

University of California, San Francisco

CURRICULUM VITAE

Name: Mark C. Walters

Position: Jordan Family Director Blood and Marrow Transplantation
Pediatric Hematology Oncology
UCSF Benioff Children's Hospital Oakland

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EDUCATION

1977-1981	University of California, Berkeley	A.B.	Genetics (Honors)
1981-1985	University of California, San Diego	M.D.	Medicine
1985-1988	University of Washington, Seattle, WA		Pediatric Internship and Residency
1988-1992	University of Washington, Seattle, WA Fred Hutchinson Cancer Research Center		Pediatric Hematology Oncology Fellowship

LICENSES, CERTIFICATION

1999	Medical licensure (active), California, Nevada, Rhode Island, Massachusetts, Kentucky, New Jersey
1986	National Board of Medical Examiners, Diplomate
1989	Board Certification, American Board of Pediatrics
1994	Board Certification, Pediatric Hematology/Oncology, renewed 2001, 2008, 2015

PRINCIPAL POSITIONS HELD

1981-1981	University of California, Berkeley Dept. of Chemistry	Research Assistant
1980-1980	NIH-Laboratory of Molecular Genetics	Research Assistant
1982-1982	NIH-Laboratory of Molecular Genetics	Research Assistant
1992-1995	Fred Hutchinson Cancer Research Center Seattle, WA	Associate in Clinical Research
1993-1995	University of Washington, Seattle, WA Department of Pediatrics	Acting Instructor
1993-1999	Odessa Brown Children's Clinic and Children's Hospital, Seattle, WA	Director, Comprehensive Sickle Cell Clinic
1995-1999	Fred Hutchinson Cancer Research Center Seattle, WA	Assistant Member
1995-1999	University of Washington, Seattle, WA Department of Pediatrics	Assistant Professor
1999-2009	Children's Hospital & Research Center,	Director, BMT Program

	Oakland, Oakland, CA	
2009-Present	Children's Hospital & Research Center, Oakland, Oakland, CA	Jordan Family Director, BMT Program
2005-Present	Children's Hospital Oakland Research Institute, Oakland, CA	Scientist
2017-Present	Adjunct Professor of Pediatrics UC San Francisco School of Medicine	Professor
2018 - Present	interim Director of Research UCSF Benioff Children's Hospital, Oakland	

OTHER POSITIONS HELD CONCURRENTLY

2000-2007	UCSF, School of Medicine Department of Pediatrics	Associate Adjunct Professor
2006-Present	ViaCord Processing Laboratory Sibling Connection Program, Hebron, KY	Medical Director
2006-2011	Alternate Member, IRB Committee	
2010-Present	All Cells, Inc.	Medical Director
2011-present	Member, Scholarship Oversight Committee	
2013-present	Co-Chair, CHORI Appointments and Promotions Committee	
2014-2016	Member, CHORI/UCSF Physician Integration Committee	
2014-present	Member, Best Practices Committee	
2016-2018	Member, CHORI Scientific Management Committee	
2015-2016	Task Force Member, UCSF Children's Health Innovations Institute	
2018-present	ACCORD: Advisory Committee for Clinical Research & Development	
2018 - present	Chair, Scientific Advisory Committee, UCSF Benioff Children's Hosp, Oakland	
2018 - present	Chair, Task Force,	

HONORS AND AWARDS *[All inclusive, most recent last]*

	<u>In My Youth Award</u>
1977-1981	(Undergraduate)Phi Beta Kappa, Edward Kraft Prize, President's Undergraduate Fellowship Alumni Scholar, Fr. Woodward Memorial Scholarship
1992-1994	American Society of Hematology, Scholar Award/Jose Carreras Award
1992-1995	American Cancer Society, Career Development Award
1994-1998	NIH Clinical Investigators Development Award (NHLBI)[K08]
1998-1999	Principal Investigator, FIRST award (NHLBI)[R29] R29 HL 60927
1999	Principal Investigator, Globin Gene Regulation by GATA-1 and Chromatin (NHLBI)R01-HL48790

	<u>Middle Age Award</u>
2001-2007	Co- Principal Investigator, SDCB program grant (NHLBI) U01 HL 61877
2001-2007	Principal Investigator, Induction of stable chimerism for sickle cell disease (NHLBI)U01-HL68091
2006-2011	Principal Investigator, Sickle Cell Disease Clinical Research Network (NHLBI) U10-HL083704

KEYWORDS/AREAS OF INTEREST *[Please provide a set of indexing terms to describe your research and clinical interests.]*

Sickle cell disease, thalassemia, non-malignant hematopoietic disorders, stem cell transplantation, cellular therapy, gene therapy, multi-center clinical trials, curative therapy, genomic editing of the sickle mutation in hematopoietic stem cells using the CRISPR/Cas9 ribonucleoprotein system.

PROFESSIONAL ACTIVITIES

PROFESSIONAL ORGANIZATIONS

Memberships *[list all]*

1992-Present American Society of Hematology (ASH)
1999-Present American Society of Blood and Marrow Transplantation (ASBMT)
2006-present Pediatric Blood and Marrow Transplantation Consortium (PBMTC)

Service to Professional Organizations *[list all]*

2005-2009 Non-malignant Diseases Strategy Group, PBMTC, Chair
2005-2010 Non-malignant Diseases Working Group, CIBMTR, Co-chair
2010-present Medical Advisory Board, Cooley's Anemia Foundation
2008-2009 Sickle Cell Disease Clinical Research Network, Co-chair and Executive Committee Member
2013-2016 BMT-CTN Special Populations Committee, Member
2018-2019 2019 BMT Tandem Meetings Scientific Organizing Committee, ASBMT
2017 – 2019 ASH SCD guidelines development committee – stem cell transplantation for SCD
2018 Panel Chair, FDA-ASH Sickle Cell Disease Clinical Endpoints Workshop

SERVICE TO PROFESSIONAL PUBLICATIONS *[list all as appropriate]*

1999-present Ad hoc referee for Blood, Bone Marrow Transplant, Biol Blood and Marrow Transplant, Pediatric Blood Cancer, Pediatric Transplantation, New Engl J Med, Blood Advances Hematologica

INVITED PRESENTATIONS *[list all as appropriate, at least 5 years – do not include titles]*

INTERNATIONAL

2002 Visiting Professor, Ribeirao Preto, Brazil
2003 International UBCT Symposium, LA, CA, invited speaker
Brazil National BMT Meeting, Ouro Preto, Brazil
2004 International UBCT Symposium, LA, CA, invited speaker
2005 EBMT Meeting/Prague, CZ, invited speaker
ACOG Conference, Cabo San Lucas, MX, invited speaker
2006 CHS/CBBMT Plenary Talk, Edmonton, Can, invited speaker
International UCB Symposium, LA, CA, invited speaker
2007 4th Ann Thal Update, Cooley's Anemia Found, Toronto Can
CBT Conference, Rome, Italy, invited speaker
2008 EBMT, Florence, Italy
Canada BMT Group Mtg, Montreal, Canada
Brazil BMT Mtg, Sao Paulo, Brazil
International Thalassemia Conf, Singapore, Malaysia
International Cord Blood Mtg, Cannes, France
2010 Sickle Cell Disease, Cuba
2011 SCD Symposium CHORI, Oakland, CA

