THE TRANSLATIONAL SCIENCE OF RARE DISEASES

From Rare to Care V

22 - 24 April 2026

Evangelische Akademie Tutzing
Germany

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SCIENTIFIC PROGRAMME

SCIENTIFIC ORGANISATION:

RESEARCH FOR RARE
German Networks on Rare Diseases

LMU KLINIKUM

HEIDELBERG UNIVERSITY HOSPITAL

With contributions from:

DZKJ German Center for Child and Adolescent Health

With funding from the:

Federal Ministry of Research, Technology and Space

Coordinators of the Research Networks on Rare Diseases:

Christian Kratz - Hannover Medical School
ADDRess - Disorders with abnormal DNA damage response

Frank Leypoldt - University Hospital Schleswig-Holstein, Kiel
CONNECT-GENERATE - Autoimmune encephalitis

Bodo Grimbacher - Medical Center – University of Freiburg
GAIN - Multi-organ autoimmune diseases

Verena Keitel-Anselmino - University Hospital Magdeburg
HiChol - Hereditary intrahepatic cholestasis

Thomas Klopstock - LMU University Hospital, LMU Munich
mitoNET - Mitochondrial diseases

Miriam Erlacher - Medical Center – University Hospital Ulm
MyPred - Syndromes predisposing to myeloid malignancies

Tobias B. Huber - University Medical Center Hamburg-Eppendorf
STOP-FSGS - Focal segmental glomerulosclerosis

Jens König - University Hospital Münster
NEOCYST - Hereditary cystic kidney diseases

Rebecca Schüle - Heidelberg University Hospital
TreatHSP.net - Hereditary spastic paraplegias

Holger Lerche - University Hospital Tübingen
Treat-ION - Neurological ion channel and transporter disorders

Christoph Klein - LMU University Hospital, LMU Munich
DZKJ - German Center for Child and Adolescent Health, partner site Munich

WELCOME ADDRESS

Dear guests, colleagues and friends,

On behalf of the German Research Networks on Rare Diseases, we are pleased to welcome you to our international symposium "The Translational Science of Rare Diseases – from Rare to Care V" .

Since 2003, the Federal Ministry of Research, Technology and Space has supported the Rare Disease Networks through a highly successful funding program. This initiative has driven impactful improvements in diagnostics, therapeutic strategies, and patient care. In parallel, it has enabled the establishment of comprehensive disease registries and biobanks, providing a critical infrastructure for translational research and enabling numerous high-impact scientific publications and increased international visibility.

This symposium will highlight recent progress in state-of-the-art technologies applied to rare disease research, including gene therapy, small-molecule and antibody-based as well as cell-based therapies. Additional topics in precision medicine - such as drug repurposing and emerging therapeutic frontiers will also be addressed. We are honored to welcome distinguished national and international experts who will share their latest findings.

A hallmark of this meeting is its interdisciplinary scope. The Rare Disease Networks encompass a broad range of medical fields - including hematology, hepatology, immunology, nephrology, neurology, and pediatrics - and this diversity is reflected in both the program and the speaker lineup. We are confident that this event will spark insightful discussions, inspire innovative ideas, and foster new scientific collaborations.

In this spirit, we wish all participants an inspiring and enjoyable symposium on Lake Starnberg.

Thomas Klopstock

Department of Neurology
LMU University Hospital, LMU Munich

Rebecca Schüle

Department of Neurology
Heidelberg University Hospital

WELCOME ADDRESS Dorothee Bär

Federal Minister of Research, Technology and Space

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Dear readers,

The topic of health currently enjoys particular importance within our Ministry, thanks to our Science Year 2026 entitled "Medicine of the Future". This also represents a huge opportunity for research and communication about rare diseases. So many people deserve to hear about the potential that exists for diagnosis and treatment, what exactly we are doing to exploit this and how we are driving innovation forward. Of course, this is all possible thanks to your hard work and dedication.

Since 2003, the Ministry has provided €165 million to support a total of 39 national research networks. These collaborations have brought together leading experts from both basic and clinical research and have produced outstanding results for genetics, molecular biology and translational therapeutics. Our High-Tech Agenda Germany also builds on this. With its key technologies such as artificial intelligence and biotechnology, the Agenda strengthens Germany's position as a key location for medical research and healthcare and also supports our European and international collaborations in this field.

The better we are able to pool our strengths, the faster we can understand diseases and develop targeted treatments. I am therefore delighted to support what is now your fifth symposium "From Rare to Care". Your work provides hope for 300 million patients worldwide that they will live longer and better lives. What an important and, at the same time, incredibly challenging task. When it comes to rare diseases, far more needs to be done to bridge the oft-cited gap between scientific findings and clinical practice. More bridge builders like you are needed to increase the speed of progress. That's why your symposium is so valuable – research meets practice, medical expertise meets management, vision meets strategy. Thank you so much to everyone involved!

I hope that, this year, you again achieve outstanding successes that will benefit all those who are awaiting help. Rare diseases deserve our resolute commitment.

