

## **CURRICULUM VITAE**

**David A. Wilcox PhD**

**Professor**

**Department of Pediatrics**

**Division of Hematology and Oncology - Pediatrics**

### **OFFICE ADDRESS:**

MACC Fund Research Center  
8701 Watertown Plank Rd  
Milwaukee, WI 53226

### **EDUCATION:**

1987 BS Chemistry, University of Wisconsin, Platteville, WI  
1994 PhD, Cell Biology, Medical College of Wisconsin, Milwaukee, WI

### **POSTGRADUATE TRAINING AND FELLOWSHIP APPOINTMENTS:**

06/1986 - 08/1986 Summer Intern, Wisconsin State Crime Lab, University of Wisconsin, Milwaukee, WI  
07/1986 Arson Investigator Training Program, FBI Bomb Data Program, Racine, WI  
06/1987 - 06/1988 Volunteer, Emergency Room, St. Mary's Hospital, Racine, WI  
06/1988 - 09/1988 Research Assistant, Psychiatry, Medical College of Wisconsin, Milwaukee, WI  
09/1988 - 08/1989 Research Assistant, Cellular Biology and Anatomy, Medical College of Wisconsin, Milwaukee, WI  
09/1989 - 06/1994 Graduate Student, Cellular Biology and Anatomy, Medical College of Wisconsin/Blood Research Institute of the BloodCenter of Wisconsin, Milwaukee, WI  
07/1994 - 04/1999 Postdoctoral Fellow, Medicine and Pharmacology, Medicine and Pharmacology, University of North Carolina, Chapel Hill, NC

### **FACULTY APPOINTMENTS:**

1999 - 2005 Assistant Professor, Pediatrics, Hematology/Oncology/BMT, Medical College of Wisconsin, Milwaukee, WI  
1999 - Present Associate Investigator, Blood Research Institute, The BloodCenter of Wisconsin, Milwaukee, WI  
2005 - 2008 Research Associate Professor, Pediatrics, Medical College of Wisconsin, Milwaukee, WI  
2008 - Present Associate Professor, Pediatrics, Hematology/Oncology/BMT, Medical College of Wisconsin, Milwaukee, WI  
2012 - Present President and Founder, Platelet Targeted Therapeutics, Brookfield, WI  
2016 - Present Adjunct Investigator, Blood Research Institute, Blood Center of Wisconsin, Milwaukee, WI  
2018 - Present Member, Discovery & Developmental Therapeutics Research Program, MCW Cancer Center, Milwaukee, WI 53226  
2023 - Present Professor in Research Track, Department of Pediatrics, Medical College of Wisconsin, Milwaukee, WI

### **AWARDS AND HONORS:**

1992 Travel Award, American Heart Association  
1993 Travel Award, American Society of Hematology  
1994 - 1995 National Research Service Award, National Institutes of Health  
1996 Travel Award, American Society of Hematology  
1997 Travel Award, American Society of Hematology  
1998 Travel Award, American Society of Hematology  
1999 Young Investigator Award, International Society for Thrombosis and Haemostasis

2003 Invited Session Chair, International Society of Thrombosis and Haemostasis  
03/2005 Invited Discussion Leader, Gordon Research Conference on Cell Biology of Megakaryocytes and Platelets, Santa Barbara, CA  
02/2011 Best Abstract in Basic Research, American Society for Bone Marrow Transplant, Honolulu, HI  
09/2019 MCW Longevity Award for 20 years of service to the College, Medical College of Wisconsin

#### **MEMBERSHIPS IN HONORARY AND PROFESSIONAL SOCIETIES:**

American Society of Gene Therapy (ASGCT) (Member)  
American Heart Association (Scientific Council Member)  
Royal Society of Medicine-Overseas Fellow (Member)  
International Society of Thrombosis and Haemostasis (ISTH) (Member)  
American Society of Hematology (ASH) (Member)

#### **EDITORSHIPS/EDITORIAL BOARDS/JOURNAL REVIEWS:**

##### **Editorial Board**

1999 - Present Thrombosis and Haemostasis, Scientific Reviewer  
2002 - Present Journal of Thrombosis and Haemostasis, Scientific Reviewer  
2003 - Present Blood, Scientific Reviewer  
2003 - Present Biology of Blood and Marrow Transplantation, Scientific Reviewer  
2006 - Present Translational Research, Scientific Reviewer  
2007 - Present Platelets, Scientific Reviewer  
2008 - Present ILAR Journal, Scientific Reviewer  
2009 - Present Human Gene Therapy, Scientific Reviewer  
2010 - Present Gene Therapy, Scientific Reviewer  
2011 - Present Haemophilia, Scientific Reviewer  
2011 - Present Thrombosis Research, Scientific Reviewer  
2011 - Present Curator, Mt. Sinai School of Medicine/Glanzmann Thrombasthenia Database  
2011 - Present Curator, U of Cambridge Thrombogenomics Database for Glanzmann Thrombasthenia  
2011 - Present Proceedings of The National Academy of Sciences, Scientific Reviewer  
2012 - Present PLOS, Scientific Reviewer  
2012 - Present Indian Journal of Sexually Transmitted Diseases and AIDS, Scientific Reviewer  
2012 - Present European Journal of Human Genetics, Scientific Reviewer  
2013 - Present Haematologica Journal, Scientific Reviewer

##### **Journal Review**

2016 - Present Arteriosclerosis, Thrombosis, and Vascular Biology, Scientific Reviewer  
2016 - Present American Journal of Hematology, Scientific Reviewer  
2016 - Present Blood Advances, Scientific Reviewer

#### **LOCAL/REGIONAL APPOINTED LEADERSHIP AND COMMITTEE POSITIONS:**

2012 - 2016 Co-Chairman, Institutional Biosafety Committee and Joint Safety Committee, Medical College of Wisconsin

#### **NATIONAL ELECTED/APPOINTED LEADERSHIP AND COMMITTEE POSITIONS:**

2004 Ad Hoc Reviewer, Study Section for Hemostasis and Thrombosis, National Institute of Health (NIH)  
2005 Ad Hoc Reviewer, Haemophilia Research United Kingdom  
2006 Ad Hoc Reviewer, Association Francaise Contre Les Myopathies, France  
2007 Ad Hoc Reviewer, Study Section for Hemostasis and Thrombosis, National Institute of Health (NIH)  
2007 Ad Hoc Reviewer, Study Section for Clinical Hematology, National Institute of Health (NIH)  
2008 Ad Hoc Reviewer, Study Section for Hemostasis and Thrombosis, National Institute of Health (NIH)  
2009 Ad Hoc Reviewer, Study Section for Hemostasis and Thrombosis (Stimulus Grants), National Institute of Health (NIH)  
2009 Ad Hoc Reviewer, Study Section for Hemostasis and Thrombosis (Feb., National Institute of Health (NIH))  
2010 Collaborator, Thrombogenomics Committee, Glanzmann Thrombasthenia Section  
03/2011 Abstract Reviewer, International Society of Thrombosis and Haemostasis  
07/2011 Ad Hoc Reviewer, Study Section for Viruses and Immunity PPG, National Institute of Health (NIH)  
10/2011 Ad Hoc Reviewer, Study Section for Hemostasis and Thrombosis, National Institute of Health (NIH)

2013 Abstract Reviewer, American Society of Gene and Cell Therapy  
 2013 Abstract Reivewer, Iternational Society of Thrombosis and Haemostasis  
 2013 Ad Hoc Reviewer, Study Section for Vascular Biology, National Institute of Health (NIH)  
 2014 Ad Hoc Reviewer, Study Section for Vascular Biology & Hematology, National Institute of Health  
 2015 Ad Hoc Reviewer, Bioengineering Sciences & Technologies IRG (July), NIH Special Emphasis Panel  
 2015 Consultant, Meeting On Lentiviral Gene Therapy, With An Additional Focus On Gene Therapeutic  
 Treatment Options For Sickle Cell Disease And ? Thalassemia (May), Baxter Healthcare Advisory  
 Board  
 2020 Ad Hoc Reviewer, NIH-NHLBI Special Emphasis Panel: TAG Therapeutic Approaches to Gene  
 Expression, NIH-NHLBI  
 2020 Ad Hoc Reviewer, NIH-NIGMS Special Emphasis Panel; Support of Competitive Research (SCORE)  
 program, supports research at minority serving institutions, NIH-NIGMS  
 2022 Ad Hoc Reviewer, Therapeutic Approaches to Genetic Diseases (TAG) study section (March), NIH-  
 NHLBI

## RESEARCH GRANTS/AWARDS/CONTRACTS/PROJECTS:

### Active

#### Peer Review

Title:	Core Clinical Centers for the Blood and Marrow Transplant Clinical Trials Network
Source:	NIH-NHLBI RFA-HL-17-018 (1UG1HL138641-01 (Hari, P, PI)
Role:	Key Personnel (Project Director for Platelet FVIII Gene Therapy for Hem A)
PI:	Hari, P
Dates:	07/2017 - 06/2022
Title:	A Phase I Clinical Trial Testing Feasibility of Hematopoietic Stem Cell Gene Therapy Using Platelet Factor VIII to Safely Improve Hemostasis for Severe Hemophilia A with Inhibitory Antibodies to FVIII (Scored in Top 5%)
Source:	NIH-NHLBI 1 R01 HL142791-01 A1
Role:	Co-PI
Dates:	04/2019 - 03/2024
Title:	RCL Testing by the National Gene Vector Biorepository
Source:	NIH-NHLBI Contract 75N92019D00018
Role:	Co-PI
Dates:	10/2019 - 12/2023

#### Non-Peer Review

Title:	Glanzmann Thrombasthenia Research 2021
Source:	Children's Research Institute/Children's Hospital of WI
Role:	Principal Investigator
PI:	David Wilcox, PhD
Dates:	01/01/2021 - 12/31/2022

### Prior

#### Peer Review

Title: Cell & Molecular Biology of the Human Platelet Fibrinogen Receptor  
Source: American Heart Association - Wisconsin Affiliate  
Role: Predoctoral Fellowship (90% effort)  
Dates: 07/01/1992 - 06/30/1993  
Direct Funds: \$20,000

Title: Cell & Molecular Biology of the Human Platelet Fibrinogen Receptor  
Source: American Heart Association - Wisconsin Affiliate  
Role: Predoctoral Fellowship (90% effort)  
Dates: 07/01/1993 - 06/30/1994  
Direct Funds: \$20,000

Title: Platelet-Specific Expression of a Functional Human Fibrinogen Receptor  
Source: American Heart Association - North Carolina Affiliate  
Role: Postdoctoral Fellowship (% 100 effort)  
Dates: 07/01/1995 - 06/30/1997  
Direct Funds: \$60,000

Title: Molecular and Cellular Mechanisms in Transfusion Medicine  
Source: NIH NHLB HL44612-15  
Role: Collaborator, Program Project Grant (10% effort)  
Dates: 05/01/2000 - 04/30/2005  
Direct Funds: \$32,000

