Universität Zürich[UZH]

radiz - Rare Disease Initiative Zürich

Clinical Research Priority Program for Rare Diseases University of Zurich

8th Rare Diseases Summer School

Kartause Ittingen, Warth

Wednesday, July 15th to Friday, July 17th 2020

PROGRAM

The program is subject to change without notice.

Please refer to the Summer School website for updates.

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Wednesday, July 15, 2020

09:00 – 10:15	Registration
10:15 – 10:30	**Welcome,** Prof. Matthias Baumgartner, Children's Hospital Zurich and University of Zurich, lead radiz
10:30 – 11:10	Prof. Nine Knoers, Department of Genetics, University Medical Center Groningen, the Netherlands **Next generation sequencing in rare disorders: scientific impact and clinical utility** *Discussion*
11:10 – 11:50	Prof. Marshall Summar, Children's National Medical Center, George Washington University, Washington DC, USA **Clinical endpoints: selecting outcomes and surrogate markers in rare diseases** *Discussion*
11:50 – 12:50	Lunch
13:00 – 14:30	Dr. Fabienne Hartmann, University of Zurich, Cutiss AG **Regulatory framework on the basis of a case study**
14:30 – 15:30	**Oral presentations** by selected participants
15:30 – 16:00	Coffee Break
16:00 – 16:40	Prof. Andrew Dwyer, Boston College, USA **Translating rare disease research into practice using patient partnerships** *Discussion*
16:40 – 17:20	Prof. Stephan Neuhauss, University of Zurich **Studying rare diseases in the (not so rare) Zebrafish model organism** *Discussion*
17:30 – 18:30	Check-in time
18:30 – 20:00	Dinner
20:00 –	**Poster viewing**

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Thursday, July 16, 2020	
07:45 – 08:45	Breakfast
08:45 – 09:45	**Oral presentations** by selected participants
09:45 – 10:30	Dr. Jasmin Barman-Aksözen, Swiss society for porphyria **On the Dark Side of Medicines Access: Approval and Reimbursement of New Drugs** *Discussion*
10:30 – 11:00	Coffee Break
11:00 – 12:00	Dr. Jürg Streuli, Children's Hospital Zurich **Interactive workshop: Ethical considerations (case study)**
12:00 – 13:15	Lunch
13:15 – 14:30	**Workshop on NGS** led by Prof. Nine Knoers
14:30 – 16:15	**Poster viewing and coffee break**
16:15 – 17:30	**Workshop on drug development** led by Prof. Marshall Summar
17:30 – 18:15	Prof. Michele De Luca, University of Modena and Reggio Emilia, Italy **Combined cell and gene therapy for *Epidermolysis Bullosa*** *Discussion*
18:15 – 18:30	Free time
18:30 – 21:00	Grill

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Friday, July 17, 2020	
07:45 – 08:45	Breakfast
08:45 – 09:40	**Preparation and discussion of take-home messages** by participants
09:40 – 10:20	Prof. Marshall Summar, Washington DC, USA **From rare diseases to common diseases: lessons from the urea cycle**
10:20 – 10:50	Coffee Break
10:50 – 11:30	Prof. Guenter Schwarz **Cysteine catabolism: From ultra-rare neurodegenerative disease to longevity** *Discussion*
11:30 – 12:00	TBA **Patient-initiated research** *Discussion*
12:00 – 12:15	Award of poster prizes and concluding remarks
12:30	End of the Summer School and departure of the participants