

AGENDA

Thursday – October 1, 2009

3:00 **Registration**

4:00 - 6:00

FA 101: An Introduction to the Medicine and Biology of Fanconi Anemia
Grand Ballroom Salon V

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, and resources to support research on Fanconi anemia are welcome and encouraged to attend.

Introductions:

Grover C. Bagby, Jr., MD

Chair, Scientific Advisory Board, Fanconi Anemia Research Fund
Oregon Health & Science University, Portland, OR

Faculty:

Ray Monnat, Jr., MD

Scientific Advisory Board, Fanconi Anemia Research Fund
University of Washington, Seattle, WA

Akiko Shimamura, MD, PhD

Fred Hutchinson Cancer Research Center, Seattle, WA

6:00 - 8:00

Welcome Reception
Poster Viewing
Grand Ballroom Salon VI

Friday – October 2, 2009

7:00 - 8:00

Buffet Breakfast
Grand Ballroom Salons I-IV
Poster Viewing
Grand Ballroom Salon VI

Plenary Session
Grand Ballroom Salon V

- 8:00 - 8:15 **Welcome**
David Frohnmayer
Co-founder and Vice President of the Board of Directors
Fanconi Anemia Research Fund
Eugene, Oregon
- 8:15 - 8:30 **Overview**
Grover C. Bagby, Jr., MD
Chair, Scientific Advisory Board, Fanconi Anemia Research Fund
Oregon Health & Science University
Portland, Oregon
- Session I: Functions of FA Proteins I**
Chair: Larry Thompson, PhD
Lawrence Livermore National Laboratory
Livermore, California
- 8:30 - 8:55 *Beyond Crosslink Repair: A Comprehensive Literature Review of the Role of FANCM Proteins in Replicating Damaged DNA*
Larry Thompson, PhD
Lawrence Livermore National Laboratory, Livermore, California
- 8:55 - 9:00 Questions and Answers
- 9:00 - 9:05 **Session Overview: Larry Thompson, PhD**
- 9:05 - 9:15 **Thiyam Ramsing Singh, PhD**, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio: *FAAP16/FAAP10, a Novel Histone-fold-containing Protein Complex Interacts Physically and Functionally with FANCM*
- 9:15 - 9:20 Questions and Answers
- 9:20 - 9:30 **Zhijiang Yan, PhD**, National Institute on Aging, NIH, Baltimore, Maryland: *A Novel Histone-fold Complex and FANCM Constitute a DNA Remodeling Complex to Maintain Genome Stability*
- 9:30 - 9:35 Questions and Answers
- 9:35 - 9:45 **Andrew Deans, PhD**, London Research Institute, South Mimms, United Kingdom: *FANCM Connects the Fanconi Anemia Core Complex and the Bloom's Complex on Chromatin*
- 9:45 - 9:50 Questions and Answers
- 9:50 - 10:00 **Fan Zhang, MS**, Cincinnati Children's Research Foundation, Cincinnati, Ohio: *FANCM/PALB2 Physically and Functionally Connects BRCA1 and FANCD1/BRCA2 into a DNA Damage Response Pathway*
- 10:00 - 10:05 Questions and Answers

Twenty-first Annual Fanconi Anemia Research Fund Scientific Symposium

10:05 - 10:10 **Session Wrap-up: Larry Thompson, PhD**

10:10 - 10:25 **Break**

Session II: Stem Cells, Hematopoiesis, and Transplantation I

Chair: Carlo Dufour, MD
G. Gaslini Children's Hospital
Genova, Italy

10:25 - 10:30 **Session Overview: Carlo Dufour, MD**

10:30 - 10:40 **Carlo Dufour, MD**, G. Gaslini Children's Hospital, Genova, Italy: *Impact of a New Donor for Second Stem Cell Transplantation in Fanconi Anemia: Survey of the FA Committee of the EBMT Severe Aplastic Anemia Working Party*

10:40 - 10:45 Questions & Answers

10:45 - 10:55 **Carmem Bonfim, MD**, Federal University of Parana, Curitiba, Brazil: *Unrelated BMT in Patients with Fanconi Anemia: Analysis of 22 Patients Transplanted in a Single Institution*

10:55 - 11:00 Questions & Answers

11:00 - 11:10 **Mohamad Al-Rahawan, MD, MPH**, University of Illinois College of Medicine at Peoria, Peoria, Illinois: *Immunosuppressive Therapy and Future Response to Androgens or Survival After Stem Cell Transplantation in Fanconi Anemia*