Title: Therapeutic Expression of a Platelet-Specific Integrin  
Source: Medical College of Wisconsin New Faculty Grant  
Role: Principle Investigator (50% effort)  
Dates: 05/25/2001 - 04/30/2002  
Direct Funds: \$12,500

Title: Therapeutic Expression of a Platelet-Specific Integrin  
Source: American Heart Association - Northland Affiliate  
Role: Principal Investigator, Beginning Grant-in-Aid (50% effort)  
Dates: 07/01/2001 - 06/30/2003  
Direct Funds: \$80,000 (Funding Relinquished 4/1/02, Due to Overlap with NIH Grant)

Title: Lineage-Specific Gene Expression  
Source: Children's Hospital Foundation  
Role: Principal Investigator (50% effort)  
Dates: 07/01/2001 - 06/30/2003  
Direct Funds: \$66,000

Title: Therapeutic Expression of a Platelet-

Source:	Specific Integrin (Years 1-5) NIH NHLB R01-068138
Role:	Principal Investigator (50% effort)
Dates:	04/01/2002 - 06/30/2007
Direct Funds:	\$700,000 (\$250,000/year In-No-Cost-Extension)
Title:	Structure and Function of TSP1 in Acute Lung Injury
Source:	NIH NHLB R01-071618
Role:	Collaborator (10% effort)
Dates:	11/01/2004 - 10/31/2008
Direct Funds:	\$32,000 (Funding Relinquished 11/01/04, PI Relocated to University of Colorado)
Title:	Molecular and Cellular Mechanisms in Transfusion Medicine
Source:	NIH NHLB HL44612-15
Role:	Collaborator, Program Project Grant (5% effort)
Dates:	12/01/2005 - 11/30/2010
Direct Funds:	\$16,000
Title:	Platelet-Specific Gene Therapy and its Potential for Correcting Hemophilia
Source:	American Heart Association - Greater Midwest Affiliate
Role:	Grant-in-Aid (20% effort)
Dates:	07/01/2007 - 06/30/2009
Direct Funds:	\$132,000
Title:	Therapeutic Expression of a Platelet-Specific Integrin
Source:	NIH NHLB R01-068138
Role:	Principal Investigator (40% effort)
Dates:	07/01/2007 - 06/30/2012
Direct Funds:	\$1,385,587
Title:	Translating Pre-Clinical Platelet-Targeted Treatment Protocols Suitable for use in Human Gene Therapy Clinical Trials
Source:	NIH NHLB 00085/Wilcox
Role:	Principal Investigator
Dates:	02/14/2014 - 01/14/2015
Direct Funds:	\$150,000
Title:	Platelet-Targeted Gene Therapy for Hemophilia A
Source:	NIH-NHLBI-RSA Gene Therapy Resource Program RFA 1253
Role:	Principal Investigator (0% effort)
Dates:	07/01/2014 - 06/30/2015
Direct Funds:	\$70,000
Title:	Platelet-Targeted Gene Therapy for

Source: Hemophilia A  
NIH-NHLBI-RSA Gene Therapy  
Resource Program RFA 1253 (Renewal)  
Role: Principal Investigator  
Dates: 04/07/2016 - 06/30/2017  
Direct Funds: \$260,000 (Indiana University Vector  
Production )

Title: Pilot and Feasibility Study of  
Hematopoietic Stem Cell Gene Transfer  
to Utilize Platelet-Derived Factor VIII  
for Hemophilia A: Transduction process  
development

Source: NIH-NHLBI-RSA-Growing Gene &  
Cell Therapy (GGACT) Cooperative  
Project Support Consortium  
Role: Project PI (U01 TR0018414, Williams et  
a., PI) Goal: Receive advisory assistance  
from Gene Therapy Cooperative to  
Advance Gene Therapy for Hemophilia  
A with Platelet FVIII to first in human  
phase I clinical trial  
PI: Williams et a.  
Dates: 05/2017 - 06/2021

**Non-Peer Review**

Title: Glanzmann Thrombasthenia Research  
Source: Glanzmann's Research Foundation  
Role: Principal Investigator  
Dates: 07/01/2001 - 06/30/2011  
Direct Funds: \$189,000 (\$10,000/year)

Title: Glanzmann Thrombasthenia and  
Hemophilia A Human and Animal  
Research  
Source: Children's Research Institute (Private  
Donor work Specific for Wilcox Lab)  
Role: Principal Investigator  
Dates: 2008 - 2015  
Direct Funds: \$1,800,000 (\$350,000/year)

Title: Glanzmann Thrombasthenia Research  
Source: Cure Glanzmann's Foundation  
Role: Principal Investigator  
Dates: 07/01/2010 - 06/30/2011  
Direct Funds: \$4,410 (\$1000/year)

Title: Lineage Specific Gene Expression  
Source: MACC Fund  
Role: Principal Investigator  
Dates: 07/01/2010 - 06/30/2012  
Direct Funds: \$832,159 (\$81,184.69/year)

Source: Children's Research Institute  
Dates: 2015 - Present  
Direct Funds: \$638,806

Source:	Children's Research Institute
Dates:	2016
Direct Funds:	\$726,282
Title:	Glanzmann Thrombasthenia Research 2020
Source:	Children's Research Institute/Children's Hospital of WI
Role:	Principal Investigator
PI:	David Wilcox, PhD
Dates:	01/2020 - 12/2020

## INVITED LECTURES/WORKSHOPS/PRESENTATIONS:

### National

- Invited Presentation An amino acid substitution within the fourth calcium-binding region of GPIIb results in degradation of the integrin GPIIb-IIIa and type I glanzmann thrombasthenia, Annual Meeting of the American Heart Association., 1992
- Invited Presentation Glanzmann thrombasthenia resulting from a single amino acid substitution flanking the fibrinogen  $\gamma$ -chain dodecapeptide-binding domain on GPIIb, Annual Meeting of the American Society of Hematology., 1993
- Invited Presentation Of mice and men: detection of a functional murine  $\alpha$ IIb $\beta$ 3 heterodimer complex on the surface of megakaryocytes derived from retrovirus transduced bone marrow cells from  $\alpha$ 3-knockout mice, Annual Meeting of the American Society of Hematology., 1998
- Invited Presentation Use of  $\alpha$ IIb Promoter in Retroviral Constructs, Gordon Research Conference on Hemostasis, Plymouth, NH, 07/2000
- Invited Presentation, Targeting Gene Therapy for Inherited Hematological Disorders and The Molecular Basis for Gene Therapy Using Hematopoietic Stem Cells, BMS Seminar/Goodwin Lecturer, Auburn University, AL, 04/2002
- Invited Presentation EM localization and agonist-induced release of human FVIII from megakaryocytes transduced with a FVIII transgene, 45th Annual Meeting of the American Society of Hematology, San Diego, CA, 12/2003
- Invited Presentation Therapeutic expression of a platelet-specific integrin  $\alpha$ IIb $\beta$ 3, Annual Meeting of the American Society for Gene Therapy., 2003
- Invited Scientific Review, NIH Hemostasis and Thrombosis Study Section, 2004
- Invited Discussion Leader, Gordon Research Conference on Cell Biology of Megakaryocytes and Platelets, Santa Barbara, CA, 03/2005
- Invited Presentation, Therapeutic expression of  $\alpha$ IIb $\beta$ 3 in murine and canine models of Glanzmann thrombasthenia Subcommittee Meeting on Platelets, Annual Meeting of the American Society of Hematology, Atlanta, GA, 12/2005
- Invited Presentation Therapeutic expression of a platelet-specific integrin restores hemostasis in dogs with glanzmann thrombasthenia, Annual Meeting of the American Society of Gene Therapy, Seattle, WA, 06/2007
- Invited Presentaion Gene Therapy for Inherited Platelet Bleeding Disorders, Department of Biological Sciences Colloquim Series, University of Wisconsin-Milwaukee, 02/2008
- Invited Presentation Hematopoietic Stem Cell Gene Therapy for Inherited Platelet Bleeding Disorders, Cincinnati Children's Hospital Medical Center, 03/2008
- Invited Presentation Targeting Therapeutic Agents to Platelets, Novo Nordisk Corporation, East Brunswick, New Jersey, 04/2008
- Invited Presentation Gene Therapy for Platelets Disorders, Platelets 2008 International Symposium, Woods Hole, Massachusetts, 10/2008
- Invited Presentation Department of Medicine, Rockefeller University, 11/2008
- Invited Presentation Special Symposium for Thrombosis & Hemostasis, American Society of Hematology Annual Meeting, San Francisco, CA, 12/2008
- Invited Presentation US House of Representatives, Congressman Gingrey R-Georgia and Deputy Director NHLBI-NIH, Dr. Susan Shurdin, 06/2009
- Invited Presentation, MACC Fund Scientific Symposium, Wisconsin Institute for Medical Research, 12/2009

Invited Presentation Division of Hematology, 2010-2011 Seminars at The Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, 01/2011

Invited Presentation Best Abstract in Basic Research, American Society for Bone Marrow Transplant, Honolulu, HI, 02/2011

Invited Presentation Seminar, Auburn University, Auburn, AL, 03/2011

Invited Presentation Seminar, Transfusion Medicine Grand Rounds at the Brigham and Women's Hospital and Children's Hospital Boston, Boston, Massachusetts, 11/2011

Invited Presentation Seminar, Indiana University School of Medicine, Indiana, 12/2011

Invited Participant, NHF's 11th Workshop on New Technologies and Gene Transfer for Hemophilia, Philadelphia, PA, 03/2012

Invited Presentation Emory University School of Medicine, Atlanta, GA, 11/2012

Invited Participant, XXIVth Annual Hemophilia Research Update, Baxter, Washington DC, 03/2014

Invited Presentation, National Hemophilia Foundation's 12th Workshop on New Technologies and Gene Transfer for Hemophilia Georgetown University, Washington DC, 10/2014

Invited Presentation Gene Therapy for Inherited Bleeding Disorders: From Bench to Bedside, Baxter/Baxalta HealthCare, Deerfield, IL, 07/2015

Invited Presentation, National Hemophilia Foundation's 13th Workshop on New Technologies and Gene Transfer for Hemophilia Georgetown University, Washington DC, 10/2016

Invited Presentation, American Society for Gene and Cell Therapy on Hematopoietic Stem Cell Gene Therapy For Hemophilia A with Platelet Derived FVIII Pleightlet, Growing in Gene and Cell Therapy Committee, Chicago, IL, 05/2018

Invited Session Moderator Men's Issues with GT, Glanzmann Thrombasthenia Symposium, Austin, TX, 04/2019

Invited Presentation Future of GT Research, Glanzmann Thrombasthenia Symposium, Austin, TX, 04/2019

Invited Presentation, American Society for Gene and Cell Therapy on Hematopoietic Stem Cell Gene Therapy For Hemophilia A with Platelet Derived FVIII Pleightlet, Growing in Gene and Cell Therapy Committee, Washington DC, 05/2019