Dorothee Bär

Dorothee Bär MdB
Federal Minister of Research, Technology and Space

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PROGRAMME

WEDNESDAY, 22 APRIL 2026

13:00	**REGISTRATION**
14:00	**WELCOME ADDRESS** **Rebecca Schüle**, Heidelberg University Hospital **Christoph Klein**, LMU University Hospital, LMU Munich **Thomas Klopstock**, 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 Tobias Cantz, Hanover - Katja Lohmann, Lübeck - Katia Maalouf, Beirut
17:20	New approaches in science communication
18:00	END OF SESSIONS
18:15	DINNER

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** Olivier 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** Kris Kahlig, 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

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 Osteogenesis imperfecta** 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** Gamze Özata, Munich - Angela Schulz, Hamburg - Lea Terbeck, Muenster - Zeynep Ünlütürk, Pisa/Kocaeli

17:30 END OF SESSIONS

18:15 DINNER

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

Petter Brodin

Petter Brodin is Garfield Weston Chair and Professor of pediatric immunology at Imperial College London and professor of Pediatric immunology at Karolinska Institutes in Stockholm. The Brodin lab develops and applies novel experimental and computational methods to describe human immune system variation with a particular interest in the immune systems of children, its development early in life, and its role in health and disease during childhood. He is a consultant in pediatric immunology combining clinical work at Astrid Lindgren children's hospital, unit for pediatric rheumatology, Karolinska University Hospital with leading two research teams of 25 members at Karolinska Institutes and Imperial College. These teams develop and apply system immunology approaches to study human immune system variation in health and disease and human immune system development early in life. The work involves the elucidation of heritable and non-heritable factors that shape human immunity, immune system development and adaptation to microbial factors early in life as well as a research program in clinical application of systems immunology in children to diagnose and describe immune system dysregulation and enable precision medicine. To enable these activities the group develops novel experimental and computational methods to more comprehensively and accurately monitoring immune systems in humans. Brodin also founded and led the SciLifeLab national facility for Mass cytometry (2014-24) and served as head of the unit for clinical pediatrics at the department of Women's and Children's Health, Karolinska Institutet (2018-24).

Toni Cathomen

Toni Cathomen is Professor of Cell and Gene Therapy at the University of Freiburg, Germany, and Founding Director of the Institute for Transfusion Medicine and Gene Therapy at the University Medical Center Freiburg. He received his Ph.D. from the University of Zurich, Switzerland, and subsequently held research and faculty positions at the Salk Institute in San Diego, the Charité - Universitätsmedizin Berlin, and Hannover Medical School.

Prof. Cathomen's research centers on the development of next-generation immune cell therapies and genetically engineered hematopoietic stem cell products. His laboratory leverages genome editing technologies to design therapeutic strategies for patients with primary immunodeficiencies, HIV infection, and select malignancies. A substantial part of his work has focused on improving the safety of genome editing by establishing innovative platforms to quantify genotoxicity and characterize off-target effects. Through these contributions, Prof. Cathomen has helped define key mechanistic principles that guide the safe and effective translation of genome editing into clinical practice.

Olivier Devuyst

Olivier Devuyst, MD, PhD, is a full professor at the University of Zurich (UZH) since 2010, with a joint clinical appointment at Saint-Luc academic hospital in Brussels (UCLouvain). After studying at UCLouvain, he continued his training at the Technion Institute in Haifa (Israel) and at Johns Hopkins Medical School in Baltimore (USA). Prof. Devuyst and his group have been studying the mechanisms of genetic kidney diseases. These studies, which have generated more than 35 doctoral theses and over 420 original articles cited more than 55,000 times (h-index, 109), have been funded by national and international agencies, including the European Union, the Swiss National Science Foundation and the National Institute of Health (USA), as well as by various foundations. This work has led to seminal advances in our understanding of the genetic architecture of kidney disease and the mechanisms involved - relevant to common diseases and the discovery of new therapeutic targets. Olivier Devuyst has been the recipient of several international awards, including the ERA Award for Outstanding Contributions to Nephrology, the Donald Seldin Award from the International Society of Nephrology, the Chan Woon Cheung Memorial Fund Award from the Hong Kong Society of Nephrology, and the Oreopoulos Award from the Canadian Society of Nephrology. He has been a member of the Académie Royale de Médecine de Belgique since 2005. O. Devuyst is associate editor of Kidney International, Nephrology Dialysis Transplantation and Orphanet Journal of Rare Diseases, and is on the editorial board of Clin J Am Soc Nephrol, Peritoneal Dialysis International, Frontiers in Physiology and Pflügers Archiv.

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Ulrich Dirnagl

In preclinical as well as in clinical studies, Ulrich Dirnagl's research has revealed pathobiology which impact on the outcome after a stroke. Several of these mechanisms can be therapeutically targeted, clinical trials are under way. In addition, through meta-research he was able to identify opportunities for improving research practice and to obtain evidence for the impact of interventions targeted to increase the value of biomedical research. At the Charité Universitätsmedizin Berlin Ulrich Dirnagl is Professor of Clinical Neuroscience and served as founding director of the Department of Experimental Neurology from 1999 until 2022. Since 2017 he is also the founding director of the QUEST Center for Responsible Biomedical Research at the Berlin Institute of Health. QUEST aims at overcoming the roadblocks in translational medicine by increasing the value and impact of biomedical research through maximizing the quality, reproducibility, generalizability, and validity of research.

Claudia Finis

Claudia Finis is a psychologist and works as a research associate at the Berlin Institute of Health at Charité in the field of rare diseases. In her work, she always combines the scientific and patient perspectives. She is active as a health policy representative for the German OI association (DOIG). She works at European and national level as a patient representative in the development of guidelines and with regulatory agencies. At European level, she represents people with a rare bone disease in the European Reference Network for Rare Bone Diseases (ERN BOND) as a patient advocate and is involved in the European Registry for people with a Rare Bone Disease (EuRREB) as a patient expert.

Richard Finkel

Richard S. Finkel, MD, pediatric neurologist, directs the Center for Experimental Neurotherapeutics at St. Jude Children's Research Hospital. Clinical practice and research interests have focused upon neuromuscular disorders, especially Spinal Muscular Atrophy, Duchenne Muscular Dystrophy, and Charcot-Marie-Tooth. Dr. Finkel has participated in numerous clinical trials and natural history studies, and has contributed to the development of standard-of-care guidelines, outcome measures and clinical trial design. He has published over 200 manuscripts. Dr. Finkel received the Sidney Carter Award in Child Neurology from the American Academy of Neurology (2018) and shared the International Prize for Translational Neuroscience from the Max Plank Institute (2019).

