

Nineteenth Annual
Fanconi Anemia Research Fund
Scientific Symposium
The Westin Hotel Michigan Avenue, Chicago, Illinois

AGENDA

Monday – Afternoon, October 8, 2007

3:00 **Registration**

4:00 - 5:30

**Fanconi Anemia 101: A Sophisticated Introduction to the
Science and Medicine of Fanconi Anemia**
Wellington Ballroom

*This session is intended especially for those new to Fanconi anemia
research and clinical care. All attendees interested in an overview of
unanswered questions, new research directions, and resources to
support research on Fanconi anemia are welcome.*

Introductions:

Grover C. Bagby, Jr., MD
Chair, FA Research Fund Scientific Advisory Board
Oregon Health & Science University Cancer Institute, Portland, OR

Faculty:

Raymond Monnat, Jr., MD
FA Research Fund Scientific Advisory Board
University of Washington, Seattle, WA

Akiko Shimamura, MD, PhD
Children's Hospital, Boston, MA

6:00 - 8:00

Welcome Reception
Poster Viewing
Cotillion Ballroom

Tuesday – Morning, October 9, 2007

7:00 - 8:00

Continental Breakfast
Foyer
Poster Viewing
Cotillion Ballroom

Plenary Sessions
Wellington Ballroom

8:00 - 8:15

Welcome

David Frohnmayer, Conference Moderator
Co-Founder, Fanconi Anemia Research Fund, Inc.
President, University of Oregon
Eugene, Oregon

8:15 - 8:30

Overview

Grover C. Bagby, Jr., MD
Chair, Scientific Advisory Board
Fanconi Anemia Research Fund, Inc.
Oregon Health & Science University Cancer Institute
Portland, Oregon

8:30 - 9:00

Keynote Address:

*Developmental Defects: Ideas as to the Molecular and Cellular Deficits
Underlying the Pathophysiology*

Lee Niswander, PhD
University of Colorado Health Sciences Center
Denver, Colorado

9:00 - 9:10

Questions and Answers

Session I:

DNA Damage and Repair I

Chairperson: Raymond Monnat, Jr., MD
Scientific Advisory Board, FA Research Fund
University of Washington
Seattle, Washington

9:10 - 9:15

Session Overview: Raymond Monnat, Jr., MD

9:15 - 9:25

**Xiao-Yin Zhang, BA, MRC Laboratory of Molecular Biology,
Cambridge, United Kingdom: *Comprehensive Genetic Analysis of a
Functionally Conserved FA Pathway in the Social Amoeba.***

9:25 - 9:30

Questions and Answers

- 9:30 - 9:40 **George-Lucian Moldovan, PhD, Department of Radiation Oncology, Dana-Farber Cancer Institute, Harvard Medical School, Boston, Massachusetts:** *Regulation of Translesion Synthesis by the Fanconi Anemia Core Complex.*
- 9:40 - 9:45 Questions and Answers
- 9:45 - 9:55 **Alexandra Sobeck, PhD, Department of Biochemistry and Molecular Biology, Oregon Health & Science University, Portland, Oregon:** *Differential Activation of Core Complex Member FANCM and Downstream Target FANCD2 during the DNA Damage Response.*
- 9:55 - 10:00 Questions and Answers
- 10:00 - 10:20 **Break**
- 10:20 - 10:30 **Angelos Constantinou, PhD, Department of Biochemistry, University of Lausanne, Epalinges, Switzerland:** *Recognition and Translocation of Branched DNA Structures by FANCM Protein.*
- 10:30 - 10:35 Questions and Answers
- 10:35 - 10:45 **Wojciech Niedzwiedz, PhD, MRC Laboratory of Molecular Biology, Cambridge, United Kingdom:** *FANCM: Motoring Forward in DNA Crosslink Repair?*
- 10:45 - 10:50 Questions and Answers
- 10:50 - 11:00 **Johannes Walter, PhD, Department of Biological Chemistry and Molecular Pharmacology, Harvard Medical School, Boston, Massachusetts:** *Biochemical Mechanism of Replication-coupled DNA Interstrand Cross-link Repair.*
- 11:00 - 11:05 Questions and Answers
- 11:05 - 11:15 **Stacie Stone, Department of Biochemistry and Molecular Biology, Oregon Health & Science University, Portland, Oregon:** *Identification of a Novel Partner Protein for FANCM.*
- 11:15 - 11:20 Questions and Answers
- 11:20 - 11:30 **Jungmin Kim, PhD, Dana-Farber Cancer Institute, Boston, Massachusetts:** *The Role of FANCM/FAAP24 in the Chromatin Association of the FA Core Complex.*
- 11:30 - 11:35 Questions and Answers
- 11:35 - 11:40 **Session Wrap-up: Raymond Monnat, Jr., MD**