Invited Presentation, National Hemophilia Foundation's 15th Workshop on New Technologies and Gene Transfer for Hemophilia, Georgetown University, Washington DC, 09/2019

Invited Presentation, National Hemophilia Foundation's 71th Annual Bleeding Disorders Conference 'Medical Pre-con' Curative Options for GT-, Anaheim, CA, 10/2019

Invited Presentation, Hematopoietic Stem Cell Gene Therapy for Inherited Bleeding Disorders, Cincinnati Children's Hospital Medical Center, 11/2019

Invited Presentation Platelet-Targeted Gene Therapy for Hemophilia A, American Society for Gene and Cellular Therapy 23rd Annual Meeting, 05/2020

Invited Presentation: Pilot and Feasibility Study of Hematopoietic Stem Cell Gene Transfer to Utilize Platelet-Derived Factor VIII for Hemophilia, American Society for Gene and Cellular Therapy 23rd Annual Meeting, 05/2020

Invited Presentation Platelet-Targeted Gene Therapy for Hemophilia A, Gene Therapy for Blood Disorders 2rd Annual Meeting, 03/2021

Curative Options: Gene Therapy for Glanzmann Thrombasthenia, Annual Glanzmann Research Foundation Society Meeting, Boston, MA, 07/29/2023

### **International**

Organizing Committee, International Symposium on Gene Therapy for Hemophilia, Chapel Hill, NC, 09/1997

Invited Presentation, Expression of a functional murine  $\alpha$ IIb-human  $\beta$ 3 heterodimer complex on the surface of megakaryocytes derived from  $\beta$ 3-knockout mice, Semi-Annual Meeting of the International Society for Thrombosis and Haemostasis, Washington DC, 1999

Invited Presentation, Transgene expression targeted in canine megakaryocytes as a model for gene therapy of lineage-specific disorders, Semi-Annual Meeting of the International Society for Thrombosis and Haemostasis, Paris France., 2001

Consultant European Network on Inherited Diseases of Platelet Production and Function, 2002

Invited Session Chair, Semi-Annual Meeting of the International Society for Thrombosis and Haemostasis, Birmingham UK., 2003

Invited Presentation, Lineage-Specific Correction of a hemorrhagic disorder affecting platelets: gene therapy for glanzmann thrombasthenia, Semi-Annual Meeting of the International Society for Thrombosis and

Haemostasis, Birmingham UK., 2003

Invited Presentation Gene therapy for Inherited Platelet Disorders, Telethon Institute of Genetics and Medicine, Naples, Italy, 04/2004

Invited Plenary Presentation Gene therapy for Inherited Platelet Disorders, European Science Foundation Exploratory Workshop: Applying new technologies to the study of inherited disorders of megakaryocytes and platelets, Naples, Italy, 04/2004

Invited Presentation, Gene therapy for inherited platelet bleeding disorders, Annual Conference of the Haematology Society of Australia and New Zealand, Hobart Tasmania, 10/2006

Invited Presentation, Targeting Coagulation Factor VIII to platelets for gene therapy of Hemophilia A, Annual Conference of the Haematology Society of Australia and New Zealand, Hobart Tasmania, 10/2006

Invited Presentation for Meet the Expert Session, Use of Hematopoietic Stem Cells (From Mice Dogs and Humans) for Preclinical Gene Therapy Studies, Annual Conference of the Haematology Society of Australia and New Zealand, Hobart, Tasmania, 10/2006

Invited Presentation, Platelet-targeted gene therapy for inherited bleeding disorders, Osaka University Graduate School of Medicine, Osaka, Japan, 05/2007

Invited Presentation Platelet-targeted gene therapy for inherited bleeding disorders, AIM International Meeting for Recent Advances in Hemostasis, Kumamoto, Japan, 05/2007

Invited Presentation Recombinant human HPA-1A antibodies for treatment of feto-maternal allo-immune thrombocytopenia (FMAIT): proof of principle in an in vivo murine model, Semi-Annual Meeting of the International Society for Thrombosis and Haemostasis, Geneva, Switzerland, 07/2007

Invited Presentation Correction of haemorrhagic disorders affecting platelets: gene therapy restores hemostasis within a canine model for glanzmann thrombasthenia, Semi-Annual Meeting of the International Society for Thrombosis and Haemostasis, Geneva, Switzerland, 07/2007

Invited Presentation, Hematopoietic stem cell gene therapy for inherited platelet bleeding disorders, IVth Congress on Stem Cell Gene Therapy, Thessaloniki, Halkidiki, Greece, 09/2007

Invited Presentation, Hematopoietic stem cell gene therapy for inherited platelet bleeding disorders, University of Crete, Heraklion, Crete, Greece, 09/2007

Invited Dissertation Committee, Defense of Lisebeth DeWaele, Katholieke University of Leuven, Leuven, Belgium, 05/2009

Invited Presentation Hematopoietic stem cell gene therapy for inherited platelet bleeding disorders, Katholieke University of Leuven, , Leuven, Belgium, 05/2009

Invited Presentation The First Affiliated Hospital of Nanjing Medical University, Nanjing, Jiangsu Province, P.R. China, 11/2010

Invited Session Chair Platelet Disorders, Semi-Annual Meeting of the International Society for Thrombosis and Haemostasis, Toronto, Canada, 06/2015

Invited Presentation Platelets and Cancer, Semi-Annual Meeting of the International Society for Thrombosis and Haemostasis, Toronto, Canada, 06/2015

Invited Abstract Reviewer Platelet Disorders, Semi-Annual Meeting of the International Society for Thrombosis and Haemostasis, Toronto, Canada, 06/2015

Invited Presentation Platelets as vehicles for drug delivery, Semi-Annual Meeting of the International Society for Thrombosis and Haemostasis SSC, Montpellier, France, 05/2016

Invited Presentation Hematopoietic Stem Cell Gene Therapy For Hemophilia A with Platelet FVIII, CSL Behring Corporation, International Webinar Presentation, 02/2017

Invited Presentation, A Novel F153S ITGB3 Mutation Found in a Glanzmann Thrombasthenic Patient Reveals a Structural Clasp that Modulates Integrin Activation, (Oral Communication) J Thromb Haemost, 13 (Suppl): a: Semi-Annual Meeting of the International Society for Thrombosis and Haemostasis SSC, Mo, Australia, 07/2019

Invited Webinar Presentation, Glanzmann Thrombasthenia Symposium (Sponsored by Novo Nordisk), Manchester UK, 10/2019

Invited Presentation, Platelet-Targeted FVIII "Pleightlet™"LV-HSC for Severe Hemophilia A with Inhibitors, 2nd Annual Meeting on Gene Therapy for Blood Disorders, Webinar Presentation, London UK, 2021

Invited Presentation, Platelet-Targeted FVIII "Pleightlet™"LV-HSC for Severe Hemophilia A with Inhibitors, Hematology Research Study Update, Takeda Pharmaceutical Corporation Limited (05/18/21), Webinar Presentation, 2021

Invited Chair for Day 1,, 3rd Annual Meeting on Gene Therapy for Blood Disorders, Virtual Presentation Boston, MA, 2022

Invited Presentation, Platelet-Targeted FVIII "Pleightlet™"LV-HSC for Severe Hemophilia A with Inhibitors,  
Virtual Presentation, 3rd Annual Meeting on Gene Therapy for Blood Disorders, Boston, MA, 2022  
Invited Presentation, Platelets as vehicles for drug delivery, Annual Meeting of the International Society for  
Thrombosis and Haemostasis SSC, London, UK, 2022

## **PEER REVIEWED WORKSHOPS/PRESENTATIONS:**

### **National**

- Wilcox DA, Wautier JL, Pidard D, Newman PJ, An amino acid substitution within the fourth calcium-binding region of GPIIb results in degradation of the integrin GPIIb-IIIa and type I glanzmann thrombasthenia, Annual Meeting of the American Heart Association, 1992
- Wilcox DA, Gill J, Newman PJ, Glanzmann thrombasthenia resulting from a single amino acid substitution flanking the fibrinogen  $\gamma$ -chain dodecapeptide-binding domain on GPIIb, American Society of Hematology (ASH) Annual Meeting, 12/1993
- Wilcox DA, Hodivala-Dilke KM, Hynes RO, White GC II, Of mice and men: detection of a functional murine  $\gamma$ Ib $\beta$  heterodimer complex on the surface of megakaryocytes derived from retrovirus transduced bone marrow cells from  $\beta$ 3-knockout mice, American Society of Hematology (ASH) Annual Meeting, 12/1998
- Use of  $\gamma$ Ib Promoter in Retroviral Constructs, Gordon Research Conference on Hemostasis, Plymouth, NH, 07/2000
- Wilcox DA, Hodivala-Dilke KM, Johnson BD, Hynes RO, White GC II, Therapeutic expression of a platelet-specific integrin,  $\gamma$ Ib $\beta$ 3, American Society for Gene Therapy Annual Meeting, 2003
- Wilcox DA, Fang J, Jensen ES, Du LM, Boudreaux MK, Therapeutic expression of a platelet-specific integrin restores hemostasis in dogs with glanzmann thrombasthenia, American Society of Gene Therapy Annual Meeting, Seattle, WA, 06/2007
- Wilcox DA, Shi Q, Nurden P, Haberichter SL, Rosenberg JB, Johnson BD, Nurden AT, White GC II, Montgomery RR, EM localization and agonist-induced release of human FVIII from megakaryocytes transduced with a FVIII transgene, American Society of Hematology Annual Meeting, San Diego, CA, 12/2009
- Du LM, Franck HWG, Merricks EP, Nurden P, Jensen ES, Haberichter SL, Hawkins TB, Jacobi PM, Fang J, Koukouritaki SB, Nurden AT, Shi Q, Montgomery RR, Wilcox DA, Gene therapy targeting synthesis of coagulation factor viii in platelets reduces bleeding in canine hemophilia a, Best Abstract in Basic Research at the American Society for Bone Marrow Transplant Annual Meeting, Hawaii, 02/2011

