

**TWENTIETH ANNUAL  
FANCONI ANEMIA RESEARCH FUND  
SCIENTIFIC SYMPOSIUM  
HILTON HOTEL AND CONFERENCE CENTER, EUGENE, OREGON**

**AGENDA**

**SATURDAY – AFTERNOON, OCTOBER 4, 2008**

3:00            **Registration**

4:00 - 5:45

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

*This session is intended especially for those new to Fanconi anemia research and clinical care. However, all attendees interested in an overview of unanswered questions, new research directions, resources for Fanconi anemia research and meeting highlights are welcome and encouraged to attend.*

**Introductions:**

**Grover C. Bagby, Jr., MD**

Chair, Scientific Advisory Board, Fanconi Anemia Research Fund  
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**

Fred Hutchinson Cancer Research Center, Seattle, WA

6:00 - 8:00    **Welcome Reception**

**Poster Viewing**

*Composers Hall*

**SUNDAY – MORNING, OCTOBER 5, 2008**

7:00 - 8:00    **Buffet Breakfast**

*Hellman and Wilder Rooms*

**Poster Viewing**

*Composers Hall*

**PLENARY SESSION**  
***O'NEILL AND WILLIAMS HALLS***

- 8:00 - 8:15      **Welcome**  
**David Frohnmayer**  
Co-Founder, Fanconi Anemia Research Fund  
President, University of Oregon  
Eugene, Oregon
- 8:15 - 8:30      **Overview**  
**Grover C. Bagby, Jr., MD**  
Chair, Scientific Advisory Board, Fanconi Anemia Research Fund  
Oregon Health & Science University Cancer Institute  
Portland, Oregon
- 8:30 - 8:45      ***Challenge to Researchers: Needed Research Directions to Address Bone Marrow Failure and Acute Myeloid Leukemia in FA Patients***  
**Franklin O. Smith, MD**  
Cincinnati Children's Hospital Medical Center  
Cincinnati, Ohio
- Session I:**      **Telomeres, Transcription, and Repair**  
**Chairperson: Kevin Hiom, PhD**  
**MRC Laboratory of Molecular Biology**  
**Cambridge, United Kingdom**
- 8:45              **Session Overview: Chair**
- 8:50              **Blanche Alter, MD, MPH**, National Cancer Institute, Rockville, Maryland:  
*Telomeres are Normal Length in Patients with Fanconi Anemia.*
- 9:00              Questions and Answers
- 9:05              **Yie Liu, PhD**, National Institute on Aging, Baltimore, Maryland: *Inactivation of FANCC Rescued Critically Shortened Telomeres in Late Generation of Telomerase Null Animals.*
- 9:15              Questions and Answers
- 9:20              **Madeleine Carreau, PhD**, Laval University, Quebec, Canada: *The Fanconi Anemia Core Complex Acts as a Transcriptional Co-regulator in HES1 Signaling.*
- 9:30              Questions and Answers
- 9:35              **Allison M. Green**, Yale University School of Medicine, New Haven, Connecticut:  
*Interaction of the FA Pathway with Transcription.*
- 9:45              Questions and Answers
- 9:50              **Koji Nakanishi, MD**, Memorial Sloan-Kettering Cancer Center, New York, New York: *Homology-directed Repair is a Major Pathway for Intercrosslink Repair in Mammalian Cells.*
- 10:00             Questions and Answers
- 10:05             **Break**

- 10:25 **Puck Knipsheer, PhD**, Harvard Medical School, Boston, Massachusetts:  
*Biochemical Mechanism of Replication-Coupled DNA Interstrand Cross-Link Repair.*
- 10:35 Questions and Answers
- 10:40 **Lily Wang**, Institute of Cancer Genetics, Columbia University, New York, New York:  
*Fanconi Anemia Proteins in DNA Replication and Repair.*
- 10:50 Questions and Answers
- 10:55 **Session Wrap-Up: Kevin Hiom, PhD, Chair**
- 11:00 **Keynote Address: Squamous Cell Carcinoma in Fanconi Anemia Patients: A Challenge for Basic Science Researchers**  
**Jennifer Rubin Grandis, MD, FACS**  
Director, Head and Neck Cancer Program  
University of Pittsburgh Cancer Institute, Pittsburgh, Pennsylvania
- 11:30 Questions and Answers

**Session II:**

**Gene Therapy**

**Chairperson: Eva Guinan, MD**  
**Scientific Advisory Board, Fanconi Anemia Research Fund**  
**Dana-Farber Cancer Institute**  
**Boston, Massachusetts**