- ASBMT Meeting, Honolulu, HA
 CIRM Meeting, Osaka, Japan
- 2012 Thalassemia/SCD Transplant Mtg, Rome, Italy
 SCD Meeting Riyadh Saudi Arabia
 CHORI International SCD Symposium, Oakland, CA
- 2013 Chori SCD International Wrkshp: Alternate Donor HCT for SCD, Oakland CA
 2014 EHA Meeting -Milan Italy
- 2015 Umbilical Cord Blood Mtg, Monaco
 Global Iron Mtg, Berlin, Germany
- 2016 Kiadis Meeting, Amsterdam
 ASH Highlight Meeting, Asia, Brisbane, Australia
 Chandigarh, India; joint US-India hemoglobinopathies Workshop
- 2017 Saudi Scientific Society of Blood & Marrow Transplantation, Riyadh, Saudi Arabia
 2nd Annual Cell & Gene Therapy Symposium, Vellore, India
 European Hematology Assoc. Meeting, Madrid, Spain (oral presentation)
- 2018 European Blood & Marrow Transplant meeting, Lisbon, Portugal (oral presentation)
 HAEMATOCAN – Joint meeting between ASH and 59th Annual conference of the Indian Society of Haematology and Blood Transfusion (ISHBT), Kochi, India
 Innovations in Pediatric Medicine Conference, invited speaker, London
- NATIONAL*
- 2000 Stroke Mtg, NIH, Bethesda, MD, invited speaker
 BMT NIH Conference, Bethesda, MD, invited speaker
 SCD Symposium St Jude CRH, invited speaker
 SCD Conference, Mobile, AL, oral presentation
 NY Methodist SC Symposium, NYC, NY, invited speaker
 National SCD Mtg, Philadelphia, PA, oral presentation
 BMT for SCD Grand Rounds,-COH, Duarte, CA
 Thalassemia Conf, Los Angeles, CA, invited speaker
 ASH, San Francisco, CA, Education Session
- 2001 ASH, Orlando, FL, oral presentation
 Grand Rounds, Kansas City, invited speaker
 Grand Rounds, Univ of Kentucky, Louisville, KY
 SCD Meeting, NYC, NY, invited speaker
 ASBMT/IBMTR, Keystone, CO, invited speaker
- 2002 Visiting professor, CHOC, Irvine, CA
 SCD Symposium, Cincinnati, OH, invited speaker
 TAG Conference, Philadelphia, PA, invited speaker
 Visiting Professor, Children's LA, Los Angeles, Ca
 ASH, Philadelphia, PA, oral presentation
 Amgen Sickle Cell Panel, Thousand Oaks, consultant
 National SCD Meeting, Washington DC, oral presentation
- 2003 Stem Cell Trans in Child, Hilton Head, SC, invited speaker
 ASH, San Diego, CA, Education Session Chair
 Visiting Professor, Children's Memorial, Chicago, IL
 SCD Research Mtgs, Bethesda, MD
 Pedi BMT Consortium, KC, study chair
- 2004 ASH Meeting, San Diego, CA, oral presentation
 NHLBI Study Selection Bethesda, MD, reviewer

Visiting Professor, grand rounds, Cincinnati, OH
 SCD Meeting, Los Angeles, CA, oral presentation
 2005 Cooley's Anemia Meeting, Orlando, FL, invited speaker
 ASBMT Meeting, Keystone/Denver, CO, invited speaker
 National SCD Prog/Cincinnati, OH, invited speaker
 NHLBI Gene Therapy/Bethesda, MD
 Viacell Mtg/Cincinnati, OH
 Univ of Kentucky-Dr. Ildstad Program, invited speaker
 ABCCC Oncology Conf, Half Moon Bay, CA invited speaker
 PBMTC Meeting, Vancouver, BC, Board Member
 PBMTC Retreat, Dallas, TX, Board Member
 ASH Scholarship Review, Washington DC, reviewer
 Columbia University, NYC, invited speaker
 ASH Meeting, Atlanta, GA, invited speaker
 2006 ASBMT Tandem Mtgs, Honolulu, HI
 National SCD Program Mtg, Memphis, TN
 2007 ASBMT Tandem Mtgs, Keystone, CO, invited speaker
 NMDP Lecture, St. Louis, MO, invited speaker
 State of the Science Symp, Ann Arbor, MI, invited speaker
 2008 PBMTC Fall Mtg, Denver, CO
 Cell Therapy: Stem Cell Trans Related/Unrelated Donors, SF, CA
 ASH Mtg, San Francisco, CA
 2009 BBANYS Meeting, NYC, New York
 Cooley's Anemia Mtg, NYC, New York
 2010 SCD Mtg, Florida
 UCB Symposium, San Francisco, CA
 Grand Rounds, Montefiore Childrens, NYC
 NMDP Council Mtg, Minneapolis, MN
 2011 NHLBI Workshop Cellular Therapy, Bethesda, MD
 Stem Cell USA Mtg, Boston, MA
 ASH Mtg, San Diego, CA
 2012 NIH/MCH Study Section, Bethesda, MD
 ASH Meeting
 2013 American Society of Hematology-New Orleans, LA
 Cooley's Anemia Foundation Conference (Philadelphia)
 SCD Meeting, Miami, FL
 Jim Eckman Symposium, Emory-Atlanta, Georgia
 2014 ACBSCT Meeting-Rockville, MD
 ASBMT-Tandem, San Diego
 American Society of Hematology Annual Meeting, San Francisco, CA
 Chori: Thalassemia Family Conference Oakland, CA
 SCD Meeting, Miami
 AABBUcB Symposium - SF
 2015 SCD Research Mtg, Hollywood Florida
 SCD Meeting, Florida
 SCDA Lectures, Baltimore, MD
 Cooley's Anemia Meeting, Chicago
 ASH Annual Meeting, Orlando, FL
 2016 CBBS Meeting San Diego Talk
 Annual Global Healthcare Conference ,NY

- ASBMT Tandem Meeting, Honolulu, HI
 SCD Meeting, Hollywood, FL
 ASPHO LTFU Conference, Minneapolis, MN
 HRSA Advisory Council on Blood Stem Cell Transplant, Bethesda
 NHLBI Sickle Cell Disease Advisory Committee, Bethesda
 Grand Rounds at St. Jude's Children's Hospital, Memphis, TN
 2017 ASBMT Tandem, Orlando, invited speaker
 CBBS meeting, Tahoe, invited speaker
 NHLBI annual SCD clinical research meeting, Bethesda, invited speaker
 University of Pittsburgh, grand rounds
 NHLBI Sickle Cell Disease Advisory Committee, Bethesda
 Foundation for Research in SCD Meeting, Hollywood, Florida
 2018 STELLAR annual advisory board meeting and invited speaker, Emory Univ
 ASBMT Tandem, Salt Lake City, invited speaker pediatric symposium
 Chair, NHLBI Sickle Cell Disease Advisory Committee meeting, Bethesda
 Bioerativ medical advisory board meeting, Bethesda, MD
 16th annual International Cord Blood Symposium (AABB sponsored), San Diego, CA
 Invited speaker, Council Meeting, National Marrow Donor Program, Minneapolis, MN
 ASH satellite symposium, Dec. 2018
 NHLBI annual SCD clinical research meeting, Bethesda, invited speaker, Aug 2018
 Sickle Cell in Focus (SCiF) annual meeting, NIH, Bethesda, invited speaker, Oct 2018

REGIONAL AND OTHER INVITED PRESENTATIONS [past 5 years or as appropriate]

- 2000 ACCP:ACS/BMT Talk, San Francisco, Ca, invited speaker
 Stanford BMT Mtg, Palo Alto, CA, invited speaker
 Cerus Corp Symposium, Santa Cruz, CA, invited speaker
 Reno Grand Rounds, Reno, NV
 2001 Lucile Packard Grand Rds, Palo Alto, CA
 AAP Mtg, San Francisco, CA, invited speaker
 2002 Stanford Pediatrics Grand Rounds, Palo Alto, CA
 Visiting professor, CHOC, Irvine, CA
 Spanish Bay Symposium, Monterey, CA, invited speaker
 Stanford BMT Symposium, Palo Alto, CA, invited speaker
 2004 OB/GYN meeting by UCSF, San Francisco, CA
 John Muir Med Ctr, Walnut Creek, CA, Grand Rounds
 Grand Rounds, Doctors Hosp, Modesto, CA
 UC Davis symposium, Davis, CA
 2006 Health Science Ctr for CME, Oakland, CA, invited speaker
 Resident Noon Conference, Oakland, CA, invited speaker
 Asilomar Stem Cell Retreat, Asilomar, CA
 CHRCO Grand Rounds, Oakland, CA
 CHORI Research Symposium, Oakland, CA
 Stanford BMT Symposium, Palo Alto, CA, invited speaker
 UCSF OB/GYN UCB Talk, San Francisco, CA, invited spkr
 2007 Madera Children's Hospital, Madera, CA, invited speaker
 4th Annual SCD Symposium, Oakland, CA, invited speaker
 CHRCO BMT RN Seminar, Oakland, CA, invited speake
 CHRCO Dinner Lecture Series, Tracy, CA, invited speaker
 CHRCO PICU RN Conference, Oakland, CA, invited speaker

