2017 FA Scientific Symposium Agenda

Thursday, 14

3:00 *Prefunction*	**Symposium Check-in and Registration Opens**	
4:30 – 6:00 *Grand Ballroom 2&3*	**Living With FA: Natural History of Disease & Clinical Perspectives** *Chair: Mark Quinlan, Executive Director, Fanconi Anemia Research Fund, Eugene, United States*	
4:30 – 4:35	**Introduction:** Mark Quinlan, Executive Director, Fanconi Anemia Research Fund	
4:35 – 4:55 4:55 – 5:00 Q&A	**Early Childhood (0 – 10 yrs)** *Akiko Shimamura, Dana-Farber/Boston Children's Hospital, United States* *Lisa Mingo, FA Parent, Vancouver, Canada*	
5:00 – 5:20 5:20 – 5:25 Q&A	**Late Childhood/Young Adults (11 – 20 yrs)** *Stella Davies, Cincinnati Children's Hospital Medical Center, United States;* *Board of Directors, Fanconi Anemia Research Fund* *Stan and Michelle Kalemba, FA Parents, St. John, United States*	
5:25 – 5:45 5:45 – 5:50 Q&A	**Adulthood** *Farid Boulad, Memorial Sloan Kettering Cancer Center, New York, United States* *Jason Brannock, Adult with FA, Apex, United States*	
5:50 – 6:00 Q&A	*All panelists*	
6:00 – 8:00 *Prefunction and Buckhead Ballroom*	**Welcome Reception and Poster Viewing**	
	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*	

Friday, 15

7:00 – 8:00 *Grand Ballroom 1*	**Breakfast**	
8:00 – 8:10 *Grand Ballroom 2&3*	**Welcome and Introduction**	
	Kevin McQueen, President, Board of Directors, Fanconi Anemia Research Fund, Midlothian, United States *Ray Monnat, Jr., University of Washington, Seattle, United States; Chair of Scientific Advisory Board, Fanconi Anemia Research Fund*	
8:10 – 9:50 *Grand Ballroom 2&3*	**Cancer in FA** *Chair: Agata Smogorzewska, The Rockefeller University, New York, United States*	
8:10 – 8:15	**Session Overview: Agata Smogorzewska**	
8:15 – 8:40 8:40 – 8:45 Q&A	**Keynote Address: Epidemiology of Cancer in Fanconi Anemia** *Blanche P. Alter, National Cancer Institute, Rockville, United States*	*p. 6*
8:45 – 8:55 8:55 – 9:00 Q&A	**Lipidomic Profiling of Fanconi Anemia Deficient Normal and Transformed Keratinocytes Reveals Perturbed Glycosphingolipid Metabolism** *Susanne Wells, Cincinnati Children's Hospital Medical Center, Cincinnati, United States*	*p. 8*

