

SYMPOSIUM AGENDA

Registration will take place in Bay Laurel Foyer
General session (all talks) will take place in Bay Laurel Ballroom South & Central
Poster receptions will take place in Bamboo Garden
Breakfasts & lunches will take place in Bamboo Garden
Banquet dinner will take place in Bay Laurel Ballroom South & Central

Note: regular talks are 15 minutes: 10 for the presenter, 5 for Q & A

THURSDAY 27TH

- 3:00 pm Registration
- 4:30 - 6:00 Opening Session
Stella Davies, Cincinnati Children's Hospital, United States
Grover Bagby, Oregon Health & Sciences University, United States
Mary-Beth Johnson, Oregon, United States
Alan D'Andrea, Dana-Farber Cancer Institute, United States
Sean Breininger, Minnesota, United States
John Wagner, University of Minnesota, United States
Isis Sroka, Fanconi Anemia Research Fund, United States
Sudhir Borgonha, Fanconi Anemia Research Fund, United States
- 6:00 - 8:00 Poster Reception
Presenters of odd-numbered posters will be at their posters 6:00 - 7:00 pm
Presenters of even-numbered posters will be at their posters 7:00 - 8:00 pm

FRIDAY 28TH

- 7:00 - 8:00 Breakfast
- 8:00 - 8:10 Welcome and Introduction
Mark Quinlan, Executive Director, Fanconi Anemia Research Fund
- 8:10 - 9:45 Cancer in FA**
- 8:10 - 8:15 Session Overview
Raymond Monnat, University of Washington, United States
- 8:15 - 8:30 Repurposing anticancer drugs inducing cancer-specific lethality in Fanconi anemia head and neck tumors p. 6
Jordi Surrallés, Genetics Department, Hospital de Sant Pau, Spain
- 8:30 - 8:45 Towards improved clinical management of FA-related cancer via a novel functional genomics approach p. 7
Josephine Dorsman, Amsterdam UMC, Netherlands

8:45 - 9:15 Keynote Address: Genomic Pathway network, and immunologic features distinguishing squamous carcinomas p. 1
Carter Van Waes, National Institutes of Health, United States

9:15 - 9:45 Keynote Address: Noninvasive molecular diagnosis and targeted chemopreventive treatment of oral precancers p. 2
Ruud Brakenhoff, VU University Medical Center, Netherlands

9:45 - 10:05 Break

10:05 - 11:05 Head & Neck Squamous Cell Carcinoma Panel

John Wagner, University of Minnesota, United States
Carter Van Waes, National Institutes of Health, United States
Joel Greenberger, University of Pittsburgh, United States
Ruud Brakenhoff, VU University Medical Center, Netherlands

11:05 - 12:00 Cancer in FA, continued

11:05 - 11:20 Cancer in Heterozygote Carriers of Fanconi Anemia p. 8
Blanche P. Alter, National Cancer Institute, United States

11:20 - 11:35 Detection of oral lesions and DNA aneuploidy in transplanted vs. non-transplanted Fanconi Anemia patients p.9
Eunike Velleuer, University of Duesseldorf, Germany

11:35 - 11:50 Lipidomic profiling links the Fanconi anemia pathway to glycosphingolipid metabolism in normal and transformed keratinocytes p.10
Susanne I. Wells, Cincinnati Children's Hospital Medical Center/University of Cincinnati College of Medicine, United States

12:00 - 2:00 Lunch
Mentorship Lunch

2:00 - 3:05 Cancer in FA, continued

2:00 - 2:15 Sustained reduction of oxidative stress in children with Fanconi Anemia treated with optimized dose Quercetin p.11
Parinda A. Mehta, Division of Bone Marrow Transplantation and Immune Deficiency, Cincinnati Children's Hospital Medical Center, United States

2:15 - 2:30 Protection of Hematopoietic Stem Cells By Aldehyde Dehydrogenase 2 (ALDH2) Activation p.13
Jennifer Tsai, Stanford University, United States

2:30 - 2:45 Identification of small molecule activators of the FA pathway p.14
Wayne Crismani, St. Vincent's Institute of Medical Research, Australia

