



University of
Zurich^{UZH}

URPP ITINERARE - Innovative Therapies in Rare Diseases



ITINERARE

Rare Disease Summer School

Zurich - Switzerland



About the Summer School

8th Rare Disease Summer School

July 13th to 15th, 2022

Kartause Ittingen, Warth

The ITINERARE Rare Disease Summer School will cover a broad spectrum of subjects in the field of rare diseases. Participants will engage with experts on topics ranging from disease mechanisms and animal models to improving diagnoses and developing novel therapies. Moreover, they will address the ethical, social, and legal (ELSI) challenges associated with rare diseases.

The high interdisciplinary program builds on a long tradition established by radiz, the Rare Disease Initiative Zurich, and consists of lectures and workshops given by national and international rare disease experts. Focus will be given to drug development, model organisms, how to choose clinical endpoints, clinical trials, regulatory aspects, patient registries, patient-initiated research, and ELSI considerations.

All participants will have the chance to present their own research projects during dedicated poster sessions and authors of selected abstracts will give a short oral presentation about their work. There will be ample time for discussions, teamwork, as well as for socializing.

This summer school lasts two and a half days and is open to clinicians, physician-scientists, postdocs, and PhD students from various disciplines such as medicine, biology, psychology, sociology, and related fields. Attendance is restricted to 30 people to allow for extensive interaction with the experts, enabling participants to optimally benefit from the varied expertise of the faculty.

Scientific organizing committee

Prof. Matthias Baumgartner

Prof. Nikola Biller-Andorno

Prof. Olivier Devuyst

Prof. Janine Reichenbach

Prof. Brigitte Tag

Confirmed speakers

Lectures

Let the sunshine in! Getting a drug approved for the rare genetic light intolerance erythropoietic protoporphyria

Dr. Jasmin Barman-Aksözen, Swiss society for porphyria, Switzerland

Combined cell and gene therapy for Epidermolysis Bullosa

Prof. Michele De Luca, Modena and Reggio Emilia University, Italy

Person-centered approaches for bridging health disparities for rare disease patients

Prof. Andrew Dwyer, Boston College, USA

Regulatory framework for ATMPs on the basis of a case study

Dr. Fabienne Hartmann, CUTISS AG, Switzerland

Using AI for rare diseases diagnosis: my personal journey

Mr. Julian Isla, Dravet Syndrome Foundation, Spain

New nerve cells for old brains

Prof. Sebastian Jessberger, University of Zurich, Switzerland

Towards better and humane science: The 3Rs principle

Dr. Paulina Jirkof, University of Zurich, Switzerland

Latest developments in exome and genome sequencing: impact for rare diseases

Prof. Nine Knoers, University Medical Center Groningen, the Netherlands

Studying rare diseases in the (not so rare) Zebrafish model organism

Prof. Stephan Neuhauss, University of Zurich, Switzerland

Designing rare disease clinical trials: outcomes, sample size, natural history, and recruitment

Prof. Marshall Summar, Children's National Medical Center, USA

Workshops

Ethical considerations (case study)

Dr. Jürg Streuli, University of Zurich, Switzerland

So you want to develop a rare disease drug

Prof. Marshall Summar, Children's National Medical Center, USA

Next generation sequencing

Prof. Nine Knoers, University Medical Center Groningen, the Netherlands

More Information on the Summer School



www.itinerare.uzh.ch

ITINERARE - Innovative Therapies in Rare Diseases – is a University Research Priority Program (URPP) of the University of Zurich. Researchers and clinicians from the University of Zurich, Children's Hospital Zurich, and the University Hospital Zurich have joined forces in this program. Through its interdisciplinary and translational research projects, the URPP ITINERARE aims to develop novel molecular and gene therapies for selected genetic disorders and to establish a broad ethical-legal and educational framework of multidisciplinary expertise to address associated societal issues.



Location: Kartause Ittingen, Warth (Canton Thurgau)

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