietic stem cells." *Mol Ther Methods Clin Dev.* 2:15012, 2015. PMC4445009

213. Corrigan-Curay J, O'Reilly M, **Kohn DB**, Cannon PM, Bao G, Bushman FD, Carroll D, Cathomen T, Joung JK, Roth D, Sadelain M, Scharenberg AM, von Kalle C, Zhang F, Jambou R, Rosenthal E, Hassani M, Singh A, Porteus MH. Genome editing technologies: defining a path to clinic. *Mol Ther* 23(5):796-806, 2015. PMID: 25943494

214. Abdel-Azim H, Mahadeo KM, Zhao Q, Khazal S**, Kohn DB**, Crooks GM, Shah AJ, Kapoor N. Unrelated donor Hematopoietic Stem Cell Transplantation for the treatment of non-malignant genetic diseases: An alemtuzumab based regimen is associated with cure of clinical disease; earlier clearance of alemtuzumab may be associated with graft rejection. Am J Hematol. 90(11):1021-6, 2015. PMID:26242764

215. Komarow HD, Sokolic R, Hershfield MS, **Kohn DB**, Young M, Metcalfe DD, Candotti F. Impulse oscillometry identifies peripheral airway dysfunction in children with adenosine deaminase deficiency. Orphanet J Rare Dis.10:159, 2015. PMID: 26682746

216. Hoban MD, Romero Z, Cost G, Mendel M, Holmes M, and **Kohn DB**. Delivery of genome editing reagents to hematopoietic stem/progenitor cells. Current Protocols in Stem Cell Biology. 90(11):1021-6, 2016.

217. Griffith LM, Cowan MJ, Notarangelo LD, **Kohn DB**, Puck JM, Shearer WT, Burroughs LM, Torgerson TR, Decaluwe H, Haddad E, on behalf of the workshop participants. Primary Immune Deficiency Treatment Consortium (PIDTC) Update. J Allergy Clin Immunol, 138:375-85, 2016. PMID: 27262745

218. Chin CJ, Cooper AR, Lill GR, Evseenko D, Zhu Y, He CB, Casero D, Pellegrini M, **Kohn DB**, and Crooks GM. Genetic Tagging During Human Mesoderm Differentiation Reveals Tripotent Lateral Plate Mesodermal Progenitors. Stem Cells 34:1239-50, 2016.

219. Young CS, Hicks MR, Ermolova NV, Nakano H, Jan M, Younesi S, Karumbayaram S, Kumagai-Cresse C, Wang D, Zack JA, **Kohn DB**, Nakano A, Nelson SF, Miceli MC, Spencer MJ, Pyle AD. A Single CRISPR-Cas9 Deletion Strategy that Targets the Majority of DMD Patients Restores Dystrophin Function in hiPSC-Derived Muscle Cells. Cell Stem Cell. 18(4):533-40, 2016.

PUBLICATIONS (Cont'd)

220. Pinello L, CaNver MC, Hoban MD, Orkin SH, **Kohn DB**, Bauer DE and Yuan GC. Analyzing CRISPR genome-editing experiments with CRISPResso. Nat Biotech 34, 695–697, 2016.

221. Bjurström CF, Mojadidi M, Phillips J, Kuo C, Lai S, Lill GR, Cooper A, Kaufman M, Urbinati F, Wang X, Hollis RP, and **Kohn D**B. Reactivating fetal hemoglobin expression in human adult erythroblasts through BCL11A knockdown using targeted endonucleases. Mol Ther Nucleic Acids. 2016;5:e351.

222. **Kohn DB**, Porteus MH, Scharenberg AM. Ethical and regulatory aspects of genome editing. Blood 127:2553-60 2016. PMID: 27053531

223. Hoban MD, Lumaquin D, Kuo C, Romero Z, Long J, Ho M Young C, Mojadidi M, Fitz-Gibbons S, Cooper AR, Lill G, Urbinati F, Campo B, Bjurstrom C, Pellegrini M, Hollis RP, and **Kohn DB**. CRISPR/Cas9-mediated correction of the sickle mutation in human hematopoietic stem/progenitor cells. Mol Ther, 2016 Sep;24(9):1561-9. PMCID: PMC5113113

224. Punwani D, Kawahara M, Yu J, Sanford U, Roy A, Patel K, Khan S, Malech HL, Cornetta K, Carbonaro DA, **Kohn DB**, Rothe M, Schambach A, McIvor RS, Puck JM, and Cowan MJ. Preclinical demonstration of lentivirus vector mediated correction of Artemis-deficient Severe Combined Immunodeficiency. Hum Gene Ther, Hum Gene Ther. 2016 Sep 9. [Epub ahead of print]

225. Smith DJ, Lin LJ, Moon H, Pham AT, Wang X, Liu S, Ji S, Rezek V, Shimizu S, Ruiz M, Lam J, Steward R, Janzen DM, Memarzadeh S, **Kohn DB**, Zack JA, Kitchen SG, An DS, and Yang L. Propagating Humanized BLT Mice for the Study of Human Immunology and Immunotherapy. Stem Cells and Devel. 25:1863-1873, 2016.

226. DeWitt MA, Magis W, Carroll D, Bray NR, Wang JT, Berman JR, Urbinati F, Muñoz D, **Kohn DB**, Walters MC, Martin DK, and Corn JE. Efficient Correction of the Sickle Mutation in Human Hematopoietic Stem Cells Using a Cas9 Ribonucleo-protein Complex. Sci Transl Med, Sci Transl Med. 2016 Oct 12;8(360):360ra134.

227. Bethune MT, Gee MH, Bunse M, Lee MS, Gschweng EH, Pagadala MS, Zhou J, Cheng D, Heath JR, **Kohn DB**, Kuhns MS, Uckert W, and Baltimore D. Domain-swapped T cell receptors improve the safety of TCR gene therapy. Elife. 2016 Nov 8;5. pii: e19095.

228. **Kohn DB** and Kuo CY. New frontiers in the therapy of primary immunodeficiency: from gene addition to gene editing. J Allergy Clin Immunol. 139(3):726-732, 2017. PMID: 28270364

PUBLICATIONS (Cont'd)

229. **Kohn DB** and Gaspar HB. How we manage adenosine deaminase-deficient severe combined immune deficiency (ADA SCID). J Clin Immunol 37(4):351-356, 2017. PMID: 28194615

230. Shaw KL, Garabedian E, Mishra S, Barman P, Davila D, Carbonaro D, Shupien S, Silvin C, Geiger S, Nowicki B, Smogorzewska EM, Brown B, Wang X, de Oliveira, Choi C, Ikeda A, Terrazas D, Fu PY, Yu A, Campo B, Cooper A, Engel B, Podsakoff G, Balamurugan A, Anderson S, Muul L, Jagadeesh J, Kapoor N, Tse J, Moore TB, Purdy K, Rishi R, Mohan K, Skoda-Smith S, Buchbinder D, Abraham R, Scharenberg A, Yang OO, Cornetta K, Gjertson D, Hershfield M, Sokolic R, Candotti F and **Kohn DB**. Clinical efficacy of gene-modified stem cells in adenosine deaminase-deficient immunodeficiency. J Clin Invest. 127(5):1689-1699, 2017. PMID: 28346229

231. Cooper AR, Lill GR, Shaw K, Carbonaro D, Davila A, Sokolic R, Candotti F, Pellegrini M, and **Kohn DB**. Cytoreductive conditioning intensity predicts clonal diversity in ADA-SCID retroviral gene therapy patients. Blood 129(19):2624-2635, 2017. PMID: 28351939

232. Seet CS, He C, Bethune MT, Li S, Chick B, Gschweng EH, Zhu Y, Kim K, **Kohn DB**, Baltimore D, Crooks GM, Montel-Hagen A. Generation of mature T cells from human hematopoietic stem and progenitor cells in artificial thymic organoids. Nat Methods. 2017 14(5):521-530.

233. Masiuk KE, Brown D, Laborada J, Hollis RP, Urbinati F, and **Kohn DB**. Improving gene therapy efficiency through the enrichment of human hematopoietic stem cells. Mol Ther, 25:2163-2175, 2017. PMID: 28663101

234. Urbinati F, Wherley J, Geiger S, Campo-Fernandez B, Kaufman ML, Cooper A, Romero Z, Marchioni F, Reeves L, Read E, Nowicki B, Grassman E, Viswanathan S, Wang X, Hollis RP and **Kohn DB**. Pre-clinical studies for a phase I clinical trial of autologous hematopoietic stem cell gene therapy for sickle cell disease. Cytotherapy, 19:1096-1112, 2017. PMID: 28733131

235. Heimall J, Logan B, Cowan MJ, Notarangelo L, Griffith LM, Puck JM, **Kohn DB**, Pulsipher MA, Parikh S, Martinez C, Kapoor N, O'Reilly R, Boyer M, Pai SY, Goldman F, Burroughs L, Chandra S, Kletzel M, Thakar M, Connelly J, Cuvelier G, Davila B, Shereck E, Knutsen A, Sullivan KE, DeSantes K, Gillio A, Haddad E, Petrovic A, Quigg T, Smith AR, Stenger E, Yin Z, Shearer WT, Fleisher T, Buckley RH, and Dvorak CC. Immune Reconstitution and Survival of 100 SCID Patients Post Hematopoietic Cell Transplant: A PIDTC Prospective Study. Blood, 130:2718-2727, 2017. PMID: 29021228 PMCID: PMC5746165

PUBLICATIONS (Cont'd)

236. Hazim RA, Karumbayaram S, Dimashkiee A, Jianga M, Lopesa VS, Burgessa BL, Vijayarajd P, Alva-Ornelash JA, Zack JA, **Kohn DB**, Gomperts BN, Pyle AD, Lowry WE, and Williams DS. Differentiation of RPE cells from integration-free iPS cells and their cell biological characterization. Stem Cell Research & Therapy, 8:217, 2017. PMID: 28969679

237. Morgan RA, Gray, D, Lomova A, and **Kohn DB**. Hematopoietic stem cell gene therapy – progress made and lessons learned. (Perspective). Cell Stem Cell, 21(5):574-590, 2017. Review. PMID: 29100011

238. Long J, Hoban MD, Kuo C, Campo B, Cooper AR, Lumaquin D, Hollis RP, **Kohn DB**, and Romero Z. Characterization of Chromosomal Alterations Using a Zinc-Finger Nuclease Targeting the Beta-Globin Gene Locus in Hematopoietic Stem/Progenitor Cells. Mol Ther 26(2):468-479, 2018.

239. Almarza Novoa, E, Kasbekar, S, Thrasher AJ, **Kohn DB**, Sevilla J, Schwartz JD, and Bueren JA. Leukocyte Adhesion Deficiency-I: A comprehensive review of all published cases. JACI In Practice, 6:1418-1420, 2018. PMID: 29371071

240. Dunbar CE, High KA, Joung JK, **Kohn DB**, Ozawa K, and Sadelain M. Gene Therapy Comes Of Age. Science. 359:175, 2018. PMID: 29326244

241. Kuo CY, Long JD, Campo-Fernandez B, de Oliviera S, Cooper AR, Romero Z, Hoban MD, Joglekar AV, Lill G, Kaufman ML, Fitz-Gibbon S, Wang X, Hollis RP, and **Kohn DB**. Targeted Gene Insertion for the Treatment of X-Linked Hyper-IgM Syndrome. Cell Reports, 23(9):2606-2616, 2018.

242. Miggelbrink A, Logan BR, Buckley RH, Parrott RE, Dvorak CC, Kapoor N,Abdel-Azim H, Prockop SE, Shyr D, Decaluwe H, Hanson IC, Gillio A, Dávila Saldaña BJ, Eibel H, Hopkins G, Walter JE, Whangbo JS, **Kohn DB**, Puck JM, Cowan MJ,Griffith LM, Haddad E, O'Reilly RJ, Notarangelo LD, and Pai SY. B cell differentiation and IL-21 response in IL2RG/JAK3 SCID patients after hematopoietic stem cell transplantation. Blood, 2018 131:2967-2977. PMID: 29728406 PMCID: PMC6024640

243. Urbinati F, Poletti V, Campo-Fernandez B, Masuik K, Hollis RP, Kozol C, Kaufman ML, Brown D, Martin S, Miccio A, Mavilio F, **Kohn DB**. Gene Therapy for Sickle Cell Disease: a lentiviral vector comparison study. Hum Gene Ther. 2018 Oct;29(10):1153-1166. PMID: 30198339

244. **Kohn DB**, Hershfield MS, Puck JM, Aiuti A, Blincoe A, Gaspar HB, Notarangelo LD and Grunebaum E. Consensus approach for the management of severe combined immune deficiency caused by adenosine deaminase deficiency. J Allergy and Clin Immunol, S0091-6749(18)31268-5, 2018. PMID: 30194989

PUBLICATIONS (Cont'd)

245. Haddad E, Logan BR, Griffith LM, Buckley RH, Parrot RE, Prockop SE, Small TN, Chaisson J, Dvorak CC, Murnane M, Kapoor N, Abdel-Azim H, Hanson IC, Martinez C, Bleesing JJH, Chandra S, Smith AR, Cavanaugh ME, Jyonouchi S, Sullivan KE, Burroughs L, Skoda-Smith S, Haight AE, Tumlin AG, Quigg TC, Taylor C, Dávila Saldaña BJ, Keller MD, Seroogy CM, Desantes KB, Petrovic A, Leiding JW, Shyr DC, Decaluwe H, Teira P, Gillio AP, Knutsen A, Moore TB, Kletzel M, Craddock JA, Aquino V, Davis JH, Yu LC, Cuvelier GDE, Bednarski JJ, Goldman FD, Kang EM, Shereck E, Porteus MH, Connelly JA, Fleisher TA, Malech HL, Shearer WT, Szabolcs P, Thakar MS, Vander Lugt MT, Heimall J, Yin Z, Pulsipher MA, Pai SY, **Kohn DB**, Puck JM, Cowan MJ, O'Reilly RJ, Notarangelo LD.. SCID Genotype and 6-Month Post-Transplant CD4 Count Predict Survival and Immune Recovery. Blood 132:1737-1749, 2018. PMID: 30154114

246. Puig-Saus C, Parisi G, Garcia-Diaz A, Krystofinski PE, Sandoval S, Zhang R, Champhekar AS, McCabe J, Cheung-Lau GC, Truong NA, Vega-Crespo A, Komenan MDS, Pang J, Macabali MH, Saco JD, Goodwin JL, Bolon B, Seet CS, Montel-Hagen A, Crooks GM, Hollis RP, Campo-Fernandez B, Bischof D, Cornetta K, Gschweng EH, Adelson C, Nguyen A, Yang L, Witte ON, Baltimore D, Coming-Anduix B, **Kohn DB**, Wang X, Cabrera P, Kaplan-Lefko PJ, Berent-Maoz B, Ribas A. IND-enabling studies for a clinical trial to genetically program a persistent cancer-targeted immune system. Clin Cancer Res. 2018 Nov 8. [Epub ahead of print] PMID: 30409823

247. Hu P, Bi Y, Ma H, Suwanmanee T, Zeithaml B, Fry NJ, **Kohn DB**, Kafri T. Superior lentiviral vectors designed for BSL-0 environment abolish vector mobilization. Gene Ther. 2018 Sep 6. PMID: 30190607

248. Clarke EL, Connell AJ, Six E, Kadry NA, Abbas AA, Hwang Y, Everett JK, Hofstaedter CE, Marsh R, Armant M, Kelsen J, Notarangelo LD, Collman RG, Hacein-Bey-Abina S, **Kohn DB**, Cavazzana M, Fischer A, Williams DA, Pai SY, Bushman FD. T cell dynamics and response of the microbiota after gene therapy to treat X-linked severe combined immunodeficiency. Genome Med. 2018 Sep 28;10(1):70. PMID: 30261899.

249. Dvorak CC, Haddad E, Buckley RH, Cowan MJ, Logan B, Griffith LM, **Kohn DB**, Pai SY, Notarangelo L, Shearer W, Prockop S, Kapoor N, Heimall J, Chaudhury S, Shyr D, Chandra S, Cuvelier G, Moore T, Shenoy S, Goldman F, Smith AR, Sunkersett G, Vander Lugt M, Caywood E, Quigg T, Torgerson T, Chandrakasan S, Craddock J, Dávila Saldaña BJ, Gillio A, Shereck E, Aquino V, DeSantes K, Knutsen A, Thakar M, Yu L, Puck JM. The Genetic Landscape of SCID in the US and Canada in the Current Era (2010-2018). J Allergy Clin Immunol. S0091-6749(18)31271-5, 2018. PMID: 30193840

PUBLICATIONS (Cont'd)

250. Lomova A, Clark DN, Miyahira EY, Campo-Fernandez B, Flores-Bjurstrom C, Kaufman ML, Fitz-Gibbon S, Wang X, Brown D, DeWitt MA, Corn JE, Hollis RP, Romero Z, and **Kohn DB**. Improving Gene Editing Outcomes in Human Hematopoietic Stem and Progenitor Cells by Temporal Control of DNA Repair. Stem Cells. 37:284-294, 2018. PMID: 30372555

251. Poletti V, Urbinati F, Charrier S, Corre G, Hollis RP, Campo Fernandez B, Martin S, Rothe M, Schambach A, **Kohn DB** and Mavilio F. Pre-clinical development of a lentiviral vector expressing the anti-sickling βAS3 globin for gene therapy for sickle-cell disease. Mol Ther - Meth & Clin Devel, 11:167-179, 2018.

252. Masiuk KE, Laborada J, Roncarolo MG, Hollis RP and **Kohn DB**. Lentiviral Gene Therapy in HSCs Restores Lineage-Specific Foxp3 Expression and Suppresses Autoimmunity in a Mouse Model of IPEX Syndrome. Cell Stem Cells, 24(2):309-317, 2019. PMID: 30639036

253. Amatuni GS, Currier RJ, Church JA, Bishop T, Grimbacher E, Nguyen AA, Agarwal-Hashmi R, Aznar CP, Butte MJ, Cowan MJ, Dorsey MJ, Dvorak CC, Kapoor N, **Kohn DB**, Markert ML, Moore TB, Naides SJ, Sciortino S, Feuchtbaum L, Koupaei RA, Puck JM. Newborn Screening for Severe Combined Immunodeficiency and T-cell Lymphopenia in California, 2010-2017. Pediatrics. 2019 Jan 25. pii: e20182300.. [Epub ahead of print] PMID: 30683812

254. **Kohn DB**. Gene therapy for blood diseases. Curr Opin Biotech 60:39-45, 2019. Review. PMID: 30599357

255. Soni S and **Kohn DB**. Chemistry, Manufacturing and Controls for Gene Modified Stem Cells. Cytotherapy, 21:358-366, 2019. PMID: 30745225

256. Masiuk KE, Osborne K, Zhang R, Hollis RP, Campo-Fernandez B, and **Kohn DB**. PGE2 and Poloxamer-F108 Enhance Transduction of Human HSPC with a β-Globin Lentiviral Vector. Mol Ther – Meth & Devel, 13:390-398, 2019. PMID: 31024981

257. Kwon HS, Logan AC, Chhabra A, Pang WW, Czechowicz A, Tate K, Le A, Poyser J, Hollis R, Kelly BV, **Kohn DB**, Weissman IL, Prohaska SS, and Shizuru JA. Anti-human CD117 antibody-mediated bone marrow niche clearance in non-human primates and humanized NSG mice. Blood, 2019 133:2104-2108, 2019 PMID: 30617195

258. Romero Z, Lomova A, Said S, Miggelbrink A, Kuo CY, Campo-Fernandez B, Hoban MD, Masiuk KE, Clark DN, Long J, Sanchez JM, Velez M, Miyahira E, Zhang R, Brown D, Wang X, Kurmangaliyev YZ, Hollis RP, **Kohn DB**. Editing the sickle cell disease mutation in primary human hematopoietic stem cells: comparison of endonucleases and homologous donor templates. Mol Ther. 27:1389-1406, 2019. PMID: 31178391

PUBLICATIONS (Cont'd)

259. Marsh RA, Leiding JW, Logan BR, Griffith LM, Arnold DE, Haddad E, Falcone EL, Yin Z, Patel K, Arbuckle E, Bleesing JJ, Sullivan KE, Heimall J, Burroughs LM, Skoda-Smith S, Chandrakasan S, Yu LC, Oshrine BR, Cuvelier GDE, Thakar MS, Chen K, Teira P, Shenoy S, Phelan R, Forbes LR, Chellapandian D, Dávila Saldaña BJ, Shah AJ, Weinacht KG, Joshi A, Boulad F, Quigg TC, Dvorak CC, Grossman D, Torgerson T, Graham P, Prasad V, Knutsen A, Chong H, Miller H, de la Morena MT, DeSantes K, Cowan MJ, Notarangelo LD, **Kohn DB**, Stenger E, Pai SY, Routes JM, Puck JM, Kapoor N, Pulsipher MA, Malech HL, Parikh S, Kang EM; submitted on behalf of the Primary Immune Deficiency Treatment Consortium. Chronic Granulomatous Disease-Associated IBD Resolves and Does Not Adversely Impact Survival Following Allogeneic HCT. J Clin Immunol, 39:653-667, 2019. PMID: 31376032.

