



University of  
Zurich<sup>UZH</sup>

URPP ITINERARE - Innovative Therapies in Rare Diseases

A large, stylized illustration of a diverse crowd of people walking in various directions. The figures are rendered in a simplified, colorful manner with long shadows, suggesting a bright, sunny day. The crowd is spread across the entire page, framing the central text.

# ITINERARE *Symposium*

November 12th, 2021  
University Children's Hospital  
Zurich | 14:00 – 18:00

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University Children's Hospital Zurich  
Hörsaal | 14:00 – 18:00

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We are very pleased to invite you to the ITINERARE Symposium 2021. ITINERARE - Innovative Therapies in Rare Diseases – is a University Research Priority Program (URPP) of the University of Zurich. Through an interdisciplinary network of researchers and clinicians, the URPP ITINERARE fosters excellent translational research on rare diseases at the University Children's Hospital Zurich, the University Hospital Zurich, and the University of Zurich. 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.

Join our ITINERARE Symposium to get an insight into cutting-edge research and social innovation aspects in rare diseases through the contributions of invited international speakers and our project leaders.

We look forward to welcoming you at the University Children's Hospital Zurich!

The URPP ITINERARE Directorate

**Director**

Prof. Janine Reichenbach, Institute for Regenerative Medicine  
University of Zurich

**Co-directors**

Prof. Olivier Devuyst, Institute of Physiology  
University of Zurich

Prof. Matthias Baumgartner, Division of Metabolic Diseases  
University Children's Hospital Zurich

**Deputy-directors**

Prof. Brigitte Tag, Faculty of Law

Prof. Nikola Biller-Andorno, Institute of Biomedical Ethics and History of Medicine  
University of Zurich

## Program

### 14:00 - 14:10 **Opening**

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Prof. Janine Reichenbach, Institute for Regenerative Medicine,  
University of Zurich

*Chair:* Prof. Olivier Devuyst, Institute of Physiology, University of Zurich

### 14:10 - 14:40 **Integrating research and care for rare genetic diseases: the experience of the Imagine Institute (Paris)**

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Prof. Stanislas Lyonnet, Imagine Institute, Paris

### 14:40 - 15:10 **Correcting monogenic liver diseases by in vivo genome editing**

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Prof. Gerald Schwank, Institute of Pharmacology and Toxicology,  
University of Zurich

### 15:10 - 15:40 **Advancing gene and cell therapies beyond their affordability**

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Matt Bolz-Johnson, EURORDIS - Rare Diseases Europe, Germany

### 15:40 - 16:20 **Coffee Break**

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*Chair:* Prof. Matthias Baumgartner, Division of Metabolic Diseases,  
University Children's Hospital Zurich

### 16:20 - 16:50 **Swiss Rare Disease Registry**

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Prof. Claudia Kuehni, Institute of Social and Preventive Medicine (ISPM),  
University of Bern

### 16:50 - 17:20 **Drug discovery for endolysosomal disorders**

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Prof. Olivier Devuyst, Institute of Physiology, University of Zurich

### 17:20 - 17:50 **The Undiagnosed Diseases Network: National and International**

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Prof. Bill Gahl, National Human Genome Research Institute, NIH, US

### 17:50 - 18:00 **Closing**

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Prof. Janine Reichenbach, Institute for Regenerative Medicine,  
University of Zurich

More information here



[www.itinerare.uzh.ch](http://www.itinerare.uzh.ch)

Registration deadline: 31st October 2021



A valid Covid certificate along with an official ID are required  
in order to attend the event.