2008 CHRCO VOD Lecture 5 South, Oakland, CA, invited speaker
UCB Stem Cell Retreat, Asilomar, CA
CHORI Research Symposium, Oakland, CA
Stanford Biannual Symposium, Palo Alto, CA
CIRM Gene Therapy Symposium, Los Angeles, CA

2009 Thalassemia Regional Mtg, CHORI, Oakland, CA
CIRM/UCB Stem Cell Retreat, Asilomar, Ca
SCAPHON Mtg, Dana Point, San Diego, CA

2010 Perinatal Med Conf, Coronado, CA
CHORI Resident Research Seminar, Oakland, CA
Stem Cell Retreat, Asilomar, CA
Fetus as Pt Conference, San Diego, Ca
SCD Workshop, CHORI, Oakland, CA

2011 U of Washington Hematology Grand Rounds, Seattle, WA
Healthcare Heroes, San Francisco
Stanford Meeting, Palo Alto, CA
CIRM UCLA Meeting
Berkeley Stem Cell Center Retreat, Asilomar CA
Thalassemia Research Conference, Oakland, CA
TAG Conference, Anaheim, CA

2012 CIRM/UCB Stem Cell Retreat, Asilomar, Ca
CHORI Faculty Fellow Research Symposium
Jean Sanders Retirement Symposium, Seattle, WA
CHRCO Residents Noon Conference, Oakland CA

2013 Chori Annual Faculty-Fellow Symposium Presentation
UCSF Ob/Gyn Mtg- Cord Blood Bank Presentation – SF
George Daley Seminar: Berkeley Stem Cell Center
CIRM Stem Cell Retreat (UCB), Asilomar, CA
Chori Annual Faculty-Fellow Symposium Presentation
UCSF Ob/Gyn Mtg- Cord Blood Bank Presentation (SanFran)

2014 SCD Symposium, CHORI-Oakland, Ca - talk
UCSF Hem/Onc Div Talk-San Francisco, CA
UCSF/CHORI Translational Research Retreat-Oakland, CA
CHRCO Hem/Onc Fellows Conference-Oakland, CA
UCSF-CHRCO PICU Fellows Conference-Oakland, CA
CHLA Talk-Los Angeles, CA
FHCRC Talk – Seattle, WA
Berkeley Stem Cell Center Retreat
Chori: Thalassemia Family Conference Oakland, CA
AABB/UCB Symposium – SF
CIRM Presentation BCL11a – Burlingame

2015 Samuel Merritt RN Students “SCA-Transplant & Donor Pools
UCSF Benioff CHO BMT RN Training (Overview & TBI)
Berkeley Stem Cell Center Asilomar Retreat - Talk
CAR-T Consortium Mtg – Seattle, WA
SSSCR Conference-UC Berkeley, Berkeley, CA
Pacific Sickle Cell Regional Collaborative, Oakland, CA

2016 BMT Nursing Program Presentation, Oakland, CA
AABB Cord Blood Symposium, San Francisco, CA
Chori: Thalassemia Family Conference, Oakland, CA

- 2017 Chori: CIRM Light a Spark to High School Students Summer Program, Oakland, CA
 Reno CME-Hem/Onc Outreach, Reno NV
 HRSA Hemoglobinopathies Regional meeting, Los Angeles, CA
 Siebel Institutes workshop, UC Berkeley/Stanford, Berkeley, CA
 Chori: CIRM Light a Spark to High School Students Summer Program, Oakland, CA
 CRISPRCon, UC Berkeley, invited panelist
 5th Annual Re-writing Genomes Symposim, UC Berkeley/QB3, invited speaker
 Sangamo Biosciences, invited speaker
- 2018 Global Blood Therapeutics, South SF, invited speaker
 CHORI faculty lecture series, Oakland
 CHORI summer student series lecture
 Pediatric Grand Rounds, Oregon Health Sciences Univ, Portland, OR
 UCSF Pediatric Hem/Onc/BMT faculty lecture series, Mission Hall, Aug 2018

GOVERNMENT and OTHER PROFESSIONAL SERVICE *[list all as appropriate, at least 5 yrs]*

- 2004-2008 NHLBI Mentored Scientist Special Emphasis Panel
 2005-2007 ASH Scholar/Junior Faculty Awards
 2012-Present Molecular and Cellular Hematology, NIH study section, ad hoc member
 2013-2018 HRSA Advisory Council on Blood Stem Cell Transplantation, DHHS, Appointee
 2013 U19 NICHD Newborn Screening and genomics study section
 2015(April) BSC site visit reviewer for intramural Hematology Branch, NHLBI
 2015(Aug) SEP for sickle cell disease, 2015/10 ZRG1 VH-J (91) B, NHLBI
 2016- Rapid Assessment Zika SEP for NHLBI
 2016-2020 NHLBI Sickle Cell Disease Advisory Committee (SCDAC), Chair
 2016- NHLBI Working Group on Clinical Trials for Rare Diseases and Therapeutics, Invited Member
 2016 - Advisory Board Member, STELLAR project for long-term followup after BMT for SCD, Emory University
 2018 - Member (pending), Cellular, Tissue and Gene Therapies Advisory Committee (CTGTAC), FDA/CBER.
 2018 SEP for NIH U01 – CIBMTR renewal application – primary reviewer
 2018 Panel Chair, FDA-ASH Sickle Cell Disease Clinical Endpoints Workshop

UNIVERSITY AND PUBLIC SERVICE

- 2006-2009 CIRM T1-0007: UC Berkeley/Children’s Hospital Oakland Research Institute
 Human Stem Cell Training, Clinical Fellowship Program
 2006- present Faculty, Berkeley Stem Cell Program (CHORI)
 2008-2009 CIRM DT1-00697-1: Children’s Hospital Oakland Research Institute/Stanford
 Disease Team Planning Award: Stem Cell Therapy for Sickle Cell Anemia
 2010-2016 CIRM TG2-01164: UC Berkeley/Children’s Hospital Oakland Research Institute
 Interdisciplinary Training in Stem Cell Biology, Engineering and Medicine,
 Clinical Fellowship Program
 2010-2014 CIRM 2301-S-NA586: UCLA/Children’s Hospital Oakland Research Institute
 Stem Cell Gene Therapy for Sickle Cell Consortium
 2014–2016 DeCAL lectures – stem cell transplantation and gene therapy for thalassemia (audit course
 for Cal undergraduates)

- 2017 – Invited participant, Genome Surgery Center, Innovative Genomics Institute, UCB/UCSF Program Director, UCSF alpha stem cell clinic
- 2018 - UC Berkeley Bioengineering Dept, member, medical advisory board
- 2018-9 interim Director of Research, BCHO

PUBLIC SERVICE

SUMMARY OF SERVICE ACTIVITIES *[Please summarize highlights of your service activities since your last advancement; please limit to 1-2 paragraphs.]*

TEACHING and MENTORING

Residency: I have been a regular interviewer for the residency selection program since 2010; typically I interview roughly 10 – 15 candidates per year, generally the applicants who have a background/interest in research.

Fellowship: I have a mentoring relationship with all the hem/onc fellows. With the fellowship leadership and division director, we meet every 6 months with each of the fellow (30 – 60 min face-to-face mtg) during which we review the career trajectory, research project selection and progress, and clinical fund of knowledge/independence.

