

# THE TRANSLATIONAL SCIENCE OF RARE DISEASES

## From Rare to Care V

 22-24 April 2026

 Evangelische Akademie Tutzing, Germany

# SCHEDULE

## WEDNESDAY, 22 APRIL 2026

13:00 REGISTRATION

14:00 WELCOME ADDRESS  
Rebecca Schüle, Heidelberg University Hospital  
Thomas Klopstock, LMU University Hospital, LMU Munich  
Christoph Klein, LMU University Hospital, LMU Munich

14:15 KEY NOTE TALK  
The era of genome editing in medicine  
[Toni Cathomen](#), Medical Center - University of Freiburg

### SESSION 1 GENETIC THERAPY APPROACHES Chair: Thomas Klopstock

15:00 The state of the art of n-of-1 therapies – Individualized Antisense Oligonucleotides for rare genetic diseases  
[Nofar Mor](#), Sheba Medical Center, Tel HaShomer

15:25 Gene editing for severe congenital neutropenia – current advances and critical considerations  
[Julia Skokowa](#), University Hospital Tübingen

15:50 COFFEE BREAK

16:20 Precision RNA editing: a therapeutic modality for rare genetic diseases  
[Philipp Reautschnig](#), University of Tübingen

16:45 Application of CRISPR base editors in innovative cancer therapies  
[Shady Sayed](#), Technical University Dresden

17:10 Short talks

17:20 New approaches in science communication

18:00 END OF SESSIONS

18:15 DINNER

# SCHEDULE

THURSDAY, 23 APRIL 2026

---

## SESSION 2 SMALL MOLECULE AND ANTIBODY MEDIATED THERAPIES

Chair: Bodo Grimbacher

- 08:15 **Novel treatment options for interferonopathies**  
[Michele Proietti](#),  
Hannover Medical School (MHH) & Medical Center - University of Freiburg
- 08:40 **Success of Leniolisib in immune-mediated diseases**  
[V. Koneti Rao](#),  
NIH/National Institute of Allergy and Infectious Diseases, Bethesda
- 09:05 **Moonlighting of the CBM complex**  
[Daniel Krappmann](#), Helmholtz Munich & LMU Munich
- 09:30 **Ligand-receptor interaction as a therapeutic target in rare endocrinological diseases**  
[Martin Wabitsch](#), Ulm University Hospital

10:00 **[POSTER SESSION](#)** & COFFEE BREAK

## SESSION 3 NOVEL AND PRECISION THERAPIES

Chairs: Tobias B. Huber & Jens König

- 11:10 **Current developments in the molecular understanding and therapeutic approaches of rare kidney diseases**  
[Oliver Devuyst](#), Université Catholique de Louvain Medical School (UCL), Brussels & University of Zurich (UZH)
- 11:35 **Antagonizing ASOs as precision treatment in developmental and epileptic encephalopathies presenting as gain-of-function channelopathies**  
[Steven Petrou](#), Praxis Precision Medicines, Boston
- 11:50 **Rethinking therapy of obesity: The case for precision medicine**  
[Annette Grüters-Kieslich](#), Charité - Universitätsmedizin Berlin
- 12:15 **Highly specific small molecules against subtypes of voltage-gated calcium channels as precision treatments in developmental and epileptic encephalopathies**  
[Henning Steinhagen](#), Lario Therapeutics, Edinburgh

12:30 LUNCH BREAK

# SCHEDULE

THURSDAY, 23 APRIL 2026

---

## SESSION 4 DRUG REPURPOSING - PANEL

Chair: Frank Leypoldt

- 13:30 Therapies for multiple sulfatase deficiency - drug repurposing in ultra-rare genetic diseases  
[Lars Schlotawa](#), University Medical Center Göttingen
- 13:55 Sildenafil as a new therapy for maternally inherited leigh syndrome  
[Markus Schülke](#), Charité - Universitätsmedizin Berlin
- 14:20 Drug repurposing and patient involvement in OI  
[Claudia Finis](#),  
Deutsche Gesellschaft für Osteogenesis imperfecta Betroffene e.V., Hamburg
- 14:35 Panel Discussion  
Claudia Finis, Lars Schlotawa, Markus Schülke,  
[Carolin Schwartner-Lehr \(Galenicap\)](#), [Anton Ussi \(EATRIS\)](#)

15:30 COFFEE BREAK

## SESSION 5 DISEASE PREDISPOSITION AND PREVENTION

Chair: Christian Kratz

- 16:00 Genetic predisposition to cancer  
[Richard Houlston](#), The Institute of Cancer Research, London
- 16:25 How does the immune system recognize and target particular molecular patterns and sequences as 'foreign', and how does this affect the evolution of tumors and viruses  
[Benjamin Greenbaum](#), Memorial Sloan Kettering Cancer Center, New York
- 16:50 Making human immune systems more interpretable through systems immunology  
[Petter Brodin](#), Karolinska Institutet, Solna
- 17:15 Short Talks

17:30 END OF SESSIONS

18:15 DINNER

---

# SCHEDULE

FRIDAY, 24 APRIL 2026

---

## SESSION 6 THE FUTURE OF CELL THERAPIES

Chair: Holger Lerche

- 08:30 Emerging molecular and cellular therapies  
[Harald Prüß](#), Charité - Universitätsmedizin Berlin
- 08:55 CAR T cell therapy in autoimmune diseases – insights from novel indications  
[Fabian Müller](#), Uniklinikum Erlangen, FAU Erlangen-Nürnberg
- 09:20 Stem cell therapies in neurodegeneration: promise and pitfalls  
[Marc Thier](#), Heidelberg University Hospital & German Cancer Research Center (DKFZ), Heidelberg

09:45 COFFEE BREAK

## SESSION 7 THERAPEUTIC FRONTIERS / MEDICAL ETHICS

Chair: Rebecca Schüle

- 10:15 Risdiplam for prenatal therapy of spinal muscular atrophy: scientific, medical and ethical considerations  
[Richard Finkel](#), St. Jude Children's Research Hospital, Memphis
- 10:40 Mitochondrial donation: Outcomes of a national reproductive care pathway for women with mitochondrial DNA disease  
[Robert McFarland](#), Newcastle University, Newcastle upon Tyne
- 11:05 Genomic newborn screening - legal implications, value, ethics and society  
[Georg Marckmann](#), LMU Munich
- 11:30 The epistemic authority of science: why do scientists demand trust when their norm is organized skepticism?  
[Ulrich Dirnagl](#), Berlin Institute of Health at Charité (BIH) & Charité - Universitätsmedizin Berlin

12:00 CLOSING REMARKS AND FAREWELL

12:15 END

---