2:45 - 3:00	Evi-1 overexpression rescues DNA damage-induced defects in Fanconi anemia hematopoietic stem cells leading to AML <i>Julius Grasel, German Cancer Research Center Heidelberg, Germany</i>	p. 15
3:00 - 3:05	Session Wrap-up <i>John Wagner, University of Minnesota, United States</i>	
3:05 - 3:25	Break	
3:25 - 4:55	FARF Tank	
5:30 - 7:30	Poster Reception <i>Presenters of even-numbered posters will be at their posters 6:00 - 7:00 pm</i> <i>Presenters of odd-numbered posters will be at their posters 7:00 - 8:00 pm</i>	
7:30 - 9:30	Symposium Banquet	

SATURDAY 29TH

7:00 - 8:30	Breakfast	
8:30 - 9:30	Rare Disease Research and Drug Development	
8:30 - 8:32	Session Overview <i>Maureen Hoatlin, Oregon Health and Science University, United States</i>	
8:32 - 8:47	From butterflies to patient-engaged phenotyping to advance clinical care and rare disease science <i>Melissa Haendel, Oregon State University, United States</i>	p. 17
8:47 - 9:00	Biomedical Translator: an integrated open science data platform for mechanistic disease discovery using Fanconi anemia as a demonstrator <i>Maureen Hoatlin, Oregon Health and Science University, United States</i>	p. 18
9:00 - 9:30	Getting from ideas to medicines; the path through regulatory approval and clinical trials <i>Jeffrey Siegel, Genentech, United States</i>	p. 19
9:30 - 12:00	HSC Biology in FA	
9:30 - 9:35	Session Overview <i>Markus Grompe, Oregon Health and Science University, United States</i>	
9:35 - 9:50	Unrestrained p38MAPK activation leads to MiTF-dependent hematopoietic stem cell attrition in Fanconi anemia <i>Alessia Oppezco, Gustave Roussy Cancer Center, Université Paris Saclay - Paris Sud, France</i>	p. 20
9:50 - 10:05	Doxycycline-inducible complementation permits robust hematopoietic differentiation of Fanconi anemia human induced pluripotent stem cells <i>Grant Rowe, Boston Children's Hospital, United States</i>	p. 21

10:05 - 10:25	Break	
10:25 - 10:40	A Pilot Study of Metformin in Fanconi Anemia <i>Elissa Furutani, Boston Children's Hospital, United States</i>	p. 22
10:40 - 10:55	Development of safe and non-genotoxic anti-c-Kit (CD117) antibody-based conditioning regimens for hematopoietic stem cell transplantation in Fanconi Anemia <i>Agnieszka Czechowicz, Stanford University, United States</i>	p. 23
10:55 - 11:10	Restoration of Hematopoietic Stem Cell Function and Genome Integrity in Fanconi Anemia <i>Wei Tong, Children's Hospital of Philadelphia and University of Pennsylvania Medical School, United States</i>	p. 24
11:10 - 11:25	TGF- β pathway inhibition rescues clonogenic growth of primary bone marrow from Fanconi anemia patients <i>Alfredo Rodriguez, Dana-Farber Cancer Institute, Harvard Medical School, United States</i>	p. 25
11:25 - 11:40	Biallelic mutations in FAAP100 predispose to Fanconi anemia (FA) <i>Julia Kuehl, University of Wuerzburg, Germany</i>	p. 26
11:40 - 11:55	Towards a Porcine Model of Fanconi Anemia <i>William H. Fleming, Oregon Health and Science University, United States</i>	p. 27
11:55 - 12:00	Session Wrap-up <i>Markus Grompe, Oregon Health and Science University, United States</i>	
12:00 - 1:30	Lunch	
1:30 - 5:00	FA Function, Mechanism and Disease Pathogenesis	
1:30 - 1:35	Session Overview <i>Andrew Deans, St. Vincent's Institute of Medical Research, Australia</i>	
1:35 - 2:05	Keynote Address: The Causes and Consequences of Replication Stress <i>Karlene Cimprich, Stanford University, United States</i>	p. 4
2:05 - 2:20	The E3 ubiquitin ligase TRAP1 activates DNA interstrand crosslink repair by the FA/BRCA and NEIL3 pathways <i>Robert Wu, Harvard Medical School, United States</i>	p. 28
2:20 - 2:35	Phosphorylation of FANCD2 by CK2 inhibits the FANCD2/FANCI complex and suppresses the Fanconi anemia pathway in the absence of DNA damage <i>David Lopez Martinez, University of Oxford, United Kingdom</i>	p. 29
2:35 - 2:50	TRIP13 is a novel regulator of the Fanconi Anemia/BRCA Pathway <i>Alan D'Andrea, Dana-Farber Cancer Institute, Harvard Medical School, United States</i>	p. 30
2:50 - 3:05	Map of synthetic rescue interactions for the Fanconi anemia DNA repair pathway identifies USP48 <i>Joanna Loizou, CeMM Research Center for Molecular Medicine, Austria</i>	p. 31