OTHER COURSES

- 1999-Present CHRCO Hem/Onc Fellowship Training Program,
- 1999-Present CHRCO Resident Training Program
- 2000-Present UCSF Benioff CHO BMT RN Training (Overview & TBI) (Ongoing)
- 2015(April) Samuel Merritt RN Students “SCA-Transplant & Donor Pools,
- 2009-2016 UC Berkeley/CIRM Stem Cell Training Grant Program
- 2006-2013 Faculty Development Committee, CHORI
- 2010- present Scholar Oversight Committee, CHORI

PREDOCTORAL STUDENTS SUPERVISED OR MENTORED *[list all individual students supervised or mentored. Give dates; specify whether undergraduate, medical school, or graduate school; list current position if known; describe Mentoring role, e.g. thesis advisor, personal advisor, research advisor, etc.]*

Dates	Name	Program or School	Role	Current Position
2003-2005	Chris Zamani	Undergrad	BMT for Hg-opathies	Residency Training
2010	Shannon Ong	Undergrad	Research mentor	Medical Residency

POSTDOCTORAL FELLOWS DIRECTLY SUPERVISED OR MENTORED *[list all, defining research or clinical supervision; give dates; list current position if known; describe Mentoring role, e.g. personal advisor, research advisor, career advisor, etc.]*

Dates	Name	Fellow	Faculty Role	Current Position
1996-1999	Michael Bender, MD	Senior Fellow	Clinical Training, sickle Cell Disease	Prof of Peds, Univ WA, Dir. SCD clinic
1999-2001	Jason Fixler, MD	Fellow	Clinical Training, BMT	Attg Hematologist

				Sinai Hosp. Baltimore, MD
2001-2002	Robert Chad Atkins, MD	Fellow	Clinical Training, BMT	Private Practice
2007	Robert Raphael, MD	Fellow	Clinical Training, Epigenetic Research and First author in book chapter	Assoc Hematologist UCSF Benioff Children's Oakland
2007-2008	Wasil Jastaniah, MD	Fellow	Iron Overload after BM and First author	Prof and consultant Jedda Saudi Arabia
2010-2012	Myra Mizokami, MD, PhD	Fellow	Research/Journal auth	Private Practice, Kaiser Permanente
2014-2016	Parul Rai, MD	Fellow	Clin Training/Research mentor/Poster ASPHO mtg 2016	3 rd year Hem/Onc fellow, UCBO
2013-2016	Nahal, Lalefar, MD	Sr. Fellow	CIRM clinical fellow Research mentor	Assoc, Pedi Hem/Onc UCBO
2016 - present	Mark DeWitt, PhD	Post-doc	Post-doc, IGI, UC Berkeley	project manager, CIRM TRAN-1, UC Berkeley
2018	Rofida Nofal, MD	fellow	Fellowship mentor	

INFORMAL TEACHING *[Describe nature of this teaching since last advancement]*

1999-present Attending rounds, Aqua Service (6 – 12 weeks per year)

FACULTY MENTORING *[list all; list the current position of the faculty mentored; list dates; describe Mentoring role, e.g. assigned faculty Mentor, informal career advisor, personal advisor, research/grant review, etc.]*

Dates	Name	Position while Mentored	Mentoring Role	Current Position
2002-2004	John Horan, MD	Jr. Faculty	BMT for hg'pathies/First author article BMT	Prof of Peds, Emory Univ
2003-2003	Robert Iannone, MD	Jr. Faculty	Academic & Research Collaborator/Advisor	Merck & company, Inc.
2000-2003	Ellen Bolotin, MD	Jr. Faculty	Reviewed grant application	Genzyme
2004-2006	Laura Burroughs, MD	Jr. Faculty	Career Advisor	Assoc. Member, Fred Hutch Cancer Research Center
2005-2008	Zahra Pakbaz, MD	Research MD	Research Guide/Journal Auth	Physician UC Davis
2010-2014	Bindu Kanathezath, MD	Jr. Faculty	Pilot Clinical Trial and CIRM Fellow Advisor	Private Practice
2010	Ashutosh Lal, MD	Jr. Faculty	PI, Pilot Trial	Assoc. Hematologist, UCBO

2010-2012	Joanna Halkias, MD	Jr. Faculty	T-32 grantee/CIRM Clinical Fellow Advisor	Jr Faculty, Neonatology, UCBO
2016-Pres	Nahal Lalefar, MD	Jr. Faculty	BMT Associate MD	Assoc Pedi Hem/Onc, UCBO

OTHER VISITING FACULTY SUPERVISED *[list all]*

Wasil Jastaniah, fellow, University of British Columbia (2007-08)

TEACHING AIDS *[Include here, even if listed again below as a formal publication.]*

Clinical Guidelines for BMT – as the BMT program director I conduct a semi-annual review of the clinical guidelines and approve all updates and new procedures. These are reviewed by the clinical fellows during their clinical rotations and are available as a learning resource.

OTHER**TEACHING AWARDS AND NOMINATIONS** *[list all teaching awards, even if already listed above]*

SUMMARY OF TEACHING HOURS *[All faculty are required to summarize their total hours of teaching and mentoring for the previous academic year, total hours of teaching and mentoring expected for the current academic year, and total hours of teaching and mentoring anticipated for the next academic year.]*

BMT guidelines: 75 hours

Inpatient teaching: 25 hours

Fellowship: 25 hours

SUMMARY OF TEACHING ACTIVITIES *[Please include a brief description of your teaching activities since your last advancement, as appropriate, limited to 1-2 paragraphs]*

Residency: During my inpatient rotations (6 – 8 weeks/year), I supervise 2-3 second/third year residents during a busy oncology/BMT rotation. I spend 20 – 30 min every day during rounds on various topics related to BMT and oncology, generally guided by the SOPs for clinical management in BMT that I created for our FACT accredited BMT program. These are distributed and reviewed on rounds with supporting medical literature evidence.

Fellowship: I am responsible for the BMT curriculum during the clinical training that is presented during their orientation and during the course of the initial year of clinical training. This includes didactic presentation during fellows' conference and also informal teaching during their time in BMT clinic and on the 5-South rotation. I also conduct basic training in apheresis and stem cell collections/cellular therapies.

RESEARCH AND CREATIVE ACTIVITIES

RESEARCH AWARDS AND GRANTS *[Current and Pending Grants, Contracts, etc. List grant title and number, your role on the grant (e.g., PI, co-investigator, or consultant.), funding period, source of funding, direct costs for year 1, total direct costs for entire period of grant]*

PHS 398/2590 OTHER SUPPORT

ACTIVE

Tran-1 09292 (Walters)	02/01/2017 – 7/31/2019	3.6 calendar months
CIRM	\$1,779,391	
Curing Sickle cell Disease with CRISPR-Cas9 genome editing		

The major goal of the project is to complete pre-clinical studies leading to a pre-IND meeting that will complete pre-clinical toxicology and clinical ramp-up studies for a project that will apply CRISPR-Cas9 genome editing in human hematopoietic stem cells to correct the sickle mutation. Role: Site PI

5U01HL128566-02 (Walters)	09/01/2015-08/31/2020	1.8 calendar months
NIH/NHLBI	\$183,770	
Hematopoietic Stem Cell Transplantation for Young Adults with Sickle Cell Disease		

The major goal of this clinical trial grant proposal is to conduct a multi-center clinical trial of conventional bone marrow transplantation in young adults with severe sickle cell disease, and to compare outcomes in those eligible for transplantation but who lack a suitable donor. This clinical protocol to treat eligible young adult patients with HLA-ID and unrelated donor bone marrow transplantation after a modified conditioning regimen designed to reduce toxicity in high-risk recipients will establish the efficacy of this treatment and for this indication. Role: Site PI

Protocol HGB-207 (Walters)	03/29/2016 – 12/31/2019	0.24 calendar months
Bluebird Bio, Inc.	\$1,794,870	
A Phase 3, Open Label Study Evaluating the Efficacy and Safety of Gene Therapy in Subjects with β -Thalassemia Major by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with a Lentiviral β A-T87Q-Globin Vector (LentiGlobin BB305 Drug Product) in Subjects ≥ 12 and ≤ 50 Years of Age.		

Multi-center, phase 3 trial of lentiviral gene therapy for thalassemia after conditioning with myeloablative Busulfan in young adults with transfusion-dependent thalassemia major. Role: Site PI

Protocol HGB-206 (Walters)	12/17/2015 – 11/30/2019	0.24 calendar months
Bluebird Bio, Inc	\$867,705	
A Phase 1 Study Evaluating Gene Therapy by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with the LentiGlobin BB305 Lentiviral Vector in Subjects with Severe Sickle Cell Disease.		

Multi-center, phase I-II trial of lentiviral gene therapy for sickle cell disease after conditioning with myeloablative Busulfan in young adults with severe sickle cell disease. Role: Site PI

Protocol HGB-212 (Lal) 8/25/2017 – 11/30/2019 0.24 calendar months

Bluebird Bio, Inc \$527,103
 A Phase 3, Single Arm Study Evaluating the Efficacy and Safety of Gene Therapy in Subjects with Transfusion-dependent β -Thalassemia Major, who have a Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with a Lentiviral β A-T87Q-Globin Vector in Subjects \leq 50 Years of Age

Phase 3, single arm trial of lentiviral gene therapy for β^0/β^0 thalassemia after conditioning with myeloablative Busulfan in patients with transfusion-dependent thalassemia major.

