**CURRICULUM VITAE**

**University of Alabama at Birmingham School of Medicine Faculty**

Date: May 30, 2020

**PERSONAL INFORMATION**

Name: Frederick Douglass Goldman

Citizenship: United States of America

Home Address: 3665 Brookwood Road, Mountain Brook, AL 35223

Office Phone: 205 638-5855

**RANK/TITLE**:

Department: Pediatrics, UAB Division of Pediatrics

Professor, UAB Division of Pediatric Hematology Oncology

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**HOSPITAL AND OTHER (NON-ACADEMIC) APPOINTMENTS:**

2009- Present Professor; University of Alabama, Birmingham, AL

2009- Present Medical Director, Lowder Blood and Marrow Transplant Program, Children’s of Alabama, Birmingham, AL

2000-2009 Director, Pediatric Immune Disorders Clinic University of Iowa Hospitals and Clinics, Iowa City, IA

1999-2009 Associate Professor, University of Iowa Hospitals and Clinics, Iowa City

2002 -2009 Associate, Blank Children's Hospital Iowa Health System Maternal/Child Health in Pediatrics, Des Moines, IA

2002-2009 Director, University of Iowa Hematopoietic Stem Cell Bank

2001-2006 Director, Pediatric Blood and Marrow Transplantation Program, University of Iowa Hospitals and Clinics, Iowa City, IA

1997-2001 Interim Director, Pediatric Bone Marrow Transplant Program, University

of Iowa Hospitals and Clinics, Iowa City, IA

1993-1999 Assistant Professor, University of Iowa Hospitals and Clinics, Iowa City

1992-1993 Associate, University of Iowa Hospitals and Clinics, Iowa City, IA

1991-1992 Instructor, Children’s Hospital Denver, CO

**EDUCATION:**

1981-1985 Doctor of Medicine Louisiana State University of New Orleans

1979-1981 Master of Immunology University of New Orleans

1975-1979 Bachelor of Science Tulane University

**LICENSURE:**

Alabama Medical License (2009- present) #29486

Iowa Medical License (1992-2009) #28926

Colorado Medical License (1988-1992) #29029

Louisiana Medical License (1985-1988)

**BOARD CERTIFICATION:**

American Board of Pediatric Hematology Oncology (1992- present) #00992

American Board of Pediatrics (1988-1995) #40114

**POSTDOCTORAL TRAINING:**

1989-1992 Post-doctoral research Fellow, National Jewish Hospital for Immunology and Respiratory Medicine, Denver, CO

1988-1991 Clinical Fellow, Pediatric Hematology Oncology, University of Colorado

1985-1988 Intern and Resident, Pediatrics, Tulane School of Medicine

**ACADEMIC APPOINTMENTS:**

2012-present Professor of Pediatrics Graduate Biomedical Sciences program

University of Alabama, Birmingham

2009-present Professor of Pediatrics University of Alabama at Birmingham

1999-2009 Associate Professor of Pediatrics University of Iowa School of Medicine

1993-1999 Assistant Professor of Pediatrics University of Iowa School of Medicine

1992-1993 Associate University of Iowa School of Medicine

**AWARDS/HONORS:**

Best Doctors in America U.S. News & World Report Listing (2006-2020)

Improving Our Workplace Award University of Iowa (2008)

Elected Member of Society of Pediatric Research (2000)

University of Iowa College of Medicine Research Award (1998)

Vere D. Wenger Award Cancer Research Travel Funds University of Iowa (1997-1998)

University of Iowa Bioscience Initiative Award (1994)

Carver Research Associate University of Iowa (1992)

Carl Hansen Fellowship for AIDS Research at National Jewish Center for Immunology and Respiratory Medicine (1990)

March of Dimes Scholarship for Genetics Research at LSU Medical School (1982)

Tri Beta Honor Society at Tulane University (1976-1979)

Dean’s List at Tulane University (1979)

**PROFESSIONAL SOCIETIES:**

American Society of Hematology (1992-present)

Children’s Oncology Group (1992-present)

American Society of Pediatric Hematology/Oncology (2000-present)

American Society for Blood and Marrow Transplantation (2001-present)

Pediatric Bone Marrow Transplant Consortium (1999-present)

International Bone Marrow Transplant Registry (2007- present)

Pediatric Immune Deficiency Transplant Consortium (2010-present)

Iowa Medical Society (2003-2009)

Midwest Society of Pediatric Research (2000-2005)

International Society for Experimental Hematology (2003-2008)

Society for Pediatric Research, Member (2000-2004)

American Academy of Pediatrics (1986-1999) (2017-present)

**COUNCILS AND COMMITTEES:**

2012-present Member, UAB School of Medicine Scholarly Activity Mentor

2015-present Consultant, Undiagnosed Diseases Program, UAB

2013-present Member, Clinical Care Consortium for Telomere Associated Ailments (multi-institutional consortium of scientists/clinicians working with Dyskeratosis congenita)

2013-present Lead, Cord Blood Collection Working Committee, UAB

2012-present Member, Children’s Hospital Ethics Review Committee

2012-present Member, Children’s Hospital Blood Utilization Committee

2009-present Director, Annual Symposium for Pediatric Cancer and Blood Disorders, Children’s Hospital of Alabama

2009-present Member, Central Line Blood Stream Infection Committee

2007-present Member, Non-Malignant Marrow Disorders Working Committee CIBMTR

2008 Member, Pediatric Medical Mission-Jamaica, ISSA Trust Foundation

2008 Staff, Community Health Free Medical Clinic, Cedar Rapids, IA

2003-2009 Judge, Annual Medical Student Research Day

2006-2009 Honorary Chairman, American Cancer Society Relay for Life, Johnson Co. 2006-2008 Advisory Board, North American Shwachman Diamond Syndrome Registry

