

AGENDA

Thursday, 15

3:00 Grand Foyer	Symposium Check-in and Registration Opens
4:00 – 6:00 Grand A-G	Fanconi Anemia 101 Introductory Presentation
	<i>Note: This session is intended for those new to Fanconi anemia research and clinical care. However, all participants interested in an overview of unanswered questions, new research and clinical directions in Fanconi anemia are encouraged to attend.</i>
4:00 – 4:10	Introduction: Ray Monnat Jr., MD, University of Washington, Seattle, United States; Chair of Scientific Advisory Board, Fanconi Anemia Research Fund
4:10 – 4:55 4:55 – 5:05 Q&A	Clinical: Blanche P. Alter, MD, MPH, National Cancer Institute, Rockville, United States
5:05 – 5:50 5:50 – 6:00 Q&A	Biology: Agata Smogorzewska, MD, PhD, The Rockefeller University, New York, United States
6:00 – 8:00 Grand Foyer	Welcome Reception and Poster Viewing
	<i>Presenters of odd-numbered posters will be at their posters 6:00 to 7:00 Presenters of even-numbered posters will be at their posters 7:00 to 8:00</i>

Friday, 16

7:00 – 8:00 Grand IJK	Breakfast
8:00 – 8:10 Grand A-G	Welcome and Introduction
	<i>Kevin McQueen, MBA, Board President, Fanconi Anemia Research Fund, Midlothian, United States Ray Monnat, Jr., MD, University of Washington, Seattle, United States; Chair of Scientific Advisory Board, FARF</i>
8:10 – 9:20 Grand A-G	Novel Mechanisms & New Approaches <i>Chair: Alan D'Andrea, MD, Dana-Farber Cancer Institute, Harvard Medical School, Boston, United States</i>
8:10 – 8:15	Session Overview: Alan D'Andrea, MD
8:15 – 8:25 8:25 – 8:30 Q&A	Fanconi Anemia Proteins Function in Mitophagy and Immunity p. 8 <i>Rhea Sumpter, Jr., MD, PhD, UT Southwestern, Dallas, United States</i>
8:30 – 8:40 8:40 – 8:45 Q&A	How Environment Shapes the Consequences of Mutation within the Fanconi Anemia Pathway p. 9 <i>Georgios Karras, PhD, Whitehead Institute for Biomedical Research, Cambridge, United States</i>
8:45 – 8:55 8:55 – 9:00 Q&A	Directed Differentiation of FA iPSCs into Keratinocyte Lineages and 3D Epidermis p. 10 <i>Susanne Wells, PhD, Cincinnati Children's Hospital Medical Center, Cincinnati, United States</i>
9:00 – 9:10 9:10 – 9:15 Q&A	Regulation of Squamous Epithelial Growth in Development and Cancer p. 11 <i>Boba Beronja, PhD, Fred Hutchinson Cancer Research Center, Seattle, United States</i>
9:15 – 9:20	Session Wrap-up: Alan D'Andrea, MD
9:20 – 9:40	Break
9:40 – 11:35 Grand A-G	Aldehydes, Genotoxicity & Disease <i>Chair: Kenneth Weinberg, MD, Stanford Medicine, Stanford, United States</i>
9:40 – 9:45	Keynote Introduction: Kenneth Weinberg, MD
9:45 – 10:25 10:25 – 10:30 Q&A	Keynote Address: "Aldehydes and the Phenotype of Fanconi Anemia - A Complex Illness with a Simple Explanation?" <i>KJ Patel, MD, PhD, MRC Laboratory of Molecular Biology, Cambridge, United Kingdom</i>