260. Zhu Y, Smith DJ, Zhou Y, Li YR, Yu J, Lee D, Wang YC, Di Biase S, Wang X, Hardoy C, Ku J, Tsao T, Lin LJ, Pham AT, Moon H, McLaughlin J, Cheng D, Hollis RP, Campo-Fernandez B, Urbinati F, Wei L, Pang L, Rezek V, Berent-Maoz B, Macabali MH, Gjertson D, Wang X, Galic Z, Kitchen SG, An DS, Hu-Lieskovan S, Kaplan-Lefko PJ, De Oliveira SN, Seet CS, Larson SM, Forman SJ, Heath JR, Zack JA, Crooks GM, Radu CG, Ribas A, **Kohn DB**, Witte ON, Yang L. Development of Hematopoietic Stem Cell-Engineered Invariant Natural Killer T Cell Therapy for Cancer. Cell Stem Cells, 25:542-557, 2019. PMID: 31495780

261. Morgan RA, Unti MJ, Aleshe B, Brown D, Osborne KS, Koziol C, Ayoub PG, Smith OB, O'Brien R, Tam C, Miyahira E, Ruiz M, Quintos JP, Senadheera S, Hollis RP, **Kohn DB**. Improved Titer and Gene Transfer by Lentiviral Vectors Using Novel, Small β-Globin Locus Control Region Elements. Mol Ther. pii: S1525-0016(19)30449-6, 2019. PMID: 31628051

262. Farrell AT, Panepinto J, Desai AA, Kassim AA, Lebensburger J, Walters MC, Bauer DE, Blaylark RM, DiMichele DM, Gladwin MT, Green NS, Hassell K, Kato GJ, Klings ES, **Kohn DB**, Krishnamurti L, Little J, Makani J, Malik P, McGann PT, Minniti C, Morris CR, Odame I, Oneal PA, Setse R, Sharma P, Shenoy S. End points for sickle cell disease clinical trials: renal and cardiopulmonary, cure, and low-resource settings. Blood Adv. 3:4002-4020, 2019. PMID: 31809537

263. Carbonaro-Sarracino DA, Tarantal AF, Chang I. Lee CCI, Kaufman ML, Wandro S, Jin X, Martinez M, Clark DN, Chun K, Koziol C, Hardee CL, Wang X and **Kohn DB**. Dosing and Re-Administration of Lentiviral Vector for *In Vivo* Gene Therapy in Rhesus Monkeys and ADA-Deficient Mice. Mol Ther Methods Clin Dev. 16:78-93, 2019.

264. **Kohn DB,** Booth C, Kang EM, Pai SY, Shaw KL, Santilli G, Armant M, Buckland KF, Choi U, De Ravin SS, Dorsey MJ, Kuo CY, Leon-Rico D, Rivat C, Izotova N, Gilmour K, Snell K, Dip JX, Darwish J, Morris EC, Terrazas D, Wang LD, Bauser CA, Paprotka T, Kuhns DB, Gregg J, Raymond HE, Everett JK, Honnet G, Biasco L, Newburger PE, Bushman FD, Grez M, Gaspar HB, Williams DA,

Malech HL, Galy A, Thrasher AJ; Net4CGD consortium. Lentiviral gene therapy for X-linked chronic granulomatous disease. Nat Med. 26:200-206, 2020. PMID: 31988463

265. Chan AY, Leiding JW, Liu X, Logan BR, Burroughs LM, Allenspach EJ, Skoda-Smith S, Uzel G, Notarangelo LD, Slatter M, Gennery AR, Smith AR, Pai SY, Jordan MB, Marsh RA, Cowan MJ, Dvorak CC, Craddock JA, Prockop SE, Chandrakasan S, Kapoor N, Buckley RH, Parikh S, Chellapandian D, Oshrine BR, Bednarski JJ, Cooper MA, Shenoy S, Davila Saldana BJ, Forbes LR, Martinez C, Haddad E, Shyr DC, Chen K, Sullivan KE, Heimall J, Wright N, Bhatia M, Cuvelier GDE, Goldman FD, Meyts I, Miller HK, Seidel MG, Vander Lugt MT, Bacchetta R, Weinacht KG, Andolina JR, Caywood E, Chong H, de la Morena MT, Aquino VM, Shereck E, Walter JE, Dorsey MJ, Seroogy CM, Griffith LM, **Kohn DB**, Puck JM, Pulsipher MA, Torgerson TR. Hematopoietic Cell Transplantation in Patients With Primary Immune Regulatory Disorders (PIRD): A Primary Immune Deficiency Treatment Consortium (PIDTC) Survey. Front Immunol. 2020 Feb 21;11:239. PMID: 32153572 PMCID: PMC7046837

266. Burroughs L, Petrovic A, Brazauskas R, Liu X, Griffith LM, Ochs HD, Bleesing J, Edwards S, Dvorak CC, Chaudhury S, Prockop S, Quinones R, Goldman F, Quigg T, Chandrakasan S, Smith AR, Parikh SH, Dávila Saldaña BJ, Thakar MS, Phelan R, Shenoy S, Forbes LR, Martinez CA, Chellapandian D, Shereck E, Miller H, Kapoor N, Barnum JL, Chong H, Shyr D, Chen K, Abu-Arja RF, Shah A, Weinacht K, Moore TB, Joshi A, DeSantes K, Gillio AP, Cuvelier GDE, Keller MD, Rozmus J, Torgerson TR, Pulsipher MA, Haddad E, Sullivan K, Logan BR, **Kohn DB**, Puck JM, Notarangelo LD, Pai SY, Rawlings D, Cowan MJ. Excellent Outcomes Following Hematopoietic Cell Transplantation for Wiskott-Aldrich Syndrome: A PIDTC Report. Blood 135:2094-2105, 2020. PMID: 32268350

267. Richard A. Morgan, Feiyang MA, Mildred Unti, Devin Brown, Curtis Tam, Lindsay Lathrop, Ryo Kurita, Yukio Nakamura, Shantha Senedhara, Roger P. Hollis, Matteo Pellegrini, **Donald B. Kohn**. Creating β-globin-Expressing Lentiviral Vectors by High Resolution Mapping of Locus Control Region Enhancer Sequences. Mol Ther Meth and Clin Devel, 17:999-1013, 2020.

268. Luigi Notarangelo, Marita Bosticardo, Francesca Pala, Enrica Calzoni, Ottavia Delmonte, Kerry Dobbs, Cameron Gardner, Nicolo' Sacchetti, Tomoki Kawai, Elizabeth Garabedian, Debbie Draper, Jenna Bergerson, Suk De Ravin, Alexandra Freeman, Tayfun Güngör, Nicholas Hartog, Steven Holland**, Donald Kohn**, Harry Malech, Mary Markert, Katja Weinacht, Anna Villa, Christopher Seet, Amelie Montel-Hagen, and Gay Crooks. Artificial thymic organoids are a reliable tool to study T cell differentiation in patients with T cell lymphopenia. Blood Adv 4:2611-2616, 2020. PMID: 32556283

PUBLICATIONS (Cont'd)

269. **Kohn DB** and Kuo CY. Overview of the Current Status of Gene Therapy for Primary Immune Deficiencies (PID). JACI, 146:229-233, 2020.

270. Bradford KL, Krajinovic M, Ansari M, Garabedian E, Tse J, Wang X, Shaw KL, Gaspar H, Candotti F, **Kohn DB**. Busulfan Pharmacokinetics in ADA-SCID Gene Therapy. Biol Blood and Marrow Transplant, 26:1819-1827, 2020. PMCID: PMC7529956

271. Peng Yang, Shih-Jie Chou, Qian Ban, Jindian Li, Na Sun, Wenqiao Hui, Yazhen Zhu, Fang Wang, Ryan Yue Zhang, Zulema Romero, Jiayuan Chen, Zunfu Ke, Alex Zou, Steve J. Jonas, Paul S. Weiss, Chin-Fa Lee, * **Donald B. Kohn**, * Kai Chen, Shi-Hua Chiou and Hsian-Rong Tseng. Supramolecular Nanosubstrate-Mediated Delivery System Enables CRISPR/Cas9 Knockin of Hemoglobin Beta Gene – A Potential Therapeutic Solution for Hemoglobinopathies, Science Advances, 6:eabb7107, 2020

272. Kathryn L. Bradford, Meghan Pearl, **Donald B. Kohn,** Patricia Weng, Ora Yadin, La Vette Bowles, Satiro N. De Oliveira, and Theodore B. Moore. AT1R Activating Autoantibodies in Hematopoietic Stem Cell Transplantation. Biol Blood and Marrow Transplant, 26:2061-2067, 2020.

273. Masterman KA, Haigh OL, Tullett KM, Leal-Rojas IM, Walpole C, Pearson FE, Cebon J, Schmidt C, O'Brien L, Rosendahl N, Daraj G, Caminschi I, Gschweng EH, Hollis RP, Kohn DB, Lahoud MH, Radford KJ. Human CLEC9A antibodies deliver NY-ESO-1 antigen to CD141+ dendritic cells to activate naïve and memory NY-ESO-1-specific CD8+ T cells. J Immunother Cancer. 2020 Jul;8(2):e000691. PMID: 32737142

274. Daniel S. Mytelka, William M. Cassidy, **Donald B. Kohn**, Mark R. Trusheim. Managing uncertainty in drug value: Outcomes-based contracting supports value-based pricing. Health Affairs, Jan 20, 2020.

275. Caroline Y. Kuo, Elizabeth Garabedian, Jennifer Puck, Morton J. Cowan, Kathleen E. Sullivan, Rebecca H. Buckley, Charlotte Cunningham-Rundles, Mary Ellen Conley, Rebecca Marsh, Fabio Candotti, and **Donald B. Kohn**. Adenosine Deaminase (ADA)-Deficient Severe Combined Immune Deficiency (SCID) in the U.S. Immunodeficiency Network (USIDNet) Registry. J Clin Immunol, 40:1124 -1131, 2020.

276. Han J, Tam K, Ma F, Tam C, Aleshe B, Wang X, Quintos J, Morselli M, Pellegrini M, Hollis RP, and **Kohn DB.** β-Globin Lentiviral Vectors Have Reduced Titers Due to Incomplete Vector RNA Genomes and Lowered Virion Production. Stem Cell Reports, 16:198-211, 2021.

PUBLICATIONS (Cont'd)

277. Dorsey MJ, Wright NAM, Chaimowitz NS, Dávila Saldaña BJ, Miller H, Keller MD, Thakar MS, Shah AJ, Abu-Arja R, Andolina J, Aquino V, Barnum JL, Bednarski JJ, Bhatia M, Bonilla FA, Butte MJ, Bunin NJ, Chandra S, Chaudhury S, Chen K, Chong H, Cuvelier GDE, Dalal J, DeFelice ML, DeSantes KB, Forbes LR, Gillio A, Goldman F, Joshi AY, Kapoor N, Knutsen AP, Kobrynski L, Lieberman JA, Leiding JW, Oshrine B, Patel KP, Prockop S, Quigg TC, Quinones R, Schultz KR, Seroogy C, Shyr D, Siegel S, Smith AR, Torgerson TR, Vander Lugt MT, Yu LC, Cowan MJ, Buckley RH, Dvorak CC, Griffith LM, Haddad E, **Kohn DB**, Logan B, Notarangelo LD, Pai SY, Puck J, Pulsipher MA, Heimall J. Infections in Infants with SCID: Isolation, Infection Screening, and Prophylaxis in PIDTC Centers. *J Clin Immunol* **41,** 38–50 (2021). PMID: 33006109

278. Elizabeth K. Benitez, Anastasia Lomova Kaufman, Lilibeth Cervantes, Danielle N. Clark, Paul Ayoub, Shantha Senadheera, Kyle Osborne, Julie M. Sanchez, Ralph V. Crisostomo, Xiaoyan Wang, Nina Reuven, Yosef Shaul, Roger P. Hollis, Zulema Romero, and **Donald B. Kohn**. Global and Local Manipulation of DNA Repair Mechanisms to Alter Site-Specific Gene Editing Outcomes in Hematopoietic Stem Cells. Front Genome Ed 2020 2:601541. eCollection 2020. PMID: 34713224 PMCID: PMC8525354

279. Cannon P, Asokan A, Czechowicz A, Hammond P, **Kohn DB**, Lieber A, Malik P, Marks P, Porteus M, Verhoeyen E, Weissman D, Weissman I, Kiem HP. Safe and effective in vivo targeting and gene editing in hematopoietic stem cells: strategies for accelerating development National Institutes of Health/Bill & Melinda Gates Foundation expert scientific roundtable webinar meeting. Hum Gene Ther. 2021 32:1-2, 31-42 PMID: 33427035

280. Gray DH, Villegas I, Long J, Santos J, Keir A, Abele A, Kuo CY, and **Kohn DB**. Optimizing expression from a BTK transgene following CRISPR/Cas9 mediated gene editing for X-linked agammaglobulinemia (XLA). CRISPR Journal, 4:191-206, 2021

281. Kohn LA and **Kohn DB**. Gene therapies for primary immune deficiencies. Front Immunol. 12:648951, 2021.

282. Carbonaro-Sarracino DA, Chun K, Clark DN, Kaufman ML, Jin X, Wang X, **Kohn DB.** Gene delivery using AAV8 *in vivo* for disease stabilization in a bimodal gene therapy approach for the treatment of ADA-deficient SCID. Mol Ther Methods Clin Dev. 20:765-778, 2021. PMID: 3738330; PMCID: PMC7940710.

283. Garcia G Jr, Sharma A, Ramaiah A, Sen C, Purkayastha A, **Kohn DB**, Parcells MS, Beck S, Kim H, Bakowski MA, Kirkpatrick MG, Riva L, Wolff KC, Han B, Yuen C, Ulmert D, Purbey PK, Scumpia P, Beutler N, Rogers TF, Chatterjee AK, Gabriel G, Bartenschlager R, Gomperts B, Svendsen CN, Betz UAK,

Damoiseaux RD, Arumugaswami V. Antiviral drug screen identifies DNA-damage response inhibitor as potent blocker of SARS-CoV-2 replication. Cell Rep. 35:1089402021 PMID:33784499

284. Baloh CH, Borkar SA, Chang KF, Yao J, Hershfield MS, Parikh SH, **Kohn DB**, Goodenow MM, Sleasman JW, Yin L. Normal IgH Repertoire Diversity in an Infant with ADA Deficiency After Gene Therapy. J Clin Immunol. 2021 41:1597-1606. PMID: 34184208.

285. **Kohn DB**, Booth C, Shaw KL, Xu-Bayford J, Garabedian E, Trevisan V, Carbonaro-Sarracino DA, Soni K, Terrazas D, Snell K, Ikeda A, Leon-Rico D, Moore TB, Buckland KF, Shah AJ, Gilmour KC, De Oliveira S, Rivat C, Crooks GM, Izotova N, Tse J, Adams S, Shupien S, Ricketts H, Davila A, Uzowuru C, Icreverzi A, Barman P, Campo Fernandez B, Hollis RP, Coronel M, Yu A, Chun KM, Casas CE, Zhang R, Arduini S, Lynn F, Kudari M, Spezzi A, Zahn M, Heimke R, Labik I, Parrott R, Buckley RH, Reeves L, Cornetta K, Sokolic R, Hershfield M, Schmidt M, Candotti F, Malech HL, Thrasher AJ, Gaspar HB. Autologous Ex Vivo Lentiviral Gene Therapy for Adenosine Deaminase Deficiency. N Engl J Med. 384:2002-2012, 2021 PMID: 33974366

263. Reinhardt BC, Habib O, Shaw KL, Garabedian EK, Carbonaro-Sarracino DA, Terrazas DR, Campo-Fernandez B, De Oliveira S, Moore TB, Ikeda A, Engel BC, Podsakoff GM, Hollis RP, Fernandes A, Jackson CR, Shupien SA, Mishra S, Davila A, Mottahedeh J, Vitomirov A, Meng W, Rosenfeld AM, Roche AM, Hokama P, Reddy S, Everett JK, Wang X, Luning Prak ET, Cornetta K, Hershfield M, Sokolic R, De Ravin SS, Malech HL, Bushman FD, Candotti F, **Kohn DB**. Long-term Outcomes after Gene Therapy for Adenosine Deaminase Severe Combined Immune Deficiency (ADA SCID). Blood 138(15):1304-1316, 2021. PMID: 33974038; PMCID: PMC8525336.

264. Abou-El-Enein M, Angelis A, Appelbaum FR, Andrews NC, Bates SE, Bierman AS, Brenner MK, Cavazzana M, Caligiuri MA, Clevers H, Cooke E, Daley GQ, Dzau VJ, Ellis LM, Fineberg HV, Goldstein LSB, Gottschalk S, Hamburg MA, Ingber DE, **Kohn DB**, Krainer AR, Maus MV, Marks P, Mummery CL, Pettigrew RI, Rutter JL, Teichmann SA, Terzic A, Urnov FD, Williams DA, Wolchok JD, Lawler M, Turtle CJ, Bauer G, Ioannidis JPA. Evidence generation and reproducibility in cell and gene therapy research: A call to action. Mol Ther Methods Clin Dev. 22:11-14, 2021. PMID: 34377737; PMCID: PMC8322039.

265 Li YR, Zhou Y, Kim YJ, Zhu Y, Ma F, Yu J, Wang YC, Chen X, Li Z, Zeng S, Wang X, Lee D, Ku J, Tsao T, Hardoy C, Huang J, Cheng D, Montel-Hagen A, Seet CS, Crooks GM, Larson SM, Sasine JP, Wang X, Pellegrini M, Ribas A, **Kohn DB,** Witte O, Wang P, Yang L. Development of allogeneic HSC-engineered iNKT cells for off-the-shelf cancer immunotherapy. Cell Rep Med. 2:100449, 2021. PMID: 34841295; PMCID: PMC8607011.

PUBLICATIONS (Cont'd)

266. Paul Ayoub, Arunima Purkayastha, Jason Quintos, Curtis Tam, Lindsay Lathrop, Kevin Tam, Marlene Ruiz, Roger P Hollis, Brigitte N Gomperts, **Donald B. Kohn.** Improved SARS-CoV-2 Spike Glycoproteins for Pseudotyping Lentiviral Vectors. Frontiers in Virology, section Translational Virology, In Press.

267. Jiaying Han, Kevin Tam, Curtis Tam, Roger P. Hollis, and **Donald B. Kohn**. Improved Lentiviral Vector Titers from a Multi-Gene Knock-out Packaging Line. Mol Ther Oncolytics, 23:582-592, 2021. PMID: 34938858.

268. Ibarrondo FJ, Hofmann C, Ali A, Ayoub P, **Kohn DB**, Yang OO. Previous Infection Combined with Vaccination Produces Neutralizing Antibodies with Potency against SARS-CoV-2 Variants. mBio. 2021 Dec 21;12(6):e0265621. PMID: 34872349, PMCID: PMC8649753.

269. Magis W, DeWitt MA, Wyman SK, Vu JT, Heo SJ, Shao SJ, Hennig F, Romero ZG, Campo-Fernandez B, Said S, McNeill MS, Rettig GR, Sun Y, Wang Y, Behlke MA, **Kohn DB**, Boffelli D, Walters MC, Corn JE, Martin DIK.. Genome editing of the sickle mutation is amplified *in vivo. iScience,* 25(6):104374, 2022 PMID: 35633935

270. Arnold DE, Chellapandian D, Parikh S, Mallhi K, Marsh RA, Heimall JR, Grossman D, Chitty-Lopez M, Murguia-Favela L, Gennery AR, Boulad F, Arbuckle E, Cowan MJ, Dvorak CC, Griffith LM, Haddad E, **Kohn DB**, Notarangelo LD, Pai SY, Puck JM, Pulsipher MA, Torgerson T, Kang EM, Malech HL, Leiding JW. Granulocyte Transfusions in Patients with Chronic Granulomatous Disease Undergoing Hematopoietic Cell Transplantation or Gene Therapy. J Clin Immunol. 2022 Apr 21:1–10. PMID: 35445907; PMCID: PMC9022412.

271. Geoffrey D.E. Cuvelier[1], Brent R. Logan[2], Susan E. Prockop[3], Rebecca H. Buckley[4], Caroline Y. Kuo[5], Linda M. Griffith[6], Xuerong Liu[2], Alison Yip[7], Michael S. Hershfield[4], Paul G. Ayoub[35], Roberta E. Parrott[4], Christen L. Ebens[8], Theodore B. Moore[9], Richard J. O'Reilly[3], Sung-Yun Pai[10], Malika Kapadia[11], Neena Kapoor[12], Lisa R. Forbes Satter[13], Caridad Martinez[14], Lauri M. Burroughs[15], Aleksandra Petrovic[15], Monica S. Thakar[15], Deepak Chellapandian[16], Jennifer Heimall[17], David C. Shyr[18], Ahmad Rayes[19], Jeffrey J. Bednarski[20], Sharat Chandra[21], Shanmuganathan Chandrakasan[22], Alfred P. Gillio[23], Lisa Madden[24], Troy C. Quigg[25], Emi H. Caywood[26], Blachy J Dávila Saldaña[27], Kenneth DeSantes[28], Hesham Eissa[29], Frederick D. Goldman[30], Jacob Rozmus[31], Ami J. Shah[18], Mark T. Vander Lugt[32], Michael A. Pulsipher[12], Luigi D. Notarangelo[33], Morton J. Cowan[7], Christopher C. Dvorak[7], Elie Haddad[34], Jennifer M. Puck[7], **Donald B. Kohn[35]**. Outcomes Following Treatment for Adenosine Deaminase Severe Combined Immune Deficiency: A Report from the PIDTC. *Blood*, 18;140(7):685-705, 2022.

PUBLICATIONS (Cont'd)

272. Shanna White, Kevyn Hart and **Donald B. Kohn**. Diverse Approaches to Gene Therapy of Sickle Cell Disease. Annual Rev Med, In Press.

273. White SL, Lee TD, Toy T, Carroll JE, Polsky L, Campo Fernandez B, Davila A, **Kohn DB**, Chang VY. Evaluation of Clonal Hematopoiesis in Pediatric ADA-SCID Gene Therapy Participants. Blood Adv. 2022 Aug Epub ahead of print. PMID: 35914227.