2006-2009 Newborn Screening Task Force University of Iowa

2003-2004 Medical Director, UI Dance Marathon

2001-2009 Holden Cancer Centers Research Review Committee

2001-2009 Reviewer, General Clinical Research Center (GCRC)

2004-2009 Advisor, Flow Cytometry Facility, University of Iowa

2005-2007 Scientific Board of Advisors Talecris

1999-2009 Interviewer, Medical School Admissions Committee, University of Iowa

1994-2009 Member, Holden Comprehensive Cancer Center

1994-1998 Director, Pediatric Hematology/Oncology Seminar Committee

1989-1992 Executive, Board of Directors, One day Pediatric AIDS Care Facility

1985-1988 Member, Aplastic Anemia Foundation of America, Louisiana Chapter

**UNIVERSITY ACTIVITIES**

2011-present Member, UAB Comprehensive Cancer Center

2009-2016 Member, Stem Cell Institute of Alabama

2019-present Protocol Review member, UAB Comprehensive Cancer Center

**EDITORIAL BOARD MEMBERSHIPS**

#### Ad hoc reviewer for:

*New England Journal of Medicine*

*JAMA*

*Blood*

*British Journal of Hematology*

*Journal of Pediatrics*

*Journal of Pediatric Hematology Oncology*

*Pediatric Blood and Cancer*

*Biology of Blood and Marrow Transplantation*

*Pediatric Transplantation*

*PLOS One*

*Immunology*

**MAJOR RESEARCH INTERESTS:**

1. Gene therapy for Sickle Cell Disease:

In collaboration with Tim Townes, CEO of HemEdits and former Department Chair of Biochemistry at UAB, we have been testing gene correction strategies using CRISPR/Cas/RNP complex in human hematopoietic stem cells isolated from sickle cell patients (cord blood, PBSC, BM). Pre-clinical Phase 1 studies have demonstrated efficiency and minimal off target toxicity. Studies have now been performed *in vivo* in mouse models of sickle cell disease, and *in vitro*, in NOD SCID mice. The long-term goal is to obtain FDA approval for a phase 1 study in infants with sickle cell disease that uses CRISPR/Cas gene correction in cord blood, and transplant using non ablative conditioning.

2. Dyskeratosis congenita (DC) and DNA damage repair responses:

We have been using cells acquired from patients with DC and studying the interplay of oxidative stress, DNA damage response, and telomere shortening. Our goal is to identify regulators of oxidative stress as a means of halting the progression of the disease phenotype.

3. Creation of patient-specific gene corrected hematopoietic stem cells using inducible pluripotent stem cell technology:

We have several IRB approved protocols to study gene repair in diseases of hematopoiesis, including Diamond Blackfan Anemia, Dyskeratosis congenita, and severe combined immune deficiency. iPS technology allows us to create cells for disease modeling as well as therapeutic intervention. Our goal is to create gene-corrected hematopoietic stem cells (HSC) that can be given back to patients, ameliorating their underlying disease and forgoing the need for an allogeneic bone marrow transplant.

4. Optimizing BMT in SCIDs:

Our program is part of a collaborative with the Pediatric Immune Deficiency Transplant Consortium, and I am a CSIDE investigator. This study was developed to allow for a standardized reduced intensity preparation therapy approach for infants with SCIDs.

**TEACHING EXPERIENCE**

Mock Interviews for medical students, UAB-Volker Hall (2018-present)

A teaching module for medical students providing experience and feedback for future interviews.

UAB Graduate Biomedical Science Program

Phenotyping Human Disease course (2017-present)

Pediatric Hematology/Oncology fellows teaching conferences, UAB (2009-present)

Present didactic lectures on areas related to bone marrow transplantation, immune deficiencies and bone marrow failure syndromes.

Adult Hematology/Oncology fellows teaching conference (2015, 2018)

Present lectures on inherited bone marrow failure syndromes

Pediatric BMT nurses (2009-present)

Lectures consist of best clinical practices in BMT, and background on diseases for upcoming patients admissions.

UAB Pediatric Tumor Board (2009-present)

Morbidity and mortality case conferences related to BMT patients, done quarterly.

Foundations for Clinical Practice: Personal and Professional Development (2001-2009)

A teaching module for 1st year medical students at the University of Iowa that integrated ethics, history taking, and problem solving in a group setting.

Pediatric Resident Orientation, University of Iowa (1995-2009)

Responsible for didactic lectures on immune deficiencies, neutrophil disorders, BMT emergencies, aplastic anemia