9:00 – 9:10 9:10 – 9:15 Q&A	**Sequencing of Clinically Relevant Somatic Gene Mutations in Brush Biopsies of Oral Potentially Malignant Lesions of FA and non-FA Patients** *Nadarajah Vigneswaran, University of Texas School of Dentistry at Houston, United States*	*p. 9*
9:15 – 9:25 9:25 – 9:30 Q&A	**Quercetin Decreases the Proliferation and Causes Cell Death in Fanconi Anemia Deficient Head and Neck Cancer Cell Lines In Vitro** *Ruby Khoury, Cincinnati Children's Hospital Medical Center, Cincinnati, United States*	*p. 10*
9:30 – 9:40 9:40 – 9:45 Q&A	**Identification of a Therapeutic Strategy Targeting Fanconi Anemia (FA) Pathway Defects in Cancers** *Ali Suliman, Yale University School of Medicine, New Haven, United States*	*p. 11*
9:45 – 9:50	**Session Wrap-up: Agata Smogorzewska**	
9:50 – 10:10	**Break**	
10:10 – 12:00 *Grand Ballroom 2&3*	**Cancer Immunotherapy** *Chair: Premal Patel, Immuno-Oncology Early Development, Pfizer, Redwood City, United States*	
10:10 – 10:15	**Session Overview: Premal Patel**	
10:15 – 10:40 10:40 – 10:45 Q&A	**Advances in Immuno-Oncology** *Premal Patel*	*p. 12*
10:45 – 11:10 11:10 – 11:15 Q&A	**Effects of Head and Neck Tumor Heterogeneity on Immune Response** *John Sunwoo, Stanford University, United States*	*p. 12*
11:15 – 11:40 11:40 – 11:45 Q&A	**Keynote Address: Immune Escape by Head and Neck Cancer and Therapeutic Reversal** *Robert Ferris, University of Pittsburg, United States*	*p. 7*
11:45 – 12:00	**Panel Discussion: All Speakers**	
12:00 – 2:00 *Grand Ballroom 1*	**Lunch Buffet**	
12:15 – 2:00 *Grand Ballroom 1*	**Mentorship Lunch for Early Investigators**	
2:00 – 2:35 *Grand Ballroom 2&3*	**Rare Disease Research and Development: The NF Story**	
2:00 – 2:05	**Introduction: Maureen Hoatlin, Oregon Health & Science University, Portland, United States**	
2:05 – 2:30 2:30 – 2:35 Q&A	**The Children's Tumor Foundation Business Model: an Emerging Niche in the Rare Disease Ecosystem** *Salvatore La Rosa, Children's Tumor Foundation, New York, United States*	*p. 13*
2:35 – 5:00 *Grand Ballroom 2&3*	**Preclinical Models** *Chair: Elaine Ostrander, National Human Genome Research Institute, NIH, Bethesda; Scientific Advisory Board, Fanconi Anemia Research Fund, United States*	
2:35 – 2:40	**Session Overview: Elaine Ostrander**	
2:40 – 3:05 3:05 – 3:10 Q&A	**Engineering Hematopoietic Stem Cells from Human IPS Cells – Application to Fanconi Anemia** *Grant Rowe, Boston Children's Hospital, Boston, United States*	*p. 14*
3:10 – 3:20 3:20 – 3:25 Q&A	**Porcine Model of FANCA** *William H. Fleming, Oregon Health & Science University, Portland, United States*	*p. 15*
3:25 – 3:35 3:35 – 3:40 Q&A	**Identification and Characterization of a Blood Stem Cell-Enriched Cell Population for Gene Therapy** *Stefan Radtke, Fred Hutchinson Cancer Research Center, Seattle, United States*	*p. 16*
3:40 – 3:45	**Session Wrap-up: Elaine Ostrander**	
3:45 – 4:05	**Break**	

4:05 – 4:15	**Fanconi Anemia: The Bone Side of the Disease** *Melody Mazon, Laval University, Québec, Canada*	*p. 17*
4:15 – 4:25	**Fanconi Anemia (FA) Fancd2-/- Mouse Bone Marrow Stromal Cells Demonstrate Increased Irradiation Induced Senescence** *Aranee Sivananthan, University of Pittsburg, United States*	*p. 18*
4:25 – 4:35	**DNA Crosslink Repair is Essential for Germ Cell Development** *Gerry Crossan, MRC Laboratory of Molecular Biology, Cambridge, England*	*p. 19*
4:35 – 4:45	**A FANCI Knock-out Mouse Model Supports New Roles for FANCI, Independently of FANCD2** *Laure Guitton, Centre de Recherche du CHU de Québec, Canada*	*p. 20*
4:45 – 5:00	**Panel Discussion and Wrap-up**	
5:30 – 7:30 *Prefunction and Buckhead Ballroom*	**Poster Reception**	
	Presenters of even-numbered posters will be at their posters from 5:30 to 6:30 *Presenters of odd-numbered posters will be at their posters from 6:30 to 7:30*	
7:30 – 9:30 *Grand Ballroom 2&3*	**Symposium Banquet**	

Saturday, 16

7:00 – 8:30 *Grand Ballroom 1*	**Breakfast**	
8:30 – 9:55 *Grand Ballroom 2&3*	**The FA Pathway: Functions & Interactions – Part 1** *Chair: Alan D'Andrea, Dana-Farber Cancer Institute, Harvard Medical School, Boston, United States*	
8:30 – 8:35	**Session Overview: Alan D'Andrea**	
8:35 – 8:45 8:45 – 8:50 Q&A	**FANCD2 and FANCI Function Independently During the Cellular Replication Stress Response** *Jung Eun Yeo, Ulsan National Institute of Science and Technology, Ulju-gun, South Korea*	*p. 21*
8:50 – 9:00 9:00 –9:05 Q&A	**FANCB Mediates Dimerization and Subcellular Localization of the Fanconi Anemia Core Complex** *Sylvie van Twest, St Vincent's Institute of Medical Research, Fitzroy, Australia*	*p. 22*
9:05 – 9:15 9:15 – 9:20 Q&A	**Identification of the UHRF2 (RNF107) Protein as an ICL Sensor, Assisting in Recruiting FANCD2 to Chromatin** *Anna Motnenko, University of Oxford, United Kingdom*	*p. 23*
9:20 – 9:30 9:30 – 9:35 Q&A	**The Replisome Promotes Hierarchical Usage of Two Replication-Coupled ICL Repair Pathways** *Dan Semlow, Harvard University, Boston, United States*	*p. 24*
9:35 – 9:45 9:45 – 9:50 Q&A	**Regulation of FANCD2 Monoubiquitination and Function in Replication Fork Maintenance via a Conserved Phosphosite Cluster Proximal to K561** *David Vierra, University of Rhode Island, Newport, United States*	*p. 25*
9:50 – 9:55	**Session Wrap-up: Alan D'Andrea**	
9:55 – 10:15	**Break**	

