AGENDA

Thursday – October 20, 2011

15:00  Registration opens

16:00 - 18:00  
FA 101: An Introduction to the Medicine and Biology of Fanconi Anemia
Tarragona-Girona Rooms
Note: 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 encouraged to attend.

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

Faculty:
Ray Monnat, Jr., MD
Scientific Advisory Board, Fanconi Anemia Research Fund
University of Washington, Seattle, Washington

Akiko Shimamura, MD, PhD
Fred Hutchinson Cancer Research Center, Seattle, Washington

Research Opportunities:
Pankaj Qasba, PhD
Program Director, Blood Diseases Branch
National Heart, Lung, and Blood Institute, NIH, Bethesda, Maryland

18:00 - 20:00  
Welcome Reception
Poster Viewing
Lleida Room

Friday – October 21, 2011

06:30 - 08:20  
Buffet Breakfast
Batlló-Güell Rooms, Mezzanine Level
Poster Viewing
Lleida Room

08:30  
Plenary Session
Tarragona-Girona Rooms
23rd Annual Fanconi Anemia Research Fund Scientific Symposium

08:30 - 08:45  Welcome
David Frohnmayer, JD
Co-founder and Advisor, Board of Directors
Fanconi Anemia Research Fund
Eugene, Oregon

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

Session I:  FA Proteins and Crosslink Repair
Chair: Ray Monnat, Jr., MD
Scientific Advisory Board, Fanconi Anemia Research Fund
University of Washington, Seattle, Washington

08:45 - 08:50  Session Overview: Ray Monnat, Jr., MD

08:50 - 09:00  Jean Gautier, PhD, Columbia University Medical Center, New York, New York:
Mechanism of Replication-independent DNA Interstrand Crosslink Repair

09:00 - 09:05  Questions and Answers

09:05 - 09:15  Blanka Sengerova, PhD, Weatherall Institute of Molecular Medicine, University of Oxford, Oxford, United Kingdom:
Human SNM1A and XPF-ERCC1 Collaborate to Initiate DNA Interstrand Cross-link Repair

09:15 - 09:20  Questions and Answers

09:20 - 09:30  David Thomas Long, PhD, Harvard Medical School, Boston, Massachusetts:
RAD51 Promotes Repair of DNA Double-strand Breaks Created by the Fanconi Pathway During Interstrand Cross-link Repair

09:30 - 09:35  Questions and Answers

09:35 - 09:45  Indrajit Chaudhury, PhD, University of Minnesota, Minneapolis, Minnesota:
FANCD2 Regulates Functions of the BLM DNA Repair Pathway Independently of FANCI

09:45 - 09:50  Questions and Answers

09:50 - 10:00  Marina Bellani, PhD, National Institute on Aging, Baltimore, Maryland:
Rapid Multiphasic Recruitment of FA Associated Nuclease 1 (FAN1) to Sites of Crosslinks in Live Cells