## **COMMITTEE SERVICE:**

### **Medical College of Wisconsin**

- 2000 - 2001 Member, Search Committee Candidates for Medical College of Wisconsin/Blood Research Institute Joint Recruitment for Faculty in Stem Cell Biology, Medical College of Wisconsin
- 2000 - 2001 Member, Search Committee for Candidates for Medical College of Wisconsin Faculty in Hematopoietic Stem Cell Gene Therapy, Medical College of Wisconsin
- 2002 - Present Member, Fellowship Selection Committee, Hematology/Oncology/BMT, Pediatrics, Medical College of Wisconsin
- 2003 - Present Member, Research /Mentorship Committee for Qizhen Shi, MD, PhD (Hematology/Oncology Research Fellow who has received three national grants), Medical College of Wisconsin
- 2004 - Present Member, Research Task Force Committee, Pediatrics, Medical College of Wisconsin
- 2004 - 2006 Voting Member, Institutional Animal Care and Use Committee (IACUC), Medical College of Wisconsin
- 2006 - Present Chairman, Advisory Board for the Lentivirus Core Facility, Medical College of Wisconsin
- 2006 Co-Chairman During Summer, Institutional Animal Care and Use Committee (IACUC), Medical College of Wisconsin
- 2006 - Present Chairman, Advisory Board for the MCW Lentivirus Core Facility, Medical College of Wisconsin
- 2006 Member, Scientific Review Committee, Children's Hospital Foundation, Medical College of Wisconsin
- 2006 Member, MCW & Blood Research Institute Search for Stem Cell Biology Faculty, Medical College

of Wisconsin  
 2007 - 2011 Member, Biosafety Committee, Medical College of Wisconsin  
 2007 Member, Institutional Animal Care and Use Committee (IACUC) Committee to ?-test the online protocol for Animal Research, Medical College of Wisconsin  
 2007 - 2016 Reviewer, MCW Institutional Biosafety Committee and Joint Safety Committee, Medical College of Wisconsin  
 2012 - 2014 Member, Committee for Design of IBC Safety Module for MCW Ebridge, Medical College of Wisconsin  
 2012 - 2016 Co-Chairman, MCW Institutional Biosafety Committee and Joint Safety Committee, Medical College of Wisconsin  
 2015 Invited Attendee, The Leadership for Social Inclusion (NCBI) workshop, Medical College of Wisconsin  
 2015 - Present Appointed Member, MCW Dual Use in Research of Concern Committee, Medical College of Wisconsin  
 2018 - 2019 Member, Department of Pediatrics Intern Selection Committee, Medical College of Wisconsin  
 2018 - 2024 Member, Appointed, Scientific Review Committee: Non-Cancer Human Gene Transfer (11/18)  
 2018 - 2021 Appointed, Scientific Review Committee: Non-Cancer Human Gene Transfer, Medical College of Wisconsin  
 2019 Member, Department of Pediatrics-Dr. Lane's Basic Research Committee, Medical College of Wisconsin  
 2020 Member, Honors Thesis Review Committee, Medical Student Summer Research Program (MSSRP) (01/06/20) Daniel Keesler/ Dr Flood Student, Medical College of Wisconsin

### **Hospital**

2000 - 2001 Member, Scientific Review Committee, Children's Hospital Foundation  
 2006 Member, Scientific Review Committee, Children's Hospital Foundation

## **MEDICAL COLLEGE TEACHING ACTIVITIES:**

### **Community/Lay Public**

05/2012 - 07/2012 Mentor, Summer Undergraduate Student, Kelsey Gardetto, U of Iowa  
 07/2012 - 08/2012 Mentor, Summer Undergraduate Student, Lisa Friedman, Virginia  
 06/2013 - 08/2013 Mentor, Summer High School Student, Rachel Gardetto, Wisconsin  
 07/2014 - 08/2014 Mentor, Summer High School Student, Haley Slater, Wisconsin  
 07/2014 - 08/2014 Mentor, Summer High School Student, Rachel Gardetto, Wisconsin  
 07/2015 - 08/2015 Mentor, Summer High School Student, Haley Slater, Wisconsin  
 05/2016 - 08/2016 Mentor, Summer High School Student, Haley Slater, Wisconsin  
 06/2018 - 08/2018 Mentor, Summer High School Student, Elisabeth Wong, Wisconsin  
 06/2019 - 08/2019 Mentor, Summer High School Student, Elisabeth Wong, Wisconsin

### **Continuing Medical Education**

1999 Department of Pediatrics, Seminar on Megakaryocyte-Targeted Gene Therapy for Inherited Hematological Disorders  
 2000 Department of Biochemistry, Signal and Gene Expression Group, Seminar on Gene Therapy for Platelet Disorders  
 2001 Department of Biochemistry, Signal and Gene Expression Group, Seminar on Gene Therapy for Platelet Disorders  
 2002 Biomedical Resource Center, Research Training Seminar  
 2003 Blood Research Institute, Seminar on Targeting Gene Therapy to Specific Hematopoietic Lineages  
 2003 Department of Pediatrics, Seminar on Targeting Gene Therapy to Specific Hematopoietic Lineages  
 2005 Department of Biochemistry, Signal and Gene Expression Group, Seminar on Gene Therapy for Inherited Bleeding Disorders  
 2006 Department of Physiology, Seminar on Gene Therapy for Inherited Bleeding Disorders  
 2006 Department of Pediatrics, Seminar on Gene Therapy for Inherited Bleeding Disorders  
 2006 Hematology/Oncology Medical Fellows Lecture Series, Platelet Disorders  
 2007 Hematology/Oncology Medical Fellows Lecture Series, Platelet Disorders

10/2008 Blood Research Institute, Presentation to Scientific Review Committee  
 12/2008 MACC Fund, Presentation to Scientific Review Committee  
 2008 Children's Research Institute, Seminar on Gene Therapy for Inherited Bleeding Disorders  
 2008 Pediatric Hematology/Oncology Medical Fellows Lecture Series, Platelet Disorders  
 2008 Children's Research Institute Noon Academic Conference, Seminar on Hematopoietic Stem Cell Gene Therapy for Inherited Bleeding Disorders  
 2008 Invited Presentation, Children's Hospital President Council Meeting, 2002 PIR Award Lineage Specific Gene Expression  
 10/2011 Blood Research Institute, Presentation to Scientific Review Committee  
 01/2014 Invited Service for Interview, Association for the Accreditation of Human Research Protections Programs, or AAHRPP for MCW site visit  
 10/2014 Blood Research Institute, Presentation to Scientific Review Committee  
 01/2015 Children's Research Institute, Seminar on Gene Therapy for Inherited Bleeding Disorders  
 05/2017 Blood Research Institute, Presentation Milwaukee Thrombosis & Hemostasis  
 08/2017 Presentation, Pediatric Hematology, Oncology, and BMT Research Retreat  
 10/2018 Invited Presentation, Blood Center of Wisconsin Scientific Review Board  
 10/2018 Presentation for Intern Research Day, Dept Pediatrics, MCW  
 11/2018 Invited Presentation, Carroll University, Gene Therapy for Bleeding Disorders  
 03/2019 Children's Research Institute, Seminar on Gene Therapy for Inherited Bleeding Disorders From Bench to Bedside  
 07/2020 IACUC Essentials Invited Speaker: Gene Therapy for Canine Glanzmann Thrombasthenia

#### **EXTRAMURAL TEACHING:**

##### **Community/Lay Public**

11/27/2022 Glanzmann Research Foundation PODCAST GT for GT, Invited Guest

##### **Continuing Medical Education**

02/2008 University of Wisconsin-Milwaukee, IDepartment of Biological Sciences Colloquium Series  
 Presentation - Gene Therapy for Inherited Platelet Bleeding Disorders  
 02/27/2023 Medical College of Wisconsin - Department of Pediatrics, Scholarship in Progress Series  
 Speaker

#### **MCW STUDENTS, FACULTY, RESIDENTS AND CLINICAL/RESEARCH FELLOWS MENTORED:**

##### **Postdoctoral Students**

Runqui Jiang, Medical College of Wisconsin, 03/01/2009 - 09/29/2009 Invited Foreign Dissertation Advisor  
 Yoshinori Nishijima, Medical College of Wisconsin, 04/25/2011 - Present Mentor for Postdoctoral Fellow

#### **EXTRAMURAL STUDENTS, FACULTY, RESIDENTS, AND CLINICAL/RESEARCH FELLOWS MENTORED:**

##### **High School Students**

High School Summer Student, Medical College of Wisconsin, 07/07/2010 - 08/23/2010 Mentor

##### **Postdoctoral Students**

Lisebeth DeWaele, Katholieke University of Leuven, Belgium, 05/06/2009 Invited Dissertation Committee Defense  
 Ming Yao, China, 01/2011 - 06/2013 Invited Foreign Dissertation Advisor

#### **PROGRAMMATIC DEVELOPMENTS:**

##### **Research Programs**

2011 Co-inventor, Methods and Compositions for Enhancing Cell Adhesion Properties; U.S. Patent Application No. 11/118,712; International No. PCT/US2005/015120

**COMMUNITY SERVICE ACTIVITIES:**

2002 - 2008 Invited Presentation, Annual Fund Raiser for Glanzmann Thrombasthenia Research, Augusta, GA  
2014 Invited Ambassador of MCW, Chili's MACC Golf Outing for Cancer & Blood Disorders  
2015 Invited Ambassador of MCW, Chili's MACC Golf Outing for Cancer & Blood Disorders  
2017 Invited Ambassador of MCW, Chili's MACC Golf Outing for Cancer & Blood Disorders  
2018 Invited Ambassador of MCW, Chili's MACC Golf Outing for Cancer & Blood Disorders  
2019 Invited Ambassador of MCW, Chili's MACC Golf Outing for Cancer & Blood Disorders  
2021 Invited Presentation, MACC Quarterly Meeting Gene Therapy for Hemophilia A 7/21/21  
2022 Host, Site Visit CureGT.Org Society tour and meeting Gene Therapy for GT 04/29/22

**PATENT APPLICATIONS:**

10/24/2012 Co-inventor, Platelet Targeted Treatment. U.S. Provisional Patent Application No. 61/717,951; filed October 24, 2012  
10/24/2013 Co-inventor, Platelet Targeted Treatment. International Patent Application No. PCT/US2013/066651 filed October 24, 2013  
2015 Co-inventor, Platelet Targeted Treatment. U.S. Provisional Patent Application No. 61/717,951; Int'l Patent Application No.: PCT/US2013/066651 filed April 24, 2015  
2019 Israel Application No.: 238417 Based on PCT/US2013/066651 Filed 24-Oct-2013  
2021 Europe Divisional Validation Certificates issued: Belgium, France, Germany, Luxembourg, Switzerland/Liechtenstein, United Kingdom, Denmark, Sweden, Austria, Italy, Poland and Spain 06-Jan-2021

**PATENT AWARDS:**

2011 Co-inventor, Methods and Compositions for Enhancing Cell Adhesion Properties. Awarded May 10, 2011 U.S. Patent No. 7,939,326  
2016 South African Patent Application No.: 2015/02945 awarded on Sept 07, 2016 Based on PCT/US2013/066651 Filed: 24-Oct-2013 Entitled: Platelet Targeted Treatment Our Ref: WILCO-32935/ZA-1/PCT  
2018 U.S. Patent Application No.: 14/437,45 U.S. National Entry Of Pct/Us2013/066651 Int'l Filing Date: 24-Oct-2013 entitle: Platelet Targeted Treatment: 9,982,034 Issued 29-May-2018  
2018 Korean Patent Application No.: 10-2015-7012821 Awarded Based on PCT/US2013/066651 Filed: 24-Oct-2013 Patent No.: 10-1819803 Issued: 11-Jan-2018 Entitled: Platelet Targeted Treatment  
2019 U.S. Patent Application No.: 15/689,875 U.S. National Entry Of Pct/Us2013/066651 Int'l Filing Date: 24-Oct-2013 entitle: Platelet Targeted Treatment: 10,294,291 Issued 21-May-2019  
2020 Brazil Patent Approved October 2020  
2020 Canadian Patent Application No.: 2888982 entitled: Platelet Targeted Treatment: Issued 21-July-2020  
2020 European Patent Accepted October 2020  
2021 India Patent No. 377388 Issued 21-Sept-2021  
2021 Japan Patent No. 6975105 Issued 09-Nov-2021  
2021 Brazilian Application No. BR1120150092292 Issued 21-Jun-2021  
2021 European Patent No. 2912186 entitled: Platelet Targeted Treatment Issued 06-Jan-2021  
2021 Hong Kong Patent No. 1211986 Issued 30-April-2021

**BIBLIOGRAPHY****Refereed Journal Publications/Original Papers**

1. Liu S, Wilcox DA, Sieber-Blum M, Wong-Riley M. Developing neural crest cells in culture: correlation of cytochrome oxidase activity with SSEA-1 and dopamine-beta-hydroxylase immunoreactivity. Brain Res. 1990 Dec 10;535(2):271-80.
2. Wilcox DA, Wautier JL, Pidard D, Newman PJ. A single amino acid substitution flanking the fourth calcium binding domain of alpha IIb prevents maturation of the alpha IIb beta 3 integrin complex. J Biol Chem.