Benjamin Greenbaum

Dr. Greenbaum is an Attending in the Computational Oncology Service. He has a PhD in theoretical physics from Columbia University and has trained at Los Alamos National Laboratory and the Institute for Advanced Study. He previously held the role of Associate Professor at Icahn School of Medicine at Mount Sinai in the Departments of Medicine and Oncological Sciences, where he was the founding director of the Center for Computational Immunology. His research seeks to quantify the interaction of tumors with the immune system and to predict immune driven evolution of tumors and viruses. His work utilizes a broad range of mathematical tools, from statistical physics and information theory to evolutionary biology. Dr. Greenbaum has established the new program of Computational Immuno-Oncology that brings computational approaches to immunotherapy. It acts as a bridge between the Computational Oncology Service and Immunooncology investigators from across MSK. He is co-leading the Olayan Center for Cancer Vaccines (established in 2024) as Co-Director, Neoantigen Discovery alongside surgeon Dr. Vinod Balachandran. He is spearheading the computational efforts needed to advance personalized vaccines against cancer neoantigens. Over the years Dr. Greenbaum has authored many publications in high-ranking journals, and he holds several awards including a Phillip A. Sharp Award for Innovation in Collaboration from Stand Up to Cancer, a Pershing Square Sohn Prize, and an ASPIRE Award from the Mark Foundation.

Annette Grüters-Kieslich

Prof. Dr. Annette Grüters-Kieslich is a leading pediatrician and internationally recognized expert in pediatric endocrinology and metabolic disorders. She studied medicine in Bochum and Berlin and received her habilitation in pediatrics in 1992. Since 1998, she has served as a tenured professor at Charité – Universitätsmedizin Berlin. As long time director of the Department of Pediatrics, she developed one of Europe' s foremost centers for pediatric medicine. Her research focuses on the genetic causes of rare hormonal and metabolic diseases in children, and she has published more than 100 scientific papers. A member of the German National Academy of Sciences Leopoldina, she has held major leadership roles, including Dean of Charité and Chair of Heidelberg University Hospital. Beyond her academic work, she has been actively engaged in the Eva Luise Köhler Foundation, promoting awareness and research for people with rare diseases.

Richard Houlston

Professor Richard Houlston MD PhD FRS qualified in medicine from Imperial College London and after medical internships took up a research position at the Institute of Cancer Research, where he is now head of the division of genetics and epidemiology. His research focuses on the inherited and somatic genetic architecture of cancer and has made seminal contributions to understanding cancer risk, evolution, and translation of genomics into clinical practice.

Kris Kahlig

Kris Kahlig is Vice President of Biology at Praxis Precision Medicines, a clinical-stage biopharmaceutical company translating genetic insights into therapies for patients with CNS disorders. He leads Discovery across programs in epilepsy, movement disorders, and other undisclosed indications; he also leads an early development clinical program for a Praxis-discovered asset. Prior to this role, Kris served in research and leadership roles at Gilead Sciences; he holds a Ph.D. in Pharmacology from UT Health San Antonio and a B.S. in Chemistry from Angelo State University.

Daniel Krappmann

Daniel Krappmann is the director of the Research Unit 'Signaling and Translation' at the Molecular Targets and Therapeutics Center of Helmholtz Munich. He is Professor at the Biology Faculty of the Ludwig-Maximilians-University in Munich. As PhD student, postdoctoral fellow and junior group leader at the Max-Delbrück-Center for Molecular Medicine in Berlin, he worked on signaling mechanisms in cancer and immune cells. His Research Unit at Helmholtz Munich studies how physiological signaling networks control immune homeostasis and activation and how defective or aberrant signaling leads to immune diseases and cancer. His studies aim to bridge the gap between biomedical research and clinical translation. He has been leading successful drug discovery programs and is involved in the pre-clinical development of anti-cancer therapeutics and new immune modulatory agents.

Georg Marckmann

Georg Marckmann studied medicine and philosophy at the University of Tübingen (Germany) and Public Health at Harvard School of Public Health (Boston, MA, USA). From 1998 to 2010, he was Assistant Professor at the Institute of Ethics and History of Medicine at the University of Tübingen. Since 2010 he is full professor of medical ethics and director of the Institute of Ethics, History, and Theory of Medicine at the Ludwig-Maximilians-University Munich. From 2012 to 2022 he was president of the German Medical Ethics Society. His main research interests include ethical implications of modern biotechnology, ethical issues in end-of-life care, clinical ethics consultation, distributive justice in health care, and public health ethics.

Robert McFarland

Prof Robert (Bobby) McFarland trained in paediatrics, specialising in paediatric neurology, in London and Newcastle upon Tyne, UK. He began his research career studying the molecular consequences and clinical problems associated with mutations of mitochondrial DNA. His research interests have grown to embrace gene discovery, determinants of pathogenicity in mitochondrial disease, longitudinal modelling to assess mitochondrial disease progression and clinical research projects including the development of clinical assessment tools (NMDAS, NPMDS), outcome measures and drug trials.

Prof. McFarland has made significant contributions to mitochondrial research with over 200 peer-reviewed publications, many in high impact journals such as Lancet and NEJM. He has been at the forefront of the introduction of mitochondrial donation, a pioneering technique to prevent transmission of mitochondrial disease, to the UK and leads the neurodevelopmental outcome study to assess the safety and efficacy of this technique. He has championed public and policy engagement to improve public understanding of decision-making in life-limiting diseases affecting children, including the introduction of mitochondrial donation as a reproductive option.