Role: Investigator

Protocol LTF-303 (Walters) 8/21/2017 – 11/30/2019 0.24 calendar months
 Bluebird Bio, Inc \$325,421

Long-Term Follow-Up Of Subjects With Hemoglobinopathies Treated With Ex Vivo Gene Therapy Using Autologous Hematopoietic Stem Cells Transduced With A Lentiviral Vector

Role: Site PI

Protocol GC P#01.01.030 (Walters) 10/21/2015 – 11/30/2019 0.24 calendar months
 Gamida Cell, Ltd \$67,440

Allogeneic Stem Cell Transplantation of CordIn™, Umbilical Cord Blood-Derived Ex Vivo Expanded Stem and Progenitor Cells, in Patients with Hemoglobinopathies.

Role: PI

Protocol ST-400 (Walters) 3/15/18 – 3/14/2021 0.24 calendar months
 Sangamo Biotherapeutics, Inc

A Phase 1/2, Open-label, Single-arm Study to Assess the Safety, Tolerability, and Efficacy of ST-400 Autologous Hematopoietic Stem Cell Transplant for Treatment of Transfusion-dependent β -thalassemia (TDT)

INFR4-10361 (Walters) 12/01/2017 – 11/30/2021 3.6 calendar months
 CIRM \$1,903,253

University of California, San Francisco (UCSF) CIRM Alpha Stem Cell Clinic

The goal of this project is to expand clinical trial activity in cellular therapies. Our specific aims are designed to accelerate the tempo of (1) pre-award planning with sponsors, (2) clinical trial activation, (3) patient accrual and trial completion, to (4) expand access to these therapies by underserved populations with disorders studied by the ASCC network, and to (5) establish a disease-team approach that promotes participation in the CIRM-alpha Network trials. Role: Site PI

PAR-15-172, CTSA U01 (Park) 09/01/2018-08/31/2023 1.2 calendar months
 NIH/NHLBI \$70,714

Accelerate cellular immunotherapy development for treatment of life-threatening childhood disorders

The major goal of this proposal is to develop novel cellular therapies for pediatric disorders with a focus on malignancies Role: Subaward PI

PAST

1. American Society of Hematology, Scholar Award/Jose Carreras Award 1992 - 1994
2. American Cancer Society, Career Development Award, 1992 - 1995
3. K08 NIH Clinical Investigators Development Award 1994 - 1998
NIH/NHLBI
4. Consultant, Newborn Screening Laboratories 1997 - 1999
Washington State Dept of Health
5. Genetics SPRANS Grant Award: (PI Walters, M) 1995 - 1998
DHHS/MCH
Washington State Sickle Cell Disease Program
6. PO1 Investigator and supplement leader, 1997 - 2001
HL36444
Stem Cell Transplantation, Clinical/Basic Research
7. R29-HL60927-01 (PI Walters, M) 1998 - 1999
NHLBI
Mechanisms of Globin Gene Silencing
8. 5R01-HL48790-07, (PI Walters, M) 1999
NIH/NHLBI
Globin Gene Regulation by GATA-1 and Chromatin
9. U01-HL65239-01 (Chair Walters, M) 2000-2004
NHLBI
BMT Committee, Thalassemia Clinical Trial Network
10. U01HL061877-06 (PI Bert Lubin/Project Lead Mark Walters) 01/15/99-08/31/07
NIH/NHLBI \$218,728 direct
Sibling Donor Cord Blood Banking and Transplantation
The major goal of this project is to establish a sibling cord blood program that will collect, characterize and cryopreserve cord blood samples from suitable siblings of children who have disorders treatable by transplantation. The initial phase of this program will be expanded to include an investigation of cord blood transplantation for sickle cell disease and β -thalassemia major that will utilize a novel conditioning regimen to reduce the incidence of graft rejection.
11. U01HL068091-04 (PI Mark Walters) 08/25/01-07/31/07
NIH/NHLBI \$463,391 direct
Induction of Stable Chimerism for Sickle Cell Anemia
The major goal of this proposal is to establish stable donor-host hematopoietic chimerism after non-myeloablative preparation for sickle cell patients who have received few or no RBC transfusions. The proposal, based on supporting pre-clinical and clinical investigations, aims to investigate a modified transplantation procedure in a phase I-II multicenter investigation. It aims to reduce the toxicity of transplant yet retain its therapeutic benefit. If successful, it

might expand the availability of transplantation for patients with clinically significant hemoglobinopathies.

12. 1U01DD000310-01 (PI: Vichinsky, E/Walters, M) 09/30/02-09/29/08
 DHHS/CDC \$161,462 direct
 \$1,133,975 direct/yrs 1-6

Prevention of the Complications of Thalassemia

The major goal of this project is to provide comprehensive care to thalassemia patients and those at risk for thalassemia by incorporating widespread educational and clinical services for the region, including collaborations with satellites, community health agencies and research facilities.

13. CIRM T1-00007 (Mark Walters/Randy Schekman) 4/1/06-12/31/09
 CIRM \$2,360,457 direct/yrs 1-3
Human Stem Cell Training at UC Berkeley and Children's Hospital Oakland

14. 1U10 HL083704-01 (PI Walters, M) 4/1/06-3/31/12
 NIH/NHLBI \$110,000 direct/yr 1
 Co-Chair \$18,091 direct/yr
 \$590,054 direct/yrs 1-6

Northern California Consortium for Sickle Cell Disease

The major goal of this proposal is to participate in a clinical trials network for conducting multicenter clinical trials for sickle cell disease. The Northern California Consortium for Sickle Cell Disease is a cohesive network of regional clinics and comprehensive medical centers in Northern California and Nevada that are committed to providing care for sickle cell disease, and promoting participation in clinical trials. Two clinical investigations are proposed in these applications that include a long-term follow-up evaluation in children treated by conventional bone marrow transplantation, and an investigation to study the utility of RBC transfusions to prevent acute chest syndrome. Additional support was added when Dr. Walters was selected as co-chair of this network.

15. Cord Blood Donation Program (PI: Lubin, B) 06/01/06-05/31/10
 Project Leader/Medical Director ViCord Processing Lab (Walters, M) \$250,000 direct/yr
 ViaCell Corporation

The major goal of this proposal is to act as a national resource to collect, process, characterize and cryopreserve umbilical cord blood units from families that might benefit from this service. We have targeted enrollment of families which children who have disorders that might be treated successfully by hematopoietic cell transplantation, and have facilitated enrollments in all 50 US States. Initially supported as a demonstration project by the NHLBI, currently this program is supported by a partnership agreement with ViaCell Corp and Children's Hospital Oakland Research Institute.

16. 5U01DD000310-02 (PI Vichinsky, E/Walters,M) 09/30/07-09/29/12
 DHHS.CDC \$159,884 direct/yr 1
Prevention of the Complications of Thalassemia \$732,945 direct/yrs 1-5

The major goal of this project is to provide comprehensive care to thalassemia patients and those at risk for thalassemia by incorporating widespread educational and clinical services for the region, including collaborations with satellites, community health agencies and research facilities.

17. DT1-00697-1 (PI Walters, M) 08/01/08-01/31/09
 CIRM Disease Team Planning Award \$55,000/direct/yr 1
Stem Cell Therapy for Sickle Cell Anemia

The major goal is to plan a larger grant application that will propose stem cell research for its therapeutic application in sickle cell anemia. This will be a collaborative effort involving basic, clinical and patient oriented research scientists.