Session II:

Telomeres and Other Pathways

Chairperson: Stephen Meyn, MD, FRCP(C)
Scientific Advisory Board, FA Research Fund
The Hospital for Sick Children
Toronto, Canada

- 11:40 - 11:45 **Session Overview: Stephen Meyn, MD, FRCP(C)**

11:45 - 11:55 **Heather Root, The Hospital for Sick Children, Toronto, Canada:** *The Fanconi Anemia Pathway Plays a Critical Role in Recombinational Telomere Maintenance in ALT-immortalized Human Cells.*

11:55 - 12:00 Questions and Answers

12:00 - 1:00

Hosted Lunch
Cotillion, Buckingham, Windsor Rooms
Poster Viewing
Cotillion Ballroom

Tuesday – Afternoon, October 9, 2007

Session II:

Telomeres and Other Pathways (continued)

1:00 - 1:10 **Toshiyasu Taniguchi, MD, PhD, Fred Hutchinson Cancer Research Center, Seattle, Washington:** *Regulation of the Fanconi Anemia-BRCA Pathway by microRNAs.*

1:10 - 1:15 Questions and Answers

1:15 - 1:25 **Alex Lyakhovich, PhD, Department of Genetics and Microbiology, Universitat Autònoma de Barcelona, Barcelona, Spain:** *FANCD2-mediated Regulation of PARP Activity Confers Telomere Maintenance.*

1:25 - 1:30 Questions and Answers

1:30 - 1:40 **Jordi Surrallés, PhD, Department of Genetics and Microbiology, Universitat Autònoma de Barcelona, Barcelona, Spain:** *A Critical Role for Fanconi Anemia Proteins FANCG and FANCD2 in Centrosome Stability and Function.*

1:40 - 1:45 Questions and Answers

1:45 - 1:55 **Niall Howlett, PhD, Department of Cell and Molecular Biology, University of Rhode Island, Kingston, Rhode Island:** *Functional Interaction between FANCD2 and PCNA via a Conserved PCNA-Interaction Motif.*

1:55 - 2:00 Questions and Answers

2:00 - 2:10 **Anna Olsen, DNA Repair, Cancer Research UK Laboratories, Oxford University, Oxford, United Kingdom:** *Processing of DNA Interstrand Cross-links by XPF-ERCC1 is Required for the Efficient Localization of FANCD2 to Chromatin.*

2:10 - 2:15 Questions and Answers

2:15 - 2:20 **Session Wrap-up: Stephen Meyn, MD, FRCP(C)**

Session III:

DNA Damage and Repair II

Chairperson: Maureen Hoatlin, PhD
Oregon Health & Science University
Portland, Oregon

- 2:20 - 2:25 **Session Overview: Maureen Hoatlin, PhD**
- 2:25 - 2:35 **Lily Wang, Institute of Cancer Genetics, Columbia University, New York, New York: *Fanconi Anemia Proteins are Recruited to Chromatin during Replication and Play a Role in Stabilizing Replication Forks.***
- 2:35 - 2:40 Questions and Answers
- 2:40 - 2:50 **Robert Brosh, Jr., PhD, National Institute on Aging, NIH, Baltimore, Maryland: *A Role for the FANCD1 Helicase in Resolution of G4 Alternate DNA Structures.***
- 2:50 - 2:55 Questions and Answers
- 2:55 - 3:15 **Break**
- 3:15 - 3:25 **Paul Andreassen, PhD, Division of Experimental Hematology, Fanconi Anemia Comprehensive Care Center, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio: *Understanding How FANCD1 Functions in the FA/BRCA Pathway.***
- 3:25 - 3:30 Questions and Answers
- 3:30 - 3:40 **Zhijiang Yan, PhD, Laboratory of Genetics, National Institute on Aging, NIH, Baltimore, Maryland: *A Novel Component of Fanconi Anemia Core Complex is Essential for Activation of the FA-associated DNA Repair Pathway.***
- 3:40 - 3:45 Questions and Answers
- 3:45 - 3:55 **Jean-Yves Masson, PhD, Laval University Cancer Research Center, Québec, Canada: *Interplay between Human FANCD2 and the MRE11-RAD50-NBS1 Complex.***
- 3:55 - 4:00 Questions and Answers
- 4:00 - 4:05 **Session Wrap-up: Maureen Hoatlin, PhD**