As Director of the NHS Highly Specialised Service for Rare Mitochondrial Disorders he leads a team of clinicians, nurses and allied health professionals dedicated to the diagnosis and management of mitochondrial disease. The close association of his research and clinical work pays dividends for both in facilitating recruitment to the extremely successful national mitochondrial disease patient cohort study (n=2100) and allows rapid translation of research breakthroughs into clinical practice.

Nofar Mor

Dr. Nofar Mor heads the RNA Therapy Unit at Sheba Medical Center, where she leads the development of personalized RNA-based therapies for patients with ultra-rare genetic disorders. Her research focuses on designing and validating antisense oligonucleotides (ASOs) tailored to individual mutations, for clinical purposes. By integrating genomic data, molecular biology, and clinical insight, Dr. Mor's team aims to translate genetic diagnoses into actionable therapies.

Fabian Müller

Prof. Müller obtained his MD in Freiburg, Germany and started his clinical education in Hamburg. Initially with a focus on targeting malignant B cells through BCR recognition, he then did his postdoc with Dr. Ira Pastan, NCI, NIH, on resistance to CD22-targeted therapy in ALL and aggressive lymphoma. After his post-doc he continued the work on B cell targeting in Erlangen, Germany using antibody directed therapies at first and then CAR T cells with a focus on translational concepts including tumor microenvironment and functional aspects of a patient' s immune system. As head of the CAR T cell unit, he pioneers CAR T cell therapies in novel indications including solid tumors and autoimmune diseases.

Michele Proietti

Michele Proietti is a physician-scientist with a strong background in clinical and experimental immunology, human genetics and genomics. His research aims to elucidate how genetic variation in the human genome contributes to increased susceptibility to infections and immune mediated diseases, with a particular focus on genes involved in nucleic acid sensing and type I interferon responses. The research group led by Dr Proietti combines experimental laboratory work with a strong computational component, developing bioinformatic platforms for the management and analysis of multi omics data.

Harald Prüß

Harald Prüss is group leader for Autoimmune Encephalopathies at the German Center for Neurodegenerative Diseases (DZNE) Berlin, consultant neurologist at the Department of Neurology, Director of the department of Experimental Neurology, Charité-Universitätsmedizin Berlin, and Head of the autoimmune encephalitis center and outpatient memory clinic at Charité Berlin, Germany.

He received his medical degree and qualification in Neurology from the Charité-Universitätsmedizin Berlin, as well as the habilitation (venia legendi) in Experimental Neurology and Neurology. The postdoctoral training was performed in Berlin, Germany, and at Harvard Medical School, Boston. His group is active in the field of cellular and molecular neuroscience, in particular in autoimmune contribution to neurological and psychiatric disorders, animal models of antibody-mediated CNS diseases, synaptic changes caused by pathogenic auto-antibodies and nervous system–immune system 'crosstalk' in acute and chronic brain diseases. His studies on the first monoclonal human autoantibodies targeting NMDA and many further brain receptors were a paradigm shift as they proofed the direct pathogenicity of these antibodies and at the same time were starting points for the development of antibody-selective treatment options. His work is published in the top journals of the field, including Cell, Science, Nature Neuroscience, Annals of Neurology and Biological Psychiatry.

V. Koneti Rao

Dr. Rao's current research and clinical activities focus on autoimmune lymphoproliferative syndrome (ALPS) and the related RAS-associated leukoproliferative disorder (RALD). ALPS is due to defects in the FAS gene leading to unusually high numbers of lymphocytes that accumulate in lymph nodes, liver, and spleen, which can lead to enlargement of these organs with an increased lifetime risk of malignant lymphoma.

Paradigms learnt from diagnosing and managing ALPS are applicable to other similar rare immune disorders due to genetic alterations. For example, defects in PI3Kinase, CTLA4, LRBA, MagT1, and STAT3 genes can also lead to nonmalignant lymphoproliferation and autoinflammatory complications due to an aberrant lymphocyte life cycle. These diseases affect both children and adults and have a predilection to hypersplenism and lymphomas.

Many of these novel genetic defects are being identified in patients presenting with cytopenias and lymphoproliferation mimicking ALPS and referred to the NIH Clinical Center. Hence Dr. Rao is interested in studying patients and families with cytopenias, nonmalignant lymphoproliferation, immunodeficiency, and as yet undetermined genetic defects.

Philipp Reautschnig

Philipp Reautschnig, Research Fellow at the University of Tübingen, is a pioneer in RNA biology and gene correction. He earned his degree in Biology at the University of Tübingen, completed his Ph.D. in Biochemistry in 2019, and secured an Innovation Grant for Life Sciences shortly, thereafter, awarded through the Excellence Strategy of the German Federal and State Governments.

His research is focused on the engineering of guide RNAs for gene correction via site-directed RNA base editing. In this endeavor he works right at the interface of bioinformatics and life sciences. His dedication to advance RNA editing technologies towards therapeutic applications has resulted in three filed patents and several high impact publications.

Shady Sayed

During his PhD, Shady Sayed pioneered CRISPR-Cas9-based strategies to identify and precisely correct cancer driver mutations, demonstrating for the first time that CRISPR screening can uncover novel cancer-driving mutations and enable targeted correction of mutations in the KRAS oncogene and TP53 tumor suppressor. As a postdoctoral researcher, he advanced the translational potential of Base Editing by establishing its safety and therapeutic efficacy in a cancer mouse model. He's a Fellow of the National Translational Tandem Program for Gene- and Cell-based Therapies (nTTP-GCT), coordinated by the Biomedical Innovation Academy of the Berlin Institute of Health at Charité and funded by the Federal Ministry of Research and Technology (BMFTR). Recently, he accepted a Junior Group Leader position to pursue Base Editing approaches for lung cancer in patient-derived organoids cooperating with a Clinician Scientist.