**MAJOR LECTURES AND VISITING PROFESSORSHIPS**

1. “Gene therapy in clinical Practice”. 10th Annual Advancements in the Care of Pediatric Cancer and Blood Disorders Symposium, Children’s Hospital, University of Alabama, Birmingham, Oct 18, 2019.
2. “Translational Research in Pediatric Hem/Onc: From the bench to the patient and how I got there”. CARES summer program, UAB Medical School, July 8, 2019
3. “Hematopoietic Stem Cell Transplantation for Genetic Disorders”. Genetics Grand Rounds. UAB, Birmingham AL January 8, 2019
4. “Sickle Cell Disease: A CRISPR way to a Cure”. 9th Annual Advancements in the Care of Pediatric Cancer and Blood Disorders Symposium, Children’s Hospital, University of Alabama, Birmingham, Nov 20, 2018.
5. “Gene Therapy in Sickle Cell Disease”: University of South Alabama School of Medicine, Pediatric Grand Rounds, Mobile, AL, June 15, 2018
6. “Translational Research in Pediatric Hem/Onc: From the bench to the patient and how I got there”. CARES summer program, UAB Medical School, July 16, 2018
7. “Inherited Bone Marrow Failure Disorders”: UAB Department of Medicine, Hematology, Oncology Fellows, April 15, 2018
8. “CRISPR Gene Editing: Our Quest to Cure Sickle Cell Disease”, Child Health Investigative Forum (CHIF), Bradley Lecture Center, Birmingham, AL, March 2, 2018
9. “Stem Cell Transplantation for Primary Immune Deficiencies - Current and Future Curative Strategies” Immune Deficiency Foundation Education Meeting, Sheraton Birmingham Hotel, May 6, 2017.
10. “CRISPR gene editing to cure Sickle Cell Disease: Hurdles to Overcome in Developing an IRB Approved Clinical Trial” PRIM&R Regional Connections Meeting, Birmingham AL, September 9, 2016.
11. “SCIDs: Curative Role of Allogeneic Hematopoietic Stem Cell Transplant” Satellite Conference and Live Webcast for Alabama Newborn Screening Program, Montgomery AL, August 23, 2016.
12. “Bone Marrow Failure in Children: Diagnostic Dilemmas and Novel Therapies” University of South Alabama School of Medicine, Pediatric Grand Rounds, Mobile AL, August 19, 2016.
13. “Bone Marrow Failure in Children: Diagnostic Dilemmas and Novel Therapies” LSU School of Medicine, Pediatric Grand Rounds, New Orleans LA, June 22, 2016.
14. “Correction of Diamond-Blackfan Induced Pluripotent Stem Cells via CRISPR”. Diamond Blackfan Anemia International Foundation Consensus Meeting. Atlanta GA. March 7, 2016
15. “Sickle cell disease: Clinical features and current treatment strategies”. Deerfield Healthcare,

UAB Research and Development, September 16, 2015.

1. “Elevated reactive oxygen species in DC: Is there a role for antioxidant therapy in DC”

Annual **C**linical **C**are **C**onsortium for **T**elomere **A**ssociated **A**ilments, Chicago IL September

2014

1. “CAR T Cells from Inducible Pluripotent Stem Cells for Leukemia Treatment”. Children’s

Hospital, Denver CO, May 14, 2014

1. “Inherited Bone Marrow Failure Syndromes & Novel Stem Cell Therapies”, 4th Annual

Advancement in the Care of Pediatric Cancer & Blood Disorders Symposium, Children’s of

Alabama, Birmingham, AL, October 24, 2013

1. “Stem Cell Transplantation in Marrow Failure Disorders: Buyer Beware”, UAB Advanced

Cell Therapy and Manufacturing Symposium, University of Alabama at Birmingham,

Birmingham, AL, October 3, 2013

1. “Inherited Bone Marrow Failure and the Role of Novel Stem Cell Therapies”, Pediatric

Medical Grand Rounds, Children’s of Alabama, Birmingham AL, July 11, 2013

1. “Introduction to Pediatric Hematopoietic Stem Cell Transplantation”, Pediatric Grand

Rounds, Columbus Regional Healthcare System, Columbus, GA, April 2013

1. “Introduction to Pediatric Bone Marrow Transplantation”, Pediatric Grand Rounds, University

of South Alabama, Mobile, AL, January 2013

1. ”Primary and secondary Pediatric Immune Disorders”, 2nd Annual Advancements in the Care

of Pediatric Cancer and Blood Disorders Symposium, Children’s Hospital, University of

Alabama, Birmingham, Oct 14, 2011

1. “Pediatric Blood and Marrow Transplant: State of the Art and Future of Stem Cell Therapies”,

Special Biology lecture series, Chandler-Gilbert Community College, Gilbert, AZ, Sept 2011

1. “Inherited Bone Marrow Failure and the Role of Novel Stem Cell Therapies,” Aflac Cancer

Center Visiting Lecture Series, Children’s Healthcare of Atlanta at Egleston on the Emory

University Campus, Atlanta, GA, May 4, 2011.

1. **“Expanding the Role of Bone Marrow Transplantation in Pediatric Diseases”, Alabama**

**Chapter-AAP 2010 Annual Meeting & Fall Pediatric Update, September 24, 2010**

1. “Pediatric Blood and Marrow Transplant: State of the Art and Future of Stem Cell Therapies",

Pediatric Medical Grand Rounds, Children’s Hospital, University of Alabama, Birmingham,

Sept 9, 2010

1. “Pediatric Blood and Marrow Transplant: State of the Art and Future of Stem Cell Therapies",

Montgomery Baptist South Hospital, Pediatric Grand Rounds, May 2010

1. “Umbilical Cord Blood: An update on its application in the clinical transplant setting”,

Pediatric Grand Rounds, Huntsville, AL, November 2009

1. “Immune Function and Monitoring in the Hematopoietic Stem Cell Transplant Recipient”,

Nurse Education Day, Children’s of Alabama, September 2009

1. “Role of BMT in Immune Disorders” Pediatric Grand Rounds, Mobile, University of South

Alabama, November 2009

1. “Hematopoietic Stem Cell Transplantation for Immune Disorders”, Allergy Immunology

Grand Rounds at UAB, Birmingham AL, September 2009

1. “Child with Immune Deficiency without Classic Signs of Inflammation,” Special Infectious

Disease Grand Rounds, University of Iowa Department of Medicine, October, 2008

1. “Umbilical Cord Blood Stem Cells: Clinical and Research Applications,” 34th Annual Iowa

Conference on Perinatal Medicine, Des Moines, IA, April 2008

1. “Pediatric Bone Marrow Failure Disorders” Pediatric Grand Rounds, Tulane University, New

Orleans LA, April 2008

1. “Pediatric Bone Marrow Failure Syndromes: Diagnostic and Therapeutic Dilemmas”,

Pediatric Hematology /Oncology / Transplant Conference, Children’s Memorial Hospital,

Chicago IL, March 2008

1. “Pediatric Bone Marrow Failure Disorders: Diagnostic and Therapeutic Dilemmas” Pediatric

