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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

Further information – see online: www.research4rare.de

Organisation/ Registration
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Registration: www.rare2care2014.wordpress.com
until September 10 th, 2014

CONFIRMED SPEAKERS

- **RONALD FRANK, Leibniz Institute for Molecular Pharmacology, Berlin (Germany):**
  EU-OPENSCREEN Research Infrastructure
- **WILLIAM A. GAHL, Undiagnosed Diseases Program, NIH, Washington D.C. (USA):**
  Clinical diagnosis of rare diseases
- **RICHARD GREGORY, Head of Genzyme R&D Center (USA):**
  Innovation in rare disease R&D
- **MICHAEL HAYDEN, President of Global R&D and Chief Scientific Officer ,Teva Pharmaceutical Industries (Israel):**
  From rare diseases to treatment of common disorder
- **DAN KASTNER, Director of Intramural Research, NIH, Washington D.C.(USA):**
  HorrorAutoinflammaticus: The Expanding Spectrum of Systemic Autoinflammatory Diseases
- **PETRA KAUFMANN, Director of the Division of Clinical Innovation at NIH`s National Center for Advancing Translational Sciences (NCATS), NIH, Washington D.C. (USA):**
  NCATS - Catalizing Translational Innovation
- **JÜRGEN KNOBLICH, Institute of Molecular Biotechnology, Vienna (Austria):**
  Using 3D culture organoid models to model rare brain disorders
- **HANNS LOCHMÜLLER, Institute of Genetic Medicine, Newcastle University, Newcastle upon Tyne (UK):**
  RD-Connect
- **JAMES LUPSKI, Baylor College of Medicine, Houston (USA):**
  Genome-wide diagnosis of rare diseases
- **SHOUKHRAT MITALIPOV, Oregon Health & Science University, Portland (USA):**
  Germline gene therapy of mitochondrial diseases
- **JOSEF PENNINGER, Institute of Molecular Biotechnology, Vienna (Austria):**
  Model system for diagnosis and therapies of rare diseases
- **OLE B. SUHR, Umea University, Umea (Sweden):**
  Development of treatment of transthyretin amyloidosis, safety and efficacy of Si-RNA treatment
- **TAKANORI TAKEBE, Department of Regenerative Medicine, Yokohama University, Yokohama (Japan):**
  Pluripotent stem cells and organ reconstitution for rare diseases

SCIENTIFIC ORGANISATION:

RESEARCH FOR RARE
Forschung für seltene Erkrankungen

IMBA Institute of Molecular Biotechnology of the Austrian Academy of Sciences

Federal Ministry of Education and Research

LMU Ludwig-Maximilians-Universität München

Care-for-Rare Foundation
Stiftung für Kinder mit seltenen Erkrankungen

Wed, October 8th 2014

1.00 p.m.	Opening Registration

Session 1

2.00 p.m.	Welcome Address
2.15 p.m.	Opening Ceremony: Roundtable Discussion: Unmet needs in rare diseases
3.15 p.m	William A. Gahl: Clinical diagnosis of rare diseases
3. 45 p.m.	James Lupski: Genome-wide diagnosis of rare diseases
4.15 p.m.	Coffee Break

Session 2

4.30p.m.	Ronald Frank: EU-OPENSCREEN Research Infrastructure
5.00 p.m.	Richard Gregory: Innovation in rare disease R&D
6.00 p.m.	Herrenchiemsee Castle - inspirations from history
7.30 p.m.	Dinner

Thu, October 9th 2014

Session 3

9.00 a.m.	Dan Kastner: Horror Autoinflammaticus: The Expanding Spectrum of Systemic Autoinflammatory Diseases
9.30 a.m.	Highlights German Rare Disease Consortium AID-NET
9.50 a.m.	Highlights German Rare Disease Consortium PID-NET
10.15 a.m.	Takanori Takebe: Pluripotent stem cells and organ reconstitution for rare diseases
10.45 a.m.	Presentation of selected abstracts
11.15 a.m.	Coffee break

Thu, October 9th 2014

Session 4

11.30 a.m.	Michael Hayden: From rare diseases to treatment of common disorder
12.00 a.m.	Petra Kaufmann: NCATS - Catalyzing translational innovation
12.30 a.m.	Highlights German Rare Disease Consortium GALENUS
12.50 a.m.	Highlights German Rare Disease Consortium CARPuD2
1.10 p.m.	Lunch

Session 5

2.15 p.m.	Shoukrat Mitalipov: Germline gene therapy of mitochondrial diseases
2.45 p.m.	Highlights German Rare Disease Consortium mitoNET
3.05 p.m.	Highlights German Rare Disease Consortium MNDnet
3.25 p.m.	Highlights German Rare Disease Consortium HOPE
3.45 p.m.	Coffee break & dedicated poster session

Session 6

4.45 p.m.	Jürgen Knoblich: Using 3D culture organoid models to model rare brain disorders
5.15 p.m.	Highlights German Rare Disease Consortium IonNeurONet
5.35 p.m.	Presentation of selected abstracts
6.00 p.m.	End Day 2
8.00 p.m.	Speaker‘s Dinner

Fri, October 10th 2014

Session 7

9.00 a.m.	Hanns Lochmüller: RD-Connect
9.30 a.m.	Highlights German Rare Disease Consortium Imprinting
9.50 a.m.	Highlights German Rare Disease Consortium FACE
10.10 a.m.	Ole B. Suhr: Development of treatment of transthyretin amyloidosis, safety and efficacy of Si-RNA treatment
10.40 a.m.	Josef Penninger: Model system for diagnosis and therapies of rare diseases
11.00 a.m.	Coffee break

Session 8

11:15 a.m.	Highlights German Rare Disease Consortium TranSaRNet
11.35 a.m.	Highlights German Rare Disease Consortium NIRK
11.55 a.m.	Highlights German Rare Disease Consortium EB-Net
12.15 a.m.	Highlights German Rare Disease Consortium GERAMY
12.45 a.m.	Presentation of selected abstracts
1.00 p.m.	Summary, Final Remarks, Farewell
1.15 p.m	Lunch
2.15 p.m.	Meeting at the pier for return to Prien
3.00 p.m.	Bus return to Munich