274. Collon K, Gallo M, Bell J, Chang S, Rodman J, Sugiyama O, **Kohn DB**, Lieberman JR. Improving Lentiviral Transduction of Human Adipose-Derived Mesenchymal Stem Cells. Hum Gene Ther. 2022 Jul 20. doi: 10.1089/hum.2022.117. Epub ahead of print. PMID: 35859364.

275. Campagna GA, Chew L, Pettenkofer M, Nicoletti E, Schwartz JD, Choi G, Fernandes AO, Terrazas D, **Kohn DB**, Tsui I. Findings on Optical Coherence Tomography in Malignant Infantile Osteopetrosis. Ophthalmic Surg Lasers Imaging Retina. 2022 Jul;53(7):398-402. PMID: 35858232.

276. Labrosse R, Boufaied I, Bourdin B, Gona S, Randolph HE, Logan BR, Bourbonnais S, Berthe C, Chan W, Buckley RH, Parrott RE, Cuvelier GDE, Kapoor N, Chandra S, Dávila Saldaña BJ, Eissa H, Goldman FD, Heimall J, O'Reilly R, Chaudhury S, Kolb EA, Shenoy S S, Griffith LM, Pulsipher M, **Kohn DB**, Notarangelo LD, Pai SY, Cowan MJ, Dvorak CC, Haddad É, Puck JM, Barreiro LB, Decaluwe H. Aberrant T-cell exhaustion in SCID survivors with poor T-cell reconstitution post transplant. J Allergy Clin Immunol. 2022 Aug 17:S0091-6749(22)01057-0. PMID: 35987350

277. Sosa RA, Mone T, Naini BV, **Kohn DB**, Reed EF, Wheeler K, Campo-Fernandez B, Davila A, Chaffin DJ, DiNorcia J, Kaldas FM, Cohen A, Lum EL, Veale JL, Kogut NM. Apheresis of Deceased Donors as a New Source of Mobilized Peripheral Blood Hematopoietic Stem Cells for Transplant Tolerance. Transplantation. 2022 Aug 17. PMID: 35974436

278. Wong RL, Sackey S, Brown D, Senadheera S, Masiuk K, Quintos JP, Colindres N, Riggan L, Morgan RA, Malech HL, Hollis RP, **Kohn DB**. Lentiviral Gene Therapy for X-Linked Chronic Granulomatous Recapitulates Endogenous CYBB Regulation and Expression. Blood. 2022 Nov 4:blood. Epub ahead of print. PMID: 36332160.

279. Dvorak CC, Haddad E, Heimall J, Dunn E, Buckley RH, **Kohn DB**, Cowan MJ, Pai SY, Griffith LM, Cuvelier GDE, Eissa H, Shah AJ, O'Reilly RJ, Pulsipher MA, Wright NAM, Abraham RS, Satter LF, Notarangelo LD, Puck JM. The diagnosis

of severe combined immunodeficiency (SCID): The Primary Immune Deficiency Treatment Consortium (PIDTC) 2022 Definitions. J Allergy Clin Immunol. 2022 Nov 28:S0091-6749(22)01479-8. Epub ahead of print. PMID: 36456361.

280. Dvorak CC, Haddad E, Heimall J, Dunn E, Cowan MJ, Pai SY, Kapoor N, Satter LF, Buckley RH, O'Reilly RJ, Chandra S, Bednarski JJ, Williams O, Rayes A, Moore TB, Ebens CL, Davila Saldana BJ, Petrovic A, Chellapandian D, Cuvelier GDE, Vander Lugt MT, Caywood EH, Chandrakasan S, Eissa H, Goldman FD, Shereck E, Aquino VM, Desantes KB, Madden LM, Miller HK, Yu L, Broglie L, Gillio A, Shah AJ, Knutsen AP, Andolina JP, Joshi AY, Szabolcs P, Kapadia M, Martinez CA, Parrot RE, Sullivan KE, Prockop SE, Abraham RS, Thakar MS, Leiding JW, **Kohn DB**, Pulsipher MA, Griffith LM, Notarangelo LD, Puck JM. The diagnosis of severe combined immunodeficiency: Implementation of the PIDTC 2022 Definitions. J Allergy Clin Immunol. 2022 Nov 28:S0091-6749(22)01478-6.. Epub ahead of print. PMID: 36456360.

281 Cornetta K, Yao J, House K, Duffy L, Adusumilli PS, Beyer R, Booth C, Brenner M, Curran K, Grilley B, Heslop H, Hinrichs CS, Kaplan RN, Kiem HP, Kochenderfer J, **Kohn DB**, Mailankody S, Norberg SM, O'Cearbhaill RE, Pappas J, Park J, Ramos C, Ribas A, Rivièra I, Rosenberg SA, Sauter C, Shah N, Slovin SF, Thrasher A, Williams DA, Lin TY. Replication Competent Retrovirus Testing (RCR) in the National Gene Vector Biorepository: no evidence of RCR in 1595 post-treatment peripheral blood samples obtained from 60 clinical trials. Mol Ther. 2022 Dec 13:S1525-0016(22)00710-9. PMID: 36518078.

282. Cornetta K, Lin TY, Pellin D, **Kohn DB**. Meeting FDA Guidance recommendations for replication-competent virus and insertional oncogenesis testing. Mol Ther Methods Clin Dev. 2022 Dec 2;28:28-39. PMID: 36588821 Free PMC article.

283. Eapen M, Brazauskas R, Williams DA, Walters MC, St Martin A, Jacobs BL, Antin JH, Bona K, Chaudhury S, Coleman-Cowger VH, DiFronzo NL, Esrick EB, Field JJ, Fitzhugh CD, Kanter J, Kapoor N, **Kohn DB**, Krishnamurti L, London WB, Pulsipher MA, Talib S, Thompson AA, Waller EK, Wun T, Horowitz MM. Secondary Neoplasms After Hematopoietic Cell Transplant for Sickle Cell Disease. J Clin Oncol. 2023 Jan 9:JCO2201203. PMID: 36623245.

284. Grunebaum E, Booth C, Cuvelier GDE, Loves R, Aiuti A, **Kohn DB**. Updated management guidelines for adenosine deaminase deficiency. J Allergy Clin Immunol Pract. 2023 Feb 1:S2213-2198(23)00118-6. Epub ahead of print. PMID: 36736952.

285. McDonald CL, Qasba P, Anderson DG, Bao G, Colvin RA, **Kohn DB**, Malik P, Mitchell MJ, Pu WT, Rawlings DJ, Williams DA, Flotte TR. Future Directions and Resource Needs for National Heart, Lung, and Blood Institute (NHLBI) Gene

Therapy Research: A Report of an NHLBI Workshop. Hum Gene Ther. 2023 Feb;34(3-4):83-89. PMID: 36794978.

286. Grace E. McAuley, Gloria Yiu, Patrick C Chang, Gregory A. Newby, Beatriz Campo-Fernandez, Sorel Fitz-Gibbon, Xiaomeng Wu, Sung-Hae L. Kang, Amber Garibay, Jeffrey Butler, Valentina Christian, Ryan L. Wong, Kelcee A. Everette, Christopher S. Seet, Aru Narendran, Luis Murguia Favela, Zulema Romero, Nicola Wright, David R. Liu, Gay M. Crooks, **Donald B. Kohn**. Human T Cell Generation is Restored in CD3δ Severe Combined Immunodeficiency Through Adenine Base Editing. *CELL*, 186:, 2023.

287. Segura EER, Ayoub PG, Hart KL, **Kohn DB**. Gene Therapy for β-Hemoglobinopathies: From Discovery to Clinical Trials. Viruses. 2023 Mar 9;15(3):713. PMID: 36992422

288. Thakar MS, Logan BR, Puck JM, Dunn EA, Buckley RH, Cowan MJ, O'Reilly RJ, Kapoor N, Satter LF, Pai SY, Heimall J, Chandra S, Ebens CL, Chellapandian D, Williams O, Burroughs LM, Saldana BD, Rayes A, Madden LM, Chandrakasan S, Bednarski JJ 2nd, DeSantes KB, Cuvelier GDE, Teira P, Gillio AP, Eissa H, Knutsen AP, Goldman FD, Aquino VM, Shereck EB, Moore TB, Caywood EH, Lugt MTV, Rozmus J, Broglie L, Yu LC, Shah AJ, Andolina JR, Liu X, Parrott RE, Dara J, Prockop S, Martinez CA, Kapadia M, Jyonouchi SC, Sullivan KE, Bleesing JJ, Chaudhury S, Petrovic A, Keller MD, Quigg TC, Parikh S, Shenoy S, Seroogy C, Rubin T, Decaluwe H, Routes JM, Torgerson TR, Leiding JW, Pulsipher MA, **Kohn DB**, Griffith LM, Haddad E, Dvorak CC, Notarangelo LD. Measuring the effect of newborn screening on survival after haematopoietic cell transplantation for severe combined immunodeficiency: a 36-year longitudinal study from the Primary Immune Deficiency Treatment Consortium. Lancet. 2023 Jun 20:S0140-6736(23)00731-6. Online ahead of print. PMID: 37352885

289. Leiding JW, Arnold DE, Parikh SH, Logan BR, Marsh RA, Griffith LM, Wu R, Kidd S, Mallhi KK, Chellapandian D, Si Lim S, Grunebaum E, Falcone EL, Murguia-Favela L, Grossman D, Prasad VK, Heimall JR, Touzot F, Burroughs L, Bleesing J, Kapoor N, Dara J, Williams O, Kapadia M, Oshrine BR, Bednarski JJ, Rayes A, Chong H, Cuvelier GDE, Forbes Satter LR, Martinez C, Vander Lugt MT, Yu LC, Chandrakasan S, Joshi A, Prockop SE, Dávila Saldaña BJ, Aquino V, Broglie L, Ebens CL, Madden LM, DeSantes K, Milner J, Rangarajan HG, Shah A, Gilio AP, Knutsen AP, Miller HK, Moore TB, Graham P, Bauchat A, Bunin NJ, Teira P, Petrovic A, Chandra S, Abdel-Azim H, Dorsey M, Birbrayer O, Cowan MJ, Dvorak CC, Haddad E, **Kohn DB**, Notarangelo LD, Pai SY, Puck JM, Pulsipher MA, Torgerson TR, Malech HL, Kang EM. Genotype, Oxidase Status, and Preceding Infection or Autoinflammation Do Not Affect Allogeneic HCT Outcomes for CGD. Blood. 2023 Aug 10. Epub ahead of print. PMID: 37562003.

290. Bell JA, Collon K, Mayfield C, Gallo MC, Chang SW, Sugiyama O, Tang AH, Hollis R, Chopra S, **Kohn DB**, Lieberman JR. Biodistribution of lentiviral transduced adipose-derived stem cells for "ex-vivo" regional gene therapy for bone repair. Gene Ther. 2023 Aug 11. Epub ahead of print. PMID: 37568039.

291. Frost IM, Mendoza AM, Chiou TT, Kim P, Aizenberg J, **Kohn DB**, De Oliveira SN, Weiss PS, Jonas SJ. Fluorinated Silane-Modified Filtroporation Devices Enable Gene Knockout in Human Hematopoietic Stem and Progenitor Cells. ACS Appl Mater Interfaces. 2023 Aug 24. doi: 10.1021/acsami.3c07045. Epub ahead of print. PMID: 37616579.

292. Chandrasekaran, P., Han, Y., Zerbe, C. S., Heller, T., DeRavin, S. S., Kreuzberg, S. A., Marciano, B. E., Siu, Y., Jones, D. R., Abraham, R. S., Stephens, M. C., Tsou, A. M., Snapper, S., Conlan, S., Subramanian, P., Quinones, M., Grou, C., Calderon, V., Deming, C., Leiding, J. W., …**Kohn DB**,…. Falcone, E. L. (2023). Intestinal microbiome and metabolome signatures in patients with chronic granulomatous disease. *The Journal of allergy and clinical immunology*, S0091-6749(23)01101-6. Advance online publication. https://doi.org/10.1016/j.jaci.2023.07.022

293. Eissa H, Thakar M, Shah AJ, Logan B, Griffith LM, Dong H, Parrott RE, O'Reilly R, Dara J, Kapoor N, Satter LF, Chandra S, Kapadia M, Chandrakasan S, Knutsen A, Jyonouchi SC, Molinari L, Rayes A, Ebens CL, Teira P, Dávila Saldaña BJ, Burroughs LM, Chaudhury S, Chellapandian D, Gillio AP, Goldman F, Malech HL, DeSantes K, Cuvelier GDE, Rozmus J, Quinones R, Yu LC, Broglie L, Aquino V, Shereck E, Moore TB, Lugt MV, Mousallem TI, Oved JH, Dorsey M, Azim HA, Martinez C, Bleesing J, Prockop S, **Kohn DB**, Bednarski JJ, Leiding J, Marsh RA, Torgerson T, Notarangelo LD, Pai SY, Pulsipher MA, Puck JM, Dvorak CC, Haddad E, Buckley RH, Cowan MJ, Heimall J. Post-Transplant Late Complications Increase Over Time for Patients with SCID: A Primary Immune Deficiency Treatment Consortium (PIDTC) Landmark Study. J Allergy Clin Immunol. 2023 Oct 2:S0091-Epub ahead of print. PMID: 37793572.

294. **Kohn, D.B.,** Chen, Y.Y. & Spencer, M.J. Successes and challenges in clinical gene therapy. *Gene Ther* (2023).

295. Long-Boyle, J. R., **Kohn, D. B**., Shah, A. J., Spencer, S. M., Sevilla, J., Booth, C., López Lorenzo, J. L., Nicoletti, E., Shah, A., Reatz, M., Matos, J., & Schwartz, J. D. (2023). Busulfan and subsequent malignancy: An evidence-based risk assessment. *Pediatric blood & cancer*, e30738. Advance online publication.

296. Anne CA Mudde, Caroline Y. Kuo, **Donald B Kohn**, Claire Booth. What a Clinician Needs to Know about Genome Editing: Status, and Opportunities for and Application to Inborn Errors of Immunity. J Allergy Clin Immunol Pract. 2024 Jan 19:S2213-2198(24)00071-0. PMID: 38246560

297. Abraham RS, Basu A, Heimall JR, Dunn E, Yip A, Kapadia M, Kapoor N, Satter LF, Buckley R, O'Reilly R, Cuvelier GDE, Chandra S, Bednarski J, Chaudhury S, Moore TB, Haines H, Dávila Saldaña BJ, Chellapandian D, Rayes A, Chen K, Caywood E, Chandrakasan S, Lugt MTV, Ebens C, Teira P, Shereck E, Miller H, Aquino V, Eissa H, Yu LC, Gillio A, Madden L, Knutsen A, Shah AJ, DeSantes K, Barnum J, Broglie L, Joshi A, Kleiner G, Dara J, Prockop S, Martinez C, Mousallem T, Oved J, Burroughs L,

Marsh R, Torgerson TR, Leiding JW, Pai SY, **Kohn DB,** Pulsipher MA, Griffith LM, Notarangelo LD, Cowan MJ, Puck J, Dvorak CC, Haddad E. Relevance of lymphocyte proliferation to PHA in severe combined immunodeficiency (SCID) and T cell lymphopenia. Clin Immunol. 2024 Feb 15:109942. PMID: 38367737.

298. Michael D Keller, Patrick J. Hanley, Yueh-Yun Chi5, Paibel Aguayo-Hiraldo6, Christopher C. Dvorak, Michael R. Verneris, **Donald B. Kohn**, Sung-Yun Pai, Blachy J. Dávila Saldaña, Benjamin Hanisch, Troy C. Quigg, Roberta H. Adams, Ann Dahlberg, Shanmuganathan Chandrakasan15, Hasibul Hasan6, Jemily Malvar6, Mariah A. Jensen-Wachspress1, Christopher A. Lazarski1, Gelina Sani1, John M. Idso1, Haili Lang1, Pamela Chansky1, Chase D. McCann1, Jay Tanna1, Allistair A. Abraham1,3,4, Jennifer L. Webb, Abeer Shibli1, Amy K. Keating, Prakash Satwani18, Pawel Muranski, Erin Hall, Michael Eckrich21, Evan Shereck22, Holly Miller13, Ewelina Mamcarz23, Rajni Agarwal, Satiro N. De Oliveira, Mark T. Vander Lugt, Christen L. Ebens, Victor M. Aquino, Jeffrey J. Bednarski, Julie Chu, Suhag Parikh, Jennifer Whangbo, Michail Lionakis, Elias T. Zambidis, Elizabeth Gourdine, Catherine M. Bollard, Michael A. Pulsipher. Antiviral Cellular Therapy for Enhancing T-cell Reconstitution Before or After Hematopoietic Stem Cell Transplantation (ACES): a two-arm, open label phase II interventional trial of pediatric patients with risk factor assessment. *Nat Commun* 15, 3258 (2024).

299. Hart KL, Liu B, Brown D, Campo-Fernandez B, Tam K, Orr K, Hollis RP, Brendel C, Williams DA, **Kohn DB.** A novel high-titer, bifunctional lentiviral vector for autologous hematopoietic stem cell gene therapy of sickle cell disease. Mol Ther Methods Clin Dev. 2024 Apr 24;32(2):101254. PMID: 38745893 Free PMC article.

300. Enhancing Pediatric Access to Cell and Gene Therapies. Crystal L Mackall, Catherine M. Bollard, Nancy Goodman, Casey Carr, Rebecca Gardner, Rayne Rouce, Elena Sotillo, Rich Stoner, Fyodor D. Urnov, Alan Wayne, Julie Park, **Donald B. Kohn.** *Nat Med*, In Press.

301. Li YR, Zhou Y, Yu J, Kim YJ, Li M, Lee D, Zhou K, Chen Y, Zhu Y, Wang YC, Li Z, Yu Y, Dunn ZS, Guo W, Cen X, Husman T, Bajpai A, Kramer A, Wilson M, Fang Y, Huang J, Li S, Zhou Y, Zhang Y, Hahn Z, Zhu E, Ma F, Pan C, Lusis AJ, Zhou JJ, Seet CS, Kohn DB, Wang P, Zhou XJ, Pellegrini M, Puliafito BR, Larson SM, Yang L. Generation of allogeneic CAR-NKT cells from hematopoietic stem and progenitor cells using a clinically guided culture method. Nat Biotechnol. 2024 May 14. Online ahead of print. PMID: 38744947

302. Lentiviral Vectors for Precise Expression to Treat X-Linked Lymphoproliferative Disease. Paul Ayoub, Julia Gensheimer, Lindsay Lathrop, Colin Juett, Kevin Tam, Jack Reid, Fei-Yang Ma, Curtis Tam, Jason Quintos, Grace E. McCauley, Devin Brown, Xiaomeng Wu, Ruixue Zhang, Kathryn Bradford, Roger P. Hollis, Gay M. Crooks, **Donald B. Kohn** *Molecular Therapy Methods & Clinical Development*, In Press

303. DNA Contamination Within Recombinant Adeno Associated Virus (AAV) Preparations Correlates with Decreased CD34+ Cell Clonogenic Potential. Christopher Luthers, Sung-Min Ha, Annika Mittelhauser, Marco Morselli, Joseph D. Long, Caroline Y. Kuo, Zulema Romero, **Donald B. Kohn** *Molecular Therapy Methods & Clinical Development*, In Press

PUBLICATIONS (Cont'd)

304. Allogeneic hematopoietic cell transplantation is effective for p47phox chronic granulomatous disease: a PIDTC study. Eyal Grunebaum, Danielle E. Arnold, Brent Logan, Suhag Parikh, Rebecca A. Marsh, Linda M. Griffith, Kanwaldeep Mallhi, Deepak Chellapandian, Stephanie Si Lim, Christin L. Deal, Neena Kapoor, Luis Murguía-Favela, Emilia Liana Falcone, Vinod K. Prasad, Fabien Touzot, Jack J. Bleesing, Shanmuganathan Chandrakasan, Jennifer R. Heimall, Jeffrey J. Bednarski, Larisa A. Broglie, MD, Hey Jin Chong, Malika Kapadia, Susan Prockop, Blachy J. Dávila Saldaña, Edo Schaefer, Andrea L. Bauchat, Pierre Teira, Sharat Chandra, Mark Parta, Morton J. Cowan, Christopher C. Dvorak, Elie Haddad, **Donald B. Kohn,** Luigi D. Notarangelo, Sung-Yun Pai, Jennifer M. Puck, Michael A. Pulsipher, Troy Torgerson, Harry L. Malech, Elizabeth M. Kang and Jennifer W. Leiding. J All Clin Imm 153:1423-62, 2024

Submitted:

Successful Gene Editing of Apolipoprotein E4 to E3 in Brain of Alzheimer Model Mice After a Single IV Dose of Synthetic Exosome-Delivered CRISPR . Bruce Teter, Jesus Campagna. Chunni Zhu, Grace E. McCauley, Patricia Spilman, **Donald B. Kohn**, and Varghese John.