10:00 - 10:05  Questions and Answers

10:05 - 10:10  Session Wrap-up: Ray Monnat, Jr., MD

Special Session:  Gene Therapy/Embryonic Stem Cells & Induced Pluripotent Stem Cells
Chair: Jakub Tolar, MD, PhD
Scientific Advisory Board, Fanconi Anemia Research Fund
University of Minnesota, Minneapolis, Minnesota
(for more information, see page 11)
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<td>10:10 - 10:20</td>
<td><strong>Meeting Report: International FA Gene Therapy Work Group</strong></td>
<td>Jakub Tolar, MD, PhD</td>
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<td>10:20 - 10:25</td>
<td>Questions and Answers</td>
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<td>10:25 - 10:45</td>
<td><strong>Break</strong></td>
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<td>10:45 - 10:50</td>
<td><strong>Session Overview and Keynote Introduction: Jakub Tolar, MD, PhD</strong></td>
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<td><strong>Keynote Address:</strong> Modeling Bone Marrow Failure Syndromes Using Induced Pluripotent Stem Cells</td>
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<td>10:50 - 11:10</td>
<td>Suneet Agarwal, MD, PhD, Children’s Hospital Boston, Boston, Massachusetts</td>
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<td>11:00 - 11:15</td>
<td>Questions and Answers</td>
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<td>11:15 - 11:25</td>
<td>Montserrat Barrağán, PhD, Centro de Medicina Regenerativa de Barcelona, Barcelona, Spain: Targeted Gene Correction of Patient-specific Fibroblasts for Cell-therapy of Spanish Gypsy Fanconi Anaemia</td>
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<td>11:30 - 11:40</td>
<td>Susana Navarro, PhD, CIEMAT, Madrid, Spain: Generation and Hematopoietic Differentiation of Disease-free Induced Pluripotent Stem Cells from FA-D1 Mouse Fibroblasts</td>
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<td>11:40 - 11:45</td>
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<td><strong>iPSC Panel – Moderated by Jakub Tolar, MD, PhD</strong></td>
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<td>11:45 - 11:55</td>
<td>Thomas Graf, PhD, Center for Genomic Regulation, Barcelona, Spain</td>
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<td>11:55 - 12:05</td>
<td>Angel Raya, MD, PhD, Institute for Bioengineering of Catalonia, Barcelona, Spain</td>
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<td>12:05 - 12:15</td>
<td>Toni Cathomen, PhD, Hannover Medical School, Hannover, Germany</td>
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<td>12:15 - 12:25</td>
<td>Juan Bueren, PhD, CIEMAT, Madrid, Spain</td>
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<td>12:25 - 12:35</td>
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<td>12:40 - 13:55</td>
<td><strong>Hosted Lunch</strong></td>
<td>Batlló-Güell Rooms, Mezzanine Level</td>
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<td><strong>Poster Viewing</strong></td>
<td>Lleida Room</td>
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<td><strong>Session II: Stem Cells, Hematopoiesis, and Transplantation</strong></td>
<td>Chair: Eva Guinan, MD Scientific Advisory Board, Fanconi Anemia Research Fund Dana-Farber Cancer Institute, Boston, Massachusetts</td>
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<td>13:55 - 14:00</td>
<td><strong>Session Overview: Eva Guinan, MD</strong></td>
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<td>14:00 - 14:10</td>
<td>Margaret MacMillan, MD, University of Minnesota, Minneapolis, Minnesota:</td>
<td>Hematopoietic Cell Transplantation for FA Patients with Acute Leukemia or Advanced MDS</td>
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<td>14:10 - 14:15</td>
<td>Questions and Answers</td>
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<td>14:15 - 14:25</td>
<td>Ashley Kamimae-Lanning, Oregon Health &amp; Science University, Portland, Oregon: Prenatal Origins of Hematopoietic Failure in a Mouse Model of Fanconi Anemia</td>
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<td>Kathrin Scheckenbach, MD</td>
<td>Heinrich-Heine University, Duesseldorf, Germany</td>
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<td>Kalindi Parmar, PhD</td>
<td>Dana-Farber Cancer Institute, Boston, Massachusetts</td>
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<td>Laura Hays, PhD</td>
<td>Oregon Health &amp; Science University, Portland, Oregon</td>
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<td>15:00</td>
<td>Paula Rio, PhD</td>
<td>CIEMAT, Madrid, Spain</td>
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<td>Session Wrap-up: Eva Guinan, MD</td>
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<td>Session Overview: Stephen Meyn, MD, PhD</td>
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<td>Kailin Yang</td>
<td>Dana-Farber Cancer Institute, Boston, Massachusetts</td>
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<td>Agata Smogorzewska, MD, PhD</td>
<td>The Rockefeller University, New York, New York</td>
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<td>Kim-Hien Dao</td>
<td>Oregon Health &amp; Science University, Portland, Oregon</td>
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16:55 - 17:05  Maria Jasin, PhD, Memorial Sloan-Kettering Cancer Center, New York, New York: Unified Model for Fanconi Anemia and Breast Cancer Gene-network in Preventing Genome Instability at Stalled DNA Forks
17:05 - 17:10  Questions and Answers
17:10 - 17:20  Weidong Wang, PhD, National Institute on Aging, Baltimore, Maryland: Crystal Structures Reveal that FANCM Remodels the MHF Tetramer in Favor of Binding Branched DNA
17:20 - 17:25  Questions and Answers
17:25 - 17:35  Tomoko Shigechi, MD, Kyoto University, Kyoto, Japan: An Absolute Requirement of ATRIP-ATR Kinase in Replication Stress-induced Triggering of the FA Pathway Activation
17:35 - 17:40  Questions and Answers
17:40 - 17:50  Filippo Rosselli, PhD, Institut Gustave Roussy, Villejuif, France: FANC Pathway Limits 53BP1 Accumulation to DSBs Associated with the Collapse of Stalled Replication Forks
17:50 - 17:55  Questions and Answers
17:55 - 18:00  Session Wrap-up: Stephen Meyn, MD, PhD