18. 2301-S-NA586 (PI Kohn, Donald/Walters, M) 03/01/10-2/28/14
 CIRM \$82,059 direct/yr 1
Stem Cell Gene Therapy for Sickle Cell Disease \$393,404 direct/yrs 1-5
 This consortium research effort lead by Dr. Kohn is to test the feasibility of replacement gene therapy in individuals with severe sickle cell disease. The initial phases of this study involve the development of a viral transduction vehicle for stem cell insertion, the collection of marrow samples from individuals with sickle cell disease, and in vitro studies of gene expression in hematopoietic progenitor cells. If the pre-clinical studies are promising and achieve benchmark targets for transduction frequency and gene expression, a clinical trial to study the safety of the gene therapy vector will be initiated.
19. TG2-01164 (Mark Walters/Ellen Robey) 01/01/10-6/30/16
 CIRM \$200,100 direct/yr 1
 \$6,901,914 direct yrs 1-6
Interdisciplinary Training in Stem Cell Biology, Engineering and Medicine
20. R341 HL108761-01 (co-PI Krishnamurti, L/Walters, M) 9/1/11-4/30/16
 NIH/NHLBI \$18,848 direct/yr 1
Hematopoietic Cell Therapy for Young Adults with Severe Sickle Cell Disease \$54,854 direct/yrs 1-5
 The major goal of this planning grant proposal is to develop a multi-center clinical trial of conventional bone marrow transplantation in young adults with severe sickle cell disease. This planning grant will develop a clinical protocol to treat eligible young adult patients with HLA-ID and unrelated donor bone marrow transplantation after a modified conditioning regimen designed to reduce toxicity in high-risk recipients. The preliminary data will be used to develop a subsequent comparative transplantation trial for adults with sickle cell disease.
21. R01 R01FD004090-01A1 (co-PI)(PI: Cairo) 4/16/12-8/31/15
 FDA \$26,502 direct/yr 1
Ph2 of T-Cell Depl Familial Haploidentical SCT for tx-Hi-Risk Sickle Cell Anemia \$93,611 direct/yrs 1-4
 The purpose of this investigation is to address a leading gap in the availability of SCT for sickle cell anemia, which is a lack of donors. This study aims to identify a novel conditioning regimen with a focus on patient safety that might ensure engraftment after HLA-mismatched family member donor SCT.
22. Protocol HGB-204 (Walters) 04/11/2013 – 03/31/2018 0.24 calendar months
 Bluebird Bio, Inc. \$1,127,561
A Phase 1/2, Open Label Study Evaluating the Safety and Efficacy of Gene Therapy in Subjects with β Thalassemia Major by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with a Lentiviral β^{AT87Q} Globin Vector (LentiGlobin® BB305 Drug Product)

Multi-center, phase I-II trial of lentiviral gene therapy for thalassemia after conditioning with myeloablative Busulfan in young adults with transfusion-dependent thalassemia major. Role: Site PI

PEER REVIEWED PUBLICATIONS *[All inclusive. Numbered. List publications in chronological order (oldest first). Include full list of authors (no "et al."), full title, and full citation and date. Manuscripts accepted but not yet published can be included as "In Press" after the name of the Journal. Submitted manuscripts may be included; do not list manuscripts in preparation]*

1. **Walters, M.**, Kim, C., and Gelinas, R. Characterization of a DNA binding activity in DNase I hypersensitive site 4 of the human globin Locus Control Region, *Nucl Acids Res* 19:5385-5393, 1991.
2. **Walters, M.**, and Martin, D. Functional erythroid promoters created by interaction of GATA-1 with CACCC and AP-1/NFE-2 elements. *Proc Nat Acad Sci USA* 89:10444-10448, 1992.
3. **Walters M**, Sullivan KM, O'Reilly RJ, Boulad F, Brockstein J, Blume K, Amylon M, Johnson FL, Klemperer M, Graham-Pole J, Appelbaum FR, Hansen JA, Sanders JE, Storb R, and Thomas, E.D. Review of marrow transplantation for thalassemia in the US and Canada, *Am J Pediatr Hem/Onc* 16:11-17, 1994.
4. **Walters, M.C.**, Sullivan, K.M., Bernaudin, F., Souillet, G., Vannier, J.P., Johnson, F.L., Lenarsky, C., Powars, D., Bunin, N., Ohene-Frempong, K., Wall, D., Plouvier, M.E., Bodigoni, P., Lutz, P., Sanders, J.E., Matthews, D.C., Patience, M., Appelbaum F.R., and Storb, R. Neurologic complications after allogeneic marrow transplantation for sickle cell anemia. *Blood* 85:879-884, 1995.
5. **Walters, M.C.**, Fiering, S., Eidemiller, J., Magis, W., Groudine, M., and Martin, D.I.K. Enhancers increase the probability but not the level of gene expression. *Proc Nat Acad Sci, USA* 92:7125-7129, 1995.
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10. Sullivan KM, **Walters MC**, Ohehe-Frempong K. Informed consent for bone marrow transplantation in children (Letter). *N Engl J Med* 335:1845-46, 1996.
11. Francastel, C., Augery-Bouget, Y., Prenant, M., **Walters, M.**, Martin, D.I.K., Robert-Lezenes, J. c-Jun inhibits NF-E2 transcriptional activity by association with p18/maf in Friend erythroleukemia cells. *Oncogene* 14:873-877, 1997.

12. **Walters MC**, Patience M, Leisenring W, Rogers ZR, Dinndorf P, Davies SC, Roberts IAG, Yeager, A, Kurtzberg J, Bunin N, Scott JP, Wall DA, Wayne AS, Wiley J, Darbyshire PJ, Mentzer WC, Smith FO, and Sullivan KM. Collaborative multicenter investigation of marrow transplantation for sickle cell disease: current results and future directions. *Biol Blood Marrow Transplant*, 3:310-315, 1997.
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14. **Walters MC**, Fiering S, Bouhassira EE, Scalzo D, Goeke S, Garrick D, Whitelaw E and D. I.K. Martin . The Chicken β -globin 5'HS4 Boundary Element blocks enhancer-mediated suppression of silencing. *Mol Cell Biol*, 19:3714-26, 1999
15. C Francastel, **MC Walters**, M Groudine and DIK Martin. A Transcriptional Enhancer Suppresses Silencing of a Transgene and Prevents its Localization Close to Centromeric Heterochromatin, *Cell*, 99:259-269, 1999.
16. Lorincz MC, Schübeler D, Goeke SC, **Walters M**, Groudine M, and Martin DIK. Dynamic analysis of proviral induction and de novo methylation: implications for a histone deacetylase-independent, methylation density-dependent mechanism of transcriptional repression. *Mol Cell Biol* 20: 842-850, 2000
17. **Walters MC**, Storb R, Patience M, Leisenring W, Taylor T, Sanders JE, Buchanan GR, Rogers ZR, Dinndorf P, Davies SC, Roberts IAG, Dickerhoff R, Yeager AM, Hsu L, Kurtzberg J, Ohene-Frempong K, Bunin N, Bernaudin F, Wong W-Y, Scott JP, Margolis D, Vichinsky E, Wall, DA, Wayne AS, Pegelow C, Redding-Lallinger R, Wiley J, Klemperer M, Mentzer WC, Smith FO, and KM Sullivan. Impact of bone marrow transplantation for symptomatic sickle cell disease: an interim report. *Blood*, 95: 1918-24, 2000.
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55. Alexis A. Thompson, MD, MPH* **Mark C. Walters, MD***, Janet Kwiatkowski, MD,§ John E. J. Rasko, MBBS, PhD,§ Jean-Antoine Ribeil, MD, PhD,§ Suradej Hongeng, MD, Elisa Magrin, PhD, Gary J. Schiller, MD, Emmanuel Payen, PhD2, Michaela Semeraro, MD, PhD3, Despina Moshous, MD, PhD1,4, Francois Lefrere, MD1, Hervé Puy, MD, PhD5,6, Philippe Bourget, PharmD, PhD1, Alessandra Magnani, MD PhD1, Laure Caccavelli, PhD1, Jean-Sébastien Diana, MD1, Felipe Suarez, MD, PhD1, Fabrice Monpoux, MD7, Valentine Brousse, MD1,5, Catherine Poirot, MD, PhD8, Chantal Brouzes, MD1, Jean-François Meritet, PhD9, Corinne Pondarré MD, PhD10, Yves Beuzard, MD11, Stany Chrétien, PhD2, Thibaud Lefebvre, MD5,6, David T. Teachey, MD,2 Usanarat Anurathapan, MD,7 P. Joy Ho, MBBS, DPhil,5 Christof von Kalle, MD, PhD9 Morris Kletzel, MD,1 Elliott Vichinsky, MD, Sandeep Soni, MD, Gabor Veres, PhD, Olivier Negre, PhD, Robert W. Ross, MD, David Davidson, MD, Alexandria Petrusich, Laura Sandler, MPH, Mohammed Asmal, MD, PhD, Olivier Hermine, MD, PhD1,4,5, Mariane De Montalembert, MD, PhD1,5, Salima Hacein-Bey-Abina, PharmD, PhD1,10,14, Stéphane Blanche, MD, PhD1, Philippe Leboulch, MD,* Marina Cavazzana, MD, PhD* Gene Therapy for Transfusion-Dependent β^- Thalassemia, *N Eng J Med*, 2018 378:1479-1493. doi: 10.1056/NEJMoa1705342..
§ Drs. Kwiatkowski, Rasko and Ribeil contributed equally to this work.
* Drs. Thompson, Walters, Leboulch and Cavazzana contributed equally to this work and are co-corresponding authors, *Gene Therapy for Transfusion-Dependent β^- Thalassemia*
56. Mitchell Cairo, Jessica Hochberg, Stacey Zahler, Mark Geyer, Nan Chen, Jennifer Krajweski, Lauren Harrison, Olga Militano, Mehmet Ozkaynak, Alexandra Cheerva, Julie Talano, Theodore Moore, Alfred Gillio, **Mark Walters**, Lee Ann Baxter-Lowe, and Carl Hamby The safety and efficacy of clofarabine in combination with high-dose cytarabine and total body irradiation myeloablative conditioning and allogeneic stem cell transplantation in children, adolescents and young adults (CAYA) with poor-risk acute leukemia" *Bone Marrow Transplant*. 2018 Jun 13. doi: 10.1038/s41409-018-0247-9..
57. Jennifer Chung*, Wendy Magis*, Jonathan Vu, Seok-Jin Heo, Kirmo Wartiovaara, **Mark C. Walters**, Ryo Kurita, Yukio Nakamura, Dario Boffelli, David I. K. Martin**, Mark A. DeWitt**, Jacob E. Corn1** CRISPR-Cas9 interrogation of a putative fetal globin repressor in human erythroid cells, submitted.