- 11:40 **Session Overview: Chair**
- 11:45 **David S. Strayer, MD, PhD**, Thomas Jefferson Medical College, Philadelphia, Pennsylvania:  
*In Vivo Bone Marrow-directed Gene Delivery Leads to Widespread Transgene Expression in Many Organs.*
- 11:55 Questions & Answers

**[Gene Therapy Session to be continued at 1 p.m.]**

- 12:00 **Hosted Lunch**  
*Hellman and Wilder Rooms*  
**Poster Viewing**  
*Composers Hall*

**Session II:**

**Gene Therapy (continued):**

- 1:00 **Juan Bueren, PhD**, CIEMAT, Madrid, Spain: *The Relevance of Transducing Unpurified Bone Marrow Cells for the Gene Therapy of Fanconi Anemia Patients.*
- 1:10 Questions and Answers
- 1:15 **Linda Hartmann**, Heinrich Heine University, Duesseldorf, Germany: *Phenotypic Correction of Fanconi Anemia Cells by Lentiviral Delivery of Specifically Engineered U1 snRNAs.*
- 1:25 Questions and Answers
- 1:30 **Session Wrap-Up: Chair**

**Session III:**

**Cancer**

**Chairperson: Grover Bagby, Jr, MD**  
**Chair, Scientific Advisory Board, Fanconi Anemia Research Fund**  
**Oregon Health & Science University Cancer Institute**  
**Portland, Oregon**

- 1:40 **Session Overview: Chair**
- 1:45 **Eunike Velleuer, MD**, Heinrich Heine University, Duesseldorf, Germany: *A Novel De-escalating Procedure to Enhance the Compliance of FA Patients for a Regular Oral Squamous Cell Carcinoma Screening.*
- 1:55 Questions and Answers
- 2:00 **Laura Hays, PhD**, Oregon Health & Science University, Portland, Oregon:  
*Chromosomal Instability and Enhanced Invasiveness of Fance-deficient Murine Head and Neck Squamous Cell Carcinomas*
- 2:10 Questions and Answers
- 2:15 **Susanne Wells, PhD**, Cincinnati Childrens Hospital Medical Center, Cincinnati, Ohio: *Suppression of HPV-associated Hyperplasia by FA Complementation in Keratinocytes from Children with Fanconi Anemia.*
- 2:25 Questions and Answers
- 2:30 **Celine Jacquemont, PhD**, Fred Hutchinson Cancer Research Center, Seattle, Washington: *Fanconi Anemia Pathway Inhibitors Which Sensitize Tumor Cells to Cisplatin.*
- 2:40 Questions and Answers
- 2:45 **Igor Landais, PhD**, Oregon Health & Science University, Portland, Oregon:  
*Characterization of the Curcumin Analog EF24 as a Potent Inhibitor of the Fanconi Anemia Pathway.*
- 2:55 Questions and Answers
- 3:00 **Session Wrap-Up: Chair**
- 3:05 **Break**
- 3:20 ***Diagnosis and Management of Fanconi Anemia: Consensus Guidelines for Care***  
**Eva Guinan, MD**  
Scientific Advisory Board, Fanconi Anemia Research Fund  
Dana-Farber Cancer Institute  
Boston, Massachusetts.
- 4:00 - 7:00 **Poster Presentations**  
**Wine and Hors d'Oeuvres, Hosted**  
*Composers Hall*
- 7:00 - 9:00 **Symposium Dinner**  
All registrants are invited  
*Hellman and Wilder Rooms*

**MONDAY – MORNING, OCTOBER 6, 2008**

7:00 - 8:00      **Buffet Breakfast**  
*Hellman and Wilder Rooms*  
**Poster Viewing**  
*Composers Hall*

**Session IV:**      **Helicases in FA Pathway Function**  
**Chair: Stephen Meyn, MD, PhD**  
**Scientific Advisory Board, Fanconi Anemia Research Fund**  
**The Hospital for Sick Children**  
**Toronto, Ontario, Canada**

8:00              **Session Overview: Chair**

8:05              **Weidong Wang, PhD**, National Institute on Aging, Baltimore, Maryland: *FANCM of the Fanconi Anemia Core Complex is Required for both Monoubiquitination and DNA Repair.*

8:15              Questions and Answers

8:20              **Kerstin Gari, PhD**, University of Lausanne, Lausanne, Switzerland: *Remodeling of DNA Replication Structures by the Branchpoint Translocase FANCM.*

8:30              Questions and Answers

8:35              **Sietske Bakker, MSc**, Netherlands Cancer Institute, Amsterdam, The Netherlands: *Fancm, an Atypical Fanconi Protein.*