Grand Rounds, University of Iowa Children’s Hospital, January 2008

1. “Stem Cells and Gene Therapy for Dyskeratosis Congenita” University of Iowa

Interdisciplinary Health Group, Iowa City IA, September 2007

1. “State of Iowa Cord Blood Bank” Iowa Postnatal Tissue and Fluid Banking Task Force

Meeting, Iowa City IA, August 2007

1. “Dyskeratosis Congenita as a Model to Study the Role of Telomere Shortening in Aging,”

The Role of Telomeres in Cancer and Aging, Cancer and Aging Program, University of

Iowa Hospitals and Clinics, September 2007

1. “Cord Blood Banking: Practical Points and Research Applications,” Obstetrics/Gynecology

Grand Rounds, University of Iowa Hospitals and Clinics, March 2007

1. “Neutrophil Disorders” Association of Pediatric Hematology/Oncology Nurses Group,

University of Iowa Hospitals and Clinics, Iowa City, IA, March 2007

1. “Umbilical Cord Blood Stem Cells: Clinical and Research Applications”, Maternal Child

Nurse Conference, Cedar Rapids, IA, March 2007

1. “Emerging Targets for HCT: Genetic Disorders of Hematopoiesis and Immunity,

Dyskeratosis Congenita.” BMT Tandem Meetings, Keystone, CO, February 2007

1. “Pediatric Bone Marrow Failure Syndromes,” Pediatric Grand Rounds, University of Texas

M.D. Anderson Cancer Center, Houston, TX, November 2006

1. “Neutropenia in Shwachman Diamond Syndrome”, Shwachman Diamond Syndrome Support

Group, Maine, July 2006

1. “Translational Research in Pediatric Immunology: From the Bedside to the Lab Bench and

Back to the Patient,” Immunology Grand Rounds, University of Iowa Hospitals and Clinics,

November 2005

1. “Stem Cells: Current Controversies and Practical Applications in Medicine”, Temple Judah,

Cedar Rapids, IA, September 2005

1. “Pediatric Immune Disorders,” Pediatric Grand Rounds, Genesis Medical Center, Davenport,

IA, August 2005

1. “Dyskeratosis Congenita: Implications of Telomerase Deficiency on Hematopoiesis and

Potential Role for Gene Therapy”, Baylor College of Medicine for the Joint Cancer Center

and Center for Cell and Gene Therapy Seminar, Houston, TX, July 2005

1. “IGIV Therapy: Optimizing Care of Patients in the Oncology Setting”, Orlando, FL, April

2005

1. “Stem Cell Research in Iowa”, Molecular Biology Annual Retreat, Amana, IA, October 2004
2. “Common Immune Disorders in Children and Young Adults”, Mercy Medical Center,

Dubuque, IA, September 2004

1. “Development of a Research Cord Blood Bank at the University of Iowa,” OB/GYN Grand

Rounds, University of Iowa Hospitals and Clinics, February 2004

1. Translational Research; It Must Make Sense”, University of Iowa Hospitals and Clinics,

Medical Student Research Club, March 2004

1. “Neutrophil Motility and Chemotaxis”, 4th International Family Conference on Shwachman-

Diamond Syndrome, Chicago, IL, April 2004

1. “Bone Marrow Transplant”, 4th International Family Conference on Shwachman-Diamond

Syndrome, Chicago, IL, April 2004

1. “Rare Pediatric Bone Marrow Failure Syndromes: Characterization of Molecular Defects”,

Institut de Genetique Moleculaire de Montpellier, France, July 2004

1. “Identifying the chemotactic defects in Schwachman Diamond Syndrome”, International

Congress on Shwachman Diamond Syndrome Meeting, Toronto, Canada, September 2004

1. “Immunodeficiencies in Pediatrics”, Grand Rounds, Fort Madison Community Hospital, Fort

Madison, IA

1. “Pediatric Immune Disorders,” Pediatric Grand Rounds, Sunrise Children’s Hospital, Las

Vegas, NV, December 2003

1. “Rare Pediatric Bone Marrow Failure Syndromes: Characterization of Molecular Defects

and Transplant Strategies”, Pediatric Grand Rounds, University of Iowa Hospitals and\

Clinics, March 2003

1. “Biology of Autoimmune Lymphoproliferative Syndrome", Hematology/Oncology Grand

Rounds, Children’s Hospital of Cincinnati, 2000

1. “Immunopathogenesis of Graft Versus Host Disease and Rationale for Hydroxychloroquine

Therapy”, Hematology, Oncology, and Blood & Marrow Transplantation Grand Rounds,

University of Iowa, 2000

1. “Pediatric Bone Marrow Transplantation” Pediatric Grand Rounds, University of Iowa

Hospitals and Clinics, 1998

1. “HIV in Pregnant Woman and Infants”, Mercy Hospital Family Practice Grand Rounds,

Cedar Rapids, IA, 1997

1. “Immunodeficiency in Children”, Pediatric Grand Rounds, University of Iowa Hospitals and

Clinics, 1996

1. “Pediatric AIDS and HIV Pathogenesis” Pediatric Grand Rounds, University of Iowa

Hospitals and Clinics, 1993

**GRANT SUPPORT:**

***Current***

Title: Barriers to Transplant in Sickle Cell Disease: A Survey of Patients and Families at Children’s of Alabama

Source: Sickle Cell Foundation of Birmingham

Direct: $1000

Period of Funding 1/1/10-12/31/20

Principle Investigator: Fred Goldman and Hilary Haines

The primary goal of this study is to better understand psychologic, sociologic, and educational barriers to patients with sickle cell disease undergoing curative treatment with allogeneic bone marrow transplantation.