Lars Schlotawa

After finishing his medical training Lars Schlotawa did an internship in paediatrics at the Department for Paediatrics and Adolescent Medicine, University Medical Centre Goettingen, Germany. He specialised in paediatric neurology and paediatric metabolic medicine, and is currently working as leading consultant at the same institution. As a postdoc at the University of Cambridge, UK, he did basic research on autophagy. He was graduated to become a professor for paediatric metabolic in 2025. At the same time, he became head of the research area CNS development and neurological diseases at the German Centre for Child and Adolescent Health. His special clinical and scientific expertise is on translational medicine and lysosomal disorder with a focus on Multiple Sulfatase Deficiency.

Markus Schülke

Markus Schuelke, MD, is a tenured full professor at Charité-Universitätsmedizin Berlin. He is a member of the NeuroCure Cluster of Excellence and a Principal Investigator at the Einstein Center for Neurosciences Berlin. He performs his clinical work at the Department of Neuropediatrics at Charité. From an early stage in his career, he has been fascinated by the detective work involved in identifying the genetic basis of rare inherited diseases of the nervous system. His research group has identified numerous novel genetic defects, particularly in mitochondrial and neuromuscular diseases. To help researchers interpret the flood of data coming out of NGS projects, his group has developed bioinformatic tools such as "MutationTaster", "MutationDistiller", and "HomozygosityMapper". Alongside stem cell researcher Alessandro Prigione from Düsseldorf, he is searching for active small molecules to treat mitochondrial disorders using human induced pluripotent stem cell (iPSC)-derived neuronal models. They recently identified sildenafil as a potential treatment for Leigh syndrome, a severe mitochondrial disease. He is now the spokesperson for the DFG Research Unit "Beyond the Exome" and focuses on interpreting non-coding genetic variants in the regulatory genome of patients with congenital myopathies.

Carolin Schwartner-Lehr

Carolin Schwartner-Lehr is a pharmacist (PhD in Pharmaceutical Biology from LMU Munich, specializing in immunology and bio-guided fractionation of immunologically active plant extracts, e.g., Sinupret) and has been working in the pharmaceutical industry for more than 30 years, mainly in Business Development. Since last year, she has been working for Galenicap, an emerging pharmaceutical company specialized in the development of innovative, clinically validated Value-Added Medicines (VAMs) across large, high-impact therapeutic areas. Before she was at Helm Pharmaceuticals for In-Licensing, Hennig Arzneimittel as Head of Business Development, and as well has established the export department. Previously, she worked in business development at Altana Pharma/Nycomed, focusing on respiratory, drug delivery technologies, diabetes, and niche therapeutic areas. At Viatris / Sofotec (divisions of the former Asta Medica), she was responsible for business development for respiration and the Novolizer-a dry powder inhaler. She was responsible as project manager for the third round of S4L a German business plan competition. She started her career in the pharmaceutical industry at Schwarz Pharma, initially in R&D and later in business development for new projects and drug delivery technologies (NPTA).

Julia Skokowa

Julia Skokowa is a Full Professor of Translational Oncology at the Department of Internal Medicine II, University Hospital Tübingen. She also heads the reference laboratory for the European branch of the Severe Congenital Neutropenia International Registry (SCNIR). Her multi-disciplinary team of scientists is dedicated to advancing the research of the pathogenesis and new therapies of inherited pre-leukemia bone marrow failure syndromes and leukemia. They use cutting-edge technologies such as patient-specific iPSCs, gene editing, xenograft models, multi-OMICs and de novo protein design. Her group has achieved significant advances in understanding of pathogenesis of congenital neutropenia and leukemia, cytokine signaling, stem cell differentiation, and gene therapy, including the efficient promoter-based correction of gene mutations in primary hematopoietic stem cells from severe congenital neutropenia patients using gene editing.

Henning Steinhagen

Henning Steinhagen is a serial entrepreneur and company founder with over 25 years of experience and strong track record in Pharma, Biotech and VC. Henning currently acts as CEO and co-founder of the precision medicine focussed biotech: Lario Therapeutics Ltd. He also serves on various international company boards including Epidarex-Exeed, Rinn and Upperton Ltd. Henning previously acted as President R&D at Aptuit and prior to that as Global Head of Drug Discovery at Grünenthal. He previously held leadership roles at Sanofi-Aventis and Bayer. Henning holds a PhD in Chemistry and an MBA degree from IMD in Lausanne.

Marc Thier

Dr. Marc Christian Thier is a physician-scientist and junior group leader of the 'Neural Reprogramming and Clinical Translation' group at the German Cancer Research Center (DKFZ) and HI-STEM in Heidelberg. He is also training as a neurologist at Heidelberg University Hospital. After studying medicine in Bonn, he obtained his MD summa cum laude in 2014 at the Institute for Reconstructive Neurobiology, conducting research on cellular reprogramming. He joined HI-STEM in 2013, where he developed pioneering methods to directly reprogram human somatic cells into neural stem and neural plate border stem cells, protected by several patents. As coordinator of the BMBF-funded NEURO-STEM project and co-PI of the Horizon Europe consortium BEHIND-MS, he aims to translate stem cell technologies into regenerative therapies for neurological diseases.