58. Mitchell S. Cairo, MD, Julie-An Talano, MD, Theodore B. Moore, MD, Qiuhi Shi, PhD, Carolyn Keever-Taylor, MD, Rona Weinberg, PhD, Brenda Grossman, MD, **Mark C. Walters**, MD, Elliot Vichinsky, MD, Susan K. Parsons, MD, MRP, Suzanne Braniecki, PhD, Allen J. Dozor, MD, Deborah Friedman, MD, Robert McKinstry, MD, James Verbsky, MD, PhD, Chitti R. Moorthy, MD, Janet Ayello, MS, Mildred Semidei-Pomales, MS, Allyson Flower, MD, Yaya Chu, PhD, Erin Morris, RN, BSN, Harshini Mahanti, MS Sandra Fabricatore, RN, PNP, MSN, Olga Militano, PharmD, Liana Klejmont, PharmD, Carmella van de Ven, MA, Lee Ann Baxter-Lowe, PhD, and Shalini Shenoy, Familial Haploidentical Stem Cell Transplantation in Patients with Sickle Cell Disease, submitted.
59. Catriona H.M. Jamieson, Maria T. Millan, Abba A. Creasey, Geoff Lomax, Mary E. Donohoe, **Mark C. Walters**, Mehrdad Abedi, Daniela A. Bota, John A. Zaia, and John S. Adams. CIRM Alpha Stem Cell Clinics: Collaboratively Addressing Regenerative Medicine Challenges. *Cell Stem Cell* 22:801-805, 2018. <https://doi.org/10.1016/j.stem.2018.05.007>
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NON-PEER REVIEWED PUBLICATIONS AND OTHER CREATIVE ACTIVITIES

[All inclusive. List may be separated into subcategories such as Review Articles, Books and Chapters, Teaching Aids and Other Publications, etc.]

Invited Reviews:

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36. Shalini Shenoy, Javid Gaziev, Emanuele Angelucci, Allison King, Monica Bhatia, Angela Smith, Dorine Bresters, Anne Haight, Christine N. Duncan, Josu de la Fuente, Andrew C. Dietz, K. Scott Baker, Michael A. Pulsipher, **Mark C. Walters**. Late Effects Screening Guidelines after Hematopoietic Cell Transplantation (HCT) for Hemoglobinopathy: Consensus Statement from the Second Pediatric Blood and Marrow Transplant Consortium International Conference on Late Effects after Pediatric HCT. *Biol Blood Marrow Transplant*. 2018 Apr 10. pii: S1083-8791(18)30182-4. doi: 10.1016/j.bbmt.2018.04.002

Book Chapters

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ABSTRACTS (from 2017-18)

Julie-An Talano, MD 1, Theodore B Moore, MD2, Carolyn A. Keever-Taylor, DPhil3, Shalini Shenoy, MD4, **Mark C. Walters, MD**, Susan K Parsons, MD, MRP6*, Allen J Dozor, MD7*, Deborah Friedman, MD8*, Qiuhu Shi, PhD9*, Suzanne Braniecki, PhD7*, Brenda J. Grossman, MD10, Rona Singer Weinberg, PhD11, Elliott Vichinsky, MD12, Yaya Chu, PhD7*, Erin Morris, RN, BSN7*, Sandra Fabricatore, RN, PNP, MSN7*, Janet Ayello, MS, MT(ASCP)7*, Lee Ann Baxter-Lowe, PhD13 and Mitchell S. Cairo, MD
Promising Results at 1 Year Follow-up Following Familial Haploidentical (FHI) T-Cell Depleted (TCD) with CD34 Enrichment and T-Cell (CD3) Addback Allogeneic Stem Cell Transplantation in Patients with High-Risk Sickle Cell Disease, American Society of Hematology Meeting, 2017, 4602a (POSTER)

Janet L. Kwiatkowski, MD1, Alexis A Thompson, MD, MPH2, John Rasko, MBBS, PhD3*, Suradej Hongeng, MD4, Gary J. Schiller, MD5, Usanarat Anurathapan4*, Marina Cavazzana, MD, PhD6,7,8, Phoebe Joy Ho9, Christof von Kalle, MD10*, Morris Kletzel, MD, MBA11, Philippe Leboulch, MD4,12,13*, Elliott Vichinsky, MD14, Briana Deary15*, Mohammed Asmal, MD, PhD16* and **Mark C. Walters, MD**. Clinical Outcomes up to 3 Years Following Lentiglobin Gene Therapy for Transfusion-Dependent β -Thalassemia in the Northstar Hgb-204 Study, American Society of Hematology Meeting, 2017, 360a (ORAL)

Mark C. Walters, MD, Suradej Hongeng, MD2, Janet L. Kwiatkowski, MD3, Franco Locatelli, MD, PhD4, John B. Porter, BA MB BChir MRCP(Path) FRCP(Path) MD5*, Martin G. Sauer6, Adrian J Thrasher, MD, PhD, FMedSci7*, Isabelle Thuret, MD8*, Evangelia Yannaki, MD9*, Heidi Elliot10*, Marisa Gayron10*, Mohammed Asmal, MD, PhD10* and Alexis A Thompson, MD, MPH. Results from the Hgb-207 (Northstar-2) Trial: A Phase 3 Study to Evaluate Safety and Efficacy of Lentiglobin Gene Therapy for Transfusion-Dependent β -Thalassemia (TDT) in Patients with Non- β 0/ β 0, American Society of Hematology Meeting, 2017, 526a. (ORAL)

Julie Kanter, MD, **Mark C. Walters, MD**, Matthew Hsieh, MD3, Lakshmanan Krishnamurti, MD4, Janet L. Kwiatkowski, MD5, Rammurti Kamble, MD6, Christof von Kalle, MD7*, Marcelyne Joseney-Antoine8*, Francis J. Pierciey Jr.8*, Weiliang Shi, PhD8*, Mohammed Asmal, MD, PhD8*, Alexis A Thompson, MD, MPH9 and John F Tisdale, MD. Interim Results from a Phase 1/2 Clinical Study of Lentiglobin Gene Therapy for Severe Sickle Cell Disease, American Society of Hematology Meeting, 2017, 527a. (ORAL)