Ex-vivo Lentiviral-Mediated Gene Therapy for Severe Leukocyte Adhesion Deficiency Type-I (LAD-I). Claire Booth, Ph.D.,1 Julián Sevilla, M.D., Ph.D.,2,3 Elena Almarza, Ph.D.,4-6 Caroline Y. Kuo, M.D.,7 Josune Zubicaray, M.D., Ph.D.,2 Dayna Terrazas, RN,7 Gráinne O'Toole, RN,1 Maria Chitty-Lopez, M.D.,4 Grace Choi, B.S.,4 Eileen Nicoletti, M.D.,4 Susanna Carou-Keenan, PharmD,4 Janel Long-Boyle, PharmD, Ph.D.,8 Augustine Fernandes, Ph.D.,7 Kritika Chetty, MBBS,1 Satiro De Oliveira, M.D.,7 Crystal Banuelos,7 Jinhua Xu-Bayford, RN,1 Antonella Lucía Bastone, Ph.D.,9 Philipp John-Neek, M.Sc.,9 Connie Jackson,7 Theodore B. Moore, M.D.,7 Kimberly Gilmour, Ph.D.,1 Axel Schambach, Ph.D.,9 Michael Rothe, Ph.D.,9 Gayatri R. Rao, M.D., J.D.,4 Kinnari Patel, PharmD,4 Adrian J. Thrasher, Ph.D.,1 Juan A. Bueren, Ph.D.,6 Jonathan D. Schwartz, M.D.,4 **Donald B. Kohn, M.D**.7

Outcomes Following Matched Sibling Donor Transplant for Severe Combined Immunodeficiency: A Report from the PIDTC. Ahmad Rayes1, Brent Logan2, Xuerong Joy Liu2, Jasmeen Dara3, Rebecca H. Buckley4, Joseph H. Oved5, Neena Kapoor6, Malika Kapadia7, Sharat Chandra8, Caridad Martinez9, Nancy J. Bunin10, Shanmuganathan Chandrakasan11, Lauri M. Burroughs12, Jeffrey J. Bednarski13, Hilary Haines14, Geoff D.E. Cuvelier15, Hesham Eissa16, Julie-An Talano17, Blachy J. Dávila Saldaña18, Christen L. Ebens19, Sonali Chaudhury20, Evan Shereck21, Victor Aquino22, Alan P. Knutsen23, Jessie L. Alexander24, Alfred P. Gillio25, Deepak Chellapandian26, Ami J. Shah27, Holly Miller28, Mark Vander Lugt29, Kenneth DeSantes30, Morna Dorsey3, Roberta E. Parrott4, Richard J. O'Reilly5, Paibel Aguayo-Hiraldo6, Monica S. Thakar12, Troy Torgerson31, Jennifer Leiding32, Rebecca Marsh8, Linda Griffith33, Michael A. Pulsipher34, **Donald Kohn**35, Luigi Notarangelo36, Morton J. Cowan3, Jennifer M. Puck3, Jennifer Heimall37, Elie Haddad38*, Sung-Yun Pai39*, Christopher C. Dvorak3*.

In preparation:

Lentiviral Vectors for Gene Therapy of Alpha Thalassemia. Eva Segura, Kevyn Hart, Roger Hollis, Tippi Mackenzie, and **Donald B. Kohn**

Hematopoietic Stem Cell Gene Therapy for Adenosine Deaminase-Deficient Severe Combined Immune Deficiency (ADA SCID) Demonstrates Safe and Sustained Restoration Of Immune Function. Claire Booth, Katelyn Masiuk,…Donald B. Kohn

Non-peer review

1. **Kohn DB**, Kantoff PW. Potential applications of gene therapy. Transfusion 29:812-820, 1989.
2. Parkman R, Weinberg K, **Kohn D**, Sender L, Lenarsky C. Bone marrow transplantation in primary disorders of the hematopoietic stem cell. Bone Marrow Transplantation 4 (Suppl 4):137-38, 1989.
3. Lenarsky C, **Kohn DB**, Weinberg KI, Parkman R. Bone marrow transplantation for genetic disease. Hematol Oncol Clin North Am 4:589-602, 1990.
4. **Kohn DB**. The current status of gene therapy using hematopoietic stem cells. Curr Opin Pediatr 7 (1): 56-63, 1995.
5. Parkman R, Crooks G, **Kohn DB**, Lenarsky C, Weinberg K. Bone marrow transplantation for metabolic diseases. Cancer Treat Res 76:87-96, 1995.
6. Weinberg KI. **Kohn DB**. Gene therapy for congenital immunodeficiency disease. Organ and Bone Marrow Transplantation. Immunol All Clin N Am 16:453-76, 1996.
7. **Kohn DB**, Sarver N. Gene therapy for HIV-1 infection. Review Article. Adv Exp Med 394:421-428, 1996
8. **Kohn DB**, Weinberg KI, Parkman R. Gene therapy for immune deficiency syndromes: the past, the present, the future. The Immunologist 4:199-202, 1996.
9. **Kohn DB**, Gene therapy for hematopoietic and immune disorders. Bone Marrow Transplant 3:S55-S58, 1996.
10. **Kohn DB**. Gene therapy for hematopoietic and lymphoid disorders. Clin Exp Immunol 107:54-57, 197.
11. **Kohn DB**, Parkman R. Gene therapy for newborns. FASEB J 11:635-639, 1997.
12. Weinberg KI, **Kohn DB**. Gene therapy for congenital lymphoid immunodeficiency diseases. Seminars in Hematology 35:354-366, 1998.
13. Kapoor N, Crooks G, **Kohn DB**, Parkman R. Hematopoietic stem cell transplantation for primary immunodeficiencies. Semin Hematol 35:346-353, 1998.
14. Engel BC, **Kohn DB**. Stem cell directed gene therapy. Front in Biosci 4:26-33, 1999.

15. **Kohn DB**. Gene therapy using hematopoietic stem cells. Curr Opin Mol ther 1:437-442, 1999.

16. Parkman R, Weinberg K, Crooks G, Nolta J, Kapoor N, **Kohn DB**. Gene therapy for adenosine deaminase deficiency. Ann Rev. Med 51:33-47, 2000.

17. **Kohn DB**. Gene therapy for XSCID: The first successful gene therapy. Pediatr Res, November 48: 2000.

18. Carter B, **Kohn DB**. Clinical applications of gene therapy. Curr Opin Mol Ther 2:360-361, 2000.

19. Bauer G, Selander D, Engel B, Carbonaro D, Csik S, Rawlings S, Church J, **Kohn DB**. Gene therapy for pediatric AIDS. Ann NY Acad Sci 918:318-329, 2000.

20. **Kohn DB**. Gene therapy for genetic hematological disorders and immunodeficiencies. J Int Med 249:379-390, 2001.

21. Weinberg KI, Kapoor N, Shah AJ, Crooks GM, **Kohn DB**, Parkman R. Hematopoietic stem cell transplantation for severe combined immune deficiency. Curr Allergy Asthma Rep 5:416-420, 2001.

22. **Kohn DB**. Adenosine deaminase gene therapy protocol revisited. Mol Ther 5:96 97, 2002.

23. Engel BC, **Kohn DB**, Podsakoff GM. Update on gene therapy of inherited immune deficiencies. Curr Opin Mol Ther 5:503-507, 2003.

24. Engel BC, **Kohn DB**. Gene therapy for inborn and acquired immune deficiency disorders. Acta Haematol 110:60-70, 2003.

25. **Kohn DB**, Sadelain M, Dunbar C, Bodine D, Kiem HP, Candotti F, Tisdale J, Riviere I, Blau CA, Richard RE, Sorrentino B, Nolta J, Malech H, Brenner M, Cornetta K, Cavagnaro J, High K, Glorioso J. American Society of Gene Therapy (ASGT) ad hoc subcommittee on retroviral-mediated gene transfer to hematopoietic stem cells. Mol Ther 8:180-187, 2003.

26. Smogorzewska EM, Weinbert KI, **Kohn DB**. Transplantation of genetically modified cells in the treatment of children with SCID: great hopes and recent disappointments. Med Wieku Rozwoj 7:27-34, 2003.

27. Podsakoff GM, Engel BC, **Kohn DB**. Perspectives on gene therapy for immune deficiencies. Biol Blood Marrow Transplant 11:972-976, 2005.

28. **Kohn DB**. Lentiviral vectors ready for prime-time. Natl Biotechnol 25:65-66, 2007.

29. Rossi JJ, June CH, **Kohn DB**. Genetic therapies against HIV. Nat Biotechnol 25:1444-1454, 2007

30. **Kohn DB**. Gene therapy for childhood immunological deficiencies. Special Issue: Pediatric Blood and Marrow Transplantation: State of the Science. BMT, 2007

31. **Kohn DB** and Candotti F. Gene therapy fulfilling its promise. N Engl J Med, 360:518-521, 2009.

32. Shah AJ, and **Kohn DB**. Neurocognitive function of patients with severe combined immunodeficiency. Immunol Allergy Clin North Am. 2010 Feb;30(1):143-51

33. Shaw K and **Kohn DB**. Perspective: A Tale of Two SCIDS. Science Translational Medicine 2011 Aug 24;3(97):97ps36.

34. **Kohn DB**, Pai, SY, and Sadelain M. Gene Therapy through Autologous Transplantation of Gene-Modified Hematopoietic Stem Cells. Biol Blood Marrow Transplant. 2013 Jan;19(1 Suppl):S64-9.

35. Gschweng E, De Oliveira SM, and **Kohn DB**. Hematopoietic Stem Cells for Cancer. Immunotherapy. Immunological Reviews, 257:237-49, 2014.

36. Kuo CY and **Kohn DB**. Gene Therapy for the Treatment of Primary Immune Deficiencies. Curr Allergy Asthma Rep. 2016 May;16(5):39. doi: 10.1007/s11882-016-0615-8. Review. PMID: 27056559

37. **Kohn, DB**. Gene Therapy for Adenosine Deaminase Severe Combined Immune Deficiency – An Unexpected Journey of Four Decades. Immunological Reviews, In press.

Letter to Editor

1. **Kohn DB**, Horowitz SD, Finlay J, Trigg ME, Chesney PJ. Sarcoidosis presenting as scrotal mass. Pediatr Infec Dis 4:302-03, l985.
2. **Kohn DB**, Krall W. MFG is no more immune to “silencing” than the N2 backbone. Gene Ther 1995.
3. Kiem HP, **Kohn DB** and Cannon PM. Hope of HIV cure in Boston patients dashed. Nature News Dec 06 2013.

BOOK CHAPTERS

1. Weinberg K, Lenarsky C, **Kohn D**, Parkman R. Bone marrow transplantation for congenital and acquired immunodeficiency syndromes. In Bone Marrow Transplantation: Current Controversies, Gale RP, Champlin RE (Eds), New York: Alan R. Liss, 337-44, 1989.
2. Lenarsky C, **Kohn DB**, Weinberg KI, Parkman R. Bone marrow transplantation for genetic diseases. In Hematology/Oncology Clinics of North America, Yeager E (Ed), Philadelphia: WB Saunders, 1990.
3. Lenarsky C, **Kohn D**, Parkman R. Bone marrow transplantation for immunodeficiency and genetic diseases. In Bone Marrow Transplantation, Champlin R (Ed), Boston: Kluwer Academic Publishers, 167-84, 1990.
4. Lenarsky C, Weinberg K, Peterson J, Nolta J, Brooks J, Annett G, **Kohn D**, and Parkman R. Autologous bone marrow transplantation with 4-Hydroperoxycyclo-phosphamide purged marrows for children with acute nonlymphoblastic leukemia in second remission. In Autologous Bone Marrow Transplantation. Dicke KA, Armitage JO, Dicke-Evinger MJ (eds). University of Nebraska Medical Center, Nebraska, 1990, pp 105-112.
5. Parkman R, **Kohn DB**. Gene therapy for inborn errors of metabolism. Genetics, Ethics and Human Values - Human Genome Mapping, Genetic Screening and

Gene Therapy. Proceedings of the XXIVth CIOMS Round Table Conference Edited by Z. Bankowski and A.M. Capron held in Tokyo and Inuyama, Japan, July 1990.

6. Weinberg KI, **Kohn DB**. Somatic gene therapy for severe combined immune deficiency (SCID). In Somatic Gene Therapy, CRC Press, Inc. Patricia L. Chang (Ed). 31-47, 1994.
7. **Kohn DB**, Sarver N. Gene therapy for HIV-1 infection. In Antiviral Chemotherapy 4, Plenum Press, J Mills et al (ED).421-428, 1996.
8. **Kohn DB**, Conrad C. Gene therapy and viruses. Clinical Virology, Churchill Livingstone, Richman, Whitley, Hayden (Ed), New York, NY, 1996.
9. Nolta JA, **Kohn DB**. Hematopoietic Stem Cells for Gene Therapy. 447-462, Stem Cell Handbook, C.S. Potten (ed) Academic Press Limited, London, 1997.
10. **Kohn DB**, Schuening F, Dunbar C, Penserio M, Kiem HP, Beach K, Scott R, Zaboikin M, Miller D, Storb R, Darovsky B, Wells S, Nolta J, Esplin J, Barton N, Brady R, Karlsson S. Early trials of gene transfer for Gaucher disease. Advances in Jewish Genetic Diseases (ed) Desnick RJ. Oxford Press, 1997.
11. Nolta JA, **Kohn DB**. Human hematopoietic cell culture, transduction, and analyses. Current Protocols in Human Genetics, 1997.
12. **Kohn DB**, Nolta JA, Crooks GM. Clinical trials of gene therapy using hematopoietic stem cells. Hematopoietic Stem Cell Transplantation, 2nd edition ed by Forman S, Blume K, Thomas ED. Blackwell Scientific Publications, Boston MA. 1998.
13. **Kohn DB**. Gene therapy for adenosine deaminase (ADA)-deficient severe combined immunodeficiency. Progress in Gene Therapy: Basic and Clinical Frontiers. (ed) Bertolotti R, Parvez SH, Nagatsu T, VSP 2000.
14. Bauer G, Selander D, Engel B, Carbonaro D, Csik S, Rawlings S, Church J, **Kohn DB**. Gene Therapy for Pediatric AIDS. Prevention and Treatment of HIV Infection in Infants and Children. (ed) Ammann AJ, Rubinstein A. Ann NY Acad Sci, 2000.
15. **Kohn DB**, Carter B, Glorioso J. Gene Therapy and Viruses. In Clinical Virology, 2nd edition. (ed) Richman DD, Whitley RJ, Hayden FG. ASM, 2002.
16. Bonde J, Wirthlin L, **Kohn DB**, Nolta JA. Human Hematopoietic Cell Culture, Transduction, and Analyses. Genetics: Laboratory Techniques & Procedures. (ed) Jonathan L. Hains, et al. Currr Protoc Hum Genet, 2008.
17. Ikeda A and **Kohn DB**. Hematopoietic Stem Cell Transplantation and Gene Therapy for Primary Immune Deficiency Diseases. Pediatric Allergy:Principles and Practices-Second Edition. (ed) Leung DYM, et al. Elsevier. 2010.
18. Kuftinec G, Wherley J and **Kohn DB**. Gene Therapy of Genetic Diseases of Blood Cells. Pp. 391-422. Stem Cells: From Biology to Therapy. Advances in Molecular Biology and Medicine. (ed) Meyers RA. Wiley-Blackwell, 2013.
19. **Kohn DB**. Gene Therapy for Primary Immune Deficiencies. In Stiehm's Immune Deficiencies, edited by Kathleen Sullivan and E. Richard Stiehm. Academic Press, San Diego, CA, 2015.

20. **Kohn DB** and Notarangelo LN. Hematopoietic Stem Cell Therapy. In: Pediatric Allergy, 3rd edition, edited by Donald Leung, Stanley Szefler, Francisco Bonilla, Cezmi Akdis, and Hugh Sampson. Elsevier, Hoboken, NJ, 2016.

21. **Kohn DB**. Historical Perspective on the Current Renaissance for Hematopoietic Stem Cell Gene Therapy. Hematology/Oncology Clinics of North America. 32:721-735, 2017.

22. Grunebaum, E., and **Kohn**, **D.B**. (2018). Adenosine deaminase deficiency: Treatment. In E. TePas (ed.), *UpToDate*. May 2, 2108.

23. Grunebaum, E. and **Kohn, D.B**. (2018). Adenosine deaminase deficiency: Pathogenesis, clinical manifestations, and diagnosis. In E. TePas (ed.), *UpToDate*. May 1, 2018.

24. **Kohn DB**. Hematopoietic Stem Cell Therapies. In: Pediatric Allergy, 4rd edition, edited by Donald Leung, et al. Elsevier, Hoboken, NJ, 2020.

25. **Kohn DB** and Kuo CY. Gene Therapy for Primary Immune Deficiency Diseases. In: Clinical Immunology: Principles and Practice, 6th edition, edited by R.Rich, T Fleischer, and J Puck. Elsevier, Hoboken, NJ, 2020

26. **Kohn DB.** Gene Therapy for Primary Immune Deficiencies. In: Stiehm's Immune Deficiencies: Inborn Errors of Immunity, 2nd edition edited by KE Sullivan and ER Stiehm. Academic Press, San Diego CA, 2020.

SYMPOSIA

1. **Kohn DB**, Nolta JA, Hong CM, Barranger JA. Expression of the human glucocerebrosidase gene using retroviral vectors. In Gene Transfer and Gene Therapy (UCLA Symposia on Molecular and Cellular Biology, New Series, Vol. 87). Beaudet AL, Mulligan R, Verma IM (Eds), New York: Alan R. Liss, 397-408, 1989.

2. **Kohn DB**, Kantoff P, Zweibel JA, Gilboa E, Anderson WF, Blaese RM. Transfer and expression of the human adenosine deaminase (ADA) gene in ADA-deficient human T lymphocytes with retroviral vectors. In Gene Transfer and Gene Therapy (UCLA Symposia on Molecular and Cellular Biology, New Series, Vol. 87). Beaudet AL, Mulligan R, Verma IM (Eds), New York: Alan R. Liss, 365-74, 1989.

3. **Kohn D**, Nolta J, Weinthal J, Bahner I. Towards gene therapy for Gaucher disease. Hum Gene Ther 2:101-105, 1991.

4. Dube I, Stewart K, Lutzko C, Kruth S, Abrams-Ogg A, Shull R, Danos O, **Kohn D**, Reddy V, Chu P, Kamel Reid S. Gene transfer into marrow-derived hematopoietic stem cells. Gene Ther 579-580, 1995.

5. **Kohn DB**. Gene therapy for hematopoietic and immune disorders. Hilton Head First International Pediatric Bone Marrow Transplant Meeting. 1995.

6. Parkman R, Lenarsky C, **Kohn D**, Sender L, Weinberg K. Bone marrow transplantation for immunodeficiency states. In New Strategies in Bone Marrow

Transplantation (UCLA Symposia on Molecular and Cellular Biology). Gale RP, Champlin R (Eds), New York: Wiley-Liss 1991.

7. **Kohn DB**, Krall W, Challita PM, Skelton D, Malik P. Gene therapy for congenital hematologic and immune disorders. Bone Marrow Transplant

K. INVITED ARTICLES

1. Blaese RM, **Kohn DB**, Moen RC. The treatment of adenosine deaminase deficiency. Immunol Today 8:296-97, 1987.
2. Eglitis MA, Kantoff PW, **Kohn DB**, Karson E, Moen RC, Lothrop Jr CD, Blaese RM, Anderson WF. Retroviral-mediated gene transfer into hematopoietic cells. Adv Exp Med Biol 241:19-27, 1988.
3. **Kohn DB**, Anderson WF, Blaese RM. Gene therapy for genetic diseases. Cancer Invest 6(4), 1989.
4. Barranger JA, Ohashi T, Hong CM, Tomich J, Aerts JFGM, Tager JM, Nolta JA, Sender LS, Weiler S, **Kohn DB**. Molecular pathology and therapy of Gaucher disease. Japanese J Inherited Metabolic Disease 51:45-71, 1989.
5. **Kohn DB**, Sarver N. Gene therapy for HIV-1 infection. In antiviral chemotherapy 4: Plenum press, J Mills et al (ed) 421-428, 1996.
6. **Kohn DB**. Gene therapy for hematopoietic and lymphoid disorders. Clin Exp Immunol 107:54-57, 1997.
7. **Kohn DB**, Weinberg KI, Parkman R. Gene therapy for primary immunodeficiencies: the past, the present and the future. The Immunologist 4:199-202, 1996.
8. **Kohn DB**. Gene therapy for hematopoietic and immune disorders. Bone Marrow Transplant 18 (Suppl 1) S55-S58, 1996.
9. **Kohn DB**, Parkman R. Gene therapy for newborns. FASEB 11:635-639, 1997.
10. Kapoor N, Crooks G, **Kohn DB**, Weinberg KI, Parkman R. Histocompatible family member bone marrow transplantation for metabolic diseases. Hematopoietic Cell Transplant Second Edition. 1998.
11. Parkman R, Weinberg K, Crooks G, Nolta J, Kapoor N, **Kohn D**. Gene therapy for adenosine deaminase (ADA Deficiency). Ann Rev of Medicine, 51:33-47, 2000.
12. **Kohn, D.B.** Inside Blood: Gene therapy as salvage. Blood 110:4, 2007.
13. **Kohn DB** and Hollis RP. Inside Blood: Envelope, please. And the award goes to…. Blood 124:1203-4, 2014.
14. **Kohn DB.** Inside Blood: Gene therapy outpaces haplo for XSCID. Blood 125:3521-2, 2015.

15. **Kohn DB.** Inside Blood: Gene Therapy: WAS (not) just for kids. Blood, 2017.

16. Podcast: Forum: Gene therapy roundtable with High and Kohn. *Nat Biotechnol* **39,** 1201 (2021). https://doi.org/10.1038/s41587-021-01083-3

17. Ayoub PG and **Kohn DB**. Blood (commentary). β-thalassemia: All about that base, no cutting. Blood. 2023 Mar 9;141(10):1098-1099.. PMID: 36893006

L. INVITED PRESENTATIONS (Selected)

1. "Retroviral-mediated Gene Transfer into Mammalian Cells." Presented at the 2nd Annual Symposium on Analysis of Hemopoiesis: Recent Advances. Valhalla New York, October 1986.

2. "Gene Therapy of Immunodeficiency Disorders." Presented at the American Academy of Allergy and Immunology workshop, A Primer on Molecular Biology. Washington DC, February 1987.