Anton Ussi

Anton Ussi (MSc) is Operations & Finance Director at EATRIS ERIC, the ESFRI European infrastructure for translational medicine. Joining EATRIS in 2010, he was part of the team responsible for the design and statutory incorporation of the infrastructure, whose membership counts 15 European Member States. In his current role as CEO since 2015, Ussi has a background in technology transfer, with previous history in mechanical engineering and automotive design, and small business administration. He specialises in public-private and public-public collaboration and translational research in medicine. Ussi is also Principal Investigator of REMEDi4ALL, a large EU-funded initiative to develop a European medicines repurposing eco-system.

Martin Wabitsch

Martin Wabitsch MD, PhD, is Professor of Paediatrics at the University of Ulm and Head of the Division of Paediatric Endocrinology and Diabetes and Chairman of the Executive Board of the Center of Rare Diseases at the Ulm University Medical Center.

His main areas of research are the energy homeostasis of the human body, the biology of the human adipocyte, and rare endocrine diseases.

Dr. Wabitsch completed his clinical and experimental training at the Universities in Berlin (Free University) and in Ulm, Germany, as well as in Baltimore (Johns Hopkins Hospital), USA and in Nice (University Sophia Antipolis), France. As an ESPE research fellowship recipient he conducted research on hormone receptors and ligand binding behaviour at the Hagedorn Research Institute, Gentofte, Denmark. Among several prestigious awards he received the ESPE Research Award in 2024.

RESEARCH POSTERS

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1. Deciphering Dup15q syndrome: Integrating the genomic architecture of supernumerary (iso-)dicentric chromosomes with longitudinal deep-phenotyping
   Presenting Author: Sebastian Burkart, Heidelberg University Hospital

2. Patient-specific iPSC-derived hepatic organoids as model for mRNA-based gene therapy in farnesoid-X receptor (FXR) deficiencies
   Presenting Author: Tobias Cantz, Hannover Medical School, Department of Pediatric Gastroenterology and Hepatology, Germany - HiChol

3. Development of a prognostic tool for kidney function decline in NPHP1-related nephronophthisis
   Presenting Author: Mareike Dahmer-Heath, Department of General Pediatrics, University Children's Hospital Münster, Germany - NEOCYST

4. Rare disease diagnostic platform megSAP/GSvar: genome diagnostics in 10.000 cases
   Presenting Author: German Demidov, Institute of Medical Genetics and Applied Genomics, Tübingen, Germany

5. Hilda Biobank supports research on rare diseases affecting children and adolescents
   Presenting Author: Ursula Kern, Medical Center - University of Freiburg, Germany - MyPred

6. PRKN alternative translation initiation mitigates Parkinson's disease onset and identifies a novel therapeutic target
   Presenting Author: Katja Lohmann, Institute of Neurogenetics, University of Lübeck, Germany

7. Standardized transcriptome analysis improves rare disease diagnosis in the pan-European Solve-RD consortium
   Presenting Author: Rebeka Luknárová, Technical University of Munich, Germany

8. Non-invasive detection of allele-specific CRISPR-SaCas9-KKH disruption of TOR1A DYT1 allele in a xenograft mouse model
   Presenting Author: Katia Maalouf, Massachusetts General Hospital and Harvard Medical School, USA & American University of Beirut, Lebanon

9. Dissecting immune dysregulation and dopaminergic stress in BPAN using patient-derived iPSC microglia and neuron models
   Presenting Author: Gamze Özata, Metabolic Biochemistry, Biomedical Center (BMC), Faculty of Medicine, LMU Munich, Germany

10. Towards Modelling of Leber's Hereditary Optic Neuropathy in Retinal Organoid
    Presenting Author: Teresa S. Rogler, University Ulm, Institute for Biophysics, Germany

11. Identification of adverse mitochondrial signatures across Lysosomal Diseases
    Presenting Author: Lina Schmidt, University Medical Center Göttingen, Germany

12. Presymptomatic and Symptomatic Disease Stage-dependent Outcomes of Cerliponase Alfa Treatment in Siblings with CLN2 Disease
    Presenting Author: Angela Schulz, University Children's Hospital, University Medical Center Hamburg-Eppendorf & German Center for Child and Adolescent Health (DZKJ), Hamburg, Germany

13. Longitudinal analysis of anti-drug antibody response against Cerliponase alfa in CLN2 patients
Presenting Author: Lena Marie Westermann, University Children's Hospital, University Medical Center Hamburg-Eppendorf & German Center for Child and Adolescent Health (DZKJ), Hamburg, Germany

14. Ketogenic diet in ARPKD: Early observations of dietary intervention on liver and kidney disease in a murine Pkhd1-knockout model
Presenting Author: Vishnu Soman, Department of Pediatric Kidney, Liver, Metabolic and Neurological Diseases, Hannover Medical School, Germany - NEOCYST

15. Establishing Scanning Electron Microscopy for air-liquid interface cultures of individuals with ciliopathies
Presenting Author: Lea Terbeck, Department of General Pediatrics, University Hospital Münster, Germany - NEOCYST

16. Clinical and Genotypic Spectrum of Twinkle-Related Disorders-Insights From a Multinational Cohort Study
Presenting Author: Zeynep Ünlütürk, Department of Clinical and Experimental Medicine, Neurological Institute, University of Pisa, Italy & Neurology Clinic, University of Health Sciences Kocaeli Derince Training Hospital, Turkey

17. Growth impairment and progressive short stature in Pearson syndrome
Presenting Author: Ayami Yoshimi, Department of Pediatric Hematology and Oncology, Medical Center, Faculty of Medicine, University of Freiburg, Germany - MyPred

18. Retrospective cohort study of complex I defects in Leigh syndrome spectrum Patients and stabilization under idebenone
Presenting Authors: Leopold Zeng & Heinrich Sasse, LMU University Hospital, LMU Munich, Germany - mitoNET

