# DONALD BARRY KOHN, M.D.

## Curriculum Vitae

## A. PERSONAL INFORMATION


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


## B. EDUCATION

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

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

## C. PROFESSIONAL EXPERIENCE

### Present Position:

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

Director, UCLA Human Gene and Cell Therapy Program, 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, 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.

## D. HONORS AND EXTRACURRICULAR ACTIVITIES

1971 National Merit Scholarship Semi-Finalist

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

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

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 Editorial Board: Human Gene Therapy 1993-present

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

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

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

1996 Member, Scientific Review Board, National Gene Vector Laboratory

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

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

1998 **American Society of Gene and Cell Therapy**:
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, September 13, 2018
Co-Organizer “Value Summit”. Washington DC September 24, 2018
Member, 2019 ASGCT Strategic Planning Workgroup
Abstract Reviewer for Annual Meeting 2019
Member, Nominating and Awards Committee 2020-2023
Member, Education Committee 2020-2023

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

## HONORS AND EXTRACURRICULAR ACTIVITIES (Cont'd)

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

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

## HONORS AND EXTRACURRICULAR ACTIVITIES (Cont'd)

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), 2006-2011

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 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

2008- Member, Primary Immune Deficiency Treatment Consortium (PIDTC) Steering Committee and Co-Director of Fellowship Program.

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

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

## HONORS AND EXTRACURRICULAR ACTIVITIES (Cont'd)

2015 Gene Transfer Safety Assessment Board (GTSAB).

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

2010-2014 Member, External Advisory Board for CIRM-funded Disease Team Projects 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

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

2011- Editorial Board,*The Journal of Clinical Immunology* - present

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

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

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

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

2016-2018 Member, External Advisory Board, SCID-NET, a European Commission Research & Innovation multinational cooperative research group

**HONORS AND EXTRACURRICULAR ACTIVITIES (Cont'd)**

2017-2019 International Society for Cellular Therapy (ISCT):
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 to: Award# DR2-05365. "A monoclonal antibody that depletes stem cells." PI: Judith Shizuru, Stanford University.

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

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

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.

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.

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-present 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 *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

## E. AWARDS AND GRANTS

Present Support

National Institutes of Health-NHLBI (T32HL086345-11) "Training in Developmental Hematology," DBK PI. 4/1/18-3/31/23.

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.

National Institutes of Health-NIAID 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. 9/1/16-8/31/22

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.

U54 AI082973 (MPI: Puck/Kohn) 9/1/2019–8/31/2024 NIH/UCSF $25,268 Primary Immune Deficiency Treatment Consortium (PIDTC)

California Institute for Regenerative Medicine (CIRM) (DR3-06945). "Clinical Trial of Stem Cell Gene Therapy for Sickle Cell Disease." DBK PI. 7/1/14-6/30/20, $2,364,631 Annual DC.

California Institute for Regenerative Medicine (CIRM) (CTS1-08231). "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 – 08/31/22. $1,548,584 Annual DC.

California Institute for Regenerative Medicine (CIRM)/Orchard Therapeutics (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 Co-I, 1/1/17-12/31/21. $1,849,342 Annual DC.

California Institute of Regenerative Medicine (CIRM) (TRAN1-10954) "Developing Engineered Autologous Leukemia Vaccines to Target Residual Leukemic Stem Cells" K. Gaensler PI, Kohn Co-I. 02/01/19-01-31-21 $239,266 Annual 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. UCLA Role: GMP cell processing.

CIRM CLIN2 (PI: S. Cherqui) 06/01/2019 - 05/31/23 UCSD $509,029 annual dc 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 Viv o Transduction using the pCCL-CTNS Lentiviral Vector. UCLA Role: GMP cell processing.

Doris Duke Charitable Foundation - 2017 Sickle Cell Disease/Advancing Cures Award "Optimizing Gene Editing for Sickle Cell Disease." DBK PI, 9/01/17 – 08/31/20 $280,368.33 Annual DC.

Doris Duke Charitable Foundation – Sickle Cell Disease Research Project (2018186) "Cas9 Fusion Proteins for Improved Sickle Cell Disease Gene Correction." DBK PI, 01/01/19-12/31/21

**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, l979, $2,000.

American Association of Immunologists (AAI) Travel Grant, VI International Immunology Congress, l986, $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.

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.

**Past Support (Continued):**

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.

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. "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.

**Past Support (Continued):**

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.

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 A1074043) "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 Annual DC.

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.

**Past Support (Continued):**

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.

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

## 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 Blood and Marrow Transplantation - 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

## G. RESEARCH INTERESTS

Gene therapy of genetic diseases of blood cells

Gene transfer, expression and correction of hematopoietic stem cells

Gene editing in human hematopoietic stem cells

Hematopoietic stem cell transplantation for primary immune deficiencies

Immunotherapy for childhood leukemia and cancer

Immune tolerance

## H. Sponsor for FDA INVESTIGATIONAL NEW DRUG 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)

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).

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 CiniMacs) Cultivated W/ Cytokines; Transduced W/ Self-Inactivating (SIN) Lentiviral Vector Expressing Human gp91phox (G1XCGD); Following Busulfan) (Open Oct. 2014)

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

| | |
|---|---|
| Kathryn Bradford (MD) | 2018- Gene editing for Common Variable Immune Deficiency |

**Previous fellows (36):**

| | |
|---|---|
| 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
Robert Drummond (MD/PhD) 2015-2017 Correction of the sickle cell mutation
Alexandra Miggelbrink, (MD) 2016-2017 CRISPR-mediated gene correction in HSC

**J. GRADUATE STUDENTS FOR THE Ph.D. (6)**

Mr. David Gray (2015- ) Gene correction for X-linked agammaglobulinemia
Ms. Nebula Han (2017- ) β-globin lentiviral vector genomes
Mr. Ryan Wong (2017- ) Bioinformatically-designed lentiviral vectors
Mr. Paul G. Ayoub (2019- ) Lentiviral vectors for gene therapy of hemoglobinopathies
Mr. Ralph V. Cristostomo (2019- ) Cell cycle and gene editing

**K. Previous graduate students (19)** (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

**9** 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*)**:**

| | |
|---|---|
| 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 |

## 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.

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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

**SYMPOSIA**

1. **Kohn DB**, Nolta JA, Hong CM, Barranger JA. Expression of the human glucocere-brosidase 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.

## 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 $\alpha$-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.

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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.

275. 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.

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.

276. 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.