3. "Retroviral Vectors Producing HIV Antisense RNA Block HIV Infection." Presented The NIH AIDS Discovery and Development Meeting. Oakland, CA, October 1988.

4. "Retroviral Vectors to Deliver Genes Inhibitory to Human Immunodeficiency Virus (HIV) Infection." Presented at Conference on Self-cleaving RNA as an Anti-HIV Agent: Design and Delivery to Cells. National Institute of Allergy and Infectious Diseases (NIAID), AIDS Program, Potomac Maryland, June 1989.

5. "Gene Therapy for Gaucher Disease." Presented at the FASEB Summer Conference on Cellular and Molecular Studies of Bone Marrow Transplantation. Saxton's River Vermont, July 1989.

6. "Retrovirally Mediated Insertion of the Human Glucocerebrosidase Gene." Presented at the Pediatric Hematology Subcommittee 23rd Annual Meeting of American Society of Hematology. Boston Massachusetts, December 1990.

7. Gene Transfer into Hematopoietic Stem Cells." Presented at the Parvin Cancer Research Laboratories Symposium On Hematopoietic Growth Factors. Honoring the Contributions of Dr. David Golde. Organized by the UCLA Tumor Cell Biology Training Program and UCLA, June 1991.

8. "Retroviral Vectors for Gene Replacement Therapy." Presented at the 6th Annual Conference of National MPS Society. Progress in MPS Research. Presented at University of California, Los Angeles June 1991.

9. "Retroviral Vector Transduction of Hematopoietic Stem Cells." Presented at Gene Therapy Approaches to Treatment of HIV-1 Infections. Sponsored by the UCLA

AIDS Institute, the UCI Cancer Research Institute, and Amgen. Presented in Palm Springs California, February 1993.

10. Nobel Forum at Karolinska Institutet - Frontiers in Medicine. “Gene Transfer in Gaucher’s Disease”. Presented in Copenhagen Stockholm, September 1993.

11. International Conference/Workshop on Cord Blood Transplantation and Biology/Immunology. “Clinical Aspects of Cord Blood Transplantation” Presented in Indianapolis, Indiana, November 1993.

12. Gene Therapy for Congenital Hematopoietic and Immune Disorders. Presented at the Keystone Symposium - Advances and Controversies in Bone Marrow Transplantation. Presented in Keystone Colorado, January 1994.

13. Marrow Transplantation in Children: Current Results and Controversies, Meeting #1 “Bone Marrow Gene Therapy” Hilton Head Island South Carolina, March 1994.

14. Educational and Programming at the American Academy of Allergy & Immunology: Basic Molecular Biology Course, Part 2. “A Practical Approach to Gene Therapy: Molecular Techniques, Anaheim California, March 1994.

15. Gene Therapy Meeting Cold Spring Harbor Laboratory “Gene Transfer for Gaucher Disease”. Presented in Cold Spring Harbor New York, September 1994.

16. Fourth International Gaucher Disease Conference - Expanding Horizons “A Perspective on the First Trials of Gene Therapy for Gaucher Disease. Presented in Philadelphia Pennsylvania, November 1994.

17. The Molecular Basis of Immune Deficiency Disorders and Strategies for Therapy “Gene Therapy for Immune Deficiency Disorders”. Presented in West Palm Beach Florida, November 1994.

18. The 34th Midwinter Conference of Immunologists “Gene Therapy for Congenital Immune Deficiencies” Presented at Asilomar, Pacific Grove California, January 1995.

19. Clinical Immunology Society - HIV Immune-based Therapies Workshop. “Gene Transfer into CD34 Cells for AIDS and SCID”. Presented in Baltimore Maryland, January 1995.

20. Translational Research in Blood and Marrow Transplantation - American Society for Blood & Marrow Transplantation “Modulation of Gene Expression”. Presented in Keystone Colorado, January 1995.

21. Stem Cells: Prospects for the Clinic, “Clinical Assessment of Stem Cell Transduction”. Presented in Palo Alto California, February 1995.

22. Gene Therapy and Molecular Medicine, “Results of Human Gene Therapy Trials” Presented at Keystone Symposia, Steamboat Springs Colorado, March 1995.

23. Gene Therapy of Bone Marrow Disorders. Presented at the 11th Annual Dr. Peter G. Danis Memorial Lecture. Presented in St. Louis Missouri, April 1995.

24. Annual 1995 ASCI/AFCR/SPR Clinical Research Meeting, Symposium on Primary Immunodeficiencies. “Gene Therapy of Primary Immunodeficiencies.” Presented in San Diego California, May 1995.

25. APS-SPR Annual Meeting-Therapies of Genetic Disease. “Gene Therapy for Neonates with ADA Deficiency by Transfer of the Human ADA cDNA into Umbilical Cord CD34+ Cells: Two Year Follow-up”. Presented in San Diego CA, May 1995.

26. The University of California, Irvine Cancer Research Institute - Basic Cancer Research. “Gene Therapy using Hematopoietic Stem Cells.” Presented in Irvine, California, May 1995.

27. Marrow Transplantation in Children: Current Results and Controversies, Meeting #2. “Gene Therapy: How Close Are We to Curing Anyone? Hilton Head Island South Carolina, June 1995.

28. Gene Therapy using Hematopoietic Stem Cells. Presented in Vancouver Canada, International Society Hematology & Graft Engineering, June 1995.

29. Board of Governors, Childrens Hospital Los Angeles. “Gene Therapy Program”. Presented in Los Angeles California, June 1995.

30. Gene Therapy for Pediatric AIDS. Presented at the Novel HIV Therapies: From Discovery to Clinical Proof-of-Concept. Joint Conference of the Strategic Program for Innovative Research on AIDS Treatment (SPIRAT) and the National Cooperative Drug Discovery Groups for the Treatment of HIV Infection (NCDDG-HIV). Presented in Bethesda Maryland, July 1995.

31. Grand Rounds Childrens Hospital Los Angeles. “What Ever Happened to Those “Bubble Babies” Who Got Gene Therapy”. Presented in Los Angeles California June 1995.

32. Presented to the Panel to Assess the NIH Investment in Research on Gene Therapy. “Gene Therapy Using Hematopoietic Stem Cells”. Presented in San Francisco California, August 1995

33. Gene Silencing III Homology-Dependent Gene Silencing in Plants. Presented at the Gordon Conference Epigenetic Effects in Gene Expression. Presented in Holderness New Hampshire, August 1995.

34. Practice of Hematologic Gene Therapy (Program of the Education Committee). "Gene Therapy for Non-Malignant Disorders of Hematopoietic Cells". Presented in Seattle Washington, December 1995.

35. Workshop on Bone Marrow Transplantation for Immune Deficiency and Metabolic Disorders: "Retroviral-Mediated Transfer and Expression of the Human α-Iduronidase cDNA in Human CD34+ Cells: Potential for Gene Therapy of Hurler's Disease. The Sixth Biennial Sandoz-Keystone Symposium on Bone Marrow Transplantation, Keystone, Colorado January 1996.

36. Workshop on Gene Therapy: "Use of Modified Retroviral Vectors to Overcome Silencing and Methylation in Murine Hematopoietic Stem Cells". The Sixth Biennial Sandoz-Keystone Symposium on Bone Marrow Transplantation, Keystone, Colorado January 1996.

37. Plenary Session on Cellular and Genetic Engineering: "Genetic Correction of Hematopoietic Stem Cells". The Sixth Biennial Sandoz-Keystone Symposium on Bone Marrow Transplantation, Keystone, Colorado January 1996.

38. Gene Therapy Workshop, Blood Cell and Marrow Transplants "Genetic Correction of Hematopoietic Stem Cells", Keystone, Colorado, January 1996.

39. Gene Therapy for Hematopoietic Stem Cells in Genetic Disease and Cancer - "Selective Accumulation of ADA Gene-Transduced T lymphocytes upon PEG-ADA Dosage Reduction After Gene Therapy with Transduced CD34+ Umbilical Cord Blood Cells". Keystone Symposia, Taos, New Mexico February 1996.

40. First Annual UCLA Gene Therapy Symposium - "Hematopoietic Stem Cells". University of California, Los Angeles, Los Angeles, California - June 1996.

41. Twenty-Fifth Annual Meeting of the International Society for Experimental Hematology "Gene Expression, Silencing and Methylation in Gene Therapy". New York, August 1996.

42. Gene Therapy of Genetic Diseases - "T Cell and Stem Cell Gene Therapy for ADA Deficiency". San Francisco, October 1996.

43. The 38th Annual Meeting & Exposition of the American Society of Hematology "Suitability of Bone Marrow from HIV-1-Infected Donors for Retroviral-Mediated Gene Transfer. Orlando, Florida, December 1996.

44. The 1997 Keystone Symposia Conference on Hematopoiesis "Gene Expression and Gene Transfer in Hematopoiesis. Durango, Colorado, February 1997.

45. PAGID/AAAI session chair and speaker, Gene Therapy for Primary Immunodeficiencies “Gene Therapy for ADA Deficiency” San Francisco, California, February 1997.

46. St. Jude Children’s Research Hospital Seminar Memphis, Tennessee, March 1997.

47. International Conference/Workshop on Cord Blood Transplantation “Biology and Gene Transfer/Therapy of Cord Blood Stem/Progenitor Cells”. Indianapolis, Indiana, March 1997.

48. The 100th Annual Congress of Japan Pediatric Society “Gene Therapy Using Hematopoietic Stem Cells. Tokyo, Japan, April 1997.

49. American Association for Cancer Research “DNA Methylation, Imprinting, and the Epigenetics of Cancer”. Fajardo Puerto Rico. 1998.

50. The 1998 Keystone Symposia Conference “Gene Therapy Strategies for Hematopoietic Cells. Keystone, Colorado. January 1998.

51. The 1998 Keystone Symposia Conference “HIV Pathogenesis and Treatment” Park City, Utah. March 1998.

52. First Annual American Society of Gene Therapy Educational Program “The Basics of Gene Therapy”. Seattle, Washington. May 1998.

53. The III International Symposium “In Utero Stem Cell Transplantation and Gene Therapy”. Portland, Oregon. September 1998.

54. University of Toronto’s Program in Molecular Medicine. “Retroviral Vector Modification of Stem Cells”. Toronto. November 1998.

55. Keystone Symposia “Molecular and Cellular Biology of Gene Therapy” Lentiviral Vectors Show Improved Gene Transfer in Human Hematopoietic Stem Cells. January 1999.

56. The 1999 National GCRC Meeting. “Stem Cell Based Gene Therapy”. Arlington, VA. March 1999.

57. Jonsson Cancer Center Research Conference Series “Immunotherapy Approaches to ALL: Feasible or Fantasy?”. Los Angeles, California. April 1999.

58. The 90th Annual Meeting American Association for Cancer Research “Advances in Stem Cell Gene Therapy.” Philadelphia, Pennsylvania. April 1999.

59. Advances in Clinical Gene Therapy Research *A Tribute to R. Michael Blaese, M.D.* “Gene Therapy using Hematopoietic Stem Cells”. Bethesda, Maryland. April 1999.

60. The 14th Annual Conference on Clinical Immunology - 5th International Symposium on Clinical Immunology "Co-Chair. Gene Therapy for Clinical Disease - "Gene Therapy for ADA-Deficient SCID." Washington, D.C. April 1999.

61. The 7th International Symposium on Recent Advances in Hematopoietic Stem Cell Transplantation "Gene and Cell Therapy". San Diego, CA. May, 1999.

62. Educational Seminars on Stem Cells "Bone Marrow Transplant and Stem Cells". Childrens Hospital Los Angeles, October 1999.

63. Marrow Transplantation in Children: Current Results and Controversies - Meeting #5 "Gene Therapy: Progress & Problems". Hilton Head Island, South Carolina, February, 2000.

64. Immune Reconstitution & Surrogate Markers in HIV/AIDS "Gene Therapy using Hematopoietic Stem Cells". Baltimore, Maryland, May, 2000.

65. ISHAGE 2000 Annual Meeting "Current Status of Gene Therapy using Hematopoietic Cells. San Diego, CA June 2000.

66. The Ethical and Social Implications of Human Medical Genetics, University of Illinois at Urbana-Champaign, September 2000.

67. The 57th Annual Brennemann Memorial Lecture "Gene Therapy: Current Status", San Diego, CA September 2000.

68. The 8th Meeting of the European Society of Gene Therapy "Gene Therapy for Genetic Hematopoietic Disorders and Immunodeficiencies" Stockholm, Sweden, October 2000.

69. "Molecular Mechanisms of Disease" Seminar at California Institute of Technology, Pasadena, CA December 2000.

70. Keystone Symposia Conference "Gene Therapy 2001: A Gene Odyssey" Conference Organizer, Snowbird, Utah January, 2001.

71. "The Future of Science and Pediatrics" Symposium at Childrens Hospital Los Angeles, Los Angeles, CA February, 2001.

72. 2001 Palm Springs Symposium on HIV/AIDS, "The Development of Antiviral Therapies". Palm Springs, CA March, 2001.

73. American Society of Gene Therapy 4th Annual Meeting, "Infectious Diseases" Seattle, WA May, 2001.

74. The Eighth West Coast Retrovirus Meeting, Keynote Lecture: "Expression from Retroviral Vectors for Gene Therapy" Palm Springs, CA October, 2001.

75. 2002 Keystone Symposium, Epigenetics in Development and Disease, "Overcoming Silencing for Somatic Gene Therapy" Taos, New Mexico Feb., 2002.

76. Third Conference, Stem Cell Gene Therapy: Biology and Technology, "Issues of Clinical Application of Gene Therapy" – Chairperson, NIDDK, NIAID, NHGRI, Rockville, Maryland, March 2002.

77. American Society of Gene Therapy 5th Annual Meeting: "Hemopoietic Gene Therapy: Progress and Prospects" Boston, Massachusetts, June 2002.

78. American Society of Gene Therapy 5th Annual Meeting: Genetic Disease – "Stem Cells for Genetic Disease" Boston, Massachusetts, June 2002.

79. 1st Annual Gene Therapy Symposium for Heart, Lung, and Blood Diseases – Keynote Speaker "Gene Therapy Using Hematopoietic Stem Cells", Davis, California, November 2002.

80. 9th Annual UCLA Human Gene Medicine Symposium, "Gene Transduction of Stem Cells", Los Angeles, California, May 2004.

81. Western Society for Pediatric Research (WSPR), Invited Speaker: State of the Art Lecture. "Gene Therapy Using Hematopoietic Stem Cells", Carmel, California, February 2005.

82. University of Southern California Board of Trustees, Invited Stem Cell Research, Invited Speaker: "Human Embryonic Stem Cells andProposition 71", Los Angeles, California March 2005.

83. Food and Drug Administration, Invited Speaker Cellular, Tissues and Gene Therapies Advisory Committee, Rockville, Maryland, March 2005.

84. The 6th Joint Annual Meeting, American Transplant Congress, Invited Speaker: Gene Therapy (Clinical State of the Art and Translational Research Reviews) – Retro and Lentiviral Vectors (XSCID Children, AIDS, Cancer, Bone Marrow Transplantation, Seattle, Washington, May 2005.

85. CalTech Biotechnology Club, California Institute of Technology. Invited speaker: "Challenges of Translating Gene Therapy". Los Angeles, California May 2005.

86. USC Systems Biology and Disease Graduate Program, Inaugural Scientific Symposium invited keynote speaker "ADA-Deficient SCID: Gene Therapy Approaches", Los Angeles, California May 2005.

87. The 4th Euregenethy International Conference, "Gene Therapy for SCID: Progress, Problems and Prospects. Paris, France June 2005.

88. American Society of Gene Therapy Annual Meeting, “ADA-deficient SCID: Pathophysiology and Treatments”. St. Louis, MO June 2005.

89. NIH, National Heart, Lung and Blood Institute Working Group: Hemoglobin Gene Transfer in Sickle Cell Disease and Cooley’s Anemia – Chair. June 2005.

90. USC 125th Anniversary University Celebration, Invited Speaker: “Inventing the Future: Stem Cell Research”. Los Angeles, California October 2005.

91. Gordon Conference on the Science of Viral Vectors for Gene Therapy, “Novel Approaches to Gene Therapy for SCID and Other Blood Cell Diseases, Ventura, California March 2006.

92. Global Horizons: America’s Challenge in Science and Innovation, “Stem Cell Research: Are We Bystanders or Key Players?” Panelist, Los Angeles, California April 2006.

93. The Hospital for Sick Children, “Gene Therapy Using HSC”, Toronto, Canada. May 2006.

94. International Society for Cellular Therapy, “Novel Developments in Gene Therapy” Berlin, Germany May 2006.

95. Seattle Children’s Symposium Primary Immune Deficiency Diseases: Molecular-Based Diagnoses and Therapy, “Current Status and Future of Gene Therapy in PIDD” Seattle, Washington August 2006.

96. NIH, Gene Therapy Conference, Gene Therapy for Inherited Immune Deficiencies: Advances and Safety Issues, “Gene Therapy for Primary Immune Deficiency Diseases: Looking Back and Looking Forward” Bethesda, Maryland Sept 2006.

97. University of California, San Diego School of Medicine: Grand Rounds, “Making and Remaking Kidneys.” San Diego, California October 2006.

98. University of Southern California, Moderator CER Research Salon, “Stem Cell Therapies: From Bench to Bedside”, Los Angeles, California April 2007.

99. University of Iowa, Gene Therapy Center Retreat, Featured Speaker: “Gene Therapy Using Hematopoietic Stem Cells”, Iowa City, Iowa April 2007.

100. Chair and Organizer, Educational Section “Topical Review: Retroviruses, Lentiviruses and Gene Therapy for Hematological Diseases”, ASGCT Seattle, Washington May 2007.

101. Chair – Hematopoietic Stem Cell Sub-Committee Scientific Symposium: “Conditioning Regimens for Hematopoietic Gene Therapy, ASGCT Seattle, Washington May 2007.

102. Invited Speaker – Industrial Liaison Sub-Committee: Drug Development and Gene Therapy "Performing Clinical Trials of ADA Gene Therapy in the New World, Seattle, Washington June 2007.

103. Chair: Corporate Symposium at the 10th Annual Meeting of ASGT by Sangamo Biosciences, Inc. "DNA as a Drug Target: Engineered Zinc Finger Protein Therapeutics" Seattle, Washington June 2007.

104. Invited Speaker: University of Washington, Department of Hematology Grand Rounds. "Gene Therapy for ADA-Deficient SCID" Seattle, Washington June 2007.

105. Invited Speaker: Immune Deficiency Foundation 2007 National Conference, "The Latest Advances in Gene Therapy" St. Louis, Missouri, June 2007.

106. Scientific Advisory Board Meeting, HSR-TIGET San Raffaele Telethon Institute for Gene Therapy, Milan, Italy, July 2007.

107. Panel Member –The Robert A. Good Immunology Society, "Immune Reconstitution of Primary Immunodeficiencies" Boston, Massachusetts, November 2007.

108. Invited Speaker: University of Alabama, Gene Therapy Program. "Gene Therapy for ADA-Deficient SCID" Birmingham, AL May 2008.

109. Invited Speaker: University of California, Los Angeles, Stem Cell Institute. "Gene Therapy for ADA-Deficient SCID" Los Angeles, CA May 2008.

---

110. Invited Speaker CIRM Town Forum: Stem Cell Science: The Pace to Cures. "Stem Cell Transplantation and Gene Therapy. USC Davidson Center, April 22, 2009.

111. Invited Debator: NIAID Workshop on Improving Cellular Therapy for Primary Immune Deficiency Diseases. "Gene Therapy or Allogeneic HSCT for SCID?" Bethesda, MD May 2009.

112. Invited Speaker: American Society of Gene Therapy 12th Annual Meeting, Scientific Symposium: "Overcoming Key Challenges in Advancing a Clinical Trial through the Various Phases of FDA Approval. ADA Gene Therapy: the Ten Years War." San Diego CA, May 2009.

113. Invited Speaker: UCLA Division of Pediatric Hematology/Oncology Research Seminar: "Gene Therapy for ADA-Deficient SCID" June 2009.

114. Invited Speaker: Clinical Immunology Society and the American Academy of Allergy, Asthma and Immunology Consortium Conference on Primary Immunodeficiency Diseases. "Gene Therapy for Primary Immune Deficiency Diseases" San Francisco, June 2009.

115. Invited Plenary Speaker: 9th Annual Meeting of the Federation of Clinical Immunology Societies. "Gene Therapy for Primary Immune Deficiency Diseases" San Francisco, June 2009.

116. Invited Speaker: 11th International Congress of Inborn Errors of Metabolism. Opening Presidential Session. "Gene Therapy Using Hematopoietic Stem Cells." San Diego CA, August 30, 2009.

117. Invited Speaker: 5th Annual Sudhir Gupta Endowed Chair in Molecular Immunology Symposium: Primary Immunodeficiency Diseases: Bench to bedside. "Gene Therapy in Primary Immunodeficiency." October 3, 2009.

118. Invited Speaker: City of Hope Graduate School - Leading Edge Lecture Series. "Gene Therapy Using Hematopoietic Stem Cells" March 11, 2010.

119. Invited Speaker: UCLA Jonsson Comprehensive Cancer Center Seminar Series. "Gene Therapy Using Hematopoietic Stem Cells" March 25, 2010.

120. Invited Speaker: Chronic Granulomatous Disease Research Trust Sympoisum: Progress and Prospects for Gene Therapy for CGD and Other Primary Immmunodeficiencies. "Update on Clinical Trials of Gene Therapy for X-SCID and ADA-Deficient SCID", Washington D.C. May 21, 2010.

121. Invited Speaker: UCLA Science Faculty Research Colloquium Series: "Gene Therapy Using Hematopoietic Stem Cells", May 24, 2010.