3:05 - 3:20	A novel role of FANCG in DNA double strand break repair <i>Niraj Joshi, Dana-Farber Cancer Institute, Harvard Medical School, United States</i>	p. 32
3:20 - 3:40	Break	
3:40 - 3:55	Mitochondrial DNA fork protection by FANCO/RAD51C suppresses inflammation <i>Katharina Schlacher, The University of Texas MD Anderson Cancer Center, United States</i>	p. 33
3:55 - 4:10	Non-genotoxic modulators of Fanconi Anemia phenotype <i>Georgios Karras, The University of Texas MD Anderson Cancer Center, United States</i>	p. 34
4:10 - 4:25	Monoubiquitination locks FANCI:FANCD2 on DNA <i>Winnie Tan, St. Vincent's Institute of Medical Research, Australia</i>	p. 35
4:25 - 4:40	Molecular architecture of Fanconi anemia core complex <i>Shabih Shakeel, MRC Laboratory of Molecular Biology, United Kingdom</i>	p. 36
4:40 - 4:55	Separate roles of the BRCA2 DNA binding domain in replication fork protection in response to hydroxyurea and DNA interstrand crosslink damage <i>Kimberly A. Rickman, The Rockefeller University, United States</i>	p. 37
4:55 - 5:00	Session Wrap-up <i>Andrew Deans, St. Vincent's Institute of Medical Research, Australia</i>	

SUNDAY 30TH

7:00 - 8:00	Breakfast	
8:00 - 9:40	Gene Editing and Gene Therapy	
8:00 - 8:05	Session Overview <i>Mark Osborn, University of Minnesota, United States</i>	
8:05 - 8:20	Investigating the impact of lentiviral transduction on Fanconi anemia hematopoietic stem cells for improved gene therapy <i>Anna Kajaste-Rudnitski, San Raffaele Telethon Institute for Gene Therapy, Italy</i>	p. 38
8:20 - 8:35	In Utero Gene Editing in Mice Mediated by PNA-Nanoparticles <i>Peter Glazer, Yale University, United States</i>	p. 39
8:35 - 8:50	Proliferative Advantage in Fanconi Anemia A Patients' Hematopoietic Stem and Progenitor Cells after correction by NHEJ-mediated Gene Editing <i>Francisco J. Roman-Rodriguez, Centro de Investigaciones Energéticas Medioambientales y Tecnológicas (CIEMAT) / Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER-ISCIII), Spain</i>	p. 40
8:50 - 9:05	Improving genome editing outcomes in hematopoietic stem cells <i>Christopher Richardson, UC Berkeley, United States</i>	p. 42

9:05 - 9:20	From bone marrow to mobilized stem cells: the circuitious path to clinical gene therapy for Fanconi Anemia <i>Pam Becker, Fred Hutchinson Cancer Research Center, United States</i>	p. 43
9:20 - 9:35	Lentiviral-mediated Gene Therapy in Non-Conditioned Fanconi Anemia Patients <i>Juan Bueren, Centro de Investigaciones Energéticas Medioambientales y Tecnológicas (CIEMAT) / Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER-ISCIII), Spain</i>	p. 44
9:35 - 9:40	Session Wrap-up <i>Mark Osborn, University of Minnesota, United States</i>	
9:40 - 10:00	Break	
10:00 - 11:00	Closing session	