11:10 - 11:15 Questions & Answers

11:15 - 11:25 **Melissa Mizesko, MD**, Indiana University School of Medicine, Indianapolis, Indiana: *Fance-/- Hematopoietic Stem and Progenitor Cells Exhibit Impaired Function Compared to Fance-/- and WT Cells*

11:25 - 11:30 Questions & Answers

11:30 - 11:40 **Patrizia Vinciguerra, PhD**, Dana-Farber Cancer Institute, Boston, Massachusetts: *Cytokinesis Failure in Fanconi Anemia Pathway Deficient Murine Hematopoietic Stem Cells*

11:40 - 11:45 Questions & Answers

11:45 - 11:55 **Jie Li, PhD**, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio: *Oxidative Stress-specific FANCD2-FOXO3a Interaction*

11:55 - 12:00 Questions & Answers

12:00 - 12:05 **Session Wrap-up: Carlo Dufour, MD**

12:05 - 1:05

Hosted Lunch
Grand Ballroom Salons I-IV
Poster Viewing
Grand Ballroom Salon VI

1:05 - 1:35

Keynote Address: *Optical Imaging Approaches to Early Detection of Oral Cancer*

Ann M. Gillenwater, MD, FACS

University of Texas M.D. Anderson Cancer Center, Houston, Texas

Dr. Gillenwater is a Professor for the Department of Head and Neck Surgery, University of Texas M.D. Anderson Cancer Center. She earned a BA in Russian studies from Brown University and received her MD from the University of Virginia. She completed residency in Otolaryngology – Head and Neck Surgery at the University of Texas Health Science Center in Houston followed by a two-year fellowship in Head and Neck Surgery Oncology at the University of Texas M.D. Anderson Cancer Center. She has been a faculty member at M.D. Anderson since 1994, and currently directs the Oral Cancer Prevention Clinic at this institution.

Dr. Gillenwater treats patients with head and neck cancer, particularly cancers of the oral cavity. Her research interests include detection and diagnosis of oral cancer and precancer, molecular imaging, and chemoprevention. A NIH-funded investigator, her research involves development of novel technologies for detection and diagnosis of oral cancer and precancer.

1:35 - 1:45

Questions and Answers

Session III: Cancer I

Chair: Susanne Wells, PhD

Cincinnati Children's Hospital Medical Center

Cincinnati, Ohio

1:45 - 1:50

Session Overview: Susanne Wells, PhD

1:50 - 2:00

Susanne Wells, PhD, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio: *Patient-derived 3D Models of FA Squamous Cell Carcinoma*

2:00 - 2:05

Questions and Answers

2:05 - 2:15

Ian Mackenzie, DDS, PhD, Barts and The London School of Medicine and Dentistry, London, United Kingdom: *Comparative Behavior of Stem Cell Populations in Fanconi Anemia Head and Neck Cancer Cell Lines*

2:15 - 2:20

Questions and Answers

2:20 - 2:30

Kajal Biswas, PhD, National Cancer Institute, Frederick, Maryland: *Functional Evaluation of BRCA2 Mutations Found in Fanconi Anaemia Patients Using a Mouse ES Cell-based Assay*

2:30 - 2:35

Questions and Answers

2:35 - 2:45

Nigel Jones, PhD, University of Liverpool, Liverpool, United Kingdom: *Significance of the Fanconi Anaemia FANCD2 Protein in Sporadic Human Breast Cancer*

2:45 - 2:50

Questions and Answers

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2:50 - 2:55 **Session Wrap-up: Susanne Wells, PhD**

2:55 - 3:10 **Break**

Session IV: Cancer II
Chair: Nigel Jones, PhD
University of Liverpool
Liverpool, United Kingdom

3:10 - 3:15 **Session Overview: Nigel Jones, PhD**

3:15 - 3:25 **Heidemarie Neitzel, PhD**, Charité-Universitätsmedizin Berlin, Berlin, Germany:
Acquired Clonal Aberrations in Primary Bone Marrow Cells of FA Patients: The First Study Utilizing High Resolution Array

3:25 - 3:30 Questions and Answers

3:30 - 3:40 **Jung Wook Park, Graduate Student**, University of Wisconsin, Madison,
Wisconsin: *Deficiency in the Fanconi Anemia Pathway Sensitizes Mice to HPV-associated Head and Neck Cancer*