Title: Primary Immune Deficiency Treatment Consortium

Source: Children’s Hospital of Philadelphia

Direct: $500-1200 per patient enrollment

Period of Funding 9/1/10-present (in 2019 received renewal for another 5 years)

Principle Investigator: Michael Pulsipher

The primary goal of this study is to study, retrospectively and prospectively, pediatric patients with immune deficiencies, to better understand benefit of early detection and role of bone marrow transplant in these conditions.

Title: Risk factors associated with the development and treatment outcomes of VOD in a pediatric bone marrow transplant cohort: A ten year retrospective analysis.

Source: JAZZ Pharmaceuticals

Direct: $41,000

Period of Funding 11/1/18-3/31/20

Principle Investigator: Frederick D. Goldman, M.D

The primary goal of this project is to determine veno-occlusive disease (VOD) risk factors and outcomes for a pediatric BMT cohort

Title: A Phase 1, Open-Label, Multicenter, Non-comparative

Pharmacokinetics and Safety Study of Intravenous Isavuconazonium Sulfate in

Pediatric Patients

Source: Astella Pharmaceuticals

Direct: $30,000 per patient enrollment

Period of Funding 9/1/17-present

Principle Investigator: Frederick Goldman at COA

The primary goal of this study is to study pharmacokinetics of a new antifungal agent that will be use as treatment or prevention of invasive fungal disease in the BMT setting.

Title: A Phase 3, Randomized, Adaptive Study Comparing the Efficacy and Safety of Defibrotide vs Best Supportive Care in the Prevention of Hepatic Veno-Occlusive Disease in Adult and Pediatric Patients Undergoing Hematopoietic Stem Cell Transplant

Source: Jazz Pharmaceuticals

Direct: ~ $25,000 per patient enrollment

Period of Funding 2/1/18-present

Principle Investigator: Frederick Goldman at COA

The primary goal of this study is to study Defibrotide in high risk BMT patients as prevention of VOD, and monitor safety and efficacy.

***Past***

Title: DNA Damage and Oxidative Stress in Dyskeratosis Congenita: Analysis of Pathways and Therapeutic Strategies Using CRISPR and iPSC Model Systems

Source: Department of Defense (Bone Marrow Failure Research Program Idea Development Award)

Direct: $400,000

Period of Funding 8/1/15-7/31/18

Principle Investigator: Frederick Goldman

The primary goals of this project are to 1) Characterize the mechanism by which short telomeres upregulate oxidative stress, 2) screen small molecules, antioxidants, and other drugs with the goal of decreasing oxidative stress and cell death, and 3) create hematopoietic progenitor cells (HPC) from induced pluripotent stem cells (iPSCs) to better understand the basis of progressive stem cell loss *in vivo* and to serve as a model for therapeutic intervention.

Title: Generation of Suicide Gene Modified Anti-CD19 CAR Redirected T Cells

Source: St. Baldrick Foundation

Direct: $50,000

Period of Funding 7/1/17-6/30/18

Principle Investigator: Corey Falcon, Goldman (Mentor)

Role: Mentor/PIThe primary goals of this project are to 1) Evaluate the functionality and specificity of ΔiC8+ΔiC9 suicide gene modified CAR-T.CD19 toward CD19+ pre-B cell ALLblasts *in vitro*, and 2) conduct *in vivo* assessments of suicide gene modified CAR-T cells with and without CD19+ pre-B cell ALL using a murine model.

Principle Title: Production of T cells from human inducible pluripotent stem cells (hIPS) expressing a chimeric antigen receptor (CAR) against precursor-B leukemia cells:

Source: Hyundai Hope on Wheels

Direct: Total $250,000

Period of Funding: 10/1/14-12/31/16

Principle Investigator: Frederick Goldman

The primary goal of this pre-clinical project is to produce, *in vitro*, autologous tumor killing T cell from reprogrammed skin cells isolated from patients with pre B acute lymphoblastic leukemia

Title: DNA Damage Responses and Oxidative Stress in Dyskeratosis Congenita

Source: KPRI

Direct: $60,000

Period of Funding: 7/1/13-6/30/15

Principle Investigator: Frederick Goldman

The primary goal of this project is to characterize the molecular mechanisms involved in telomerase deficiency mediating oxidative stress and to identify novel anti-oxidant therapies for may decrease DNA damage responses in Dyskeratosis congenita

Title: Towards a cure for DBA: Creation of patient-specific gene corrected hematopoietic stem cells using inducible pluripotent stem cell technology.

Source: Diamond Blackfan Anemia Foundation

Direct: $59,000 x 2 yrs.

Period of Funding: 7/1/12-6/30/14

Principle investigator: Frederick Goldman

The primary goal of this project is to determine the feasibility of doing gene correction of RPS 19 mutations in skin fibroblasts of DBA patients, then producing iPS cells and ultimately hematopoietic stem cells. These studies are preclinical and will utilize the GMP facility for testing and eventual expansion to the clinical arena.

Title: A Randomized, Placebo-Controlled, Multi-Site Phase II Study Evaluating the Safety and Efficacy of Preemptive Treatment with CMX001 for the Prevention of Adenovirus Disease Following Hematopoietic Stem Cell Transplantation (The ADV Halt Trial)

Source: Chimerix, Inc

Direct: $4,250

Period of Funding: 10/17/11 – 10/16/13

Title: iPS cells and gene therapy for disease of defective hematopoiesis.

Source: Walmart Research Grant

Direct: $50,000

Period of Funding: 10/1/10-9/31/12

Principal investigator: Frederick Goldman

The primary goal of this project is to determine the feasibility of utilizing inducible pluripotent stem cells for gene correction of diseases of abnormal hemoglobin synthesis. These preclinical studies will hasten the development clinical trials to treat diseases of characterized by defective hematopoiesis.

Title: Gene therapy for Diamond Blackfan Anemia using induced pluripotent stem cell technology.