8:45              Questions and Answers

8:50              **Ruhikanta Meetei, PhD**, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio: *Structure-function Analysis of FANCM by Complementation Studies in EUFA867 Cells.*

9:00              Questions and Answers

9:05              **Younghoon Kee, PhD**, Dana-Farber Cancer Institute, Boston, Massachusetts: *Regulated Degradation of FancM in the Fanconi Anemia Pathway.*

9:15              Questions and Answers

9:20              **Kevin Hiom, PhD**, University of Cambridge, Cambridge, United Kingdom: *The Role of FANCI in the Maintenance of Genome Stability.*

9:30              Questions and Answers

9:35              **Robert M. Brosh, PhD**, National Institute on Aging, Baltimore, Maryland: *FANCI Uses its Motor ATPase to Disrupt Protein-DNA Complexes, Unwind Triplexes, and Inhibit RAD51 Strand Exchange.*

9:45              Questions and Answers

9:50              **Valeria Naim, PhD**, Institut Gustave Roussy, Villejuif, France: *A Role for the FANCI/BRCA Pathway in Chromosome Segregation.*

10:00             Questions and Answers

10:05             **Session Wrap-Up: Chair**

10:10 **Break**

**Session V:**

**Survival Pathways**

**Richard Gelinas, PhD**

**Scientific Advisory Board, Fanconi Anemia Research Fund**

**Battelle Seattle Research Center**

**Seattle, Washington**

10:30 **Session Overview: Chair**

10:35 **Alex Lyakhovich, PhD**, Universitat Autònoma de Barcelona, Barcelona, Spain:  
*Role of PARP/Caspase-3 Activity in FA Cells undergoing Oxidative Stress.*

10:45 Questions and Answers

10:50 **Grover C. Bagby, Jr., MD**, Oregon Health & Science University Cancer Institute,  
Portland, Oregon: *FANCD2 Participates in JAK2 Dependent Activation of Stat5a in  
Fibroblasts.*

11:00 Questions and Answers

11:05 **Stefan Meyer, MD, PhD**, University of Manchester, Manchester, United  
Kingdom: *Bone Marrow Chromosomal Gains in 3q26q29 in FA Results in Activation of  
Oncogenes with TGF- $\beta$  Antagonistic Properties.*

11:15 Questions and Answers

11:20 **Session Overview [Session to be continued in afternoon]: Chair**

11:30 - 1:30 **Poster Viewing**  
*Composers Hall*

11:30 – 1:30 **Board Meeting: Scientific Advisory Board and Board of Directors**  
*Studio B and C*

Noon - 1:00 **Buffet Lunch**  
*Hellman and Wilder Rooms*

**Session V:**

**Survival Pathways (continued)**

1:30 **Ihn Young Song**, Boston University School of Medicine, Boston, Massachusetts: *A  
Novel Role for Fanconi Anemia Pathway Protein FANCD2 in Cell Cycle Progression of  
Untransformed Primary Human Cells.*

1:40 Questions and Answers

1:45 **Kornelia Neveling, PhD**, University of Wuerzburg, Wuerzburg, Germany: *Different  
Causes for Fanconi Anemia Patients to Reach Adulthood.*

1:55 Questions and Answers

2:00 **Petra van der Lelij**, Vrije University Medical Center, Amsterdam, The Netherlands:  
*Diagnostic Overlap between Fanconi Anemia and Roberts Syndrome.*

2:10 Questions and Answers

2:15 **Session Wrap-Up: Chair**

**Session VI:**

**Novel Animal Models**

**Chairperson: Raymond J. Monnat, Jr, MD**  
**Scientific Advisory Board, Fanconi Anemia Research Fund**  
**University of Washington School of Medicine**  
**Seattle, Washington**

2:20 **Session Overview: Chair**

2:25 **John Postlethwait, PhD**, University of Oregon, Eugene, Oregon: *A Zebrafish Fanconi Anemia C Splice Mutation Models Human FA-C.*

2:35 Questions and Answers

2:40 **Alison Clark**, Yale University School of Medicine, New Haven, Connecticut: *Genetic Screening in a Drosophila Fanconi Anemia Model.*

2:50 Questions and Answers

2:55 **Henri van de Vrugt, PhD**, Oregon Health & Science University, Portland, Oregon: *Functional Consequences of Combined Fancd2 and Mlh1 Defects.*

3:05 Questions and Answers

3:10 **Ruth A. White**, Oregon Health & Science University, Portland, Oregon: *Smad4 Deletion Leads to Spontaneous Head and Neck Cancer Formation Associated with Reduced Expression of FancA and Brca1.*