3:40 - 3:45 Questions and Answers

3:45 - 3:55 **Jen-Wei Huang, Graduate Student**, Fred Hutchinson Cancer Research Center,
Seattle, Washington: *MicroRNA-Mediated Regulation of the Fanconi anemia-BRCA Pathway*

3:55 - 4:00 Questions and Answers

4:00 - 4:05 **Session Wrap-up: Nigel Jones, PhD**

4:05 - 7:00

Poster Presentations

Wine and Hors d'Oeuvres

Grand Ballroom Salon VI

7:00 - 9:00

Symposium Dinner

All registrants are invited

Grand Ballroom Salons I-IV

Saturday – October 3, 2009

7:00 - 8:00

Buffet Breakfast
Grand Ballroom Salons I-IV
Poster Viewing
Grand Ballroom Salon VI

Session V:

Functions of FA Proteins II

Chair: Barbara Godthelp, PhD
Leiden University Medical Center
Leiden, The Netherlands

8:00 - 8:05

Session Overview: Barbara Godthelp, PhD

8:05 - 8:15

Ivan Rosado, PhD, MRC Laboratory of Molecular Biology, Cambridge, United Kingdom: *The Walker B Motif in Avian FANCM is Required to Limit Sister Chromatid Exchanges but is Dispensable for DNA Crosslink Repair*

8:15 - 8:20

Questions & Answers

8:20 - 8:30

Alexandra Sobeck, PhD, University of Minnesota, Minneapolis, Minnesota: *The Fanconi Anemia Protein FANCM is Controlled by FANCD2 and the ATR/ATM Pathways*

8:30 - 8:35

Questions & Answers

8:35 - 8:45

Peter McHugh, PhD, Weatherall Institute of Molecular Medicine, Oxford, United Kingdom: *The Yeast FANCM Homologue Mph1 is Required for the Repair of DNA Interstrand Cross-links in the Absence of Pso2*

8:45 - 8:50

Questions & Answers

8:50 - 9:00

Min Huang, Dana-Farber Cancer Institute, Boston, Massachusetts: *FAAP24 is Required for a Crosslinker-specific Checkpoint Response*

9:00 - 9:05

Questions & Answers

9:05 - 9:15

Parameswary Muniandy, PhD, National Institute on Aging, NIH, Baltimore, Maryland: *The Recruitment Kinetics of Fanconi Anemia Proteins to Laser Localized Psoralen Crosslink*

9:15 - 9:20

Questions & Answers

9:20 - 9:30

Barbara Godthelp, PhD, Leiden University Medical Center, Leiden, The Netherlands: *Elucidating the Spatio-temporal Organization of DNA Interstrand Crosslink Processing and Repair at Sites of ICL*

9:30 - 9:35

Questions & Answers

9:35 - 9:40

Session Wrap-up: Barbara Godthelp, PhD

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Session VI: Functions of FA Proteins III

Chair: Ray Monnat, Jr., MD

Scientific Advisory Board, Fanconi Anemia Research Fund

University of Washington

Seattle, Washington

- 9:40 - 9:45 **Session Overview: Ray Monnat, Jr., MD**
- 9:45 - 9:55 **Yanbin Zhang, PhD**, University of Miami School of Medicine, Miami, Florida:
Human FANCI Binds to DNA and Interacts with FANCD2 to Recognize Branched Structures
- 9:55 - 10:00 Questions & Answers
- 10:00 - 10:20 **Break**
- 10:20 - 10:30 **James Wilson, PhD**, University of Liverpool, Liverpool, United Kingdom:
FANCD2 Serine 331 Phosphorylation is Important for FA Pathway Function and BRCA2 Interaction
- 10:30 - 10:35 Questions & Answers
- 10:35 - 10:45 **Koji Nakanishi, MD**, Memorial Sloan-Kettering Cancer Center, New York, New York:
Homology-directed FA Pathway Crosslink Repair is Dependent on DNA Replication
- 10:45 - 10:50 Questions & Answers
- 10:50 - 11:00 **Puck Knipsheer, PhD**, Harvard Medical School, Boston, Massachusetts: *The FancI-FancD2 Complex is Required for Translesion DNA Synthesis During Interstrand Cross-link Repair*
- 11:00 - 11:05 Questions & Answers
- 11:05 - 11:15 **Minoru Takata, MD, PhD**, Kyoto University, Kyoto, Japan: *A Novel Role of the FA Core Complex in FANCI Phosphorylation, an Important Switch in the FA Pathway*
- 11:15 - 11:20 Questions & Answers
- 11:20 - 11:30 **Session Wrap-up: Ray Monnat, Jr., MD**