Saturday – October 22, 2011

06:30 - 08:20  Buffet Breakfast
              Batlló-Güell Rooms, Mezzanine Level
              Poster Viewing
              Lleida Room

Session V: Carcinogenesis I
           Chair: Erich Sturgis, MD, MPH
           Scientific Advisory Board, Fanconi Anemia Research Fund
           The University of Texas MD Anderson Cancer Center
           Houston, Texas

08:30 - 08:35  Session Overview and Keynote Introduction: Erich Sturgis, MD, MPH

Keynote Address: Human Papillomavirus and Head and Neck Cancer

08:35 - 09:00  Maura Gillison, MD, PhD, Ohio State University Comprehensive Cancer Center, Columbus, Ohio
               (for more information, see page 12)
09:00 - 09:15  Questions and Answers
09:15 - 09:25  Susanne Wells, PhD, Cincinnati Children’s Hospital, Cincinnati, Ohio: The Fanconi Anemia Pathway Restrictions the Human Papillomavirus Replicative Cycle
09:25 - 09:30  Questions and Answers
09:30 - 09:40  Jason Taylor, MD, PhD, Oregon Health & Science University, Portland, Oregon: 
*B-cell Defects and Quantification of Antibody Response to HPV Vaccines in FanC-/- Mice*
09:40 - 09:45  Questions and Answers

09:45 - 09:55  Gerry Crossan, MRC Laboratory of Molecular Biology, Cambridge, United 
Kingdom:  *Development of Hematological, Anogenital, and Hepatocellular Cancers in Mature 
FANCP Deficient Mice*
09:55 - 10:00  Questions and Answers

10:00 - 10:05  **Session Wrap-up: Erich Sturgis, MD, MPH**

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**Session VI:  Function of the FA Core Complex**

Chair: Christopher Mathew, PhD
Scientific Advisory Board, Fanconi Anemia Research Fund
King’s College London, London, United Kingdom

10:05 - 10:10  **Session Overview: Christopher Mathew, PhD**

10:10 - 10:20  Lei Li, PhD, MD Anderson Cancer Center, Houston, Texas:  *FANCM and 
FAAP24 Play Distinct Roles in Maintaining Genomic Stability*
10:20 - 10:25  Questions and Answers

10:25 - 10:35  Charlotte Hodson, London Research Institute, London, United Kingdom:  
*Structural Analysis of Human FANCL*
10:35 - 10:40  Questions and Answers

10:40 - 11:00  **Break**

11:00 - 11:10  Jordi Surrallés, PhD, Universitat Autonoma de Barcelona, Barcelona, Spain:  
*Is FAN1 a Novel Fanconi Anemia Gene?*
11:10 - 11:15  Questions and Answers

11:15 - 11:25  Paul Andreassen, PhD, Cincinnati Children’s Research Foundation, Cincinnati, 
Ohio:  *The Critical Function of BRCA1 in Homologous Recombination is to Localize 
FANCN/PALB2 to Sites of DNA Damage*
11:25 - 11:30  Questions and Answers

11:30 - 11:40  Maria Castella, PhD, Fred Hutchinson Cancer Research Center, Seattle, 
Washington:  *Identification of New Kinases Involved in the Regulation of the Fanconi Anemia-
BRCA Pathway*
11:40 - 11:45  Questions and Answers

