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With involvement of formerly BMBF-funded Networks.

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AUDIENCE

Physicians & scientists interested in therapies of rare diseases
Young investigators are particularly welcome!

Limited number of travel grants available.

ABSTRACTS INVITED

Venue

Evangelische Akademie Tutzing
Schlossstr. 2+4, 82327 Tutzing, Lake Starnberg

Organisation/ Registration

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Registration: www.rare2care2018.wordpress.com
until March 30, 2018

CONFIRMED SPEAKERS

- ▶ **ODILE BOESPFLUG-TANGUY, Centre Hospitalier Universitaire Robert Debré, Paris (France):**
Genetic disorders of the white matter: from animal models to patients for the development of diagnosis and therapies
- ▶ **BILL GAHL, Undiagnosed Diseases Program, NIH, Washington (USA)**
Clinical challenges in patients with RD & international networks
- ▶ **ILSE GANTOIS, McGill University, Montreal (Canada)**
Metformin ameliorates core deficits in a mouse model of fragile X syndrome
- ▶ **VOLKMAR GIESELMANN, University of Bonn (Germany)**
New insights in the therapy of lysosomal storage diseases
- ▶ **DAN KASTNER, Intramural Research, NIH, Washington (USA)**
Rare Autoinflammatory Disorders
- ▶ **ROBERT KLETA, UCL London (Great Britain)**
Rare diseases of the kidney to search for novel therapeutic concepts
- ▶ **PETRA KAUFMANN, National Center for Advancing Translational Sciences (NCATS), Washington (USA)**
- ▶ **ALEIXO MUISE, The Hospital for Sick Children, Toronto (Canada)**
New genetic defects in very early onset inflammatory bowel diseases
- ▶ **JOSEF PENNINGER, Institute of Molecular Biotechnology, Vienna (Austria)**
Innovation in Biomedicine by virtue of analysis of patients with RD
- ▶ **ANITA RAUCH, University of Zürich, Zürich (Switzerland)**
New insights into the etiology of epileptic encephalopathy
- ▶ **LAURA DE ROSA, University of Modena and Reggio Emilia, Modena (Italy)**
Epidermal stem cell-mediated cell and gene therapy for Junctional Epidermolysis Bullosa
- ▶ **SCOTT SNAPPER, Boston Children's Hospital, Boston (USA)**
Cytokine defects in children with rare inflammatory bowel diseases
- ▶ **NICO WULFFRAAT, University Medical Center Utrecht, Utrecht (The Netherlands)**
Early Anakinra therapy for Systemic Juvenile Idiopathic Arthritis (SJIA): standard of care?

Presentations from the
Research Networks on Rare Diseases in Germany



From Rare to Care III
April 11 - 13, 2018

Tutzing, Starnberger See (Bavaria)



SCIENTIFIC ORGANISATION:



RESEARCH FOR RARE
Forschung für seltene Erkrankungen



Federal Ministry
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