122. Invited Speaker: Unveil Sickle Cell 2010 - USC Community Educational Seminar. "Sickle Cell Disease, Bone Marrow Transplant and Gene Therapy." Long Beach CA Sept 11, 2010.

123. Invited Speaker and Discussion Panel Leader: Novel Therapies for Lysosomal Storage Disease Symposium, "Bone Marrow Based Gene Therapy for Primary Immune Deficiency Diseases." University of Pennsylvania, September 14, 2010.

124. Invited presenter: UCLA BioBasics/Research Administrators Layman Seminar. "Treating Diseases with Gene Medicine". September 22, 2010.

125. Invited Speaker: Eloise Giblett Memorial Symposium, Department of Hematology, University of Washington, "Therapy for ADA-deficient SCID." Seattle WA September 28, 2010.

126. Co-Organizer, Invited Speaker/Panelist, FDA Public Workshop on Cell and Gene Therapy: Clinical Trials in Pediatric Populations, Bethesda MD November 2, 2010.

127. Invited speaker, UCLA-Cal Tech Medical Scientist Training Program Tutorial Series, "Gene Therapy Using Hematopoietic Stem Cells" November 6, 2010.

128. Featured Speaker: UCLA School of Life Sciences – The Year of the New Life Sciences at UCLA Lecture Series. "Bone marrow stem cells: developing new therapies in the fight against disease." January 18, 2011.

129. UCLA Department of Pediatrics Grand Rounds "Hematopoietic Stem Cell Therapies for Blood Cell Diseases." January 21, 2011

130. UCLA Department of Neurology Third Annual Neurology Science Day Invited Speaker: Gene Therapy using Hematopoietic Stem Cells." January 26, 2011.

131. UC San Diego Program in Gene Therapy Speaker. "Gene Therapy – Hematopoietic Stem Cell Models" February 7, 2011.

132. Sickle Cell Disease Foundation of California 8th Annual Symposium. Invited speaker: "Sickle Cell Disease, Bone Marrow Transplant and Gene Therapy." Buena Park CA, April 1, 2011.

133. Invited Speaker and Session Chair. Primary Immune Deficiency Treatment Consortium (PIDTC). First Annual Scientific Workshop. "Gene Therapy for ADA and X-SCID in the U.S." April 10, 2011

134. California State Sacramento – Regenerative Medicine Lecture Series. "Going Viral: Using Viruses and Bone Marrow Stem Cells in the Fight Against Diseases." April 19, 2011.

135. Invited Speaker. University of Wisconsin, Madison, Department of Pediatrics Grand Rounds "Severe Combined Immune Deficiency: Causes, Transplants and Gene Therapy. " April 28, 2011. Madison WI.

136. Invited Speaker. Pediatric Academic Societies - Topic Symposium: Treatment of Pediatric Diseases Using Gene Therapy. "Gene Therapy for Primary Immune Deficiency Disorders". May 1, 2011. Denver CO.

137. Invited Presentation. California Institute for Regenerative Medicine: Spotlight on Disease Team Awards. "Disease Team Project Update: Stem Cell Gene Therapy for Sickle Cell Disease." May 4, 2011, Los Angeles CA.

138. Immune Deficiency Foundation National Conference 2011. Invited Speaker. "Hematopoietic Stem Cell Transplants for Primary Immune Deficiency Diseases." Scottsdale AZ, June 28, 2011.

139. Clinical Immunology School in Primary Immune Deficiency Diseases. Faculty Member. Miami Beach FL, August 25-28, 2011.

140. CIRM Grantee Meeting 2011 – Invited Plenary Speaker "Stem Cell Gene Therapy for Sickle Cell Disease". September 16, 2011.

141. ASGCT/NIH Gene Therapy Symposium. Meeting organizer, workshop leader and invited speaker. "Gene Therapy for Primary Immune Deficiencies: ADA-deficient SCID, X-SCID and Wiskott-Aldrich Syndrome." Bethesda MD, August 26-27, 2011.

142. Trans-Atlantic Gene Therapy Consortium – Participant and Invited Speaker. "Clinical, pre-clinical and pre-pre-clinical trial gene therapy studies." Brighton England, October 26-27, 2011.

143. Cedar-Sinai Hospital Center, Department of Pediatric Genetics Grand Rounds. "Gene Therapy for Blood Cell Diseases." January 19, 2012.

144. UCLA 8th Annual Stem Cell Symposium: Stem Cells and Cancer: Shared Paths, Different Destinations. "Gene Therapy Using Hematopoietic Stem Cells" February 10, 2012.

145. Institute of Medicine of the National Academies – Review of the California Institute for Regenerative Medicine. Invited presenter. Irvine CA, April 10. 2012.

146. Primary Immune Deficiency Treatment Consortium – Second Annual Scientific Workshop. "Update on Gene Therapy for ADA-SCID". , Boston MA April 28, 2012.

147. 2012 Advanced School in Primary Immune Deficiency. Invited speaker "New Innovations in Gene Therapy". Chicago IL, May 17, 2012.

148. Clinical Immunology Society 2012 Annual Meeting. Gene and Other New Therapies – Session Chair and Speaker, Chicago IL, May 20, 2012.

149. Organizer, Invited Speaker and Session Moderator. "Gene Transfer and Rare Disease Workshop". NIH Office of Biotechnology Activities and Office of Rare Diseases Research. Rockville MD, September 13, 2012.

150. D.A. Carbonaro-Sarracino, C.C.I. Lee, X. Jin, A.F. Tarantal, D.B. Kohn. Direct Intravenous Delivery of the Human ADA Gene by Lentiviral Vectors for In Vivo Enzyme Replacement Therapy. Poster presented at: The 15th Biennial Meeting of the European Society for Immunodeficiences. Florence, Italy, October, 2012. Abstract Number: 178.

151. F. Candotti, K.L. Shaw, R. Sokolic, D. Carbonaro, L. Muul, S. Mishra, E. Garabedian, P.-Y. Fu, G.J. Jagadeesh, C. Silvin, M.S. Hershfield, R.M. Blaese, D.B. Kohn. U.S. results of gene therapy for adenosine deaminase deficiency. Poster presented at: The 15th Biennial Meeting of the European Society for Immunodeficiences. Florence, Italy, October, 2012. Abstract Number: 692

152. Invited speaker. Indiana University School of Medicine, Medical and Molecular Genetics Seminar. "Gene Therapy Using Hematopoietic Stem Cells." 10/03/12.

153. Invited Speaker. “Performing Multi-national Clinical Trials for Rare Disorders.” ASGCT/ESGCT Early Phase Clinical Trials Training Course. Versailles, France 10/25/12.

154. Invited Speaker and Session Chair. “Gene Therapy for Adenosine Deaminase Deficient SCID.” Gene and Cell Therapy for PID Parallel Session. ESGCT 20th Annual Meeting, Versailles, France 10/26/12.

155. Invited presentation. “Gene Therapy: Treating Genetic Diseases by Inserting New Genes.” Science Speakers Series, Discovery Center for Science and Technology, Thousand Oak CA. 11/01/12

156. Invited lecture. “Gene Therapy for Genetic Diseases of Blood Cells.” California State University, Northridge. 11/02/12.

157. Invited Speaker. “Update on Gene Therapy for Primary Immunodeficiences.” 8th Annual Symposium on Primary Immunodeficiency Diseases. Foundation for Primary Immunodeficiency Diseases, and Division of Basic and Clinical Immunology, University of California, Irvine. Newport Beach, California, 11/04/12.

158. Invited Speaker: Case Studies of Cell Therapy Clinical Trials: Challenges and Lessons Learned. “Clinical Cell Therapies at an Academic Medical Center.” CIRM Workshop on Alpha Stem cell Clinics. Stanford University, Nov. 14, 2012.

159. Invited Speaker. “Gene Therapy Using Hematopoietic Stem Cells.” 11th Annual Gene Therapy Symposium for Heart, Lung and Blood Diseases. Sonoma CA, Nov 16, 2012.

160. Invited Session Chair and Speaker, “Hematopoietic Stem Cell Gene Therapy - An Update.” 2013 BMT Tandem Meetings, Salt Lake City, Utah, Feb 14, 2013

161. Invited Speaker. “Gene Therapy Using Hematopoietic Stem Cells.” Molecular Medicine Research (MMR) Seminar series, Children’s Hospital of Pittsburgh of UPMC, Pittsburgh, PA, March 5, 2013.

162. Invited Speaker. “Gene Therapy Using Hematopoietic Stem Cells.” Cedars Sinai Medical Center Hematology/Oncology Grand Rounds. Los Angeles, CA, April 16, 2013.

163. Session Moderator and Invited Speaker “Gene Therapy Update.” Primary Immune Deficiency Treatment Consortium (PIDTC) Third Annual Scientific Workshop. Houston, Tx, May 3, 2013.

164. Invited Speaker: “Severe Combined Immune Deficiency – Causes and Treatments” Southern California Association of Pediatric Hematology/Oncology Nurses (SCAPHON). Lake Arrowhead, CA, May 10, 2013.

165. Invited Speaker: “Gene Therapy for Sickle Cell Disease.” Frontiers in Gene & Molecular Therapies Lecture Series, Department of Pediatrics, Stanford University School of Medicine. Palo Alto, CA, May 23, 2013.

166. Invited Speaker: “Gene Therapy for Severe Combined Immune Deficiency.” Department of Pediatrics Grand Rounds. Stanford University School of Medicine. Palo Alto, CA, May 24, 2013.

167. Invited Speaker: “Gene Therapy Using Hematopoietic Stem Cells” UCLA MSTP Tutorial Series- Introduction to Biomedical Research Selective, UCLA September 16, 2013.

168. Organizer and Moderator” UCLA Human Gene Medicine 17th Annual Symposium: “Oncolytic viruses: Killing Cancer by Infection. UCLA September 23, 2013.

169. Invited Speaker: “Hematopoietic Stem Cell Transplantation for Sickle Cell Disease: Sibs, MUDs, Cords and Genes.” 41st Annual Convention, Sickle Cell Disease Association of America, Baltimore MD, September 27, 2013.

170. Invited Speaker: “Hematopoietic Stem Cell Transplantation for Sickle Cell Disease: Sibs, MUDS, Cords and Genes.” Second Annual West Coast Sickle Cell Conference 2013: Future and Current Concepts in Sickle Cell Disease**.** Children’s Hospital Los Angeles, Los Angeles CA. October 11, 2013

171. Invited Session Chair: 7th Stem Cell Clonality and Genome Stability Retreat. TransAtlantic Gene Therapy Consortium. Madrid, Spain. October 24, 2013

172: Invited presentation: “Gene Therapy for Sickle Cell Disease.” European Society of Gene and Cell Therapy, Annual meeting. Madrid Spain, October 27, 2013.

173. Invited speaker: “Bone Marrow Transplant and Gene Therapy for Sickle Cell Disease”. Update on Sickle Cell Disease – 2014. Comprehensive Blood & Cancer Center, Bakersfield CA, April 26, 2104.

174. Kit L. Shaw, Robert Sokolic, Alejandra Davila, Christopher Silvin, Elizabeth Garabedian, Satiro de Oliveira, Provaboti Barman, Berkley Brown, Denise Carbonaro, Sabine Geiger, Suparna Mishra, Monika Smogorzewska, Jayashree Jagadeesh, Michael S. Hershfield, Alan Wayne, Gay M. Crooks, Theodore Moore, Fabio Candotti, Donald B. Kohn. Phase II Clinical Trial of Gene Therapy for Adenosine Deaminase-Deficient Severe Combined Immune Deficiency (ADA-SCID). Oral presentation at the 17th annual meeting of the American Society of Gene and Cell therapy, Washington DC. May 2014.

175. Megan D. Hoban, Alok V. Joglekar, David Gray, Michael L. Kaufman, Michelle Ho, Zulema Romero, Shantha Senadheera, Gregory J. Cost, Andreas Reik, Michael C. Holmes, Philip D. Gregory, Roger P. Hollis, Donald B. Kohn. Site-Specific Correction of the Sickle Mutation in CD34+ Cells Using Zinc Finger Nucleases.

Oral presentation at the 17th annual meeting of the American Society of Gene and Cell therapy, Washington DC. May 2014.

176. Hubert B. Gaspar, Karen Buckland, Christine Rivat, Nourredine Himoudi, Kimberly Gilmour, Claire Booth, Kenneth Cornetta, Donald B. Kohn, Denise Carbonaro, Anna Paruzynski, Manfred Schmidt, Adrian J. Thrasher. Immunological and Metabolic Correction After Lentiviral Vector Mediated Haematopoietic Stem Cell Gene Therapy for ADA Deficiency. Oral presentation at the 17th annual meeting of the American Society of Gene and Cell therapy, Washington DC. May 2014.

177. Fabrizia Urbinati, Beatriz Campo, Jennifer Wherley, Sabine Geiger, Michael L. Kaufman, Aaron R. Cooper, Sally Shupien, Zulema Romero, Michelle Ho, Roger P. Hollis, Donald B. Kohn. Optimization and Characterization of Product Manufacturing for a Clinical Trial of Gene Therapy for Sickle Cell Disease. Poster presentation at the 17th annual meeting of the American Society of Gene and Cell therapy, Washington DC. May 2014.

178. Zulema Romero, Jennifer Wherley, Aaron R. Cooper, Michael L. Kaufman, Fabrizia Urbinati, Beatriz Campo, Megan D. Hoban, Kismet Baldwin, Shantha Senadheera, Roger P. Hollis, Donald B. Kohn. Novel Dual Insulated Lentiviral Vectors Expressing an Anti-Sickling β-Globin Gene. Poster presentation at the 17th annual meeting of the American Society of Gene and Cell therapy, Washington DC. May 2014.

179. Caroline Y. Kuo, Megan D. Hoban, Alok V. Joglekar, Donald B. Kohn. Targeted Gene Therapy in the Treatment of X-Linked Hyper-IgM Syndrome. Poster presentation at the 17th annual meeting of the American Society of Gene and Cell therapy, Washington DC. May 2014.

180. Kismet Baldwin, Fabrizia Urbinati, Zulema Romero, Michael Kaufman, Beatrice Campo-Fernandez, Sabine Geiger, Donald B. Kohn. Enrichment of Human Hematopoietic Stem/Progenitor Cells Increases Transduction Efficiency for Stem Cell Gene Therapy. Poster presentation at the 17th annual meeting of the American Society of Gene and Cell therapy, Washington DC. May 2014.

181. Katrin Hacke, Janet A. Treger, Brooke T. Bogan, Valerie Rezek, Munetoshi Narukawa, Saki Shimizu, Pei-Qi Liu, Andreas Reik, André M. Lieber, Gay M. Crooks, Donald B. Kohn, Dong Sung An, Scott G. Kitchen, Philip D. Gregory, Gregory J. Cost, Michael C. Holmes, Noriyuki Kasahara. Combined Preconditioning and In Vivo Chemoselection with 6-Thioguanine for Selection of Genetically Modified Hematopoietic Stem Cells. Poster presentation at the 17th annual meeting of the American Society of Gene and Cell therapy, Washington DC. May 2014.

182. Session Moderator “Update on Gene Therapy” and invited speaker “ADA-SCID and CGD.” Primary Immune Deficiency Treatment Consortium (PIDTC) Fourth Annual Scientific Workshop, Seattle WA. May 1 - 3, 2014.

183. Invited Speaker “Overview of Gene Therapy & Bone Marrow Transplantation for Primary Immune Deficiency Diseases.” Immune Deficiency Foundation Education Meeting. Anaheim CA. May 17, 2014.

184. Course Co-chair. American Society of Gene and Cell Therapy, Clinical Trials Training Course: Lab to Licensure. Washington DC, May 19-20, 2014.

185. Invited presentation. “Gene Therapy for Sickle Cell Disease.” In Scientific Symposium on Gene Therapy for Genetic & Metabolic Diseases. American Society of Gene and Cell Therapy, 17th annual meeting. Washington DC, May 21, 2014.

186. Invited presentation. “Gene Therapy for SCID – The American Perspective.” International Symposium on Gene Therapy vs. Haploidentical Stem Cell Transplantation- Concepts and Limitations. 40th anniversary of the University Medical Centre, Ulm Pediatric Stem Cell Transplantation Program. Ulm, Germany, May 31-June 1, 2014.

187. Invited Speaker. “Stem Cell Gene Therapy for Sickle Cell Disease.” Stem Cell Meeting on the Mesa. La Jolla, CA. October 8, 2014

188. Invited keynote speaker “Gene Therapy Using Hematopoietic Stem Cells.” Stem Cell Awareness Day Science Symposium, Sue & Bill Gross Stem Cell Research Center, University of California, Irvine. October 9, 2014

189. Invited Speaker – “Gene Therapy for Primary Immunodeficiency Disorders: Is Chronic Granulomatous Disease Ready for Prime-time?” Mini-symposium: New Developments in the Diagnosis, Treatment and Biology of Chronic Granulomatous Disease. Children’s Hospital Los Angeles. October 10, 2014

190. Invited speaker. UCLA Children's Discovery and Innovation Institute (CDI) Scientific Seminar Series 2014-2015**.** “Gene Therapy Using Hematopoietic Stem Cells” October 16th, 2014.

191. Invited Speaker - “Gene Therapy Using Hematopoietic Stem Cells”. Dept. of Pediatrics Grand Rounds, M.D. Anderson Cancer Center, Houston TX. October 20, 2014.

192. Invited Speaker - “Clinical Applications of Integrating Vectors for Gene Therapy” 5th International Conf. on Retroviral Integration, Asilomar, CA. October 26, 2014.

193. Invited Speaker – “Clinical Trial of Stem Cell Gene Therapy for Sickle Cell Disease.” CIRM and Johnson & Johnson Innovations symposium - Accelerating Stem Cell Treatments to Patients. San Francisco CA., November 5, 2014.

194. Invited Speaker - “Eliminating SCID Row – new approaches to SCID“ in Educational Session: Stemware: Stem Cell Therapy for Congenital Blood

Disorders. 56th ASH Annual Meeting & Exposition, San Francisco, CA., December 6-9, 2014,

195. Invited Speaker. “Gene Therapy for ADA-SCID and the Future of Gene Manipulation of Inherited Diseases”. The Biennial William T Shearer Innovations in Primary Immunodeficiency and Clinical Immunology Symposium. Texas Children’s Hospital. Houston TX, February 7, 2015.

196. Oral Presentation. “Autologous Transplant/Gene Therapy for Adenosine Deaminase-Deficient Severe Combined Immune Deficiency.” 2015 Pediatric BMT Program - Best Pediatric Abstracts session. BMT Tandem Meetings, San Diego CA, February 12, 2015.

197. Invited Presenter. “Gene therapy for adenosine deaminase deficient severe combined immune deficiency (ADA-SCID).” Clinical Immunology Society 2015 Annual Meeting, Houston TX, April 9, 2015.

198. Invited Speaker, UCLA Molecular Biology Institute Retreat 2015. “Stem Cell Gene Therapy for Blood Cell Diseases.” UCLA, April 25, 2015.

199. Invited Speaker, PAS Topic Symposium - Cord Blood Stem Cells: Biology, Banking and Future Pediatric Applications. “Cord Blood Stem Cell Therapy for neonates with Severe Combined Immunodeficiency Syndrome (SCID).” Pediatric Academic Societies Annual Meeting, San Diego CA, April 28, 2015.

200. Meeting Co-Organizer and Invited Speaker, 8th Stem Cell Clonality and Genome Stability Retreat, the TransAtlantic Gene Therapy Consortium. “Gene Editing for Sickle Cell Disease.” New Orleans, LA, May 11th, 2015.

201. Invited Speaker. USC/UCSF/UCLA Tri-Institutional Stem Cell Retreat. “Hematopoietic Stem Cell Gene Therapy.” Santa Barbara CA, May 17, 2015.

202. Invited Speaker. UC San Diego Gene Therapy Symposium Honoring Theodore Friedmann, MD. “Gene Therapy Using Hematopoietic Stem Cells.” University of California, San Diego, La Jolla CA, May 20, 2015.

203. Invited speaker. 2nd annual Children’s Discovery & Innovation Institute of UCLA, Child Health Research Symposium “Gene Therapy for Primary Immune Deficiency Diseases.” May 28, 2015.

204. Invited Speaker. 2015 American Association of Immunology - Course in Immunology. “Genetic Approaches to Immune-Mediated Diseases.” Long Beach CA, July 19, 2015.

205. Invited Speaker. Hematology and Hematopoietic Cell Transplantation Program Seminar, City of Hope Medical Center. “Gene Therapy Using Hematopoietic Stem Cells”. Duarte CA, July 14, 2015.

206. Invited Speaker. Jordan Family Lectureship Series at Children's Hospital Oakland Research Institute. "Gene Therapy Using Hematopoietic Stem Cells". Oakland CA, August 26, 2015.

207. Invited Speaker. 11th Annual Symposium on Primary Immunodeficiency Diseases. "Current Status of Gene Therapy for Primary Immune Deficiency Diseases." Newport Beach CA, October 4, 2015.

208. Invited Speaker. UCLA Broad Stem Cell Research Center (BSCRC) Cell Seminar. "Hematopoietic Stem Cell Gene Therapy. Los Angeles CA, October 8, 2105.

209. Invited Keynote Speaker. Stem Cell Meeting on the Mesa -10th Annual Scientific Symposium. "Hematopoietic Stem Cell Gene Therapy." La Jolla, CA Oct. 9, 2015.

210. Invited Speaker. AABB Annual Meeting 2015 – Scientific Session: Genome Editing in Regenerative Medicine. "Beta-Globin Gene Correction in HSC for Sickle Cell Disease." Anaheim, CA October 24, 2015.

