



RADIZ - RARE DISEASE INITIATIVE ZÜRICH
CLINICAL RESEARCH PRIORITY PROGRAM
UNIVERSITY OF ZÜRICH

2nd Rare Diseases Summer School

Tagungszentrum Schloss Au, Wädenswil (on lake Zurich)
Monday, July 14th to Wednesday, July 16th 2014

UNIVERSITY
 **CHILDREN'S HOSPITAL
ZÜRICH**
Children's Research Center CRC

 *The hospital of the
Eleonore Foundation*



radiz
Rare Disease Initiative Zurich

Program and aims

A disease is considered rare when fewer than one in 2'000 persons is affected. Today, more than 7'000 rare diseases are known, affecting approximately five to eight percent of the global population. Most rare diseases already manifest themselves in childhood. Rare diseases often lead to a grave impairment of health and may be very difficult to diagnose.

While progress has been made in recent years in the diagnosis and treatment of rare diseases, the situation for many patients is still dire. For many rare diseases, there are still no treatments. Interdisciplinary national and international collaborations are needed in order to improve the prospects for patients with rare diseases and to translate findings from research to the clinic.

The 2nd radiz Rare Diseases Summer School will focus on a wide variety of subjects in the arena of rare diseases, from disease mechanisms and animal models, to improving diagnoses, to novel therapeutics. There will be lectures and workshops on **drug development, model organisms, how to choose clinical endpoints, clinical trials, regulatory aspects, patient registries, patient initiated research, ethical considerations**, as well as what rare diseases may tell us about common diseases.

The summer school will contain lectures by national and international rare disease experts, workshops, and poster presentations by participants. A high staff to student ratio will allow participants to optimally benefit from the varied expertise of the faculty. There will be ample time for discussions, teamwork, as well as for socializing.

Organizing Committee

Prof. Matthias Baumgartner, Prof. Olivier Devuyst, Prof. Arnold von Eckardstein, Prof. Barbara Plecko, Prof. Anita Rauch, and Dr. Saskia Karg; University of Zurich, Children's Hospital Zurich, and University Hospital Zurich.

Keynote lecture

Prof. Leena Bruckner-Tuderman,
University of Freiburg, Germany

Confirmed speakers (in alphabetical order)

- **Prof. Nine Knoers**, University Medical Centre Utrecht, Utrecht, The Netherlands: Exome sequencing
- **Prof. Stephan Neuhaus**, University of Zurich: Model organisms
- **Samantha Parker**, Orphan Europe Recordati Group, France: Rare disease registries
- **Prof. Janine Reichenbach**, Children's Hospital Zurich: Clinical gene therapy
- **Dr. Tsveta Schyns-Liharska**, Founder and Secretary of ENRAH, Brussels, Belgium: Patient initiated research
- **PD Dr. Gabriela Senti**, University Hospital Zurich: Clinical trials
- **Prof. Marshall Summar**, Children's National Medical Center, George Washington University, Washington DC, USA: Clinical endpoints
- **Dr. Effy Vayena**, University of Zurich: Ethical considerations
- **Prof. Carsten Wagner**, University of Zurich: Model organisms

Eligibility and Application

The school addresses clinicians, physician-scientists, postdocs, and PhD students from various disciplines such as medicine, biology, psychology, sociology, and related fields. We plan to admit up to 40 participants. There are a limited number of spaces available to participants from industry. Participants are responsible for having valid insurance and a valid visa, if applicable, for Switzerland.

Participants are expected to present a poster about their work or research interests related to rare diseases at the Summer School. The best posters will be awarded a prize.

Please apply by sending the completed application form (to be downloaded from our website www.radiz.uzh.ch/events/summerschool.html), a short CV, a letter of motivation including a brief description of your project or research interest, and one letter of recommendation to saskia.karg@kispi.uzh.ch

The application deadline is April 11th 2014. Notification of acceptance will be given by May 14th 2014.

Language

The Summer School will be held in English.

Fee

There is a non-refundable registration fee of CHF 200 for participants from academia and clinics and CHF 500 for participants from industry. The fee becomes due upon notification of acceptance. Food (coffee, two dinners, one lunch) will be offered free of charge during the Summer School. Participants are responsible for their own travel.

Time and Venue

The summer school will take place at Tagungszentrum Schloss Au, Wädenswil (on lake Zurich), Switzerland and will start on Monday, July 14th at 1 pm and end on Wednesday, July 16th at 1 pm.

The program is subject to change without notice. Please refer to our website www.radiz.uzh.ch/events/summerschool.html for updated information.

Accommodation

Accommodation on lake Zurich, close to the conference venue, will be organized, but needs to be paid by participants. There will be a choice of rooms for either approximately CHF 105 for two nights, double occupancy in a youth hostel, approximately CHF 140 for two nights, single occupancy in a youth hostel (limited availability), or approximately CHF 300 - 400 for two nights, single occupancy in a hotel. Please indicate your choice on the application form. Choice is subject to availability. Prices may be subject to change.

Contact

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