- NETWORK PRESENTATIONS -

19. mitoNET - German Network for Mitochondrial Diseases
Presenting Author: Leopold Zeng, LMU University Hospital, LMU Munich, Germany

20. GAIN - German genetic multi-organ Auto-Immunity Network
Presenting Author: Bodo Grimbacher, Medical Center - University of Freiburg, Germany

21. Speed Translation-Oriented Progress to Treat FSGS - STOP-FSGS
Presenting Author: Maja Lindenmeyer, University Medical Center Hamburg-Eppendorf, Germany

22. Treat-ION: New Therapies for Neurological Ion Channel and Transporter Disorders
Presenting Author: Christina Vossler-Wolf, University Hospital Tübingen, Germany

23. Research for Rare – German Networks on Rare Diseases
Presenting Authors: Katja Franke-Rupp & Corinna Schultheis, LMU University Hospital, LMU Munich, Germany

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Bodo Grimbacher

Medical Center - University of Freiburg
Research network GAIN - Immunodeficiencies

Tobias Huber

University Medical Center Hamburg-Eppendorf

Research network STOP-FSGS - Speed Translation-Oriented Progress to Treat FSGS

Thomas Klopstock

LMU University Hospital, LMU Munich
Research network mitoNET - Mitochondrial diseases

Jens König

University Hospital Münster
NEOCYST - Hereditary cystic kidney diseases

Christian Kratz

Hannover Medical School
Reseach network ADDRess - Disorders with abnormal DNA damage response

Holger Lerche

University Hospital Tübingen
Research network Treat-ION - Neurological ion channel and transporter disorders

Frank Leypoldt

University Hospital Schleswig-Holstein, Kiel
Research network CONNECT-GENERATE - Autoimmune encephalitis

Rebecca Schüle

Heidelberg University Hospital
Research network TreatHSP.net - Hereditary spastic paraplegias

RESEARCH FOR RARE
German Networks on Rare Diseases

Since 2003, the Federal Ministry of Research, Technology and Space (BMFTR) has been funding research for rare diseases by supporting national networks in their efforts to understand the underlying causes of rare diseases and to develop new therapeutic approaches. The networks bring together excellent scientific and clinical capacities in order to create best conditions for systematic basic research and its translation into patient care.

The cooperation of specialists, located nationwide at German university hospitals, overcomes the hurdles for medical research caused by small patient groups and expertise spreaded over different locations. Networking activities enable a structured and coordinated compilation of data as well as evaluation of study results and new findings. The joint effort is intended first and foremost to help patients achieve a more timely and exact diagnosis and to continue developing the best personalized treatments.

For the current funding period 2023-2026, nine research networks have been supported. Together with the consortia financed in previous years, the BMFTR has been investing 165 million Euro in this very successful programme.

A board of speakers serves as decision-making and coordinating body. Elected spokespersons are Prof. Dr. Rebecca Schüle, principal investigator of the TreatHSP.net and Prof. Dr. Thomas Klopstock, principal investigator of the mitoNET research network.

A coordinating office, located in Munich at the Friedrich Baur Institute at the Department of Neurology, LMU University Hospital, LMU Munich, assists the board of speakers to manage joint tasks and to foster collaboration on national and international level.

For further information, please visit the website: www.research4rare.de

ADDRess

A common characteristic of patients with Disorders with Abnormal DNA Damage Response (DADDR) is a highly increased cancer risk. The scientific projects of the research network ADDRess (Translational Research for Persons with Abnormal DNA Damage Response) join forces to improve medical and psychosocial care, early cancer detection, cancer diagnosis and therapy for people with impaired DNA repair.

www.krebs-praedisposition.de/register/address

CONNECT-GENERATE

The established German Network for Research on Autoimmune Encephalitis (GENERATE) contributes significantly to the national networking of therapists, researchers and patients and offers a server-based registry and biomaterials. The research network CONNECT-GENERATE, for the explanation, categorisation and treatment of autoimmune encephalitis and related diseases, is part of this network and aims at improving the diagnosis and therapy for patients with autoimmune encephalitis, a rare but treatable form of brain inflammation. The scientific projects focus on genetic causes, imaging techniques as well as immunological and neuronal network mechanisms. In addition, the efficacy of plasma cell-targeted therapeutics is being investigated in a clinical trial (GENERATE-BOOST). The optimisation and expansion of the patient registry is another important activity.

www.generate-net.de/connect-generate.html

GAIN

The German genetic multi-organ Auto-Immunity Network (GAIN) focuses its research on ultra-rare multi-organ autoimmune diseases. The scientific projects aim to elucidate the underlying molecular and cellular pathomechanisms and to explore potential molecular interventions as therapeutic strategies. Another central goal is to develop standardized approaches for the identification, diagnosis, and treatment of multi-organ autoimmune disorders. To this end, a comprehensive biobank and patient registry have been established, which serve as essential resources for translational research and facilitate the integration of clinical and molecular data. In addition, an epidemiological study involving patient participation will evaluate the quality of life of affected individuals.