3:20 Questions and Answers

3:25 **Session Wrap-Up: Chair**

3:30 **Break**

**Session VII:**

**Late-breaking Presentations**

**Chairperson: Raymond J. Monnat, Jr, MD**  
**Scientific Advisory Board, Fanconi Anemia Research Fund**  
**University of Washington School of Medicine**  
**Seattle, Washington**

3:50 **Session Overview: Chair**

3:55 **Andrew J. Deans, PhD**, Cancer Research UK, South Mimms, Herts, United Kingdom: *Separation of Function Analysis of FANCM-FAAP24.*

4:05 Questions and Answers

4:10 **Sharon B. Cantor, PhD**, University of Massachusetts Medical School, Worcester, Massachusetts: *Unleashing FANCF/BACH1 from BRCA1 Alters the DNA Damage Response.*

4:20 Questions and Answers

4:25 **Agata Smogorzewska, MD, PhD**, Harvard Medical School, Boston, Massachusetts: *Whole Genome Genetic Screen to Identify Proteins Important for DNA Crosslink Resistance in Human Cells.*

4:35 Questions and Answers

- 4:40 **Jordi Surralles, PhD, Universitat Autònoma de Barcelona, Barcelona, Spain:**  
*The Fanconi Anemia Differential Proteomics.*
- 4:50 Questions and Answers
- 4:55 **Session Wrap-Up: Chair**

**TUESDAY – MORNING, OCTOBER 7, 2008**

- 7:00 - 8:00 **Buffet Breakfast**  
*Hellman and Wilder Rooms*
- Poster Viewing**  
*Composers Hall*
- 8:00 **Keynote Address:** *Biology of Genetic Disease: Parallels between Fanconi Anemia and Bloom's Syndrome*  
**Ian Hickson, PhD**  
Genome Integrity Group  
Weatherall Institute of Molecular Medicine  
University of Oxford, Oxford, United Kingdom
- 8:30 Questions and Answers

**Session VIII: Genome Stability and FA Pathway Inhibitors**  
**Chairperson: Markus Grompe, MD**  
**Oregon Health & Science University Stem Cell Center**  
**Portland, Oregon**

- 8:40 **Session Overview: Chair**
- 8:45 **Lei Li, PhD, MD,** University of Texas MD Anderson Cancer Center, Houston, Texas: *Differential Recruitment of Fanconi Anemia and Breast Cancer Susceptibility Proteins to DNA Interstrand Crosslinks.*
- 8:55 Questions and Answers
- 9:00 **Nigel J. Jones, PhD,** University of Liverpool, Liverpool, United Kingdom: *A Role for FANCG outside of the FA Nuclear Core Complex.*
- 9:10 Questions and Answers
- 9:15 **Jean-Yves Masson, PhD,** Laval University Cancer Research Center, Québec, Canada: *The Human MRE11-RAD50-NBS1 Complex is a Critical Regulator of FANCD2 Function and Stability during DNA Repair.*
- 9:25 Questions and Answers
- 9:30 **Stacy A. Williams,** Yale University School of Medicine, New Haven, Connecticut: *Functional Interaction between FANCD2 and the E3 Ubiquitin Ligase, RAD18.*
- 9:40 Questions and Answers
- 9:45 **Break**

- 10:05 **Larry Thompson, PhD**, Lawrence Livermore National Laboratory, Livermore, California: *FANCG- and RAD51D-dependent Base Substitution Mutagenesis at the Na<sup>+</sup>/K<sup>+</sup>-ATPase Locus in Mammalian Cells.*
- 10:15 Questions and Answers
- 10:20 **Barbara C. Godthelp, PhD**, Leiden University Medical Center, Leiden, The Netherlands: *Processing and/or Repair of DNA Interstrand Crosslinks in the G1 Phase of the Cell Cycle in Human Fibroblasts.*
- 10:30 Questions and Answers
- 10:35 **Stephen Meyn, MD, PhD**, The Hospital for Sick Children, Toronto, Canada: *The Role of Fanconi Anemia Proteins in the DNA Damage Response of Primitive Human Hematopoietic Cells.*
- 10:45 Questions and Answers
- 10:50 **Session Wrap-Up: Chair**
- 11:00 ***Closing Forum: Planning for the Future***  
**Chairperson: Grover C. Bagby, Jr., MD**  
Chair, Scientific Advisory Board, FA Research Fund  
Oregon Health & Science University Cancer Institute  
Portland, Oregon
- Noon **Adjournment**