11:30 - 1:30

Poster Viewing
Grand Ballroom Salon VI

11:30 - 1:30

Joint Meeting and Lunch:
Fanconi Anemia Research Fund
Board of Directors and Scientific Advisory Board
Dover A-B

12:00 - 1:00

Buffet Lunch
Grand Ballroom Salons I-IV

1:30 - 2:00

Keynote Address: *Modeling Marrow Failure with Human Pluripotent Stem Cells*

M. William Lensch, PhD

George Q. Daley Laboratory, Harvard Medical School, Boston, Massachusetts

Dr. Lensch is an Instructor in Pediatrics at the Harvard Medical School, Faculty Advisor for Education at the Harvard Stem Cell Institute, and Senior Scientist in the laboratory of George Q. Daley at Children's Hospital Boston. Dr. Lensch earned his PhD from Oregon Health & Science University in Molecular and Medical Genetics for work conducted with Grover C. Bagby, MD, studying clonal evolution in congenital and acquired bone marrow failure. He was a post-doctoral fellow with George Q. Daley, MD, PhD, at the Whitehead Institute for Biomedical Research and later at Children's Hospital Boston where he worked on modeling developmental hematopoiesis from human embryonic stem cells and cord blood-derived hemogenic endothelium. His current research interests include modeling congenital blood diseases with human pluripotent stem cells and understanding the biology of pediatric and adult germ cell tumors.

Dr. Lensch is a founding member of the Interstate Alliance on Stem Cell Research, a member of the Public Education Committee of the International Society for Stem Cell Research, and a past gubernatorial appointee of the Stem Cell Research Advisory Committee for the State of Connecticut. He has lectured internationally in scientific, medical, government, religious, and general public forums on the science, conduct, and policy of stem cell research.

2:00 - 2:10

Questions and Answers

Session VII: Stem Cells, Hematopoiesis, and Transplantation II

Chair: Juan Bueren, PhD

CIEMAT/Biomedical Center for Rare Diseases

Madrid, Spain

2:10 - 2:15

Session Overview: Juan Bueren, PhD

2:15 - 2:25

Juan Bueren, PhD, CIEMAT/Biomedical Center for Rare Diseases, Madrid, Spain: *Generation of Hematopoietic Progenitors from Genetically-corrected and Reprogrammed Fanconi Anemia Skin Cells*

2:25 - 2:30

Questions and Answers

2:30 - 2:40

Jordi Surralles, PhD, Universitat Autònoma de Barcelona, Barcelona, Spain: *Functional Studies in Fanconi Anemia Induced-pluripotent Stem Cells*

2:40 - 2:45

Questions and Answers

2:45 - 2:55

Arleen Auerbach, PhD, The Rockefeller University, New York, NY: *Combining Gene Therapy and Non-viral Reprogramming to Generate FA-C iPS Cells and Disease-free Hematopoietic Progenitors*

2:55 - 3:00

Questions and Answers

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- 3:00 - 3:10 **Kalindi Parmar, PhD**, Dana-Farber Cancer Institute, Boston, Massachusetts:
Hematopoietic Stem Cell Defects in Mice with Deficiency in Fancd2 or Usp1
- 3:10 - 3:15 Questions and Answers
- 3:15 - 3:20 **Session Wrap-up: Juan Bueren, PhD**
- 3:20 - 3:40 **Break**