www.g-a-i-n.de

HiChol

The research network HiChol focuses on the genetic defects in patients with severe hereditary intrahepatic cholestasis, a disease caused by disrupted bile flow and resulting in progressive liver damage. The aim of the consortium is to better understand these diseases and, in the long term, to provide patients with better predictions of the disease progression and tailored treatment options. The scientific projects analyse the biological consequences of novel mutations in silico and in organoid models and include the establishment of a patient registry allowing investigating genotype-phenotype relationships. Results should provide new insights regarding effective therapies.
www.hichol.hhu.de

mitoNET

The German Network for Mitochondrial Diseases (mitoNET) is dedicated to the heterogeneous group of mitochondrial diseases. The mitoNET builds on a horizontal network of clinical experts in pediatric and neurological university hospitals who enroll patients into a comprehensive registry and, by regular follow-up visits, into a natural history study. DNA, RNA and serum is collected in a central biobank. The basic science experts in the network work inter alia on genomics, transcriptomics, proteomics and complexome profiling. A current project explores the benefit of wearables to assess exercise intolerance in the real life setting. Since the mitoNET has been funded by BMBF for the maximum of 3 periods, its sustainability has been secured by different measures. Most importantly, mitoNET structures have been rolled out to an international level via the EU-funded GENOMIT project including the setup of a global mitochondrial registry. Of note, mitoNET/GENOMIT sites provide excellent infrastructure for clinical studies, and are accordingly involved in most of the current and upcoming therapy trials in mitochondrial diseases.
www.mitonet.org * www.genomit.eu

MyPred

The research network MyPred cares for young individuals with syndromes predisposing to myeloid malignancies, including myelodysplastic syndromes, myeloid leukemias and other myelo-proliferative diseases. The consortium 's overall goal is to understand specific genetic alterations that lead to myeloid neoplasms. This includes the description of still unknown syndromes that predispose to myeloid neoplasms. The research results will be used to help patients by establishing and improving early detection and treatment strategies in a targeted manner. One focus is on the comparison of different aspects of carcinogenesis and the treatment results of individual therapeutic measures in the different syndromes.
www.mypred.de

NEOCYST

Hereditary cystic kidney diseases are major causes of chronic kidney failure in children. The Network for Early Onset Cystic Kidney Disease (NEOCYST) was founded to advance understanding of these conditions by investigating the epidemiology, the phenotypic spectrum, the underlying genetic and pathophysiological mechanisms and outcomes. Through shared databases and coordinated research, the network fosters collaboration and knowledge exchange across institutions. A key element is active patient involvement, ensuring that research priorities, data collection, and care strategies reflect patient and family perspectives. Current efforts aim to improve disease prediction, identify targets for future therapies, and enhance individualized care and counselling.
www.neocyst.de

STOP-FSGS

The research network STOP-FSGS (Speed Translation-Oriented Progress to Treat FSGS) concentrates on the idiopathic focal segmental glomerulosclerosis (FSGS). The focus is on understanding the molecular basis and developing new diagnostic and therapeutic approaches to treat FSGS. Previous work has helped to reveal underlying mechanisms of the disease by identifying new disease-causing genes, biomarkers and signaling pathways. The goal of the network is to combine the identified disease mechanisms, biomarkers and therapeutic approaches, develop them further and translate them into improved diagnostics and therapy for patients. In addition, STOP-FSGS will provide a central information, contact and exchange platform for FSGS for physicians, scientists and patients in Germany.
www.research4rare.de/forschungsverbuende/stop-fsgs/

TreatHSP.net

The research network Treat-HSP.net (Translational Research in Hereditary Spastic Paraplegias) emphasises on hereditary spastic paraplegias (HSP), which are highly heterogeneous neurodegenerative disorders. The network concentrates its translation-oriented research approach on pathophysiological key pathways of HSP. To make substantial progress towards implementation of novel therapies, TreatHSP.net generates a shared infrastructure that provides access to clinical data, biological samples and OMICS data. Scientific projects develop and validate outcome parameters for clinical trials, identify shared pathways and novel therapeutic targets and prioritise drug-repurposing strategies to evaluate novel therapeutic approaches in preclinical trials.
www.treathsp.net

Treat-ION

The research network Treat-ION specialises on rare neurological ion channel and transporter disorders. The focus of the network is on improving diagnostics and establishing individualised therapeutic treatment options for these diseases. Scientific projects concentrate on molecular and cellular pathophysiology, the effects of mutations on channel functions and the mechanisms of specific drugs. In explorative n-of-1 studies, research results in clinical subprojects are to be transferred into practice. A translational process accompanies the therapeutic use, in particular of already licensed and available drugs (drug repurposing). An established registry on ion channel diseases supports the work and results will be delivered to patients through a structured molecular therapeutic board.
www.treat-ion.de

GERMAN CENTER FOR CHILD AND ADOLESCENT HEALTH (DZKJ)

The German Center for Child and Adolescent Health (DZKJ) is dedicated to addressing the specific health needs of children and adolescents in both research and clinical care. By doing so, it aims to sustainably advance the field of pediatric and adolescent medicine. Established by the German federal government in 2024, the DZKJ is one of eight German Centers for Health Research. It provides a unique platform for interdisciplinary collaboration, enabling scientists to explore the causes of both common and rare childhood diseases and to develop cutting-edge therapies and prevention strategies. By uniting leading university hospitals, academic institutions, and renowned research organizations - such as the Max Planck Society, Fraunhofer-Gesellschaft, Helmholtz and Leibniz Centers - the DZKJ brings together exceptional expertise and enables the rapid translation of new scientific findings into clinical practice.

The DZKJ is contributing to the event by the following talks:

Application of CRISPR base editors in innovative cancer therapies
Shady Sayed – Technical University Dresden

Ligand-receptor interaction as a therapeutic target in rare endocrinological diseases
Martin Wabitsch, Ulm University Hospital

Therapies for multiple sulfatase deficiency – drug repurposing in ultra-rare genetic diseases
Lars Schlotawa, University Medical Center Göttingen

Sildenafil as a new therapy for maternally inherited Leigh syndrome
Markus Schülke, Charité – Universitätsmedizin Berlin

DZKJ – Deutsches Zentrum für Kinder- und Jugendgesundheit

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