Source: Kaul Pediatric Research Institute

Direct: $100,000

Period of Funding: 2/1/11-1/31/13

Principal investigator: Frederick Goldman

The primary goal of this project is to determine the feasibility of utilizing inducible pluripotent stem cells for gene correction of Diamond Blackfan Anemia. These studies will address issues of expansion of iPS cells into erythroid precursors and serve as preclinical modeling for use in children with defects in red blood cell production.

Title: A Multi-Center, Randomized, Double Blind, Phase III Trial Evaluating Corticosteroids with Placebo as Initial Systemic Treatment of Acute GVHD

Source: NIH and BMT CTN

Direct: $24,589

Period of Funding: 1/1/10 – 12/31/11

Principal Investigator: Joseph Chewning, M.D.

Co- Investigator: Frederick Goldman, M.D

Title: Neonatal Anemia: Pathophysiology and Treatment

Source: NIH/NHL (9P01HL046925-11A1

Period of Funding: 7/1/06-6/30/2011

Principal Investigator Jack Widness

Role: Consultant

The primary goal is to establish more effective treatment of anemia in critically ill infants.  We propose to reduce the number of transfusions by optimizing the use of erythropoietin (EPO) through studies in human infants and in sheep directed at developing a comprehensive knowledge of the physiology of erythropoiesis and EPO’s complex PK/PD behavior.

Title: A Human Model for Telomerase Dysfunction and Aging

Source: NIH (1 RO1 AG027388-01-A2)

Direct: $1,357,270

Period of Funding: 4/1/07-3/31/2012

Principal Investigator: Al Klingelhutz PhD

Co-Principal Investigator: Frederick D. Goldman, M.D,

The primary goal of this project is to decipher the role of telomerase gene components in cell aging, using skin as the organ system and dyskeratosis congenita skin cells as a disease model of telomerase deficiency.

Title: A pilot Study of Matched Unrelated Donor Hematopoietic Stem Cell Transplant for Patients with High Risk Sickle Cell Disease and Other Non-Malignant red Cell Disorders Using a Reduced Intensity Preparative Regimen and Third Party Mesenchymal Stromal Cells to Achieve Stable Mixed Chimerism

Source: Third Party Payers- Hematopoietic Stem Cell Transplant, Production Assistance for Cellular Therapies (PACT), and the Department of Pediatrics at TCHA

Direct: $10,000

Period of Funding: 01/31/09 – 01/30/2011

Principal Investigator: Sandhya Kharbanda, M.D.

Co-Investigator: Frederick Goldman, M.D.

Title: Immunomodulatory Properties of IGIV in Developing Immune Systems Post Hematopoietic Stem Cell Transplants

Source: Talecris Biotherapeutics

Direct: $36,992

Period of Funding: 5/8/07-4/30/09

Principal Investigator: Frederick Goldman, M.D., 5% effort

The primary goal of this grant is to better understand how IGIV modulates T cell function and cytokine synthesis.

Title: Cell Biology of Hematopoietic Stem Cells and Malignant Stem Cells

Source: Carver Research Trust

Total Period Funding: $500,000

Date of Yearly Funding: 7/04-6/07

Principal Investigator: M. Sue O’Dorisio, M.D., Ph.D.

Co-Investigator: Frederick D. Goldman, M.D.

Title: Gene Therapy Strategies in Bone Marrow Failure Disorders

Source: Aiming for a Cure Foundation

Direct: $40,000

Period of Funding: 7/1/05-6/30/07

Principal Investigator: Frederick D. Goldman, M.D.

Title: Stem Cells for the Treatment of Bone Marrow Failure

Source: Canadian Institute for Health Research (CIHR)

Direct: $95,972/year x 5 yrs

Period of Funding: 11/1/05-3/31/2010

Principal Investigator: Peter Lansdorp, M.D., Ph.D

Co-Principal Investigator: Frederick Goldman, MD., 10% effort

The primary goal of this collaborative grant is better understand the role of telomerase and telomere length in hematopoiesis. Dr. Goldman’s role in this project entails obtaining hematopoietic stem cells from patients with dyskeratosis congenita, a disease of telomerase deficiency. In addition, his lab will employ gene transfer of telomerase components into stem cells, further defining the overall importance of this enzyme complex in cellular function and bone marrow aging.

Title: Role of Telomere Loss in Congenital Marrow Failure Syndromes and Hematopoietic Stem Cell Function

Source: University of Iowa Dance Marathon

Direct: $20,000

Period of Funding: 7/1/05-6/30/06

Principal Investigator: Frederick D. Goldman, M.D.

Title: Role of Telomeres in Aging

Source: Holden Cancer Center

Direct: $50,000

Period of Funding: 7/1/05-6/30/06

Principal Investigator: Aloysius Klingelhutz, PhD

Co-investigator: Frederick Goldman, M.D.

Title: Immune Recovery in Autologous Stem Cell Transplantation: Comparative Analysis of Immune Cell Populations within the Graft versus Blood Early Post-Transplant

Source: University of Iowa Dance Marathon

Direct: $10,000

Period of Funding: 4/15/04-12/31/04

Principal Investigator: Frederick D. Goldman, M.D

Title: Motility and Chemotaxis Defects in SDS Neutrophils

Source: Schwachman Diamond Syndrome, International

Direct: $27,000

Indirect: $3,000

Total Period Funding: $30,000

Period of Funding: 12/1/03-11/30/04

Principal Investigator: David Soll, Ph.D.

Co-Investigator: Frederick D. Goldman, M.D.

Title: Molecular Mechanisms of Action of Thymoglobulin Analysis of Potential Synergy with Immunosuppressants.

Source: University of Iowa, Children’s Miracle Network

Direct: $7,500

Period of Funding: 5/01-4/30/03

Principal Investigator: Frederick D. Goldman, M.D.