4:05 - 7:00

Poster Presentations
Wine and Hors d'Oeuvres, Hosted
Cotillion Ballroom

7:00 - 9:00

Symposium Dinner
All registrants are invited
Wellington Ballroom

Wednesday – Morning, October 10, 2007

7:00 - 8:00

Continental Breakfast
Foyer
Poster Viewing
Cotillion Ballroom

Session IV:

Hematopoiesis

Chairperson: Madeleine Carreau, PhD
Laval University
Québec, Canada

8:00 - 8:05

Session Overview: Madeleine Carreau, PhD

8:05 - 8:15

Cédric Tremblay, Laval University, Québec, Canada: *HES1 is a Novel Monoubiquitinated Partner of the FA Core Complex.*

8:15 - 8:20

Questions and Answers

8:20 - 8:30

Amy Skinner, PhD, Department of Pediatrics, Oregon Health & Science University, Portland, Oregon: *Induction of CXCR4 in FANC-A, C, and D2 -/- Progenitor Cells to Enhance Hematopoietic Homing.*

8:30 - 8:35

Questions and Answers

8:35 - 8:45

David Williams, MD, Division of Experimental Hematology, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio: *Genetic Correction of the Fanconi Anemia Stem Cell Defect by Ectopic Delivery of HOXB4.*

8:45 - 8:50

Questions and Answers

8:50 - 9:00

Frank Smith, MD, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio: *A Majority of Patients with Fanconi Anemia Respond to Low Dose Androgens: A Pilot Trial of Oxandrolone for the Treatment.*

9:00 - 9:05

Questions and Answers

9:05 - 9:15

Nupur Gangopadhyay, DVM, PhD, Department of Surgery, University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania: *Clonally Selected Hematopoietic Stem Cells as a Tool for Gene Therapy.*

9:15 - 9:20

Questions and Answers

9:20 - 9:30

John Postlethwait, PhD, Institute of Neuroscience, University of Oregon, Eugene, Oregon: *The Zebrafish Fanconi Anemia Network is Mechanistically Similar to Human.*

9:30 - 9:35

Questions and Answers

9:35 - 9:40

Session Wrap-up: Madeleine Carreau, PhD

9:40 - 10:00

Break

Session V:

Stem Cell Transplantation and Preclinical Gene Therapy

Chairperson: David Williams, MD

Scientific Advisory Board, FA Research Fund

Cincinnati Children's Hospital Medical Center

Cincinnati, Ohio

10:00 - 10:05

Session Overview: David Williams, MD

10:05 - 10:15

Carmem Bonfim, MD, Federal University of Parana, Parana, Brazil: *Hematopoietic Cell Transplantation from HLA-Matched Related Donors in 43 Patients with Fanconi Anemia.*

10:15 - 10:20

Questions and Answers

10:20 - 10:30

Margaret MacMillan, MD, Pediatric Blood and Transplant Program, University of Minnesota, Minneapolis, Minnesota: *Total Body Irradiation (TBI) Dose De-escalation Study in FA Patients Undergoing Alternate Donor Hematopoietic Stem Cell Transplantation (HSCT).*

10:30 - 10:35

Questions and Answers

10:35 - 10:45

Madeleine Carreau, PhD, Department of Pediatrics, Laval University, Québec, Canada: *In vivo Lentiviral Gene Delivery Prevents Bone Marrow Aplasia in FA Group C Knockout Mice.*

10:45 - 10:50

Questions and Answers

10:50 - 11:00

Paula Rio Galdo, PhD, Hematopoiesis and Gene Therapy Division, CIEMAT, Madrid, Spain: *Genetically Corrected Stem Cells Induce a Spontaneous Proliferation Advantage in a FA-D1 Mouse Model.*