11:45 - 11:55  Nigel Jones, PhD, University of Liverpool, Liverpool, United Kingdom:  
*Inhibition of NHEJ Reveals Distinct Roles for FANCG in the Repair of Interstrand Crosslinks and 
DNA Strand Breaks*
11:55 - 12:00  Questions and Answers
23rd Annual Fanconi Anemia Research Fund Scientific Symposium

12:00 - 12:10 Carrie Adelman, PhD, Cancer Research UK, South Mimms, United Kingdom: HELQ Helicase: A New FA Associated Protein that Phenocopies FancD2-/- When Mutated in Mice

12:10 - 12:15 Questions and Answers

12:15 - 12:25 Caroline Huard, MSc, Laval University, Québec City, Canada: Identification of CtBP1 as a New FA Core Complex Interacting Protein

12:25 - 12:30 Questions and Answers

12:30 - 12:40 Jean-Yves Masson, PhD, Laval University Cancer Research Center, Québec City, Canada: Functions of PALB2 and BRCA2 in Stimulating the Invasion Step of Homologous Recombination

12:40 - 12:45 Questions and Answers

12:45 - 12:50 Session Wrap-up: Christopher Mathew, PhD

12:50 - 14:45 Hosted Lunch

Batlló-Güell Rooms, Mezzanine Level
Poster Viewing
Lleida Room

Note: Mini-session available at the end of lunch period for interested participants

Mini-session: Next-generation Sequencing

Chair: Richard Gelinas, PhD
Scientific Advisory Board and Board of Directors
Fanconi Anemia Research Fund
Institute for Systems Biology, Seattle, Washington

13:45 - 13:50 Session Overview: Richard Gelinas, PhD


13:58 - 14:06 Najim Ameziane, MD, Vrije Universiteit Medical Center, Amsterdam, The Netherlands: Novel Comprehensive Mutation Screening Approach for FA by Multiplexed Targeted Next-generation Sequencing

14:06 - 14:14 Settara Chandrasekharappa, PhD, National Human Genome Research Institute, Bethesda, Maryland: Application of Next-gen Sequencing Technologies for Screening of Fanconi Anemia Gene Mutations

14:14 - 14:24 Questions and Answers

14:24 - 14:30 Session Wrap-up: Richard Gelinas, PhD
14:45 - 14:50  **Keynote Introduction: Grover C. Bagby, Jr., MD**

**Keynote Address:**  Fanconi Anemia and Novel Drug Targets

14:50 - 15:15  **Alan D'Andrea, MD,** Dana-Farber Cancer Institute, Boston, Massachusetts

(for more information, see page 13)

15:15 - 15:30  Questions and Answers

**Session VII-A:  Experimental Therapies**

Chair: K. J. Patel, MD, PhD
MRC Laboratory of Molecular Biology, Cambridge, United Kingdom

15:30 - 15:35  **Session Overview: K.J. Patel, MD, PhD**

15:35 - 15:45  **Michael Garbati, PhD,** Oregon Health & Science University, Portland, Oregon: *p38 MAPK Inhibition Suppresses the TLR-hypersensitive Phenotype in FANCC-deficient Macrophages*

15:45 - 15:50  Questions and Answers

15:50 - 16:00  **Jordi Villa,** Universitat Autònoma de Barcelona, Barcelona, Spain: *Downregulation of N-ethylmaleimide Sensitive Factor Protects Fanconi Anemia Cells from DNA Damage*

16:00 - 16:05  Questions and Answers

16:05 - 16:15  **Céline Jacquemont, PhD,** Fred Hutchinson Cancer Research Center, Seattle, Washington: *USP28 is a Modulator of the Fanconi Anemia Pathway and DNA Double-strand Break Repair*

16:15 - 16:20  Questions and Answers

16:20 - 16:30  **Johanna Svahn, MD,** Gaslini Childrens Hospital, Genova, Italy: *Kinase Inhibitors Reduce TNF-alpha Overproduction in Monocytes from Fanconi Anemia Group A Patients*