- 1994 Feb 11;269(6):4450-7.
3. **Wilcox DA**, Paddock CM, Lyman S, Gill JC, Newman PJ. Glanzmann thrombasthenia resulting from a single amino acid substitution between the second and third calcium-binding domains of GPIIb. Role of the GPIIb amino terminus in integrin subunit association. *J Clin Invest.* 1995 Apr;95(4):1553-60. PMID: PMC295643
  4. **Wilcox DA**, Olsen JC, Ishizawa L, Griffith M, White GC 2nd. Integrin alphaIIb promoter-targeted expression of gene products in megakaryocytes derived from retrovirus-transduced human hematopoietic cells. *Proc Natl Acad Sci U S A.* 1999 Aug 17;96(17):9654-9. PMID: PMC22265
  5. Shiraga M, Ritchie A, Aidoudi S, Baron V, Wilcox D, White G, Ybarrondo B, Murphy G, Leavitt A, Shattil S. Primary megakaryocytes reveal a role for transcription factor NF-E2 in integrin alpha IIb beta 3 signaling. *J Cell Biol.* 1999 Dec 27;147(7):1419-30. PMID: PMC2174239
  6. **Wilcox DA**, Olsen JC, Ishizawa L, Bray PF, French DL, Steeber DA, Bell WR, Griffith M, White GC 2nd. Megakaryocyte-targeted synthesis of the integrin beta(3)-subunit results in the phenotypic correction of Glanzmann thrombasthenia. *Blood.* 2000 Jun 15;95(12):3645-51.
  7. Shi Q, **Wilcox DA**, Fahs SA, Kroner PA, Montgomery RR. Expression of human factor VIII under control of the platelet-specific alphaIIb promoter in megakaryocytic cell line as well as storage together with VWF. *Mol Genet Metab.* 2003 May;79(1):25-33.
  8. **Wilcox DA**, White GC 2nd. Gene therapy for platelet disorders: studies with Glanzmann's thrombasthenia. *J Thromb Haemost.* 2003 Nov;1(11):2300-11.
  9. Yarovoi HV, Kufrin D, Eslin DE, Thornton MA, Haberichter SL, Shi Q, Zhu H, Camire R, Fakhrazadeh SS, Kowalska MA, **Wilcox DA**, Sachais BS, Montgomery RR, Poncz M. Factor VIII ectopically expressed in platelets: efficacy in hemophilia A treatment. *Blood.* 2003 Dec 01;102(12):4006-13.
  10. **Wilcox DA**, Shi Q, Nurden P, Haberichter SL, Rosenberg JB, Johnson BD, Nurden AT, White GC 2nd, Montgomery RR. Induction of megakaryocytes to synthesize and store a releasable pool of human factor VIII. *J Thromb Haemost.* 2003 Dec;1(12):2477-89.
  11. Niemeyer GP, Boudreaux MK, Goodman-Martin SA, Monroe CM, **Wilcox DA**, Lothrop CD Jr. Correction of a large animal model of type I Glanzmann's thrombasthenia by nonmyeloablative bone marrow transplantation. *Exp Hematol.* 2003 Dec;31(12):1357-62.
  12. Shi Q, **Wilcox DA**, Morateck PA, Fahs SA, Kenny D, Montgomery RR. Targeting platelet GPIIbalpha transgene expression to human megakaryocytes and forming a complete complex with endogenous GPIIbbeta and GPIX. *J Thromb Haemost.* 2004 Nov;2(11):1989-97.
  13. Fang J, Hodivala-Dilke K, Johnson BD, Du LM, Hynes RO, White GC 2nd, **Wilcox DA**. Therapeutic expression of the platelet-specific integrin, alphaIIbbeta3, in a murine model for Glanzmann thrombasthenia. *Blood.* 2005 Oct 15;106(8):2671-9. PMID: PMC1895311
  14. Shi Q, **Wilcox DA**, Fahs SA, Weiler H, Wells CW, Cooley BC, Desai D, Morateck PA, Gorski J, Montgomery RR. Factor VIII ectopically targeted to platelets is therapeutic in hemophilia A with high-titer inhibitory antibodies. *J Clin Invest.* 2006 Jul;116(7):1974-82. PMID: PMC1483176
  15. Shi Q, **Wilcox DA**, Fahs SA, Fang J, Johnson BD, DU LM, Desai D, Montgomery RR. Lentivirus-mediated platelet-derived factor VIII gene therapy in murine haemophilia A. *J Thromb Haemost.* 2007 Feb;5(2):352-61.
  16. **Wilcox DA**, White GC. Gene therapy for platelet disorders Platelets. 2007:1313-1325.
  17. Ghevaert C, **Wilcox DA**, Fang J, Armour KL, Clark MR, Ouwehand WH, Williamson LM. Developing recombinant HPA-1a-specific antibodies with abrogated Fcgamma receptor binding for the treatment of fetomaternal alloimmune thrombocytopenia. *J Clin Invest.* 2008 Aug;118(8):2929-38. PMID: PMC2483683
  18. Shi Q, Fahs SA, **Wilcox DA**, Kuether EL, Morateck PA, Mareno N, Weiler H, Montgomery RR. Syngeneic transplantation of hematopoietic stem cells that are genetically modified to express factor VIII in platelets restores hemostasis to hemophilia A mice with preexisting FVIII immunity. *Blood.* 2008 Oct 01;112(7):2713-21. PMID: PMC2556608
  19. Mendoza SA, Fang J, Gutterman DD, **Wilcox DA**, Bubolz AH, Li R, Suzuki M, Zhang DX. TRPV4-mediated endothelial Ca<sup>2+</sup> influx and vasodilation in response to shear stress. *Am J Physiol Heart Circ Physiol.* 2010 Feb;298(2):H466-76. PMID: PMC2822567
  20. Gao C, Boylan B, Fang J, **Wilcox DA**, Newman DK, Newman PJ. Heparin promotes platelet responsiveness by potentiating ?IIb?3-mediated outside-in signaling. *Blood.* 2011 May 05;117(18):4946-52. PMID: PMC3100701
  21. Fang J, Jensen ES, Boudreaux MK, Du LM, Hawkins TB, Koukouritaki SB, Cornetta K, **Wilcox DA**. Platelet gene therapy improves hemostatic function for integrin alphaIIbbeta3-deficient dogs. *Proc Natl*