John F Tisdale, MD, Francis J. Pierciey Jr.2*, Rammurti Kamble, MD3, Julie Kanter, MD4, Lakshmanan Krishnamurti, MD5, Janet L. Kwiatkowski, MD6, Alexis A Thompson, MD, MPH7, Ilya Shestopalov, PhD2*, Melissa Bonner, PhD2*, Marcelyne Joseney-Antoine2*, Mohammed Asmal, MD, PhD2* and **Mark C. Walters, MD**. Successful Plerixafor-Mediated Mobilization, Apheresis, and Lentiviral Vector Transduction of Hematopoietic Stem Cells in Patients with Severe Sickle Cell Disease, American Society of Hematology Meeting, 2017, 990a (POSTER)

Gene Therapy for Hemoglobinopathies **Mark C. Walters, MD**, Hematology/Oncology/BMT, UCSF Benioff Children's Hospital, Oakland, Oakland, CA Thursday, February 22, 2018 (ORAL)

Safety Following Autologous Transplantation with Lentiglobin Gene Therapy for Transfusion-Dependent β -Thalassemia (TDT) in the Northstar Hgb-204 Study **Mark C. Walters, MD1**, Janet L. Kwiatkowski, MD2, John E.J. Rasko, MBBS, PhD3, Suradej Hongeng, MD4, Gary J. Schiller, MD5, Usanarat Anurathapan, MD4, Marina Cavazzana, MD, PhD6, Phoebe Joy Ho, MBBS3, Christof von Kalle, MD7, Morris Kletzel, MD, FAAP, MBA8, Philippe Leboulch, MD9, Elliot P. Vichinsky, MD10, Briana Deary11, Mohammed Asmal, MD, PhD11 and Alexis A. Thompson, MD12, Friday, February 23, 2018 (ORAL)

Single-Agent Plerixafor Mobilization to Collect Autologous Stem Cells for Use in Gene Therapy for Severe Sickle Cell Disease John F. Tisdale, MD1, Julie Kanter, MD2, Matthew Hsieh, MD1, Lakshmanan Krishnamurti, MD3, Janet L. Kwiatkowski, MD4, Rammurti T. Kamble, MD5, Christof von Kalle, MD6, Alexandra Miller⁷, Francis J. Pierciey⁷, Weiliang Shi, PhD⁷, Mohammed Asmal, MD, PhD⁷, Alexis A. Thompson, MD⁸ and **Mark C. Walters, MD⁹**, Wednesday, February 21, 2018 (POSTER)

PATENTS ISSUED OR PENDING (ALLOWED) [*All inclusive; oldest first*]

None

OTHER CREATIVE ACTIVITIES [*list other forms of creative activities such as teaching aids, syllabi, web pages, etc. Dissemination of such creative work is an essential element of criteria for review.*]

SUMMARY OF RESEARCH PROGRAM *Please include a list of five significant recent publications with a description of your role/contribution to each study (one page max). In addition, a one page description of your current research interests/program is required for Ladder Rank, In Residence, Clinical X, and Adjunct faculty. Clinical Faculty should include this description as appropriate.*

Contribution to Science

My initial contributions to this discipline were focused on the transcriptional control of globin gene expression and the role of enhancers and chromatin insulators in establishing and maintaining an erythroid specific pattern of gene expression. This background continues to inform about clinical translation and selecting the best novel candidates for clinical trial development for hemoglobin disorders. More recently, I have returned to laboratory-based translational activity, focused on CRISPR/Cas 9 editing of the sickle allele in hematopoietic stem cells. Pursuing a disease team approach with colleagues at the IGI in Berkeley and at UCLA, I received a CIRM Tran-1 award to complete pre-clinical studies that will lead to an IND and early phase clinical trial.

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2. Walters, M., and Martin, D. Functional erythroid promoters created by interaction of
3. GATA-1 with CACCC and AP-1/NFE-2 elements. Proc Nat Acad Sci USA 89:10444-10448, 1992.
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5. Walters MC, Magis W, Fiering S, Eidemiller J, Scalzo D, Groudine M, and DIK Martin. Transcriptional enhancers act in cis to suppress position-effect variegation. Gene Dev 10: 185-196, 1996
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9. Mark A. DeWitt, Wendy Magis, Nicolas L. Bray, Tianjiao Wang, Jennifer R. Berman, Fabrizia Urbinati, Seok-Jin Heo, Denise P. Muñoz, Dario Boffelli, Donald B. Kohn, **Mark C. Walters**, Dana

Carroll*, David K. Martin*, and Jacob E. Corn*. Scarless Correction of the Sickle Mutation in Human Hematopoietic Stem Cells Using a Cas9 Ribonucleoprotein Complex, *Sci Transl Med.* 2016;8(360):360ra134.

10. Jennifer Chung*, Wendy Magis*, Jonathan Vu, Seok-Jin Heo, Kirmo Wartiovaara, **Mark C. Walters**, Ryo Kurita, Yukio Nakamura, Dario Boffelli, David I. K. Martin**, Mark A. DeWitt**, Jacob E. Corn1** CRISPR-Cas9 interrogation of a putative fetal globin repressor in human erythroid cells, submitted.

I have also focused on alternative sources of hematopoietic cells for clinical application, particularly umbilical cord blood, and more recently, gene therapy trials for thalassemia and SCD. This has the potential advantage of mitigating the incidence and severity of graft-versus-host disease, a leading cause of morbidity and mortality after clinical transplantation for sickle cell disease. My work has focused on cord blood banking and conducting clinical trials.

1. W Reed, R Smith, F Dekovic, JY Lee, JD Saba, E Trachtenberg, J Epstein, S Haaz, **MC Walters**, and BH Lubin. Comprehensive Banking of Sibling Donor Cord Blood for Children with Malignant and Non-Malignant Disease, *Blood*, 101: 351-357, 2003.
2. F Locatelli, V Rocha, W Reed, F Bernaudin, M Ertem, S Grafakos, B Brichard, X Li, A Nagler, G Giorgiani, P Haut, JA Brochstein, DJ. Nugent, J Blatt, P Woodard, J Kurtzberg, R Miniero, P Lutz, T Raja, I Roberts, AM Will, I Yaniv, C Vermynen, N Tannoia, F Garnier, I Ionescu, **MC Walters**, BH Lubin, E Gluckman on behalf of the Eurocord transplant group. Related umbilical cord blood transplant in patients with Thalassemia and Sickle Cell Disease, *Blood*, 101: 2137-2143, 2003.
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I have devoted my investigative career to pursuing curative therapies for hemoglobin disorders with an overarching goal of expanding this treatment more broadly to affected individuals. With NIH and industry support, I have conducted a number of multicenter clinical trials for sickle cell disease and thalassemia. Currently, we are focused on initiating early phase clinical trials to test novel genomic editing techniques in hematopoietic stem cells from individuals affected by hemoglobin disorders, starting with a high-profile trial in sickle cell disease. Other projects in the pipeline include activation of fetal hemoglobin by genomic editing in stem cells that might be applied more universally to all hemoglobin disorders regardless of genotype and in utero transplantation for alpha thalassemia major. We recently received a large CIRM grant award to pursue pre-clinical development of a Cas9 directed genomic correction of the sickle allele in hematopoietic stem cells, for which I am the principal investigator. Second, I am co-PI of two BMT – CTN trials, one funded under a MPI U01 mechanism through NHLBI (STRIDE). These studies aim to broaden the application of conventional HCT for hemoglobin disorders by comparing HLA-identical and well-matched URD bone marrow transplant to a cohort of non-transplant subjects who lack a donor and to test HLA-haploidentical HCT in children and adults with severe sickle cell disease. Finally, I have worked with industry sponsors and taken a lead investigator role in an early phase clinical trials of gene therapy for thalassemia major and SCD (bluebird bio, Inc). I am also the clinical lead investigator of a planned trial to inactivate *bcl11a* in hematopoietic stem cells to re-activate fetal hemoglobin expression in subjects with thalassemia major (Sangamo, Bioverativ). These trials will be supported in part by a large infrastructure grant award from CIRM, the UCSF alpha stem cell clinic, for which I am the program director.