16:30 - 16:35  Questions and Answers

16:35 - 16:55  **Break**

**Session VII-B:  Experimental Therapies**

Chair: Toshiyasu Taniguchi, MD, PhD
Fred Hutchinson Cancer Research Center, Seattle, Washington

16:55 - 17:05  **Ana Filipa Brinco de Oliveira Ponte,** University of Porto, Porto, Portugal: *α-Lipoic Acid and N-acetyllysisteine as an Effective Cocktail for Decreasing Spontaneous Chromosome Instability in FA*

17:05 - 17:10  Questions and Answers

17:10 - 17:20  **Markus Grompe, MD,** Oregon Stem Cell Center, Portland, Oregon: *Oral Administration of a Direct SIRT1 Activating Compound Enhances Hematopoiesis in Fancd2/- Mice*

17:20 - 17:25  Questions and Answers
17:25 - 17:35  Frédéric Langevin, MRC Laboratory of Molecular Biology, Cambridge, United Kingdom: *An Essential Requirement for the Joint Action of Acetaldehyde Catabolism and Fancd2 in Embryonic Development*

17:35 - 17:40  Questions and Answers

17:40 - 17:50  Daria Mochly-Rosen, PhD, Stanford University School of Medicine, Stanford, California: *Allosteric Activator of Aldehyde Dehydrogenase 2, Alda-1, Reduces Aldehydic Load*

17:50 - 17:55  Questions and Answers

17:55 - 18:00  Session Wrap-up: Toshiyasu Taniguchi, MD, PhD

19:00 - 20:00  Animation: Poster Presentations Reception
Wine and Hors d’Oeuvres
*Lleida Room*

20:00 - 22:00  Animation: Symposium Dinner
All registrants are invited
*Baïllo-Güell Rooms, Mezzanine Level*

**Sunday – October 23, 2011**

06:30 - 08:20  Animation: Buffet Breakfast
*Baïllo-Güell Rooms, Mezzanine Level*

**Poster Viewing**
*Lleida Room*

07:00 - 08:20  Animation: Joint Meeting and Breakfast
Board of Directors and Scientific Advisory Board
Fanconi Anemia Research Fund
*Roses Room*

08:30 - 08:35  Keynote Introduction: Eva Guinan, MD

**Keynote Address:** DNA Damage Signals and Space Radiation Risk

08:35 - 09:00  Francis Cucinotta, PhD, NASA Space Radiation Program, Houston, Texas
*(for more information, see page 14)*

09:00 - 09:15  Questions and Answers
Session VIII: Carcinogenesis II
Chair: Markus Grompe, MD
Oregon Health & Science University, Portland, Oregon

09:15 - 09:20  Session Overview: Markus Grompe, MD

09:20 - 09:30  Hein te Riele, PhD, The Netherlands Cancer Institute, Amsterdam, The Netherlands: Deficiency for FANCM, but not FANCF, Accelerates Tumorigenesis in a Mouse Model for Intestinal Cancer

09:30 - 09:35  Questions and Answers

09:35 - 09:45  Thomas Ludwig, PhD, Institute for Cancer Genetics, New York, New York: The Tumor-suppressor Activity of the FancJ/Brip1 Helicase is not Dependent on the Interaction of FancJ/Brip1 with Brca1

09:45 - 09:50  Questions and Answers

09:50 - 10:00  Spencer Luebben, University of Minnesota, Minneapolis, Minnesota: A Concomitant Loss of the Fanconi Anemia Pathway and Dormant Replication Origins Accelerates Tumorigenesis

10:00 - 10:05  Questions and Answers

10:05 - 10:15  Ian Mackenzie, DDS, PhD, Barts and The London Medical School, London, United Kingdom: Epithelial-mesenchymal Transition (EMT) in Cancer Cell Lines Lacking Fanconi Gene Function

10:15 - 10:20  Questions and Answers

10:20 - 10:25  Session Wrap-up: Markus Grompe, MD

10:25 - 10:45  Break

10:45 - 12:00  Symposium Town Hall: An Interactive Discussion about FA Research
Chair: Grover C. Bagby, Jr., MD
Chair, Scientific Advisory Board, Fanconi Anemia Research Fund
Oregon Health & Science University, Portland, Oregon