- Acad Sci U S A. 2011 Jun 07;108(23):9583-8. PMID: PMC3111318
22. Kuether EL, Schroeder JA, Fahs SA, Cooley BC, Chen Y, Montgomery RR, **Wilcox DA**, Shi Q. Lentivirus-mediated platelet gene therapy of murine hemophilia A with pre-existing anti-factor VIII immunity. *J Thromb Haemost*. 2012 Aug;10(8):1570-80. PMID: PMC3419807
  23. Zheng X, Zinkevich NS, Gebremedhin D, Gauthier KM, Nishijima Y, Fang J, **Wilcox DA**, Campbell WB, Guterman DD, Zhang DX. Arachidonic acid-induced dilation in human coronary arterioles: convergence of signaling mechanisms on endothelial TRPV4-mediated Ca<sup>2+</sup> entry. *J Am Heart Assoc*. 2013 Apr 25;2(3):e000080. PMID: PMC3698766
  24. **Wilcox DA**. Gene Therapy for Platelet Disorders Platelets. 2013:1313-1327.
  25. Fang J, Nurden P, North P, Nurden AT, Du LM, Valentin N, **Wilcox DA**. C560R $\beta$ 3 caused platelet integrin  $\alpha$ IIb  $\beta$ 3 to bind fibrinogen continuously, but resulted in a severe bleeding syndrome and increased murine mortality. *J Thromb Haemost*. 2013 Jun;11(6):1163-71. PMID: PMC3702628
  26. Nurden AT, Pillois X, **Wilcox DA**. Glanzmann thrombasthenia: state of the art and future directions. *Semin Thromb Hemost*. 2013 Sep;39(6):642-55. PMID: PMC4011384
  27. Du LM, Nurden P, Nurden AT, Nichols TC, Bellinger DA, Jensen ES, Haberichter SL, Merricks E, Raymer RA, Fang J, Koukouritaki SB, Jacobi PM, Hawkins TB, Cornetta K, Shi Q, **Wilcox DA**. Platelet-targeted gene therapy with human factor VIII establishes haemostasis in dogs with haemophilia A. *Nat Commun*. 2013;4:2773. PMID: PMC3868233
  28. Sullivan SK, Mills JA, Koukouritaki SB, Vo KK, Lyde RB, Paluru P, Zhao G, Zhai L, Sullivan LM, Wang Y, Kishore S, Gharaibeh EZ, Lambert MP, **Wilcox DA**, French DL, Poncz M, Gadue P. High-level transgene expression in induced pluripotent stem cell-derived megakaryocytes: correction of Glanzmann thrombasthenia. *Blood*. 2014 Jan 30;123(5):753-7. PMID: PMC3907760
  29. Schroeder JA, Chen Y, Fang J, **Wilcox DA**, Shi Q. In vivo enrichment of genetically manipulated platelets corrects the murine hemophilic phenotype and induces immune tolerance even using a low multiplicity of infection. *J Thromb Haemost*. 2014 Aug;12(8):1283-93. PMID: PMC4127102
  30. Ristow LC, Bonde M, Lin YP, Sato H, Curtis M, Wesley E, Hahn BL, Fang J, **Wilcox DA**, Leong JM, Bergström S, Coburn J. Integrin binding by *Borrelia burgdorferi* P66 facilitates dissemination but is not required for infectivity. *Cell Microbiol*. 2015 Jul;17(7):1021-36. PMID: PMC4478124
  31. **Wilcox DA**. Megakaryocyte- and megakaryocyte precursor-related gene therapies. *Blood*. 2016 Mar 10;127(10):1260-8. PMID: PMC4786835
  32. Simeoni I, Stephens JC, Hu F, Deevi SV, Megy K, Bariana TK, Lentaigne C, Schulman S, Sivapalaratnam S, Vries MJ, Westbury SK, Greene D, Papadia S, Alessi MC, Attwood AP, Ballmaier M, Baynam G, Bermejo E, Bertoli M, Bray PF, Bury L, Cattaneo M, Collins P, Daugherty LC, Favier R, French DL, Furie B, Gattens M, Germeshausen M, Ghevaert C, Goodeve AC, Guerrero JA, Hampshire DJ, Hart DP, Heemskerk JW, Henskens YM, Hill M, Hogg N, Jolley JD, Kahr WH, Kelly AM, Kerr R, Kostadima M, Kunishima S, Lambert MP, Liesner R, López JA, Mapeta RP, Mathias M, Millar CM, Nathwani A, Neerman-Arbez M, Nurden AT, Nurden P, Othman M, Peerlinck K, Perry DJ, Poudel P, Reitsma P, Rondina MT, Smethurst PA, Stevenson W, Szkotak A, Tuna S, van Geet C, Whitehorn D, **Wilcox DA**, Zhang B, Revel-Vilk S, Gresle P, Bellissimo DB, Penkett CJ, Laffan MA, Mumford AD, Rendon A, Gomez K, Freson K, Ouwehand WH, Turro E. A high-throughput sequencing test for diagnosing inherited bleeding, thrombotic, and platelet disorders. *Blood*. 2016 Jun 09;127(23):2791-803. PMID: PMC5016734
  33. Cao S, Anishkin A, Zinkevich NS, Nishijima Y, Korishettar A, Wang Z, Fang J, **Wilcox DA**, Zhang DX. Transient receptor potential vanilloid 4 (TRPV4) activation by arachidonic acid requires protein kinase A-mediated phosphorylation. *J Biol Chem*. 2018 Apr 06;293(14):5307-5322. PMID: PMC5892583
  34. **Wilcox DA**. Gene therapy for platelet disorders Platelets. 1 January 2019:1191-1205.
  35. Gould, J. Gene therapy: Genie in a vector. *Nature* 515, S160-161, (Editorial Interview) doi:10.1038/515S160a, 2014.
  36. Gould, J. Genie in a vector, *Scientific American*, 312, S6 (Editorial Interview), 2015.
  37. Buitrago, L., Rendon, A., Liang, Y., Simeoni, I., Negri, A., ThromboGenomics Consortium, Filizola, M., Ouwehand, W. H., Coller, B. S.  $\alpha$ IIb $\beta$ 3 variants defined by next-generation sequencing: predicting variants likely to cause Glanzmann thrombasthenia, *Proceedings of the National Academy of Science, USA* 112:15 E1898-1907, 2015.
  38. Lentaigne, C., Freson, K., Laffan, M. A., Turro, E., Ouwehand, W. H., Bridge-Bpd Consortium and the ThromboGenomics Consortium, Inherited platelet disorders: toward DNA-based diagnosis. *Blood* 127(23):2814-2823, 2016.
  39. Bariana, T. K., Ouwehand, W. H., Guerrero, J. A., Gomez, K., Bridge Bleeding, Thrombotic Platelet,

- Disorders and ThromboGenomics Consortia, Dawning of the age of genomics for platelet granule disorders: improving insight, diagnosis and management, *Br J Haematol*, 176(5): 705-720, 2017.
40. Koukouritaki, S.B., Thinn, A.M., Ashworth, K.J., Fang, J., Slater, H.S., Du, L.M., Nurden, A.T., Di Paola, J., Zhu, J., Wilcox, D.A., A Novel F153S ?3 Mutation Found in a Glanzmann Thrombasthenia Patient Reveals a Structural Clasp that Modulates Integrin Activation, (Submitted to Editor, PNAS, Nov 2020).
  41. Korishettar AM, Nishijima Y, Wang Z, Xie Y, Fang J, **Wilcox DA**, Zhang DX. Endothelin-1 potentiates TRPV1-mediated vasoconstriction of human adipose arterioles in a protein kinase C-dependent manner. *Br J Pharmacol*. 2021 Feb;178(3):709-725. PMID: PMC9121782
  42. Schroeder JA, Kuether EA, Fang J, Jing W, Weiler H, **Wilcox DA**, Montgomery RR, Shi Q. Thromboelastometry assessment of hemostatic properties in various murine models with coagulopathy and the effect of factor VIII therapeutics. *J Thromb Haemost*. 2021 Oct;19(10):2417-2427. PMID: PMC8865566
  43. Xie Y, Nishijima Y, Zinkevich NS, Korishettar A, Fang J, Mathison AJ, Zimmermann MT, **Wilcox DA**, Gutterman DD, Shen Y, Zhang DX. NADPH oxidase 4 contributes to TRPV4-mediated endothelium-dependent vasodilation in human arterioles by regulating protein phosphorylation of TRPV4 channels. *Basic Res Cardiol*. 2022 Apr 25;117(1):24. PMID: PMC9119129
  44. Koukouritaki, S.B., Thinn, A.M., Bark K.J., Fang, J., Slater, H.S., Du, L.M., Pillon, X., Nurden, A.T., DiPaola, J., Zhu, Wilcox, D.A. A Novel F153S ITGB3 A Single F153S?3 Mutation Causes Constitutive Integrin ?IIb?3 Activation in a Variant Form of Glanzmann Thrombasthenia (In Revision, Blood, May 2022)
  45. Fang, J., Yao, M., Jing, W., Koukouritaki, S., Du, L.M., Sun, B., Johnson, B.D., and Wilcox, D.A. Platelets engineered to express interleukin-24 inhibited melanoma tumor growth in mice. (In Preparation) 2022.
  46. **Wilcox DA**. Gene Therapy for Platelet Disorders Platelets, Third Edition. 1 January 2012:1313-1327.
  47. **Wilcox DA**, White GC. Gene therapy for platelet disorders Platelets, Second Edition. 1 January 2006:1313-1325.
  48. Koukouritaki SB, Thinn AMM, Ashworth KJ, Fang J, Slater HS, Du LM, Nguyen HTT, Pillon X, Nurden AT, Ng CJ, Di Paola J, Zhu J, **Wilcox DA**. A single F153S?3 mutation causes constitutive integrin ?IIb?3 activation in a variant form of Glanzmann thrombasthenia. *Blood Adv*. 2023 Jul 11;7(13):3180-3191. PMID: PMC10338211
  49. Koukouritaki, S.B., Thinn, A.M., Bark K.J., Fang, J., Slater, H.S., Du, L.M., Pillon, X., Nurden, A.T., DiPaola, J., Zhu, Wilcox, D.A. A Novel F153S ITGB3 A Single F153S?3 Mutation Causes Constitutive Integrin ?IIb?3 Activation in a Variant Form of Glanzmann Thrombasthenia (First Edition, Blood Advances, March 8 2023) *Blood Adv* 2023 Vol. 7 Issue 13 Pages 3180-3191 Accession Number: 36884296 DOI: 10.1182/bloodadvances.2022009495
  50. Foster, H.R., Herbert, N., Di Buduo, C.A., Schmidt, A.P., Fang, J., Biswas, R., Taimoor, M., Waller, A.K., Lawrence, M., Mueller, A., Howard, D., Moreau, T., Evans, A., Turro, E., Fischer, R., Wilcox, D.A., Hoffmeister, K.M., Balduini, A., Ghevaert, C. Thyroid hormones and analogues promote the acute release of platelets from megakaryocytes: from blood donor biology to the production of platelets in vitro (In Preparation) 2023).
  51. Fang, J., Yao, M., Jing, W., Koukouritaki, S.B., Du, L.M., Sun, B., Johnson, B.D., and Wilcox, D.A. Platelets engineered to express interleukin-24 inhibited melanoma tumor growth in mice. (In Preparation) 2023).

### **Books, Chapters, and Reviews**

1. Wilcox, D. A., White II, G.C. Gene therapy for platelet disorders. In: Platelets. A.D. Michelson (ed.), Academic Press, San Diego, Chapter 61: 927-37, 2002.
2. Wilcox, D.A. White II, G.C. Gene therapy for platelet disorders: studies with glanzmann's thrombasthenia. *Journal of Thrombosis and Haemostasis*. 1: 2300-2311, 2003.
3. Wilcox, D. A., White II, G.C: Gene therapy for platelet disorders. In: Platelets. Second Edition, A.D. Michelson (ed.), Academic Press, San Diego, Chapter 71: 1313-1325, 2007.
4. Wilcox, D.A. Gene therapy for platelet disorders. In: Platelets. Third Edition, A.D. Michelson(ed.), Academic Press, San Diego, Chapter 64:1313-1327,2013.
5. Nurden, A.T., Wilcox, D.A. White II, G.C. Glanzmann thrombasthenia: state of the art and future directions.Seminars Thrombosis and Haemostasis Journal 39:642-655, 2013.