10:30 – 10:35	Session Overview: Kenneth Weinberg, MD	
10:35 – 10:45	Aldehydes Mutate the Genome of Blood Stem Cells <i>Juan Garaycoechea, MRC Laboratory of Molecular Biology, Cambridge, United Kingdom</i>	p. 12
10:45 – 10:55	Vitamin B9 is a Source of Genotoxic Formaldehyde <i>Guillermo Burgos-Barragan, PhD Student, MRC Laboratory of Molecular Biology, Cambridge, United Kingdom</i>	p. 13
10:55 – 11:05	Combined Genotoxic Role of Tetrahydrofolic Acid and Formaldehyde on Hematopoietic Stem Cells <i>Iván Rosado, PhD, Institute of Biomedicine of Seville, Seville, Spain</i>	p. 14
11:05 – 11:15	Hematopoietic Stem Cell (HSC) Abnormalities Due to the Common “Asian Variant” Aldehyde Dehydrogenase 2*2 Mutation <i>Kenneth Weinberg, MD, Stanford Medicine, Stanford, United States</i>	p. 15
11:15 – 11:30	Panel Discussion: All Speakers	
11:30 – 11:35	Session Wrap-up: Kenneth Weinberg, MD	
11:35 – 11:45	New FARE Research Initiatives <i>Brad Preston, PhD, Scientific Director, Fanconi Anemia Research Fund, Seattle, United States</i>	
12:00 – 2:00 <i>Grand IJK</i>	Lunch Buffet	
12:15 – 2:00 <i>Grand IJK</i>	Mentorship Lunch for Early Investigators	
2:00 – 4:00	Oral Cancer: Treatment, Risk Factors & Early Detection <i>Chair: Susanne Wells, PhD, Cincinnati Children’s Hospital Medical Center, Cincinnati, United States</i>	
2:00 – 2:05	Keynote Introduction: Susanne Wells, PhD	
2:05 – 2:45 2:45 – 2:50 Q&A	Keynote Address: “Towards Precision Oncology for Head and Neck Cancer: Functionalizing Genomic Data for Target Selection” <i>Eduardo Méndez, MD, University of Washington/Fred Hutch Cancer Research Center, Seattle, United States</i>	
2:50 – 2:55	Session Overview: Susanne Wells, PhD	
2:55 – 3:02	The Salivary Microbiome and Risk for Oral Cancer Development in Fanconi Anemia <i>Camila Furquim, DDS, Federal University of Paraná, Curitiba, Brazil</i>	p. 16
3:02 – 3:09	Non-Invasive “Cytology-On-Chip” Assay Combined with Continuous Risk Index for Monitoring Oral Cancer in Patients with Fanconi Anemia <i>Alexander Williams, University of Texas Health Science, Houston, United States</i>	p. 17
3:09 – 3:16	Salivary Oxidative Status in Individuals with Fanconi Anemia <i>José Amenabar, PhD, Federal University of Paraná, Curitiba, Brazil</i>	p. 18
3:16 – 3:23	Long Term Visual Screening for Oral Cancer Prevention in Fanconi Anemia <i>Cassius Torres-Pereira, PhD, Federal University of Paraná, Curitiba, Brazil</i>	p. 19
3:23 – 3:30	Salivary Microbial Profiles of Patients with Fanconi Anemia and Oral Health Status <i>Geisla Soares, PhD, Federal University of Paraná, Curitiba, Brazil</i>	p. 20
3:30 – 3:45	Early Detection of Head and Neck Squamous Cell Carcinoma in Fanconi Anemia <i>Eunike Velleuer, MD, Heinrich-Heine University, Children’s Hospital, Düsseldorf, Germany</i> <i>Ralf Dietrich, Executive Director, Fanconi Anemia Patients’ Organization of Germany, Unna, Germany</i>	
3:45 – 4:00	Panel Discussion: All Speakers	
5:30 – 7:30 <i>Grand Foyer</i>	Poster Reception <i>Presenters of even-numbered posters will be at their posters from 5:30 to 6:30</i> <i>Presenters of odd-numbered posters will be at their posters from 6:30 to 7:30</i>	
7:30 – 9:30 <i>Grand A-G</i>	Symposium Banquet	