211. Invited Speaker. The Stem Cell Niche and Cancer Microenvironment. "Stem Cell Gene Therapy for Sickle Cell Disease." Cedars-Sinai Medical Center, Los Angeles CA November 14, 2015.

212. Invited Speaker. Life Science Workshop Series 2015. Bayer Health Care. "Gene Therapy Using Hematopoietic Stem Cells." San Francisco, CA November 18, 2015.

213. Invited Speaker. American Academy of Allergy, Asthma and Immunology 2016 Annual Meeting. Symposium – What Do I Do with These Abnormal Newborn Screening Results. "Transplantation Options and Novel Therapies." Workshop – "Gene Therapy as a Treatment of Primary Immune Defects." Los Angeles, CA March 6-7, 2016.

214. Keynote Speaker, CELL-PID and SCID-NET Annual Workshops. "Gene Therapy for Primary Immunodeficiencies: Results from the USA Experience." Franciacorta, Italy, March 13, 2106.

215. Invited Speaker. UC San Diego Division of Regenerative Medicine & CIRM Alpha Stem Cell Clinics Network, 2nd Annual Symposium. "Hematopoietic Stem Cell Gene Therapy." La Jolla, CA March 17, 2016.

216. Invited lecturer. SCRM 515: Bringing Stem Cells to the Clinic. "Gene Therapy Using Hematopoietic Stem Cells". Keck School of Medicine of USC. Los Angeles March 30, 2016.

217. Invited Speaker. Clinical Immunology Society 2016 Annual Meeting. Plenary Session: Gene Therapy/Editing. "Gene Therapy for ADA-Deficient SCID and Chronic Granulomatous Disease." Boston, MA. April 15, 2016.

218. Invited Plenary Speaker and Session Chair. Fourth Annual PBMTC/ASPHO Educational Meeting: New Frontiers in Allogeneic Stem Cell Transplant. “Gene therapy for ADA-Deficient SCID and XCGD.” Minneapolis MN May 11, 2016.

219. Meeting Organizer, Primary Immune Deficiency Treatment Consortium 6th Annual Scientific Workshop and Education Day. Marina del Rey, CA May 18-21, 2016.

220. Invited Speaker, Jeffrey Modell Foundation: Celebrating 30 Years of Hope, Advocacy and Action. “Gene Therapy for Primary Immunodeficiencies.” Beverly Hills, CA. June 10, 2016.

221. Meeting Co-Organizer and Invited Speaker, ISSCR/ASGCT Workshop on Clinical Translation. “Stem Cell Therapy for ADA/SCID.” San Francisco, CA. June 21, 2016.

222. Oral Presentation, Concurrent Session IV: Road to the Clinic. ISSCR Annual Meeting. “HSC Gene Therapy for ADA-Deficient SCID.” San Francisco, CA. June 25, 2016.

223. Invited presentation. “Gene Therapy Using Hematopoietic Stem Cells.” AMGEN, Thousand Oaks CA. August 19, 2016.

224. Invited Speaker. International Society of Experimental Hematology 45th Annual Scientific Meeting. “Gene Therapy for Blood Cell Diseases with Autologous Hematopoietic Stem Cells.” San Diego CA, August 26, 2016.

225. Invited Speaker. “Gene Therapy Using Hematopoietic Stem Cells.” University of Texas South Western, Department of Pediatrics Grand Rounds. Dallas TX, September 14, 2016.

226. Invited Speaker. “Bone Marrow Transplant and Gene Therapy for Sickle Cell Disease.” Fifth Annual West Coast Sickle Cell Nurses Conference. Children’s Hospital Los Angeles. September 22, 2016.

227. Invited Plenary Speaker. “Gene Therapy for Sickle Cell Disease.” International Society for Cellular Therapy North America 2016 Regional meeting. Memphis TN, September 30, 2016.

228. Meeting Co-organizer and Session Chair, 9th Stem Cell Clonality and Genome Stability Retreat. Florence, Italy, October 17-18, 2016.

229. Invited Plenary Presentation: “Gene Therapy for ADA SCID and XCGD.” European Society of Gene and Cell Therapy, Florence, Italy, October 20, 2016.

230. Invited Speaker, “Gene Therapy Using Hematopoietic Stem Cells.” U.C.L.A. Division of Pediatric Hematology/Oncology Research Seminar, Los Angeles CA, November 29, 2016.

231. Invited Speaker, “Gene Editing in Human Hematopoietic Stem Cells.” *Developing Gene Editing as a Therapeutic Strategy*. Banbury Center, Cold Spring Harbor Laboratory, Cold Spring Harbor, NY December 11-14, 2016.

232. Invited Speaker, “Hematopoietic Stem Cell Gene Therapy: Progress and Challenges” at Transforming Medicine: Innovation and Discovery at The Broad Stem Cell Research Centers. UCLA 13th Annual Stem Cell Symposium. Los Angeles CA, February 3, 2017

233. Invited Speaker, “Hematopoietic Stem Cell Gene Therapy: Progress and Challenges”. UCLA Children’s Discovery and Innovation Institute - Scientific Seminar Series. Los Angeles CA, February 16, 2017.

234. Invited Lecturer. “Hematopoietic Stem cell Gene Therapy: from Concept to Licensure.” In “Bringing Stem Cell to Clinics" SCRM 515. Department of Stem Cell Biology and Regenerative Medicine at the Keck School of Medicine of USC. April 85, 2017.

235. Invited Feature Speaker. “*Ex vivo* Gene Therapy Using Hematopoietic Stem Cells” MaxCyte Dinner Symposium at ASGCT - Cell & Gene Therapy Insights. Washington, D.C., May 10, 2017.

236. Invited Presentation. “Update on Gene Therapy for ADA SCID.” Primary Immune Deficiency Treatment Consortium. 7th Annual Scientific Workshop. Bethesda MD May 25, 2017.

237. Invited Presentation. “Advances in Primary Immunodeficiency: Is It All In The Genes?” Immune Deficiency Foundation 2017 National Conference. Anaheim CA, June 16, 2017.

238. Invited Presentation. “Gene Therapy for Adenosine Deaminase Deficient SCID.” In Special SCID and SCID Variant Symposium. Immune Deficiency Foundation 2017 National Conference. Anaheim CA, June 16, 2017.

239. Invited Presentation. “Update on Bone Marrow transplantation and Gene Therapy for Primary Immune Deficiency Diseases.” Clinical Immunology Society. Update in Primary Immune Deficiency for the Practicing Clinician. Anaheim CA, June 17, 2017.

240. Invited Speaker. “Medical Applications of CRISPR.” 2017 CRISPR Workshop: Practical Aspects of Precision Biology. UC Berkeley/UCSF. Berkeley CA, July 14, 2017.

241. Invited Speaker. “Genetic Approaches to Immune-Mediated Diseases.” American Association of Immunologists - Introductory Course in Immunology. Los Angeles, CA, July 16, 2017.

242. Invited Plenary Speaker. “Use of Gene Editing to Treat Inherited Disease.” Asian Society for Pediatric Research. Aberdeen, Hong Kong, October 6, 2017.

243. Invited Speaker. “Gene Therapy for Adenosine Deaminase (ADA) Deficient Severe Combined Immunodeficiency (SCID). Asian Society for Primary Immune Deficiencies. Aberdeen, Hong Kong, October 6, 2017.

244. Invited Speaker: State of the Clinical Science and Case Study – Hematology. Workshop on Innovation in Regenerative Medicine: Focus on Adult Stem Cells. NIH/FDA. Bethesda MD, December 6-7, 2017.

245. Invited Speaker. “Gene Therapy Approaches to Sickle Cell Disease.” UCLA Molecular Biology Institute Interdisciplinary Program annual retreat. Ventura CA, March 17, 2018.

246. Invited Speaker. National Institute of Allergy and Infectious Diseases, NIH Grand Rounds. “Gene Therapy for Adenosine Deaminase-Deficient Severe Combined Immune Deficiency.” Bethesda MD, April 6, 2018.

247. Invited Keynote Speaker. SCIDNet annual meeting. “Gene Therapy for ADA SCID.” London, UK, April 9, 2018.

248. Invited Speaker. Genethon. “Gene Therapy for Sickle Cell Disease” Evry, France, April 11, 2018.

249. Invited Keynote Speaker. Net4CGD annual investigators meeting. “The U.S. Trial of Gene Therapy for XCGD.” Evry, France, April 12, 2018.

250. Meeting Organizer, International Society of Cellular Therapy (ISCT); Plenary Session Chair: “Gene Editing in Hematopoietic Stem Cells”; Invited Speaker, Breakout session “Gene Editing in Hematopoietic Stem Cells”, Montreal Canada, May 5, 2018.

251. Invited presentation, “Hematopoietic Stem Cell Gene Therapy for IPEX Disease.” Primary Immune Deficiency Workshop. Philadelphia PA, May 10, 2018.

252. Invited presentation, “Gene Therapy for X-linked Chronic Granulomatous Disease”. Clinical Trials Spotlight session, American Society of Gene and Cell Therapy 21st Annual Meeting. Chicago IL, May 17, 2018.

Posters at the Clinical Immunology Society Annual Meeting 2018:

Kuo, CY, Puck, JM, Logan BR, Haddad, E, Cuvelier, GDE, Yin Z, Prockop SE, Buckley, R, Griffith LM, and **Kohn DB**. Adenosine Deaminase (ADA)-Deficient Severe Combined Immune Deficiency (SCID): Analysis of Cases Enrolled in Protocols of the Primary Immune Deficiency Treatment Consortium (PIDTC). J Clin Immunol 38:342, 2018.

Kohn DB, Shaw KL, Garabedian E, Carbonaro-Sarracino DA, Moore TB, De Oliveira S, Crooks GM, Tse J, Shupien S, Terrazas D, Davila A, Icreverzi A, Yu A, Barman P, Coronel M, Datt J, Campo B, Hollis R, Reeves L, Cornetta K, Sokolic R, Thrasher A, Gaspar HB, and Candotti F. Gene Therapy for Adenosine Deaminase-Deficient Severe Combined Immunodeficiency (ADA SCID) with a Lentiviral Vector. J Clin Immunol 38:364, 2018.

253. Keynote Address. "Gene Editing in Hematopoietic Stem Cells." FASEB Conference - *Genome Engineering: Cutting Edge Research and Applications*. Florence, Italy, June 27, 2018.

254. Invited presentation: "Gene Therapy for Sickle Cell Disease". University of California Hematological Malignancies Consortium Annual Meeting. Irvine CA, September 9, 2018.

255. Invited presentation: "Gene Therapy", 17th Annual Science Forum-Impactful New Science for Oncologists: Artificial Intelligence, Gene Therapies, New Agents, Diabetes and Cancer, Immunology And Your Immune Bank. U.S. Oncology. Denver CO, September 14, 2018.

256. Invited presentation Jeffrey Modell Pediatric Translational Immunology Grand Rounds, "Gene Therapy for Primary Immune Deficiencies" Department of Pediatrics Grand Rounds, Washington University School of Medicine, St. Louis MO, October 5, 2018.

257. Invited presentation "Gene Therapy Using Hematopoietic Stem Cells". Award for I3T Research Excellence. Infectious Diseases or Transplantation (I3T) Research Program, David Geffen School of Medicine at UCLA. Los Angeles CA, October 23, 2018.

258. Invited Speaker. *Cell* Symposium: Translation of Stem Cells to the Clinic: Challenges and Opportunities. "Gene Therapy Using Lentiviral Vectors." Los Angeles CA. December 3, 2018.

259. Invited speaker. Presidential Symposium: "Completing the Arc in Genomic Therapies", 60th annual meeting of the American Society of Hematology. *"Translating Science to Therapy: HSC Gene Therapy for ADA SCID.*" San Diego CA. December 4, 2018.

260. Invited presentation. Presidential Symposium: "Gene Therapy for ADA SCID", Transplantation & Cellular Therapy Meetings of ASBMT & CIBMTR 2019. "*ASBMT President's Symposia: Cell and Gene Therapy: The Next Big Challenges*." Houston TX, February 22, 2019.

261. Oral Presentation – Late Breaking Abstract Session: "Effective Lentiviral Gene Therapy for X-Linked Chronic Granulomatous Disease (X-CGD)", Transplantation & Cellular Therapy Meetings of ASBMT & CIBMTR 2019. Houston TX, February 24, 2019.

262. Invited Speaker "Adding and Editing Genes in Hematopoietic Stem Cells", 13th Colloque de l'Association de Therapie Genique du Quebec. Montreal, Canada March 14, 2019.

263. Invited Speaker, Cancer and Blood Diseases Institute Seminar Series, Cincinnati Children's Hospital Medical Center. "Hematopoietic Stem Cell Gene Therapy." Cincinnati OH, Apr. 2, 2019.

Posters at Clinical Immunology Society Annual Meeting, Atlanta GA, April 5, 2019:

1. Kohn, DB, Shaw, KL, Garabedian EK, Carbonaro-Sarracino, DA, Moore TB, De Oliveira S, Crooks GM, Tse J, Shupien S, Terrazas D, Davila A, Icreverzi A, Yu A, Barman P, Coronel M, Campo Fernandez B, Zhang R, Hollis R, Uzowuru C, Ricketts H, Xu0Bayford J, Trevisam V, Arduni S, Lynn F, Kudari M, Spezzi A, Reeves L, Cornetta K, Sokolic R, Parrott R, Buckley R,

Booth C, Candotti F, Malech HL, Thrasher AJ and Gaspar HB. Autologous ex vivo lentiviral gene therapy for the treatment of severe combined immune deficiency due to adenosine deaminase deficiency.
2. Kohn, DB, Shaw, KL, Garabedian EK, Carbonaro-Sarracino, DA, Moore TB, De Oliveira S, Crooks GM, Tse J, Shupien S, Terrazas D, Davila A, Icreverzi A, Yu A, Barman P, Coronel M, Campo Fernandez B, Zhang R, Hollis R, Uzowuru C, Ricketts H, Xu0Bayford J, Trevisam V, Arduni S, Lynn F, Kudari M, Spezzi A, Reeves L, Cornetta K, Sokolic R, Parrott R, Buckley R, Booth C, Candotti F, Malech HL, Thrasher AJ and Gaspar HB. Autologous ex vivo lentiviral gene therapy for the treatment of severe combined immune deficiency due to adenosine deaminase deficiency improves B cell function.

264. Roundtable Breakout Session Chair and Presenter: "Gene Therapy". 2019 Annual meeting: Immune Deficiency & Dysregulation North American Conference, Atlanta GA April 5, 2109.

265. Invited keynote presentation: "Hematopoietic Stem Cell Gene Therapy for Blood Cell Disorders" at 4th Annual CIRM Alpha Stem Cell Clinics Network Symposium: Mending Stem Cells: The Past, Present & Future of Regenerative Medicine, San Francisco CA, April 18, 2019.

266. Invited keynote presentation: "Gene Editing for Primary Immune Deficiency Diseases". Primary Immune Deficiency Treatment Consortium, 9th Annual Scientific Workshop 2019. New York City, NY, May 17, 2019.

267. Invited presentation: "Editing Genes Using CRISPR", Southern California Genetic Counselors, 2nd Annual Education Conference: Genetics in the Digital Age: Expansions in Diagnosis, Treatment, and Social Response. Los Angeles, CA, June 7, 2019

268. Invited speaker: "Gene Therapy for Primary Immune Deficiency Diseases". The Immunology, Infection, Inflammation and Translation (I3T) Research Theme, UCLA David Geffen School of Medicine, Annual Scientific Retreat, Los Angeles CA, June 121, 2019

269. Invited presentation: "Gene Editing in Hematopoietic Stem Cells", Cellular Therapies Symposium II: Engineering the Future. UCLA David Geffen School of Medicine, the Division of Pediatric Hematology/Oncology. Los Angeles CA, June 13, 2019

270. Invited lecture; "Genetic Approaches to Immune-Mediated Diseases." American Association of Immunologists (AAI) Introductory Course in Immunology. UCLA Luskin Conference Center, Los Angeles, CA, July 14, 2019.

271. Invited presentation: "Hematopoietic Stem Cell Gene Therapy for SCID." California Institute for Regenerative Medicine, 2019 CIRM Bridges Trainee Meeting, San Mateo CA, July 15, 2019.

272. Invited Plenary Presentation: "Haematopoietic Stem Cell Gene Therapy for Sickle Cell Disease. At ESGCT 27th Annual Congress, "Gene Therapy Clinical Trials session. Barcelona Spain, October 24, 2019.

273. Invited Speaker and Session Chair, "Hematopoietic Stem Cell Gene Therapy". UCLA Dept. of Molecular & Medical Pharmacology, Annual Retreat, Huntington Beach CA Nov. 2, 2019.
Globin Lentiviral Vectors:

274. Invited Speaker: “Gene Therapy for Primary Immune Deficiencies”. At 15th Annual International Symposium on Primary Immunodeficiency: Advances in Molecular Diagnosis and Treatment. Newport Beach, CA November 16, 2019.

275. Invited Speaker “Introduction to Lentiviral Vectors”. At Think Tank for *In Utero* Gene Therapy. San Francisco CA November 18, 2019.

276. Invited Speaker, Session Chair “The Challenges of β-Globin Lentiviral Vectors”. American Society of Hematology (ASH) 61st Annual Meeting - Friday Scientific Workshop on Novel Curative Options: Gene Editing and Gene Therapy for Hemoglobinopathies with a Focus on Sickle Cell Disease. Orlando FL, November 6, 2019.

277. Kohn DB, Shaw KL, Garabedian E, Carbonaro-Sarracino DA, Moore TB, De Oliveira S, Crooks GM, Tse J, Shupien S, Terrazas D, Davila A, Icreverzi A, Yu A, Chun KM, Casas CE, Barman P, Coronel M, Campo Fernandez B, Zhang R, Hollis RP, Uzowuru C, Ricketts H, Bayford JX, Trevisan V, Arduini S, Lynn F, Kudari M, Spezzi A, Reeves L, Cornetta K, Sokolic R, Parrott R, Buckley R, Booth C, Candotti F, Malech HL, Thrasher AJ, and Gaspar HB. Lentiviral Gene Therapy with Autologous Hematopoietic Stem and Progenitor Cells (HSPCs) for the Treatment of Severe Combined Immune Deficiency Due to Adenosine Deaminase (ADA-SCID): Results in and Expanded Cohort. Blood 134 (suppl):3345, 2019. American Society of Hematology (ASH) 61st Annual Meeting, Orlando FL, November 6, 2019.

278. Invited Speaker,”Challenges to Developing Individualized Stem cell Gene Therapies”. US Food and Drug Administration, Center for Biologics Evaluation and Research, *Facilitating End-to-End Development of Individualized Therapeutics.* Silver Spring MD, March 3, 2020.

279. Donald Kohn, Gayatri Rao, Elena Almarza, Dayna Terrazas, Eileen Nicoletti, Augustine Fernandes, Caroline Kuo, Satiro De Oliveira, Theodore Moore, Ken Law, Brian Beard, Julian Sevilla, 10, Christina Mesa. A Phase 1/2 Study of Lentiviral-mediated Ex-vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Initial Results from the First Treated Patient J Clin Immunol (2020) 40 (Suppl 1):S1–S163. Presented at Clinical Immunology Society Annual Meeting, 2020.

280. Meeting abstract selected for presentation: Kohn DB, Rao G, Almarza E, Terrazas D, Nicoletti E, Ferndandes A, Kuo C, De Oliveira S, Moore T, Law K, Beard B, Sevilla J, Mesa-Nunez C, Bueren J and Schwartz J. A Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Initial Results from the First Treated Patient. Mol Ther 28 (4S1):56, 2020.

281. Meeting abstract selected for presentation: Kohn DB, Shaw KL, Garabedia E, Carbonaro DA, Moore TB, De Oliveira SN, Crooks GM, Tse J, Shupien S, Terrazas D, Davila A, Icreverzi A, Yu A, Chun K, Casas CE, Barman P, Coronel M, Campo Fernandez B, Zhang R, Hollis RP, Bayford JX, Trevisan V, Arduini S, Lynn F, Kudari M, Uzowuru C, Ricketss H, Spezzi A, Reeves L, Corentta K, Sokolic RA, Parrott R, Buckley R, Booth C, Candotti F, Malech HL, Thrasher AJ, and Gaspar HR. Lentiviral Gene Therapy with Autologous Hematopoietic Stem and Progenitor Cells (HSPCs) for the Treatment of Severe Combined Immune Deficiency Due to Adenosine Deaminase Deficiency (ADA-SCID): Two Year Follow-Up Results. Mol Ther 28 (4S1):554, 2020.

282. Invited Speaker, “In Vivo Targeting and Gene Modification of HSCs: Lessons from Ex Vivo Targeting of HSC.” NIH-Bill and Melinda Gates Foundation Scientific Roundtable Meeting –

Safe and Effective In Vivo Targeting and Gene Editing in Hematopoietic Stem cells: Strategies for Accelerating Development. May 11, 2020.

283. Session Co-Chair. "Growing Gene and Cell Therapy (GGACT): An NCATS-funded Collaborative Effort to Support Development of Academic Clinical Trials for Rare Diseases." American Society of Gene and Cell Therapy 23rd Annual Meeting. (virtual) Boston MA, May 12, 2020.

284. Oral Presentation. "A Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Initial Results from the First Treated Patient." HSPC Gene Therapies for Blood and Immune Disorder session. American Society of Gene and Cell Therapy 23rd Annual Meeting. (virtual) Boston MA, May 12, 2020.

285. Oral Presentation "Lentiviral Gene Therapy with Autologous Hematopoietic Stem and Progenitor Cells (HSPCs) for the Treatment of Severe Combined Immune Deficiency Due to Adenosine Deaminase Deficiency (ADA-SCID): Two Year Follow-Up Results" Clinical Trials Spotlight Session. American Society of Gene and Cell Therapy 23rd Annual Meeting. (virtual) Boston MA, May 12, 2020.