10:15 – 11:55 *Grand Ballroom 2&3*	**The FA Pathway: Functions & Interactions – Part 2** *Chair: Niall Howlett, University of Rhode Island, Newport, United States*	
10:15 – 10:20	**Session Overview: Niall Howlett**	
10:20 – 10:30 10:30 – 10:35 Q&A	**FANCD2 Cooperates with the ATRX/DAXX Chromatin Remodeler to Promote HR-Mediated Replication Fork Recovery** *Maya Raghunandan, University of Minnesota, Minneapolis, United States*	*p. 26*
10:35 – 10:45 10:45 – 10:50 Q&A	**Fanconi Anaemia Deficient Cells Rely on Transcription Coupled Repair for Survival** *Jamie Langton, University of Oxford, United Kingdom*	*p. 27*
10:50 – 11:00 11:00 – 11:05 Q&A	**Acetaldehyde DNA Interstrand Crosslinks are Repaired via the Fanconi Anemia Pathway and a Potentially Novel Pathway** *Puck Knipscheer, Hubrecht Institute, Utrecht, Netherlands*	*p. 28*
11:05 – 11:15 11:15 – 11:20 Q&A	**Evidence of Interferon Stress in Hematopoietic Stem Cells (HSC) in Response to Increased Aldehydic Load** *Lauren Van Wassenhove, Stanford University, United States*	*p. 29*
11:20 – 11:30 11:30 – 11:35 Q&A	**Fatty Aldehyde Dehydrogenase as a Novel Binding Partner Of FANCD2** *Wataru Sakai, Kobe University, Japan*	*p. 30*
11:35 – 11:45 11:45 – 11:50 Q&A	**A Protein Folding Way to Precision Health for Fanconi Anemia** *Georgios Karras, Whitehead Institute for Biomedical Research, Cambridge, United States*	*p. 31*
11:50 – 11:55	**Session Wrap-up: Niall Howlett**	
12:00 – 2:00 *Grand Ballroom 1*	**Lunch Buffet**	
2:00 – 3:20 *Grand Ballroom 2&3*	**Advances in Transplantation** *Chair: John Wagner, University of Minnesota, Minneapolis, United States*	
2:00 – 2:05	**Session Overview: John Wagner**	
2:05 – 2:15	**Engraftment and Repopulation Advantage of Gene-Corrected Hematopoietic Precursors in Non-Conditioned Fanconi Anemia Patients** *Paula Rio, CIEMAT/CIBERER/IIS-Fundación Jimenez Diaz (IIS-FJD, UAM), Madrid, Spain*	*p. 32*
2:15 – 2:25	**Preliminary Conclusions of a Clinical Trial for The Mobilization Of Hematopoietic Stem Cells from Fanconi Anemia Patients with Plerixafor and Filgrastim** *Susana Navarro, CIEMAT/CIBERER/IIS-Fundación Jimenez Diaz (IIS-FJD, UAM), Madrid, Spain*	*p. 33*
2:25 – 2:35	**StemRegenin-1 Expanded Umbilical Cord Blood Hematopoietic Stem Cell Transplantation for Fanconi Anemia** *John Wagner*	*p. 34*
2:35 – 2:45	**Excellent Outcome For 91 Fanconi Anemia Patients Undergoing Matched Related Transplants Using Cyclophosphamide 60mg/Kg in Brazil** *Carmem Bonfim, Federal University of Paraná, Curitiba, Brazil*	*p. 35*
2:45 – 2:55	**Allogeneic Stem Cell Transplantation Using HLA Matched Donors in Patients with Fanconi Anaemia – Single Centre Experience from India** *Biju George, Christian Medical College, Vellore, India*	*p. 36*
2:55 – 3:05	**TGF-β Pathway Inhibition by AVID200 Rescues Genotoxicity in Hematopoietic Stem and Progenitor Cells from Fanconi Anemia Mice** *Alfredo De Jesús Rodríguez Gómez, Dana Farber Cancer Institute/Harvard Medical School, Boston, United States*	*p. 37*
3:05 – 3:20	**Panel Discussion: All Speakers**	