7:00 – 8:00 Grand IJK	Breakfast	
8:00 – 10:15 Grand A-G	The Fanconi Anemia Pathway: FANCD2/FANCI in Repair and Replication Stress - Part 1 Chair: Toshiyasu Taniguchi, MD, PhD, Fred Hutchinson Cancer Research Center, Seattle, United States	
8:00 – 8:05	Session Overview: Toshiyasu Taniguchi, MD, PhD	
8:05 – 8:15 8:15 – 8:20 Q&A	The FANCD2-FANCI Complex is Recruited to DNA Interstrand Crosslinks Prior to Monoubiquitination of FANCD2 Martin Cohn, PhD, University of Oxford, Oxford, United Kingdom	p. 21
8:20 – 8:30 8:30 – 8:35 Q&A	The Mechanism of Concerted Monoubiquitination of FANCD2 and FANCI by Recombinant FA Core Complex Andrew Deans, PhD, St Vincent's Institute, Fitzroy, Australia	p. 22
8:35 – 8:45 8:45 – 8:50 Q&A	FANCD2 Binding to Methylated H4K20 via a Chromodomain is Essential for ICL Repair Karissa Paquin, PhD Candidate, University of Rhode Island, Kingston, United States	p. 23
8:50 – 9:00 9:00 – 9:05 Q&A	A Functional Interaction Between the Fanconi Anemia Protein FANCD2 and the Chromatin Remodeling Factor ATRX Promotes Replication Fork Recovery Maya Raghunandan, University of Minnesota, Minneapolis, United States	p. 24
9:05 – 9:15 9:15 – 9:20 Q&A	The Multifaceted Role of FANCD2 in Facilitating DNA Replication at Common Fragile Sites Advaita Madireddy, PhD, Albert Einstein College of Medicine, New York, United States	p. 25
9:20 – 9:30	Session Wrap-up and Keynote Introduction: Toshiyasu Taniguchi, MD, PhD	
9:30 – 10:10 10:10 – 10:15 Q&A	Keynote Address: "A New Mechanism of ICL Repair" Johannes Walter, PhD, Harvard Medical School, Boston, United States	p. 26
10:15 – 10:35	Break	
10:35 – 12:00 Grand A-G	The Fanconi Anemia Pathway: FANCD2/FANCI in Repair & Replication Stress - Part 2 Chair: Martin Cohn, PhD, University of Oxford, Oxford, United Kingdom	
10:35 – 10:40	Session Overview: Martin Cohn, PhD	
10:40 – 10:50 10:50 – 10:55 Q&A	Lesion Proximal FANCD2 is Required for Replication Independent Repair of Interstrand Crosslinks Marina Bellani, PhD, NIA, NIH, Baltimore, United States	p. 27
10:55 – 11:05 11:05 – 11:10 Q&A	Redefining How FANCI Phosphorylation Activates the Fanconi Anemia I/D2 Complex Ronald Cheung, MD, PhD, Fred Hutchinson Cancer Research Center, Seattle, United States	p. 28
11:10 – 11:20 11:20 – 11:25 Q&A	The PTEN Phosphatase Functions Cooperatively with the Fanconi Anemia Proteins in DNA Crosslink Repair Elizabeth Vuono, University of Rhode Island, Kingston, United States	p. 29
11:25 – 11:35 11:35 – 11:40 Q&A	CTDP1 Regulates FANCI and ICL Sensitivity Nicholas Woods, PhD, University of Nebraska Medical Center, Omaha, United States	p. 30
11:40 – 11:50 11:50 – 11:55 Q&A	FANCD2 and FANCI Have Independent Functions During the Cellular Replication Stress Response Jung Eun Yeo, PhD, University of Minnesota, Minneapolis, United States	p. 31
11:55 – 12:00	Session Wrap-up: Martin Cohn, PhD	
12:00 – 2:00 Grand IJK	Lunch Buffet	

2:00 – 3:15 Grand A-G	Clinical Experience: Transplantation & Disease Challenges <i>Chair: Eva Guinan, MD, Dana-Farber Cancer Institute, Boston, United States; Scientific Advisory Board, FARF</i>
2:00 – 2:05	Session Overview: Eva Guinan, MD
2:06 – 2:13	Myelodysplastic Syndrome and Acute Myeloid Leukemia in Japanese Fanconi Anemia Patients p. 32 <i>Miharu Yabe, MD, PhD, Tokai University School of Medicine, Isehara, Japan</i>
2:13 – 2:20	Clonal Mosaicism as Biomarker of Cancer Risk and Cancer-Free Survival in Fanconi Anemia p. 33 <i>Jordi Surrallés, PhD, Universitat Autònoma de Barcelona, Barcelona, Spain</i>
2:20 – 2:27	Patterns and Frequency of Renal Abnormalities in Fanconi Anemia – Implications for Long Term Management p. 34 <i>Stefan Meyer, MD, PhD, University of Manchester, Manchester, United Kingdom</i>
2:27 – 2:34	Hematopoietic Stem Cell Transplantation Results in Adult Patients with FA p. 35 <i>Marc Bierings, MD, PhD, Utrecht University Children's Hospital, Utrecht, The Netherlands</i>
2:34 – 2:41	Haploidentical Transplantation with Post-transplantation Cyclophosphamide (PT-CY) for the Treatment of Patients with Fanconi Anemia: Experience of Curitiba p. 36 <i>Carmem Bonfim, MD, PhD, Federal University of Paraná, Curitiba, Brazil</i>
2:41 – 2:48	Outcome of HLA Identical Related Versus Unrelated Donor Transplants in Fanconi Anemia: A Single Center Experience Using Non-irradiation Regimens p. 37 <i>Carmem Bonfim, MD, PhD</i>
2:48 – 2:55	Transplant Associated Thrombotic Microangiopathy in Individuals with Fanconi Anemia p. 38 <i>Kasiani Myers, MD, Cincinnati Children's Hospital Medical Center, Cincinnati, United States</i>
2:55 – 3:15	Panel Discussion: All Speakers
3:15 – 3:35	Break
3:35 – 5:00 Grand A-G	Preclinical Models & Drug Development <i>Chair: Markus Grompe, MD, Oregon Health & Science University, Portland, United States</i>
3:35 – 3:40	Session Overview: Markus Grompe, MD
3:40 – 3:50 3:50 – 3:55 Q&A	Synergy between Checkpoint Blockade and Resolving Inflammation in a Novel Murine Transplantable FANCC^{-/-} Tumor Model p. 39 <i>Allison Gartung, PhD, Beth Israel Deaconess Medical Center, Boston, United States</i>
3:55 – 4:05 4:05 – 4:10 Q&A	Marrow From a Second Strain of Double Knockout (DKO) SMAD3^{-/-} Fancd2^{-/-} Mice (Uniform 129/Sv background) Shows Marked Reduction of Duration of Hematopoiesis in Continuous Bone Marrow Cultures p. 40 <i>Joel Greenberger, MD, University of Pittsburgh Medical School, Pittsburg, United States</i>
4:10 – 4:20 4:20 – 4:25 Q&A	The Immune Receptor Trem1 Cooperates with Diminished DNA Damage Response to Induce Fanconi Anemia Preleukemic Stem Cell Expansion p. 41 <i>Wei Du, MD, PhD, University of Arkansas for Medical Sciences, Little Rock, United States</i>
4:25 – 4:35 4:35 – 4:40 Q&A	TGF-β Pathway Inhibition by Galunisertib Rescues Genotoxicity in Hematopoietic Stem Cells from Fanconi Anemia Mice p. 42 <i>Kalindi Parmar, PhD, Dana-Farber Cancer Institute, Boston, United States</i>
4:40 – 4:50 4:50 – 4:55 Q&A	Metformin Improves Defective Hematopoiesis and Delays Tumor Formation in Fanconi Anemia Mice p. 43 <i>Qingshuo Zhang, PhD, Oregon Health & Science University, Portland, United States</i>
4:55 – 5:00	Session Wrap-up: Markus Grompe, MD

