RESEARCH FOR RARE
Forschung für seltene Erkrankungen

Else Kröner-Forschungskolleg LMU
Seltene Erkrankungen des Immunsystems

10 for RARE Academy Research Exchange

Summer School:
Translational research for rare diseases

June, 21 -22, 2017
Ludwig-Maximilians-Universität, Pettenkoferstraße 12, München
Optional: Workshop „Scientific proposal writing“ (bioXpress, June, 23)

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DAAD

ELSE KRÖNER-FRESENIUS-STIFTUNG
Forschung fördern. Menschen helfen.

The summer school *Translational research for rare diseases* gives young researchers and clinicians the opportunity to learn from experienced colleagues and present their own results in an international and interdisciplinary group. Main Topics will be in the field of innovative genetics, translational research and research ethics. The seminar will be complemented by a workshop on scientific proposal writing (23 June), which can be chosen in addition to the thematic part.

Participants:
The summer school is organised for members of the following networks: “Research for Rare” (BMBF); “Ten for Rare” (DAAD) & “Rare diseases of the immune system” (EKFS Clinical Research School Munich). Scientists not belonging to the above mentioned networks can participate upon request.

Participants are asked to hand in a poster abstract and present the poster during the seminar. From the submitted abstracts, participants will be selected for short talks. The summer school will be held in English.

Registration:
Please register with a short bio and poster abstract under info@research4rare.de.

Organisation:
RESEARCH FOR RARE / TEN FOR RARE / EKFS Clinical Research School Munich
Dr. von Haunersches Kinderspital, Klinikum der Universität München, Lindwurmstraße 4, 80337 München
Responsible: Prof. Dr. C. Klein

Program

Wednesday, June 21, 2017

18:00 – 18:15 **Welcome**
Prof. Christoph Klein, Dr. von Hauner Childrens Hospital, University Hospital Munich (LMU)

18:15 – 18:45 **Medical, scientific and ethical challenges with respect to translational research in rare diseases**
Prof. Christoph Klein

18:45 – 20:00 **Poster presentation**

20:00 **Dinner & get together**

Thursday, June 22, 2017

PART I: GENOME SEQUENCING

09.00 – 09:45 **Ethical aspects of genomics in translational research**
Prof. Winkler, EURAT working group "Ethical and Legal Aspects of Whole Genome Sequencing", Heidelberg University Hospital

09:45 – 10:30 **Genome sequencing for rare diseases –latest developments**
Prof. André Reis, Institute for Human Genetics, University Hospital Nürnberg-Erlangen

10:30 – 11:00 **Coffee break**

11:00 – 12.30 **Interactive workshop**: Regulatory aspects of genomics in rare diseases

12:30 - 13.30 **Lunch break**

PART II: FROM RESEARCH TO THERAPY

13:30 – 14:00 **Intraventricular enzyme replacement therapy in CLN2**
Dr. Angela Schulz, University Clinics Hamburg

14:00 – 14:30 **The kelch surprise: monoallelic mutations in the translation initiation codon of KLHL24 cause skin fragility**
Prof. Christina Has, University Clinics Freiburg

14:30 – 15:00 **Coffee break**

15:00 – 15:30 **Diagnosing, understanding and treating primary immunodeficiencies – "from bed to bench and back"**
Prof. Raz Somech, Faculty of Medicine, Tel Aviv University

15:30 – 16:00 **Tumor immunotherapy: Giving T cells a helping hand**
Prof. Stefan Endres, University Hospital Munich (LMU)

16:00 – 16:50 **Short talks by participants**

16:50 – 17:00 **Concluding remarks**

17:00 **Departure**