11:00 - 11:05

Questions and Answers

11:05 - 11:15

Laura Haneline, MD, Cancer Research Institute, Indiana University School of Medicine, Indianapolis, Indiana: *Molecular Targeting Strategy to Enhance Fanconi Anemia Type C-deficient Hematopoietic Stem Cell Function.*

11:15 - 11:20

Questions and Answers

11:20 - 11:30

Zejin Sun, PhD, Indiana University School of Medicine, Indianapolis, Indiana: *Lentiviral Constructs Pseudotyped with Modified Foamy Viral Envelope Corrects Fanca^{-/-} HSCs with Reduced Toxicity.*

11:30 - 11:35

Questions and Answers

11:35 - 11:45

Alena Chekmasova, PhD, Department of Pathology, Anatomy, and Cell Biology, Thomas Jefferson University, Philadelphia, Pennsylvania: *Efficient Transduction of Marrow Progenitors and Long-term Gene Expression in their Progeny by in vivo Gene Delivery.*

11:45 - 11:50

Questions and Answers

11:50 - 11:55

Session Wrap-up: David Williams, MD

11:55 - 1:00

Poster Viewing
Cotillion Ballroom

12:00 - 1:00

Hosted Lunch
Cotillion, Buckingham, Windsor Rooms

Wednesday - Afternoon, October 10, 2007

1:00 - 1:30

Keynote Address:

Endocrine “Dys”function: Molecular and Cellular Pathophysiology and Treatment

Laura Niedernhofer, MD, PhD
University of Pittsburgh Cancer Institute
Pittsburgh, Pennsylvania

1:30 - 1:40

Questions and Answers

Session VI:

DNA Damage and Repair III

Chairperson: K. J. Patel, PhD, MRCP
MRC Laboratory of Molecular Biology
Cambridge, United Kingdom

1:40 - 1:45

Session Overview: K. J. Patel, PhD, MRCP

1:45 - 1:55

Simon Boulton, PhD, Cancer Research UK, London Research Institute, Herts, United Kingdom: *Molecular Interplay between the S-phase Checkpoint and FA Core Complex.*

1:55 - 2:00

Questions and Answers

2:00 - 2:10

Hiroyuki Kitao, PhD, Research Institute for Radiation Biology and Medicine, Hiroshima University, Hiroshima, Japan: *Frequent Loss of the Immunoglobulin Heavy Chain Gene Occurs in FANCI-deficient DT40 Cell Line.*

2:10 - 2:15

Questions and Answers

2:15 - 2:25

Josephine Dorsman, PhD, Department of Clinical Genetics, Vrije Universiteit Medical Center, Amsterdam, The Netherlands: *Identification of the Fanconi Anemia Complementation Group I Gene, FANCI.*

2:25 - 2:30

Questions and Answers

- 2:30 - 2:40 **Minoru Takata, MD, PhD, Research Institute for Radiation Biology and Medicine, Hiroshima University, Hiroshima, Japan: *Role of FANCI Protein and Its Phosphorylation for the Fanconi Anemia Pathway Function.***
- 2:40 - 2:45 Questions and Answers
- 2:45 - 3:05 **Break**
- 3:05 - 3:15 **Robb Moses, MD, Department of Molecular and Medical Genetics, Oregon Health & Science University, Portland, Oregon: *Bypass DNA Polymerases Rev3, Rev1, and Pol Kappa are Required for Normal DNA Interstrand Crosslink Repair.***
- 3:15 - 3:20 Questions and Answers
- 3:20 - 3:30 **Patricia McChesney, PhD, Department of Microbiology, University of Virginia, Charlottesville, Virginia: *The Fanconi Anemia Protein FANCI Binds to and Regulates Chromatin Loading of FANCD2.***
- 3:30 - 3:35 Questions and Answers
- 3:35 - 3:40 **Session Wrap-up: K. J. Patel, PhD, MRCP**

Session VII:

Carcinogenesis, Experimental Therapeutics, and Prevention

Chairperson: Alan D'Andrea, MD

Dana-Farber Cancer Institute

Boston, Massachusetts

- 3:40 - 3:45 **Session Overview: Alan D'Andrea, MD**
- 3:45 - 3:55 **Laura Hays, PhD, Department of Hematology and Oncology, Oregon Health & Science University, Portland, Oregon: *FANCC-deficient Murine Head and Neck Cancers: Disconnection of Crosslinker-induced Toxicity and Chromosomal Instability.***
- 3:55 - 4:00 Questions and Answers
- 4:00 - 4:10 **Ian MacKenzie, DDS, PhD, FDSRCS, Institute of Cell and Molecular Science, Queen Mary University of London, London, United Kingdom: *Oral Cancer Cell Lines from FA Patients Contain Stem Cells with Unusual Apoptotic Resistance and Cell Cycle Patterns.***
- 4:10 - 4:15 Questions and Answers
- 4:15 - 4:25 **Hester van Zeeburg, MSc, Department of Otolaryngology/Head and Neck Surgery, Vrije Universiteit Medical Center, Amsterdam, The Netherlands: *Generation of Retargeted Oncolytic Adenoviruses for Treatment of Oral Preneoplastic Lesions.***
- 4:25 - 4:30 Questions and Answers

- 4:30 - 4:40 **Ruud Brakenhoff, PhD, Department of Otolaryngology/Head and Neck Surgery, Vrije Universiteit Medical Center, Amsterdam, The Netherlands:** *Development of a Non-invasive Screening Method for Early Diagnosis of Preneoplastic Lesions.*
- 4:40 - 4:45 Questions and Answers
- 4:45 - 4:55 **Céline Jacquemont, PhD, Divisions of Human Biology and Public Health Sciences, Fred Hutchinson Cancer Research Center, Seattle, Washington:** *Characterization of Small Molecule Inhibitors of the Fanconi Anemia Pathway.*
- 4:55 - 5:00 Questions and Answers

5:00 - 9:00

Poster Viewing
Cotillion Ballroom

6:00 - 9:00

Joint Meeting and Dinner
Board of Directors and Scientific Advisory Board
Fanconi Anemia Research Fund
Michigan Room

Thursday – Morning, October 11, 2007

7:00 - 8:00

Continental Breakfast
Foyer
Poster Viewing
Cotillion Ballroom

8:00 - 8:30

Keynote Address:
Cancer: Molecular and Cellular Pathophysiology and Treatment
Gary Clayman, DMD, MD, FACS
University of Texas MD Anderson Cancer Center
Houston, Texas

8:30 - 8:45

Questions and Answers

Session VII:

Carcinogenesis, Experimental Therapeutics, and Prevention
(continued)

8:45 - 8:55	Qingshuo Zhang, PhD, Oregon Stem Cell Center, Oregon Health & Science University, Portland, Oregon: <i>Chemoprevention Approaches for Fanconi Anemia.</i>
8:55 - 9:00	Questions and Answers
9:00 - 9:10	Alan D'Andrea, MD, Radiation Oncology, Division of Genomic Stability and DNA Repair, Dana-Farber Cancer Institute, Boston, Massachusetts: <i>FA Pathway Deficient Cells are Hypersensitive to CHK1 Inhibition.</i>
9:10 - 9:15	Questions and Answers
9:15 - 9:25	Wataru Sakai, PhD, Divisions of Human Biology and Public Health Sciences, Fred Hutchinson Cancer Research Center, Seattle, Washington: <i>Clinical Significance of Genetic Reversion of BRCA2/FANCD1 Mutation in Cisplatin-treated BRCA2-mutated Cancer Cells.</i>
9:25 - 9:30	Questions and Answers
9:30 - 9:40	Jun Nakamura, PhD, Department of Environmental Sciences and Engineering, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina: <i>Cells Deficient in the FANC/BRCA Pathway are Hypersensitive to Plasma Levels of Formaldehyde.</i>
9:40 - 9:45	Questions and Answers
9:45 - 9:50	Session Wrap-up: Alan D'Andrea, MD
9:50 - 10:15	Break

10:15 - Noon

Closing Forum: Planning for the Future

Chairperson:

Grover C. Bagby, Jr., MD

***Chair, FA Research Fund Scientific Advisory Board
Oregon Health & Science University Cancer Institute
Portland, Oregon***