Title: Standardization of Immunological Assays

Source:Medicaid Administrative Claiming FundingCommittee, State of Iowa

Direct: $8,000

Period of Funding: 6/00-5/01

Principal Investigator:Frederick D. Goldman, M.D.

Title: Hydroxychloroquine Therapy and Biology of Chronic GvHD

Source: NIH (RO1)

Direct: $1,700,408 (total direct for the whole project)

Indirect: $162,040 (total direct for the whole project)

Total Period of Funding: $1,862,448 (total for whole project)

Period of Funding: 9/01/00-8/31/06

Principal Investigator: Andrew L. Gilman, M.D.

Co-Principal Investigator: Frederick D. Goldman, M.D

Title: Establishment of Pediatric Immune Disorders Clinic/Peds Immune Disorders Clinic Nurse

Source: Medicaid Administrative Claiming, State of Iowa

Direct: $14,700

Period of Funding: 11/00-6/05

Principal Investigator: Frederick D. Goldman, M.D

Title: Hydroxychloroquine, A New Treatment for Graft-Versus-Host Disease

Source: University of Iowa College of Medicine

Direct: $15,000

Period of Funding: 3/1/99-2/28/00

Principal Investigator: Frederick D. Goldman, M.D.

Title: Chronic Graft-Versus-Host Disease: Analysis of Potential Immunopathogenetic Mechanisms and Therapeutic Strategies

Source: University of Iowa Cancer Center

Direct: $20,000

Period of Funding: 6/99-5/00

Title:Understanding Graft-Versus-Host Disease in Pediatric Patients Undergoing Bone Marrow Transplant

Source: University of Iowa Dance Marathon

Direct: $5,000

Period of Funding: 1999

Principal Investigator: Frederick D. Goldman, M.D.

Title: HydroxychloroquineGraft-Versus-Host Disease, FDR-001420-01-1

Source: FDA

Direct: $4,000

Period of Funding: 1998

Principal Investigator: Frederick D. Goldman, M.D.

Title: Role of T Lymphocyte Specific Protein Tyrosine Kinase p56lck in HIV Immunopathogenesis

Source: University of Iowa, Biosciences Initiative Pilot Grant

Direct: $50,000

Period of Funding: 1997-1998

Principal Investigator: Frederick D. Goldman, M.D.

Title: Graft-Versus-Host Disease in Children Post-Bone Marrow Transplant: Studies of a New Immunosuppressant, Plaquenil

Source: University of Iowa, Children’s Miracle Network

Direct: $22,500

Period of Funding: 4/98-10/99

Principal Investigator: Frederick D. Goldman, M.D.

Title: The Role of p56lck/Cytoskeletal Interactions in HIV Disease

Source: University of Iowa College of Medicine Research Award

Direct: $20,000

Period of Funding: 1997-1998

Principal Investigator: Frederick D. Goldman, M.D

Title: Sequestration of p56lck by CD4 Ligands: A Model for Regulating T cell Activation

Source: American Cancer Seed Grant

Direct: $15,000

Period of Funding: 1995-1996

Principal Investigator: Frederick D. Goldman, M.D.

Title: The role of p56lck and CD45 in gp120-mediated T cell dysfunction

Source: NIH Child Health Research Center New Project

Direct: $90,000

Period of Funding: 8/94-8/97

Principal Investigator: Frank H. Morriss, M.D.

Co-Principal Investigator: Frederick D. Goldman, M.D

Title: gp120 mediated uncoupling of T cell receptor signals

Source: NIH Clinical Investigator Award KO8 AI01039

Direct: $260,000

Period of Funding: 9/91-8/94

Principal Investigator: Frederick D. Goldman, M.D

Title: Institutional Research Training Grant

Source: (National Jewish Center for Immunology and Respiratory Medicine) NRSA A107363

Direct: $64,000

Period of Funding: 9/89-8/91

Principal Investigator: Frederick D. Goldman, M.D.

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**BOOKS**

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61. Barzaghi F., Amaya Hernandez L.C., Ambrosi A., **Goldman F**. et al. IPEX syndrome survivors following different therapeutic strategies: An international multicenter retrospective study, ASBMT, Salt Lake City, UT, Feb 2018
62. Brent Logan, Rebecca Buckley, Elie Haddad, Christopher Dvorak, Richard O’Reilly, Neena Kapoor, Lisa Forbes Satter, Caridad Martinez, Sung-Yun Pai, Jennifer Heimall, Soma Jyonouchi, Kathleen Sullivan, Sharat Chandra, Angela Smith, Sonali Chaudhury, Blachy Davila Saldana, Gauri Sunkersett, David Shyr, Lauri Burroughs, Aleksandra Petrovic, Troy Quigg, Shalini Shenoy, Jeffrey Bednarski II, Kenneth DeSantes, Geoff Cuvelier, Shanmuganathan Chandrakasan, Alred Gillio, Alan Knutsen, Hesham Eissa, **Frederick Goldman**, Theodore Moore. Transplantation Outcomes with Severe Combined Immune Deficiency (SCID) Have Improved over Time: A 36-Year Summary Report by the Primary Immune Deficiency Treatment Consortium (PIDTC), TCT Meetings - Orlando, FL, 2020
63. Daniel B Peavey, Cheryl Fitts, Ameenah Tannehill, Amber T McKell, Tinesha Coleman, Richard Johnson, Janie Roberson, Marisa Marques, Ravi Bhatia, **Frederick Goldman**, Donna Salzman, Antonio Di Stasi. Stability Program for Hematopoietic Progenitor Products from Apheresis (HPC-A) at an Academic Institution, TCT Meetings, Orlando, FL 2020.
64. Aditi Dhir, Hilary Haines, Joseph Chewning, Susmita Murthy, Justin Kim, Lisa Beatty, Emily Mixon, Karen Fowler, Jarrod Gage, Teresa Meadows, **Frederick Goldman**. Incidence and Outcomes for Veno-Occlusive Disease in a Pediatric Transplant Cohort on Retrospective Application of Modified Seattle and EBMT Criteria., TCT Meetings, Orlando, FL 2020.
65. Suk See De Ravin, M.D., Ph.D.1\*, Sandra Anaya O'Brien2\*, Nana Kwatemaa, RN, MSN, CCRP3\*, Narda Theobald, BS4\*, Siyuan Liu5\*, Janet Lee, BS6\*, Lela Kardava7\*, Taylor Liu4\*, **Frederick Goldman, MD, MS8**, Susan Moir9\*, Jack Bleesing, MD, PhD10\*, Benedicte Neven, MD, PHD11\*, Jennifer Puck, M.D.12\*, Morton J Cowan, M.D.13,14, Ewelina Mamcarz, MD15,16, Stephen Gottschalk, M.D.16,17, Michael M Meagher, Ph.D.18\*, Luigi Notarangelo, MD19\*, Elizabeth Kang, MD20, Xiaolin Wu, PhD21\* and Harry L. Malech**, MD**1,22Enhanced Transduction Lentivector Gene Therapy for Treatment of Older Patients with X-Linked Severe Combined Immunodeficiency. ASH, Orlando, FL 2019