7:00 – 8:00 Grand IJK	Breakfast	
8:00 – 11:00 Grand A-G	New Genes & Gene Therapy Co-chairs: Rich Gelinias, PhD, Institute for Systems Biology, Seattle, United States; Board of Directors and Scientific Advisory Board, FARF; Jakub Tolar, MD, PhD, University of Minnesota, Minneapolis, United States; Scientific Advisory Board, FARF	
8:00 – 8:05	Session Overview: Richard Gelinias, PhD	
8:05 – 8:15 8:15 – 8:20 Q&A	FANCV (REV7/MAD2L2), a New Fanconi Anemia Gene Dominique Bluteau, PhD, French Institute of Health and Medical Research (INSERM), Paris, France	p. 44
8:20 – 8:30 8:30 – 8:35 Q&A	Novel Fanconi Anemia E3 Ligase RFWD3 Promotes Removal of Both RPA and RAD51 from DNA Damage Sites During ICL Repair Shojiro Inano, MD, Kyoto University Radiation Biology Center, Kyoto, Japan	p. 45
8:35 – 8:45	Session Wrap-up and Keynote Introduction: Richard Gelinias, PhD	
8:45 – 9:25 9:25 – 9:30 Q&A	Keynote Address: “Selection-Free Genome Editing of the Sicklet Mutation in Human Adult Hematopoietic Stem/Progenitor Cells” Mark DeWitt, PhD, University of Berkeley, Berkeley, United States	p. 46
9:30 – 9:50	Break	
9:50 – 9:55	Session Overview: Jakub Tolar, MD, PhD	
9:55 – 10:05 10:05 – 10:10 Q&A	NHEJ-mediated Gene Editing in Fanconi Anemia Hematopoietic Cells Francisco J. Roman-Rodriguez, CIEMAT/CIBERER/IIS-Fundación Jimenez Diaz (IIS-FJD, UAM), Madrid, Spain	p. 47
10:10 – 10:20 10:20 – 10:25 Q&A	Genetic Reversion of FA Alkylator Sensitivity by a Mitotically Stable, Anchored Non-integrating Lentiviral Vector Santhosh Chakkaramakkil Verghese, PhD, Oregon Health & Science University, Portland, United States	p. 48
10:25 – 10:35 10:35 – 10:40 Q&A	Preclinical and Clinical Studies of Lentiviral-Mediated Gene Therapy with Plerixafor and G-CSF-Mobilized CD34+ Cells in FA-A Patients Paula Rio, PhD, CIEMAT/CIBERER/IIS-Fundación Jimenez Diaz (IIS-FJD, UAM), Madrid, Spain	p. 49
10:40 – 10:50 10:50 – 10:55 Q&A	Early Results of a Phase I Clinical Trial of Lentivirus Mediated Gene Therapy for Fanconi Anemia Complementation Group A Jennifer E. Adair, PhD, Fred Hutchinson Cancer Research Center, Seattle, United States	p. 50
10:55 – 11:00	Session Wrap-up: Jakub Tolar, MD, PhD	
11:00 – 12:00	Town Hall Chair: Ray Monnat Jr., MD, University of Washington, Seattle, United States; Chair of Scientific Advisory Board, Fanconi Anemia Research Fund	