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 CRSPR/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)
HAEMATOCON – 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
SCDAA Lecture, 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
STELLAR annual advisory board meeting and invited speaker, Emory Univ

2018 ASBMT Tandem, Salt Lake City, invited speaker pediatric symposium
Chair, NHLBI Sickle Cell Disease Advisory Committee meeting, Bethesda
Bioverativ 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

	CHRCO VOD Lecture 5 South, Oakland, CA, invited speaker
2008	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 Hemtology 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

	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
2017	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 ar 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	3rd 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 Positic
2002-2004	John Horan, MD	Jr. Faculty	BMT for hg'pathies/First auth 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 Kanathezhath, 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 βA-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 disase 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 βA-T87Q-Globin Vector in Subjects ≤50 Years of Age

Phase 3, single arm trial of lentiviral gene therapy for β°/β°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

<u>PAST</u>

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
Northern California Consortium for Sickle Cell Disease $590,054 direct/yrs 1-6

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 $393,404direct/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. Ifthe 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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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.

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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

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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.

18. Reed W, **Walters M**, Lubin BH. Collection of sibling-donor cord blood for children with thalassemia. J Pediatr Hematol/Onc, 22:602-604, 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 HambyThe 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..

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58. Mitchell S. Cairo, MD, Julie-An Talano, MD, Theodore B. Moore, MD, Qiuhu 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, Abla 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:

1. **Walters, M**., Matthews, D., and Sullivan, K. Bone marrow transplantation for hemoglobinopathy and sickle cell disease, Recent Advances in Haematology 7, Churchill Livingstone, Inc., NY, 119-134, 1993.

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Consortium International Conference on Late Effects after Pediatric HCT, Biol Blood Marrow Transplant, 2017 23:552-61.

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Book Chapters

1. **Walters MC**. Stem cell transplantation for sickle cell disease; in Hematopoietic Cell Transplantation, Second Edition, eds. SJ Forman, KG Blume and ED Thomas. Blackwell Science, Malden, MA, 1999.

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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-β0/β0, 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 CRSPR/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.
4. 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.
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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7. 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.
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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 Vermylen, 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.