### **Abstracts**

1. **Wilcox DA**, Sieber-Blum M. Monoclonal antibody, B-1A11, to a cell surface epitope recognizes a subpopulation of early neural crest cells. Society for Neuroscience Abstracts. 1989;15 a:352.3
2. **Wilcox DA**, Wautier JL, Pidard D, Newman PJ. An amino acid substitution within the fourth calcium-binding region of GPIIb results in degradation of the integrin GPIIb-IIIa and type I glanzmann thrombasthenia. Circulation. 1992;86 a:2713
3. Valentin N, **Wilcox DA**, Newman PJ. Disruption of the GPIIIa cys5-cys435 disulfide bonded-loop: effects on the PIA1 epitope and GPIIb-IIIa complex formation. Blood. 1993;82 a:826
4. Wilcox, D. A., Gill, J., and Newman, P. J. Glanzmann thrombasthenia resulting from a single amino acid substitution flanking the fibrinogen gamma-chain dodecapeptide-binding domain on GPIIb, Blood. 82 a:824, 1993.
5. Wilcox, D. A., Olsen, J. C., and White II, G.C. Retrovirus mediated gene therapy for glanzmann thrombasthenia: tissue-specific transcription and synthesis of the GPIIIa PIA2 alloantigen within cells homozygous for PIA1, Blood. 88 a:108, 1996.
6. Wilcox, D. A., Olsen, J. C., Ishizawa, L., Bray, P.F., French, D.L., Bell, W.R., Griffith, M., and White II, G.C. Phenotypic correction of Glanzmann's thrombasthenia following megakaryocyte-targeted synthesis of the integrin  $\beta 3$ -subunit, Blood. 90 a:1236, 1997.
7. Wilcox, D. A., Hodivala-Dilke, K.M., Hynes, R.O., and White II, G.C. Of mice and men: detection of a functional murine  $\beta 3$  heterodimer complex on the surface of megakaryocytes derived from retrovirus transduced bone marrow cells from  $\beta 3$ -knockout mice, Blood. 92 a:2885, 1998.
8. Wilcox, D. A., Hodivala-Dilke, K.M., Steeber, D.A., Shattil, S.J., Hynes, R.O., and White II, G.C. Expression of a functional murine  $\beta 3$ -human  $\beta 3$  heterodimer complex on the surface of megakaryocytes derived from  $\beta 3$  3-knockout mice, Thrombosis and Haemostasis. 82 a:1168, 1999.
9. Shiraga, M., Richie, A., Aidoudi, S., Baron, V., Wilcox, D., White, G., Ybarrando, B., Murphy, G., Leavitt, A., and Shattil, S.A. A role for transcription factor NF-E2 in the regulation of integrin  $\alpha$ IIb- $\beta 3$  signaling, Blood. 94 a:2750, 1999.
10. Wilcox, D.A., Rosenberg, J.B., White II, G.C., and Montgomery, R.R. Factor VIII (FVIII) trafficks to megakaryocyte alpha-granules following retroviral transduction of human CD34+ cells, Blood. 94 a:1962, 1999.
11. Wilcox, D.A., Rosenberg, J.B., Johnson, B.D., and Montgomery, R.R. Storage of factor VIII (FVIII) in the alpha granules of human platelets following retroviral transduction and transplantation of human CD34+ cells into nod-scid mice, Blood. 96 a:3467, 2000.
12. Wilcox, D. A., Kiem, H.-P., Johnson, B.D., Nash, R.A., Catalfamo, J.L., and Boudreaux, M.K. Transgene expression targeted in canine megakaryocytes as a model for gene therapy of lineage-specific disorders, Thrombosis and Haemostasis. 86 a:5757, 2001.
13. Boudreaux, M.K., Niemeyer, G.P., Wilcox, D.A., Brawner, W.R., Lothrop, Jr., C.D. Correction of type I glanzmann's thrombasthenia in great pyrenees dogs by nonmyeloablative marrow transplantation, Blood. 98 a:721, 2001.
14. Shi, Q., Fahs, S.A., Wilcox, D.A., Kroner, P.A., Montgomery, R.R. Endothelial, platelet, and hepatic-specific expression of human FVIII and the effect of von Willebrand factor, Blood. 100 a:1906, 2002.
15. Wilcox, D.A., Hodivala-Dilke, K.M., Johnson, B.D., Hynes, R.O., and White II, G.C. Lineage-Specific Correction of glanzmann thrombasthenia by expression of a functional hybrid murine-human  $\beta 3$  integrin complex on the surface of platelets of  $\beta 3$  knockout mice, Blood. 100 a:3419, 2002.
16. Wilcox, D.A., Targeting transgene expression in canine megakaryocytes derived from lentivirus-transduced G-CSF mobilized CD34+ peripheral blood cells, Blood. 100 a:1713, 2002.
17. Wilcox, D.A., Hodivala-Dilke, K.M., Johnson, B.D., Hynes, R.O., White II, G.C. Therapeutic expression of a platelet-specific integrin,  $\alpha$ IIb $\beta 3$ , Molecular Therapy. 7 a:398, 2003.
18. Wilcox, D.A., Targeting transgene expression in canine megakaryocytes, Molecular Therapy. 7 a:1127, 2003.
19. Wilcox, D.A., Hodivala-Dilke, K.M., Johnson, B.D., Hynes, R.O., and White II, G.C. Lineage-Specific Correction of a hemorrhagic disorder affecting platelets: gene therapy for glanzmann thrombasthenia, Journal of Thrombosis and Haemostasis. 1 a:130, 2003.
20. Yarovoi, H., Kufrin, D., Eslin, D.E., Zhu, H., Camire, R. Wilcox, D., Montgomery, R.R., Kowalska, M.A., Poncz, M. A transgenic mouse model demonstrates the efficacy of factor FVIII ectopically expressed in platelets for Haemophilia A treatment. Journal of Thrombosis and Haemostasis. 1 a:131, 2003.
21. Wilcox, D.A., Shi, Q., Nurden, P., Haberichter, S.L., Rosenberg, J.B., Johnson, B.D., Nurden, A.T., White II, G.C., Montgomery, R.R. EM localization and agonist-induced release of human FVIII from megakaryocytes transduced with a FVIII transgene, Blood. 102 a:297, 2003.

22. Yarovoi, H., King, M., Eslin, D.E., Haberichter, S.L., Shi, Q., Kowalska, M.A., Wilcox, D., Sachais, B.S., Montgomery, R.R., Poncz, M. Demonstration that platelet-expressed human B-domainless Factor VIII can ameliorate the bleeding diathesis in a murine model of Haemophilia A. *Blood*. 102 a:175, 2003.
23. Shi, Q., Wilcox, D.A., Morateck, P., Fahs, S.A., Kenny, D., Montgomery, R.R. Targeting platelet GPIb? transgene expression to megakaryocytes using a lentiviral vector and its potential for gene therapy of bernard soulier syndrome, *Blood*. 102 a:1029, 2003.
24. Patel, M., Wilcox D.A., Giddings, A.M., McKay, T.R., Olsen, J.C. Modification of HEK 293 Cell Integrin Expression Profile Allows Convenient Large-Scale Roller Bottle Production of Lentiviral Vectors, *Molecular Therapy*. 9 a:81, 2004.
25. Du, L.M., Wilcox, D.A. Gene Therapy for Platelet Disorders: Targeting Transgene Expression in Canine Platelets, *Molecular Therapy*. 9 a:919, 2004.
26. Fang, J., Johnson, B.D., Wilcox, D.A. Assessing the Risk from Transfer of an Integrin  $\alpha$ IIb $\beta$ 3 Gene that Encodes a Naturally Occurring Polymorphism Associated with Acute Coronary Thrombosis, *Molecular Therapy*. 9, a:920, 2004.
27. Yoshida, T., Gomez, J., Gama, V., Wickramasekera, N.T., Wilcox, D.A., Matsuyama, S. Bax inhibiting peptides (BIPs) derived from Ku70 suppress chemotherapy induced-cell death of megakaryocytes, *Blood*. 104, a:1262, 2004.
28. Shi, Q., Wilcox, D.A., Fahs, S.A., Fang J, Johnson, B.D., Weiler, H., Montgomery, R.R. Lentivirus-Mediated Platelet-Specific Gene Therapy for Hemophilia A, *Blood*. 104, a:2974, 2004.
29. Wilcox, D.A., Fang, J., Du, L.M., and Boudreaux, M.K. Drug-selectable as well as therapeutic gene transfer into hematopoietic stem cells may be essential for gene therapy of inherited bleeding disorders in large animals, *Molecular Therapy*. 11, a:340, 2005.
30. Wilcox, D.A., Fang, J., Johnson, B.D., Valentin, N. Modulating murine platelet function by expressing integrin  $\alpha$ IIb $\beta$ 3 locked-in its high affinity state, *Journal of Thrombosis and Haemostasis*. 3 a:OR108, 2005.
31. Boylan, B., Wilcox, D.A., Newman, D.K., Kahn, M.L., Newman, P.J. Immunodepletion of GPVI from human platelets circulating in NOD/SCID mice: development of a novel animal model for evaluating the therapeutic efficacy of compounds that affect human platelet function and survival, *Journal of Thrombosis and Haemostasis*. 3 a:OR284, 2005.
32. Shi, Q., Wilcox, D.A., Fahs, S.A., Weiler, H., Montgomery, R.R. Platelet-derived factor VIII (FVIII) is protected from inhibitor inactivation - an approach for gene therapy of hemophilia A even in the presence of FVIII inhibitors, *Journal of Thrombosis and Haemostasis*. 3 a:H05, 2005.
33. Shi, Q., Wilcox, D.A., Fahs, S.A., Fang J, Johnson, B.D., Weiler, H., Montgomery, R.R. Murine hemophilia A is phenotypically corrected by platelet-expressed factor VIII even in the absence of detectable plasma FVIII, *Journal of Thrombosis and Haemostasis*. 3 a:OR186, 2005.
34. Wilcox, D.A., Fang, J., Lily M. Du, L.M., and Boudreaux, M.K. Phenotypic correction of a canine model for glanzmann thrombasthenia demonstrates efficacy for using platelets to deliver therapeutics at the site of vascular injury, *Blood*. 106, a:3045, 2005.
35. Wilcox, D.A., Therapeutic expression of  $\alpha$ IIb $\beta$ 3 in murine and canine models of glanzmann thrombasthenia, Subcommittee Meeting on Platelets, Annual Meeting of the American Society of Hematology, Atlanta, GA, *Blood*. 106, Invited Presentation, 2005.
36. Shi, Q., Wilcox, D.A., Fahs, S.A., Cooley, B.C., Desai, D., Weiler, H., Morateck, P.A., Montgomery, R.R. Platelet-derived factor VIII (FVIII) corrects the murine hemophilia A phenotype even in the presence of FVIII inhibitors, *Blood*. 106, a:457, 2005.
37. Wilcox, D.A., Gene therapy for inherited platelet bleeding disorders, Annual Conference of the Haematology Society of Australia and New Zealand, Hobart, Tasmania, (Invited Presentation), October 15-18 2006.
38. Wilcox, D.A., Targeting Coagulation Factor VIII to platelets for gene therapy of Hemophilia A, Annual Conference of the Haematology Society of Australia and New Zealand, Hobart, Tasmania, (Invited Presentation), October 15-18 2006.
39. Fang, J., Jensen, E.S., Du, L.M., Boudreaux, M.K., Wilcox, D.A., Intravenous immunoglobulin (IVIG) diminishes immune-mediated clearance of platelets expressing an integrin  $\alpha$ IIb $\beta$ 3 transgene product that restores hemostasis in a canine model for Glanzmann Thrombasthenia, *Blood*. 108, a:3263, 2006.
40. Shi, Q., Fahs, S.A., Wilcox, D.A., Weiler, H., Haberichter, S.L., Montgomery, R.R., Endothelial and Platelet FVIII/VWF Expression - Divergence in Clinical Effect in Murine Models of Hemophilia A with and without FVIII Inhibitory Antibodies, *Blood*. 108, a:3286, 2006.
41. Wilcox, D.A., Fang, J., Jensen, E.S., Du, L.M., and Boudreaux, M.K. Therapeutic expression of a platelet-

