RESEARCH FOR RARE
German Networks on Rare Diseases

With contributions from: DZKJ German Center for Child and Adolescent Health

LMU KLINIKUM

HEIDELBERG UNIVERSITY HOSPITAL

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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	Innovative technologies for gene editing Frank Buchholz, 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, University Hospital Ulm

10:00 — POSTER SESSION & COFFEE BREAK

SESSION 3 — NOVEL AND PRECISION THERAPIES

Chair: 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

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, Laurens G. Theunis (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