**Poster exhibit**

1. Kook H, Trigg ME, Giller R, **Goldman F**, Comito M, Peters C, Padley D. Immune recovery after unrelated or partially matched T-cell depleted marrow transplants in children: Immunophenotypic analysis. Marrow Transplantation in Children: Current Results and Controversies, Hilton Head Island, South Carolina, March, 1994
2. **Goldman F,** Taylor N, Schutte B, Trigg M, Hollenback C, Koretzky G, Ballas Z. Molecular characterization of a unique signal transduction defect in a patient with severe combined immune deficiency. Marrow Transplantation in Children: Current Results and Controversies, Meeting #3, Ft. Lauderdale, Florida, 1997.
3. Pertzborn S, **Goldman FD***,* Browning M. Head and neck manifestations of post-transplant lymphoproliferative disorders. Middle Section Meeting, Triological Society, 1999.
4. Rumelhart S, Giller R, Fleitz J, Ambruso D, Quinones R, Holida M, Lee N, Tannous R, **Goldman F***.*Successful hematopoietic stem cell transplantation for Shwachman-Diamond syndrome. Marrow Transplantation in Children, Meeting #5, Hilton Head Island, South Carolina, 2000.
5. **Goldman F** and Dovat S. Congenital Pancytopenia and Absence of B Lymphocytes in a Neonate with a Mutation in the Ikaros Gene. Pediatric Immune Deficiency Transplant Consortium, San Francisco CA, April 24-26, 2011.
6. Erik Westin, Chao Li, Tim Townes, **Frederick Goldman** Gene correction and induced pluripotent stem cell production using DBA fibroblasts. 13th International Consensus Conference for Diamond Blackfan Anemia, Atlanta, GA, March8-10, 2014
7. Chewning J, ChangCW, DingL, JamayranA, Lai Y, LiC, PawlikP, WestinE,TownesT, and **Goldman F.** Investigation of Omen syndrome with a unique phenotype of T+/B-. Pediatric Immune Deficiency Transplant Consortium, Seattle, WA, May 1-3, 2015
8. Chewning J, Haines H, Buchanan H, **Goldman F**. Severe complications of immune-mediated cytopenias in pediatric primary immune deficiency patients post-HSCT: case report and review of a single center experience. . Pediatric Immune Deficiency Transplant Consortium, Montreal, April 2015.

**ORAL PRESENTATIONS**

**Scientific paper presented at national/international meeting**

1. **Goldman FD**, Hohl RJ, Crabtree J, Koretzky G. Effects of lovastatin on T cell signaling. American Society of Hematology Meeting, Nashville, 1994
2. **Goldman F**, Shutt D, and Soll D. Defective leukocyte motility as a diagnostic tool for Shwachman-Diamond syndrome. 1st Annual Shwachman-Diamond Syndrome Scientific Meeting, 2000.
3. Stepanovic V, Wessels D, **Goldman FD**, Soll DR. The Chemotaxis Defect of Shwachman-Diamond Syndrome Leukocytes. 2nd International Congress on Shwachman-Diamond Syndrome, Toronto, Ontario, Canada, June 16-17, 2003.
4. Bessler M, Bouharich R, Kulkarni S, Freeman S, Du Hong-Yan, Mason PJ, Londono-Vallejo A, **Goldman F**. Accelerated shortening of long telomeres and accumulation of short telomeres in dyskeratosis congenita. 47th American Society of Hematology Meeting, New Orleans, LA, December 2005
5. Filipovich AH, Bleesing J, **Goldman F.** Emerging Targets for Hematopoietic Cell Transplantation (HCT): Genetic Disorders of Hematopoiesis and Immunity. ASBMT, Honolulu, HI, February 2007
6. Heimall J, Logan B, Cowan M, Notarangelo L, Puck J, Fleisher T, Griffith L, Kohn D, Pulsipher M, Shearer W, Hanson I, Kapoor N, O’Reilly R, Hoyer M, Pai SY, Parikh S, **Goldman F**, Burroughs L, Buckley R. Poor T Cell Reconstitution at 100 Days after T Cell-Replete Hematopoietic Cell Transplantation for SCID is Associated with Later Risk of Death or Need for Second Transplant in the 6901 Prospective Study of the PIDTC. ASBMT, presented as Best Pediatric Abstract, Honolulu, HI Feb 2016.
7. Dhir, A, Westin ER, Goldman F. Novel SCIDs phenotype of CD8 lymphopenia with mutation in CD8A gene. PIDTC Workshop, Philadelphia, PA. 2018