286. Invited Plenary Speaker: International Society Cell & Gene Therapy (ISCT): Accelerating Cell & Gene Therapy Adoption: Proof of Concept to Standard of Care. Plenary Session – Gene Engineering: The Past, Present, and The Future. "Hematopoietic Stem Cell Gene Therapy." May 28-29 2020 (virtual).

287. Invited Speaker. CIRM Grantee Meeting 2020. "Hematopoietic Stem Cell Gene Therapy for Primary Immune Deficiencies." UC Irvine, Irvine CA. September 14, 2020 (virtual).

288. Invited Keynote Speaker. Fanconi Anemia Research Fund Scientific Symposium. Hematopoietic Stem cell Gene Therapy. September 16, 2020 (virtual).

289. Invited Speaker. USC Department of Medicine Visiting Speaker Series. Hematopoietic Stem Cell Gene Therapy. USC Keck School of Medicine. September 18, 2020 (virtual).

290. Invited speaker. UCLA Division of Pediatric Hematology/Oncology Seminar. "Severe Combined Immune Deficiency". September 22, 2020 (virtual).

291. Invited keynote Speaker, Immune Deficiency Foundation. Rare of the Rare Summit. "Gene Therapy for Primary Immunodeficiency: Past, Present and Future. September 27, 2020 (virtual).

292. Invited speaker. CIRM Alpha Stem Cell Clinic Network Virtual Symposium. "Severe Combined Immunodeficiency (SCID)." October 8, 2020 (virtual).

293. Oral abstract presentation. "A phase ½ study of lentiviral-mediated ex vivo gene therapy for pediatric patients with severe Leukocyte Adhesion Deficiency-I (LAD-I): Results from Phase I." 19th biennial meeting of the European Society for Immunodeficiences (ESID 2020 Online meeting). October 16, 2020. (virtual).

294. Invited speaker. "Gene editing in hematopoietic stem cells". World CRISPR Day Symposium (Synthego). October 20, 2020 (virtual).

295. Oral abstract presentation. “A phase ½ study of lentiviral-mediated ex vivo gene therapy for pediatric patients with severe Leukocyte Adhesion Deficiency-I (LAD-I): Results from Phase I.” 62nd annual meeting of the American Society of Hematology (ASH) Online meeting). December 7, 2020. (virtual).

296. Invited presentation. Gene Therapy Using Hematopoietic Stem Cells. Center for Cellular Gene Therapy at Johns Hopkins All Children’s Hospital, St. Petersburg FL, January 20, 2021 (virtual).

297. Invited lecture. “Hematopoietic Stem Cell Gene Therapy for Blood Cell Disease.” USC Stem Cell Translational Research Workshop. February 4, 2021 (virtual).

298. Invited presenter. “Gene therapy for ADA SCID.” Concurrent session: Gene Therapy for Non-Malignant Diseases. 2021 Transplantation & Cellular Therapy Meeting of ASTCT and CIBMTR. Thursday February 11, 2021 (virtual).

299. Meeting Co-Organizer and Invited Speaker. “Correcting the Sickle Mutation in Beta-Globin in HSC- How Hard Can That Be?” Sickle Cell Disease: What’s New with Transplant and Gene Therapy? Sponsored by the Doris Duke Charitable Foundation. St. Jude Children’s Research Hospital. Memphis TN March 2, 2021 (virtual).

300. Invited speaker. “Gene therapy for SCID.” Immune Deficiency Foundation, SCID Compass Lunch & Learn Series. March 19, 2021 (virtual).

301. Donald Kohn, Omar Habib, Bryanna Reinhardt, Kit Shaw, Elizabeth Garabedian, Dayna Terrazas, Beatriz Campo Fernandez, Satiro De Oliveira, Theodore Moore, Alan Ikeda, Barbara Engel, Gregory Podsakoff, Roger Hollis, Augustine Fernandes, Connie Jackson, Sally Shupien, Suparna Mishra, Alejandra Davila, Jack Mottahedeh, Andrej Vitomirov, John Everett, Aoife Roche, Pascha Hokama, Shantan Reddy, Xiaoyan Wang, Kenneth Cornetta, Michael Hershfield, Robert Sokolic, Harry Malech, Frederick Bushman, Fabio Candotti, Long-term Outcomes after Gene Therapy for Adenosine Deaminase Severe Combined Immune Deficiency ADA SCID. Oral presentation at 12th Annual Meeting of the Clinical Immunology Society: 2021 Virtual Annual Meeting: Immune Deficiency and Dysregulation North American Conference. April 16, 2021 (virtual).

302. Invited Speaker. “Hematopoietic Stem Cell Gene Therapy for Primary Immune Deficiencies.” Cell Therapy Transplant Canada (CTTC), (virtual) Annual Meeting June 16, 2021

303. Invited Speaker. “Hematopoietic Stem Cell Gene Therapy.” Internal Medicine Grand Rounds, UCLA David Geffen School of Medicine. July 28, 2021.

304. Invited Speaker. “Hematopoietic Stem Cell Gene Therapy”. Miltenyi Biotec Webinar. Thursday November 4, 2021 (virtual).

305. Selected for Best Abstracts oral presentation. “A Phase I/II study of lentiviral-mediated ex vivo gene therapy for pediatric patients with severe Leukocyte Adhesion Deficiency-I (LAD-I). Primary Immune Deficiency Treatment Consortium - Tenth Annual Scientific Workshop. Asilomar CA, November 16, 2021

306. Invited Speaker. Hematopoietic Stem Cell Gene Therapy. UCLA Undergraduate Science Journal Group. February 9, 2022 (virtual).

307. Invited presentation. “Primary Immune Deficiencies and Gene Therapy.” Pediatric Potpourri. Maui Hawaii, March 2, 2022.

308. Invited Speaker. “Evolution of the Field of Gene Therapy.” Future Directions and Resource Needs for NHLBI Gene Therapy Research. March 15, 2022 (virtual).

309. Meeting Co-Organizer and Co-Chair. “Sickle Cell Disease: What’s New with Transplant and Gene Therapy”. DDCF/St. Judes/UCLA. March 24, 2022. (virtual).

310. Invited Keynote Speaker. “Hematopoietic Stem Cell Gene Therapy for Primary Immune Deficiencies”. Webinar: “Looking at gene therapy for XLA” March 29, 2022 (virtual)

311. Plenary Presentation. American Society of Gene and Cell Therapy - Outstanding Achievement Award. Hematopoietic Stem Cell Gene Therapy for Severe Combined Immune Deficiency. ASGCT 25th Annual Meeting. Washington DC, May 18, 2022.

312. Clinical Trial Spotlight presentation. “Interim Results: A Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I)”. ASGCT 25th Annual Meeting. Washington DC, May 19, 2022.

Abstracts from lab at ASGCT 2022 meeting:

Treating Sickle Cell Disease with Lentiviral Vectors Combining an Anti-Sickling βAS3-Globin Gene with BCL11A and ZNF410 MicroRNA Adapted Short Hairpin RNAs Kevyn L. Hart, Boya Liu, Roger P. Hollis, Christian Brendel, David A. Williams, **Donald B. Kohn**

Quantification of DNA Contamination within Recombinant Adeno Associated Virus Preps Correlates with CD34+ Cell Potential. Christopher Luthers, Zulema Romero, Daniel Ha, **Donald B. Kohn**

Base Editing of Hematopoietic Stem Cells Rescues T-Cell Development for CD3δ Severe Combined Immunodeficiency. Grace E. McAuley, Gloria Yiu, Gregory A. Newby, Sung Hae L. Kang, Amber J. Garibay, Jeffrey A. Butler, Valentina S. Christian, Sorel Fitz-Gibbon, Ryan L. Wong, Kelcee A. Everette, Zulema Romero, Nicola Wright, David R. Liu, Gay M. Crooks, **Donald B. Kohn**

Bioinformatic-Guided Design of a Lentiviral Vector for X-Linked Chronic Granulomatous Diseases Recapitulates Endogenous CYBB Gene Regulation and Expression. Ryan L. Wong, Sarah Sackey, Devin Brown, Katelyn Masiuk, Shantha Senadheera, Jason P. Quintos, Richard A. Morgan, Harry Malech, Roger P. Hollis, **Donald B. Kohn**

Low-Cost Fluorosilane-Modified Filtroporation Devices Enabling Gene Knockout in Human Hematopoietic Stem and Progenitor Cells. Isaura Maia Frost, Alexandra Mendoza, Tzu-Ting Chiou, Philseok Kim, Joanna Aizenberg, **Donald B. Kohn**, Satiro De Oliveira, Paul S. Weiss, Steven J. Jonas

Interim Results from an Ongoing Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I). **D. B. Kohn**, J. Sevilla, G. Rao, M. Chitty Lopez, E. Almarza, D. Terrazas, J. Zubicaray, M. González-Vicent, K. Chetty, G. O’Toole, J. Xu-Bayford, E. Nicoletti, A. Fernandes, C. Kuo, S. de Oliveira, T. B. Moore, G. Choi, M. Zeini, C. Mesa-Núñez, A. J. Thrasher, J. Bueren, J. Schwartz, C. Booth

313. Invited presentation. “Gene therapy for primary immune deficiencies.” Clinical Immunology Network – Canada. Nova Scotia, Canada. June 20, 2022 (virtual).

314. Keynote Presentation. “Hematopoietic Stem Cell Gene Therapy”. *Regenerative Medicine 2022* - 2022 - Science Day at The Saban Research Institute (TSRI) of Children’s Hospital Los Angeles (CHLA). Los Angeles CA, June 30, 2022.

315. Invited presentation. “Gene editing in hematopoietic stem cells for monogenic disorders”. Genome Engineering: CRISPR Frontiers, Cold Spring Harbor Laboratory, Cold Spring Harbor, New York, August 25, 2022.

316. Invited presentation. “Hematopoietic Stem Cell Gene Therapy.” NIH Hematology Conference. NIH, Bethesda MD. September 14, 2022 (virtual).

317. Invited presentation. “Update on Gene Therapy for ADA SCID.” Immune Deficiency Foundation SCID Compass Lunch and Learn. September 14, 2022 (virtual).

318. Keynote presentation. “Hematopoietic Stem Cell Gene Therapy.” ISSCR/ASGCT Madison International Symposium: Emerging therapies at the Intersection of Genetic and Cellular Therapies. Madison WI September 21, 2022.

319. Invited presentation. “Hematopoietic Stem Cell Gene Therapy”. Dr. E. Donnall Thomas Symposium “Advancing Transplantation, Gene and Cell Therapy”. Seattle, WA Sept 30, 2022.

320. Invited plenary presentation. “Interim Results from an Ongoing Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I).” 29th annual meeting of the European Society of Gene and Cell Therapy. Edinburgh UK, October 12, 2022.

321. Invited speaker. “Hematopoietic Stem Cell Gene Therapy for ADA SCID – Promises and Challenges.” Innovative Genomics Institute- Affordability Task Force October Convening. Berkeley CA October 20, 2022.

322. Invited speaker. “Hematopoietic Stem Cell Gene Therapy.” UCLA Division of Pediatric Hematology/Oncology weekly conference. UCLA October 25, 2022.

323. Invited Speaker: “Gene Therapy for Primary Immune Deficiencies,” Cleveland Clinic Foundation – Basic Immunology Course. March 7, 2023 – virtual.

324. Invited Speaker. USC Broad Stem cell Center, Masters of Science in Stem Cells course. “Hematopoietic Stem Cell Gene Therapy”. USC Keck School of Medicine, March 8, 2023).

325. Best Abstract Awardee presentation “ Primary Immune Deficiency Treatment Consortium Scientific Workshop, Cincinnati OH, April 19, 2023.

326. Invited presentation “The Landscape of Gene Therapy for Inborn Errors of Immunity.” Session co-chair: Challenges in Translating Gene Therapy Clinical Trials into Standard Treatments for Inborn Errors of Immunity. Primary Immune Deficiency Treatment Consortium Scientific Workshop, Cincinnati OH, April 19, 2023.

327. Oral presentation from submitted abstract “Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Interim Results from An Ongoing Phase 1/2 Study.” European Bone Marrow Transplant (EBMT) 49th annual meeting, Paris France, 25 April 2023.

328. Invited presentation “Overview of Gene Therapy in Inborn Errors of Immunity” in session on Gene therapy in Inborn errors. European Bone Marrow Transplant (EBMT) 49th annual meeting, Paris France, 25 April 2023.

329. Invited Presentation “Hematopoietic Stem Cell Gene Therapy”. At Miltenyi Biotec Industry Sponsored Symposium, 26th annual meeting of the American Society of Gene and Cell Therapy. Los Angeles CA May 17, 2023.

330. Abstracts at 26th Annual Meeting of the American Society of Gene & Cell Therapy. Los Angeles CA May 2023.

1. Universal Survival and Superior Immune Reconstitution after Lentiviral Gene Therapy with Low Dose Conditioning for X-linked SCID (SCID-X1). Sung-Yun Pai, Claire Booth, **Donald B. Kohn**, Myriam A. Armant, Susan E. Prockop, Karen Buckland, Sharat Chandra, Shanmuganathan Chandrakasan, Kritika Chetty, Colleen H. Dansereau, Satiro N. De Oliveira, John Everett, Pei-Chi Kao, Wendy B. London, Rebecca A. Marsh, Theodore B. Moore, Nisha Nagarsheth, Suhag Parikh, Dayna R. Terrazas, Shanna White, Jinhua Xu-Bayford, Axel Schambach, Frederic D. Bushman, Adrian J. Thrasher, David A. Williams

2. Engineering Stage-Specific Developmental Cues to Generate Non-Allogenic, iPSC-Derived CAR T Cells for Immunotherapy. Sang Pil Yoo1, Claire Engstrom1, Suwen Li1, Patrick Chang1, Xuegang Yuan1, Ralph Valentine Crisostomo1, Christopher S. Seet2, **Donald B. Kohn**1, Gay M. Crooks1

3. Alpha Globin Lentiviral Vectors for Hematopoietic Stem Cell Gene Therapy of Alpha-Thalassemia. Eva E. R. Segura Gensler1, Kevin Tam1, Beatriz Campo1, Tippi C. MacKenzie2, Georgia Gregori3, Roger P. Hollis1, **Donald B. Kohn**4.

4. Lentiviral Vectors with Regulated Expression to Treat X-Linked Lymphoproliferative Disease. Paul G. Ayoub1, Julia Gensheimer1, Kevin T. Tam1, Lindsay E. Lathrop1, Roger P. Hollis1, Gay M. Crooks2, **Donald B. Kohn**1

4. A Lipid Nanoparticle Toolkit to Correct Cystic Fibrosis in Airway Stem Cells. Ruth Foley1, Paul Ayoub, Emily Duggan, Josh Khorsandi, Diego Banuet, Arunima Purkayastha, **Donald B. Kohn**, Brigitte Gomperts, Steven J. Jonas

6. Autologous Ex-Vivo Lentiviral Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Interim Results from an Ongoing Phase 1/2 Study. **Donald B. Kohn**, Julián Seville, Gayatri Rao, Maria Chitty Lopez, Elena Almarza, Dayna Terrazas, Josune Zubicaray, Marta González-Vicent, Kritika Chetty, Gráinne O'Toole, Jinhua Xu-Bayford, Eileen Nicoletti, Augustine Fernandez, Caroline Y. Kuo, Satiro N. De Oliveira, Theodore B. Moore, Grace Choi, Miriam Zeini, Cristina Mesa-Núñez, Adrian J. Thrasher, Juan A. Bueren, Jonathan Schwartz, Claire Booth

7. Immunodeficient Mouse Models to Assess Human HSPC Engraftment and Gene Editing: NSG vs. NBSGW. Zulema Romero Garcia, Beatriz Campo, Xiaomeng Wu, **Donald B. Kohn**

8. Education Session Chair - Vector-derived gene therapy, May 18, 2023.

331. LABEST VIP Pre-Event - Challenges & Solution to Enable Sustained Access to Curative Therapies Showcase. ADA SCID Program – Overview and Example.

332. Invited Presentation “Hematopoietic Stem Cell Gene Therapy” for Let’s Talk Science: Innovative Therapies: From the Bench to Bedside. UCLA College, Division of Life Sciences, May 31, 2023, Los Angeles CA.

333. Invited presentation: “Gene Therapy for Inborn Errors of Immunity,” 36th regional conference of the Southern California Chapter of the Association of Pediatric Hematology/Oncology Nurses. Rancho Mirage, CA June 2, 2023

334. Invited presentation: “Hematopoietic Stem Cell Gene Therapy for Inborn Errors of Metabolism”. 2023 CIRM Alpha Stem cell Clinic Network Symposium at UC Irvine, Irvine CA, Sept. 8, 2023.

335. Invited presentation: “Hematopoietic Stem Cell Gene Therapy for Inborn Errors of Metabolism”. UCLA PICI Engineered Immunity Retreat. UCLA Lake Arrowhead Lodge, Lake Arrowhead CA, Sept 8, 2023.

336. Invited presentation: “Hematopoietic Stem Cell Gene Therapy”. University of Minnesota, Department of Medicine, Departmental Research Series. Minneapolis MN (virtual), September 18, 2023.

337. Invited Keynote presentation: “Hematopoietic Stem Cell Gene Therapy for Sickle Cell Disease.” 5th King Faisal Specialist Hospital & Research Centre-J, HSCT and Cellular Therapy Annual Conference, October 24-25, 2023 Jeddah, Saudi Arabia (virtual).

338. Invited presentation: ”Hematopoietic Stem Cell Gene Therapy for Inherited Blood Cell Diseases” at the American Society of Human Genetics 73rd Annual Meeting (Many Genomes, One Humanity - Delivering on the Promise of Genomic Medicine), Presidential Symposium, November 3, 2023, Washington DC.

339. Invited presentation: “Development of a Lentiviral Gene Therapy for Alpha Thalassemia”. Presented jointly with Eva Segura. International Quarterly Seminar on Alpha Thalassemia Major. November 7, 2023 (virtual).

340. Invited presentation: “Hematopoietic Stem Cell Gene Therapy for ADA SCID – The Road to BLA.” California Institute for Regenerative Medicine (CIRM) – Rare Disease Workshop. Burlingame CA, November 15, 2023.

341. Invited speaker: “Hematopoietic Stem Cell Gene Therapy for Inborn Errors of Immunity”. Keystone Symposium on Stem Cells and Regeneration. Santa Fe NM, January 26, 2024.

342. Invited presentation “Gene Therapy for Sickle Cell Disease”. Miller Children’s Hospital (Long Beach CA), Pediatric Hematology/Oncology Division Rounds. (virtual) February 21, 2024.

343. Invited presentation “Hematopoietic Stem Cell Gene Therapy.” FDA Office of Therapeutic Products, CBER, FDA. (virtual) March 13, 2024.

344. Invited Speaker “Hematopoietic Stem Cell Gene Therapy for Adenosine Deaminase Severe Combined Immune Deficiency.” At Arthur W. Nienhuis Research Symposium, March 20, 2024, St Jude Children’s Research Hospital, Memphis TN.

345. Invited presentation “Hematopoietic Stem Cell Gene Therapy for Inborn Errors of Immunity.” UCLA Immunology/Allergy Divisional Conference, Los Angeles CA, April 18, 2024.

346. Invited Keynote Presentation “Hematopoietic Stem Cell Gene Therapy.” At CIRM Alpha Clinic Network Regenerative Medicine Nursing Education Symposium. City of Hope, Duarte CA, April 19, 2024.

347. 23rd William Krivit Lectureship in Pediatrics, Pediatric Grand Rounds, University of Minnesota Department of Pediatrics. “Gene therapy for Blood Cell Diseases.” Minneapolis MD, May 1, 2024

348. Robert A. Good Lecture. “Gene Therapy for Inborn Errors of Immunity.” Clinical Immunology Society Annual Meeting. Minneapolis MD, May 1, 2024.

349. Session Chair and Speaker. “Lentiviral Gene Therapy as a Potential Curative Treatment for Leukocyte Adhesion Deficiency Type I- Breakfast Symposium. ” Clinical Immunology Society Annual Meeting. Minneapolis MD, May 4, 2024.

350. Session Chair and Speaker. “Gene Therapy for Inborn Errors of Immunity” (Gene Therapy for ADA SCID.” Clinical Immunology Society Annual Meeting. Minneapolis MD, May 4, 2024.

351. International Society for Cell Therapy (ISCT) Annual Meeting 2024. “Gene Editing in Hematopoietic Stem Cells for Monogenic Blood Cell Diseases” MaxCyte Corporate Session, Vancouver Canada May 31, 2024.

352. International Society for Cell Therapy (ISCT) Annual Meeting 2024. Roundtable Panelist “Ultra Rare Disease: Is Perpetual IND a Solution?” Concurrent Speaker “Rare Diseases and patient Access- The Mission and the Challenge”. Vancouver Canada May 31, 2024.

353. National Organization for Rare Diseases (NORD) - 2024 Living Rare, Living Stronger NORD Patient and Family Forum. “Gene Therapy for ADA SCID”. Los Angeles CA June 8, 2024

354. Invited educational presentation “Gene Therapy”. The 2024 PI Conference Reel Talk about Primary Immune Deficiency. The Immune Deficiency Foundation, Chicago Il June 21, 2024

355. Invited educational presentation “Gene Therapy for X-linked Agammaglobulinemia”. The 2024 PI Conference Reel Talk about Primary Immune Deficiency. The Immune Deficiency Foundation, Chicago Il June 21, 2024

356. Invited Keynote Speaker “Hematopoietic Stem Cell Gene Therapy”. CIRM Trainee Meeting 2024. University of Southern California, Los Angeles Ca, August 12, 2024.