Session VIII: Late-breaking Presentations

Chair: Blanche Alter, MD, MPH
National Cancer Institute
Rockville, Maryland

- 3:40 - 3:45 **Session Overview: Blanche Alter, MD, MPH**
- 3:45 - 3:55 **Michael Hodskinson, PhD**, MRC Laboratory of Molecular Biology, Cambridge, United Kingdom: *Nuclease Activity Associated with Recombinant FANCD2 Reveals a Direct Role for this Protein in DNA Repair*
- 3:55 - 4:00 Questions and Answers
- 4:00 - 4:10 **Katharina Schlacher, PhD**, Memorial Sloan-Kettering Cancer Center, New York, New York: *BRCA2 (FANCD1) Protects Stalled Replication Forks from Degradation, A Novel Pathway Shared with BRCA1 and Fanconi Anemia*
- 4:10 - 4:15 Questions and Answers
- 4:15 - 4:25 **Jean-Yves Masson, PhD**, Laval University Cancer Research Center, Québec, Canada: *Synergistic Functions of Breast Cancer Proteins PALB2 and BRCA2 in Stimulating Homologous Recombination*
- 4:25 - 4:30 Questions and Answers
- 4:30 - 4:40 **Wojciech Niedzwiedz, PhD**, Oxford University, Oxford, United Kingdom: *ATR Activation and Replication Fork Restart are Defective in FANCM-deficient Cells*
- 4:40 - 4:45 Questions and Answers
- 4:45 - 4:55 **Paula Rio, PhD**, CIEMAT, Madrid, Spain: *The Down-regulated Expression of Specific microRNAs in FA Cells Impairs the Growth of FA Hematopoietic Progenitors*
- 4:55 - 5:00 Questions and Answers
- 5:00 - 5:05 **Session Wrap-up: Blanche Alter, MD, MPH**

Sunday – October 4, 2009

7:00 - 8:00

Buffet Breakfast
Grand Ballroom Salons I-IV
Poster Viewing
Grand Ballroom Salon VI

Twenty-first Annual Fanconi Anemia Research Fund Scientific Symposium

Session IX: Stem Cells, Hematopoiesis, and Transplantation III

Chair: Richard Gelinas, PhD

Battelle Memorial Institute

Seattle, Washington

- 8:00 - 8:05 **Session Overview: Richard Gelinas, PhD**
- 8:05 - 8:15 **Qingshuo Zhang, PhD**, Oregon Health & Science University, Portland, Oregon:
Resveratrol, a Sirt1-activating Compound, Partially Corrects Hematopoietic Defects in Fanconi Anemia Mice
- 8:15 - 8:20 Questions & Answers
- 8:20 - 8:30 **Grover Bagby, Jr., MD**, Oregon Health & Science University, Portland, Oregon:
TLR8-dependent TNF-alpha (TNFa) Over-expression in Fanconi Anemia Group C Cells
- 8:30 - 8:35 Questions & Answers
- 8:35 - 8:45 **Carlos Pipaon, PhD**, Hospital Marques de Valdecilla-IFIMAV, Santander, Spain:
Fanconi Anemia Patients Show Elevated Levels of IL-1 β due to a Constitutively Active PI3K-AKT Pathway That May Contribute to Their Cancer Predisposition
- 8:45 - 8:50 Questions & Answers
- 8:50 - 9:00 **Alex Lyakhovich, PhD**, Medical University of Vienna, Vienna, Austria: *2D-culture Model of Oxidative Stress in FA Allows Identification of Altered Pathways Upon ROS Exposure*
- 9:00 - 9:05 Questions & Answers
- 9:05 - 9:10 **Session Wrap-up: Richard Gelinas, PhD**

Session X: Functions of FA Proteins IV

Chair: K.J. Patel, MD, PhD

MRC Laboratory of Molecular Biology

Cambridge, United Kingdom

- 9:10 - 9:15 **Session Overview: K.J. Patel, MD, PhD**
- 9:15 - 9:25 **Helen Walden, PhD**, Cancer Research UK, London Research Institute, London, United Kingdom: *The Structure of FANCL, the Catalytic Subunit of the Fanconi Anemia Core Complex*
- 9:25 - 9:30 Questions & Answers
- 9:30 - 9:40 **K.J. Patel, MD, PhD**, MRC Laboratory of Molecular Biology, Cambridge, United Kingdom: *The N Terminus of FANCL Contains an "RWD"-like Domain Involved in Ubiquitin Transfer*
- 9:40 - 9:45 Questions & Answers
- 9:45 - 9:55 **Yuliang Wu, PhD**, National Institute on Aging, NIH, Baltimore, Maryland:
Functional Characterization of FANCI Mutations Genetically Linked to Fanconi Anemia or Associated with Breast Cancer
- 9:55 - 10:00 Questions & Answers

Twenty-first Annual Fanconi Anemia Research Fund Scientific Symposium

10:00 - 10:10 **Session Wrap-up: K.J. Patel, MD, PhD**

10:10 - 10:30 **Break**

10:30 - 12:00

Closing Forum: Planning for the Future

Chair: Grover C. Bagby, Jr., MD

Chair, Scientific Advisory Board, Fanconi Anemia Research Fund

Oregon Health & Science University

Portland, Oregon