3:20 – 3:40	**Break**	
3:40 – 5:05 *Grand Ballroom 2&3*	**Genetics & Gene Editing** *Chair: Richard Gelinas, Institute for Systems Biology, Seattle, United States; Board of Directors and Scientific Advisory Board, Fanconi Anemia Research Fund*	
3:40 – 3:45	**Session Overview: Richard Gelinas**	
3:45 – 3:55 3:55 – 4:00 Q&A	**Biallelic Mutations in the Ubiquitin Ligase RFWD3 Cause Fanconi Anemia** *Detlev Schindler, University of Wurzburg, Germany*	*p. 38*
4:00 – 4:10 4:10 – 4:15 Q&A	**Identification of 8 Patients with Biallelic Mutations in FANCM with Normal Hematology, Early On Set Cancer and Hypertoxicity to Chemotherapy** *Jordi Surrallés, Universitat Autónoma Barcelona/Hospital Sant Pau, Barcelona, Spain*	*p. 39*
4:15 – 4:25 4:25 – 4:30 Q&A	**FANCN Patient Mutations are Hypomorphic and Rescue Catastrophic Genomic Instability** *Elizabeth Thompson, University of Minnesota, Minneapolis, United States*	*p. 40*
4:30 – 4:40 4:40 – 4:45 Q&A	**CRISPR-Cas9 Genome Editing in Human Cells Works via the Fanconi Anemia Pathway** *Chris Richardson, UC Berkeley, United States*	*p. 41*
4:45 – 4:55 4:55 – 5:00 Q&A	**NHEJ-Mediated Gene Editing: A New Approach to Correct Different Mutations in Fanconi Anemia Hematopoietic Stem and Progenitor Cells** *Francisco J. Roman-Rodriguez, CIEMAT/CIBERER/IIS-Fundación Jimenez Diaz (IIS-FJD, UAM), Madrid, Spain*	*p. 42*
5:00 – 5:05	**Session Wrap-up: Richard Gelinas**	

Sunday, 17

7:00 – 8:30 *Grand Ballroom 1*	**Breakfast**	
8:30 – 10:25 *Grand Ballroom 2&3*	**Rare Disease Research & Drug Development** *Chair: Maureen Hoatlin, Oregon Health & Science University, Portland, United States*	
8:30 – 8:35	**Session Overview: Maureen Hoatlin**	
8:35 – 9:00 9:00 – 9:05 Q&A	**The Translator Project: Turning Biomedical Data Into Knowledge** *Christine Colvis, Drug Development Partnership Programs, National Institutes of Health, Bethesda, United States*	*p. 43*
9:05 – 9:15 9:15 – 9:20 Q&A	**Data Translator: an Open Science Data Platform for Mechanistic Disease Discovery** *Melissa Haendel, Oregon Health & Science University, Portland, United States*	*p. 43, 105*
9:20 – 9:30 9:30 – 9:35 Q&A	**A Data Translator for Fanconi Anemia** *Maureen Hoatlin*	*p. 43, 105*
9:35 – 10:00 10:00 – 10:05 Q&A	**Challenges and Opportunities in Rare Disease Drug Development** *Jeffrey Siegel, Genentech, San Francisco, United States*	*p. 44*
10:05 – 10:15 10:15 – 10:20 Q&A	**Enabling Clinical Trials and Translational Research Through a Fanconi Database** *Erica Jonlin, University of Washington, Seattle, United States*	*p. 43*
10:20 – 10:25	**Session Wrap-up: Maureen Hoatlin**	
10:25 – 10:45	**Break**	
10:45 – 11:45	**Living With FA: Symposium Recap & Research Priorities** *Co-chairs: Ray Monnat Jr., University of Washington, Seattle, United States; Chair of Scientific Advisory Board, Fanconi Anemia Research Fund* *Bradley D. Preston, Scientific Director, Fanconi Anemia Research Fund, Seattle, United States*	