- specific integrin restores hemostasis in dogs with glanzmann thrombasthenia, *Molecular Therapy* . , a:767, 2007.
42. Shi, Q., Fahs, S.A., Wilcox, D.A., Morateck, P.A., Montgomery, R.R. Transplant bone marrow that is genetically modified to express FVIII only in platelets can restore hemostasis to hemophilia A mice on a strong inhibitor background, *Journal of Thrombosis and Haemostasis*. a:P-W-232, 2007.
  43. Ghevaert C., Wilcox, D.A., Fang, J., Ouwehand, W.H., Williamson, L.M. Recombinant human HPA-1A antibodies for treatment of feto-maternal allo-immune thrombocytopenia (FMAIT): proof of principle in an in vivo murine model, *Journal of Thrombosis and Haemostasis*. a:O-T-025, 2007.
  44. Wilcox, D.A., Fang, J., Jensen, E.S., Du, L.M., and Boudreaux, M.K. Correction of haemorrhagic disorders affecting platelets: gene therapy restores hemostasis within a canine model for glanzmann thrombasthenia, *Journal of Thrombosis and Haemostasis*. a:O-S-024, 2007.
  45. Qizhen Shi, Scot A Fahs, David A Wilcox, Erin L Kuether, Hartmut Weiler and Robert R Montgomery. In the Presence of Pre-existing Factor VIII (FVIII) Immunity, Hematopoietic Stem Cells (HSC) that Are Genetically Modified to Express FVIII in Platelets Were Successfully Transplanted into Hemophilic Mice Under Myeloablative and Various Non-myeloablative Conditions, *Blood*. a: 768, 2007.
  46. Gomez JA, Gama V, Yoshida T, Stapleton M, Paddock C, Wilcox D, Newman P, Matsuyama S, Blockade of chemotherapy-induced thrombocytopenia by Bax Inhibiting Peptides (BIPs) in mouse model. *Blood*, a:281 2007.
  47. David A. Wilcox, Juan Fang Eric S. Jensen, Lily M. Du, Mary K. Boudreaux. Hematopoietic stem cell gene therapy for inherited platelet bleeding disorders, *Blood Cells, Molecules, and Diseases*. 40,(2) a:291, 2008.
  48. Ghevaert C., Wilcox, D.A., Fang, J., Armour, K.L., Clark, M.R., Ouwehand, W.H., Williamson, L.M. Recombinant Human HPA-1A Antibodies for Treatment of Fetomaternal AlloimmuneThrombocytopenia (FMAIT) : Proof of Principle in an in Vivo Murine Model and Human Volunteer Studies. *Blood* a:85, 2008.
  49. Wilcox, D.A., Fang, J., North, P., Nurden P., Nurden A.T., Valentin, N. High Mortality in Mice with Platelets Expressing Integrin  $\alpha\text{IIb}\beta 3$  Locked-In Its High Affinity State, *Blood* a:1832, 2008.
  50. David A. Wilcox, Lily M. Du, Sandra L. Haberichter, Paula M. Jacobi, Juan Fang, Eric S. Jensen, Qizhen Shi and Robert R. Montgomery. Platelet Targeted Expression of Coagulation Factor VIII (FVIII) Shows efficacy for using the dog as a large animal model for gene therapy of hemophilia A. *Blood* a:3525, 2008.
  51. D.A. Wilcox, J. Fang, P. North, P. Nurden, A.T. Nurden, N. Valentin. There is a high mortality rate in mice when platelet integrin  $\alpha\text{IIb}\beta 3$  is locked-in its high affinity state, *J Thromb Haemost*, 7, a:OC-TH-097, 2009.
  52. C. Gao, B. Boylan, J. Fang, D.A. Wilcox, D.K. Newman, P.J. Newman. Antibody-independent,  $\alpha\text{IIb}\beta 3$ -mediated activation of human platelets by unfractionated heparin, *J Thromb Haemost*, 7, a:AS-TH-009, 2009.
  53. Qizhen Shi, Erin L. Kuether, Scot A. Fahs, Jocelyn Schroeder, David A. Wilcox, and Robert R. Montgomery. Sustained phenotypic correction of murine hemophilia A with pre-existing anti-FVIII immunity using lentivirus-mediated platelet-specific FVIII gene transfer. (Oral Presentation). *Blood* a:29, 2009.
  54. Lily M. Du, Timothy C. Nichols, Sandra L. Haberichter, Paula M. Jacobi, Eric S. Jensen, Juan Fang, Qizhen Shi, Robert R. Montgomery, and David A. Wilcox. Platelet-Targeted Expression of Human BDD-FVIII Reduces Bleeding in Canine Hemophilia A. (Oral Presentation). *Blood* a:691, 2009.
  55. Qizhen Shi, Erin L. Kuether, Jocelyn A. Schroeder, Scot A. Fahs, David A. Wilcox, and Robert R. Montgomery. The Important Role of Von Willebrand Factor In Platelet-Derived FVIII Gene Therapy of Murine Hemophilia A In The Presence of Inhibitors. *Blood* a:2201, 2010
  56. Lily M. Du, Helen W.G. Franck, Elizabeth P. Merricks, Paquita Nurden, Eric S. Jensen, Sandra L. Haberichter, Troy B. Hawkins, Paula M. Jacobi, Juan Fang, Sevasti B. Koukouritaki, Alan T. Nurden, Qizhen Shi, Robert R. Montgomery, and David A. Wilcox. De novo synthesis & storage of Human FVIII in platelets reduces bleeding in canine Hemophilia A. *Blood* a:2198, 2010.
  57. Lily M. Du, Helen W.G. Franck, Elizabeth P. Merricks, Paquita Nurden, Eric S. Jensen, Sandra L. Haberichter, Troy B. Hawkins, Paula M. Jacobi, Juan Fang, Sevasti B. Koukouritaki, Alan T. Nurden, Qizhen Shi, Robert R. Montgomery, and David A. Wilcox. Gene therapy targeting synthesis of coagulation factor viii in platelets reduces bleeding in canine hemophilia a. Awarded Best Abstract in Basic Research to be presented at the American Society for Bone Marrow Transplant, Hawaii a:3, Feb 19, 2011.

58. Cunji Gao, Juan Fang, Hu iying Zhi, David A. Wilcox , Peter J. Newman. Disaggregation signals in initiated by ligand binding and platelet aggregation. *Blood* a:2196. 2011
59. Spencer K. Sullivan, Jason A. Mills, Li Zhai, Prasuna Paluru, Karen Yo, Guohua Zhao, Michele Lambert, Sevasti B. Koukouritaki, Juan Fang, David A. Wilcox, Deborah L. French, Mortimer Poncz, Paul Gadue. Tissue-specific transgene expression in induced pluripotent stem (iPS) cell-derived megakaryocytes: correction of glanzmann thrombasthenia (GT).(Oral Presentation)*Blood* a:387, 2012.
60. Tim Thijs, Katleen Broos, Wim Maes, Aline Vandenbulcke, Juan Fang, David A. Wilcox, Karen Vanhoorelbeke, Hans Deckmyn. Establishment of conditions for in vitro and in vivo production of genetically modified human megakaryocytes and platelets. *J Thromb Haemost*, a:PB1.29-2, 2013.
61. Lily M. Du, Paquita Nurden, Alan T. Nurden, Timothy C. Nichols, Dwight A. Bellinger, Eric S. Jensen, Sandra L. Haberichter, Qizhen Shi, Kenneth Cornetta, David A. Wilcox. Genetic targeting of human coagulation factor VIII into platelet a-granules resulted in long-term improvement of hemostatic function in canine hemophilia A. (Oral Presentation)*J Thromb Haemost*,a:OC-80.1, 2013.
62. Jocelyn A. Schroeder, Yingyu Chen, David A. Wilcox, Robert R. Montgomery, Qizhen Shi. In vivo selection of genetically manipulated hematopoietic stem cells for platelet gene therapy of hemophilia A *Blood* a:2329, 2013
63. Jocelyn A. Schroeder, Yingyu Chen, David A. Wilcox, and Qizhen Shi. In vivo selection of genetically manipulated platelets corrects murine hemophilic phenotype and induces immune tolerance even using a low multiplicity of infection for transduction. (Oral Presentation ISTH SSC) Milwaukee, WI July 2014.
64. Juan Fang, Ming Yao, Weiqing Jing, Beicheng Sun, Bryon D. Johnson, and David A. Wilcox. Platelets engineered to store interleukin-24 inhibited melanoma growth in mice. (Oral Presentation) *J Thromb Haemost*,13 (Suppl S2):225 a:OR342, (06/24/15).
65. Cao S, Nishijima Y, Fang J, **Wilcox DA**, Zhang DX. Arachidonic acid-induced TRPV4 activation is enhanced by serine phosphorylation in human coronary artery endothelial cells. *FASEB J* 30:1281.2, 2016.
66. Juan Fang, Ming Yao, Weiqing Jing, Beicheng Sun, Bryon D. Johnson, and David A. Wilcox. Platelets engineered to express interleukin-24 inhibited melanoma tumor growth in mice. *Molecular Therapy*. a:668, (05/06/2016).
67. David A. Wilcox. Hematopoietic Stem Cell Gene Therapy. Invited Presentation, National Hemophilia Foundation's 13th Workshop on New Technologies and Gene Transfer for Hemophilia Georgetown University, Washington, DC (10/21/16)
68. Sarah K Westbury, Bridge Consortium and ThromboGenomics Consortium, Pathogenic Variants in Thrombocytopenia Genes in 119 Cases with Undiagnosed Heritable Thrombocytopenia. *Blood* 128 a:362, 2016.
69. Suthesh Sivapalaratnam, Willem Ouwehand, Bridge Consortium and ThromboGenomics Consortium, Rare Variants of GPIBB underlie Autosomal Dominant Macrothrombocytopenia: Findings of Large Unique Bleeding and Platelet Disorders Cohort. *Blood* 128 a:1359, 2016.
70. Claire Lentaigne, Bridge Consortium and ThromboGenomics Consortium, High Throughput Sequencing in 3449 Patients with Bleeding and Platelet Disorders: Novel Gene Discovery and Robust Diagnosis. *Blood* 130 a:5, 2017.
71. Koukouritaki, S., Thinn, A.M., Bark K.J., Fang, J., Slater, H.S., Du, L.M., Nurden, A.D., DiPaola, J., Zhu, J., Wilcox, D.A. A Novel F153S ITGB3 Mutation Found in a Glanzmann Thrombasthenic Patient Reveals a Structural Clasp that Modulates Integrin Activation, (Oral Communication) *J Thromb Haemost*,13 (Suppl ): a:, (07/08/19). International Society for Thrombosis and Haemostasis, Melbourne, AUS. (07/05/19 - 07/10/19).
72. Frederic Bushman, Aoife Roche, John Everett, Angie Petrichenko, Carole Lee, Shantan Reddy, Mary Eapen, David Wilcox, Denise Sabatino, Genomics of gene transfer and vector integration. (Oral Presentation 17th Workshop of Gene Therapy for Hemophilia, National Hemophilia Foundation (03/23/23, Washington DC).
73. David A. Wilcox, LV HSC Gene Therapy using Platelet Derived Factor VIII “Pleightlet™”: Ongoing Phase I First In Human Clinical Trial for Hemophilia A with a History of Inhibitors (03/24/23 Wash DC).