

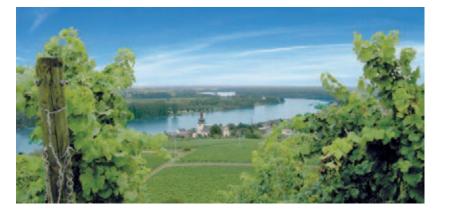


## **BEST WESTERN Wein- und Parkhotel Nierstein**

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# 14th International Postgraduate Course on Lysosomal Storage Disorders

Nierstein (Mainz), Germany May 31 - June 4, 2015



Arrangement by Prof. Julia B. Hennermann and Prof. Michael Beck, Department of Pediatric and Adolescent Medicine Johannes Gutenberg-University of Mainz, Germany

Supported by an educational grant from Shire HGT, a business unit of Shire plc IMS is providing logistic support for the course.





## 14th International Postgraduate Course on Lysosomal Storage Disorders

Applicants should be physicians with some years of clinical experience, should be seeing patients and have an interest in LSDs.

Applicants with active research projects will have priority.

The number of participants will be limited to approximately twenty five.

The faculty consists of experienced lecturers in different specialities.

The following topics will be addressed during the

- ✓ Cell biology and pathoyphysiology of lysosomes
- ✓ Genetics
- ✓ Diagnosis and Treatment
- √ Fabry Disease
- ✓ Gaucher Disease
- ✓ MPS syndromes
- ✓ Pompe disease

#### ✓ Leukodystrophies

- ✓ Other LSDs
- ✓ Patients' Organisations

A good command of the English language is necessary for active participation.

Questions regarding the scientific content of the course should be addressed to:

#### Professor Michael Beck or Professor Julia Hennermann

Pediatric Department, Johannes Gutenberg-University of Mainz michael.beck@unimedizin-mainz.de

Questions regarding logistics can be addressed to:

#### IMS GmbH, Sabine Michels

Tel: +49-6131/17-6552 • Fax: +49-6131/17-6608 ims@um-mainz.de

### **FACULTY**

#### **Prof. Michael Beck**

Department of Pediatric and Adolescent Medicine University Medical Center, Johannes Gutenberg-University of Mainz, GERMANY

### Dr. David J. Begley

Centre for Neuroscience Research, King's College London, UNITED KINGDOM

#### Prof. Andreas Gal

Institute for Human Genetics, University of Hamburg, GERMANY

#### Prof. Jutta Gärtner

Center for Child and Adolescent University Medical Center Göttingen **GERMANY** 

#### Prof. Volkmar Gieselmann

Institute for Physiological Chemistry Rheinische Friedrich-Wilhelms-Universität Bonn, GERMANY

#### Prof. Roberto Giugliani Medical Genetics Service.

Hospital de Clínicas de Porto Alegre, BRAZIL

#### Prof. Hans H. Goebel

Department of Neuropathology. Charité - Universitätsmedizin Berlin Berlin, GERMANY

#### Prof. Julia B. Hennermann

Department of Pediatric and Adolescent Medicine University Medical Center, Johannes Gutenberg-University of Mainz, GERMANY

#### Prof. Christoph Kampmann

Department of Pediatric and Adolescent Medicine University Medical Center, Johannes Gutenberg-University of Mainz, GERMANY

#### Dr. Nesrin Karabul

Department of Pediatric and Adolescent Medicine University Medical Center, Johannes Gutenberg-University of Mainz, GERMANY

#### Mrs. Christine Lavery

The Society for Mucopolysaccharide Diseases, Amersham, Buckinghamshire UNITED KINGDOM

#### Dr. Eugen Mengel

Department of Pediatric and Adolescent Medicine University Medical Center. Johannes Gutenberg-University of Mainz, GERMANY

#### Prof. Ans van der Ploed

Center for Lysosomal and Metabolic Diseases, Erasmus MC University Medical Center, NETHERLANDS

**Prof. Paul Saftig** Biochemical Institute. Christian-Albrechts-Universität Kiel, GERMANY

#### Prof. Konrad Sandhoff LIMES, c/o Kekulé-Institut f.

Organische Chemie und Biochemie Rheinische Friedrich-Wilhelms-Universitaet Bonn, GERMANY

#### Prof. M. Scarpa, MD, PhD Dr. Horst Schmidt Kliniken

Wiesbaden GERMANY

#### Prof. Kurt Ullrich

International Center for Lysosomal Disorders University Medical Center Hamburg GERMANY

### Dr. Marie T. Vanier

Laboratoire Fondation Gillet Mérieux Lyon-East Hospital Bron, FRANCE

### PD Catharina Whybra-Trümpler

Department of Pediatric and Adolescent Medicine University Medical Center, Johannes Gutenberg-University of Mainz, GERMANY

## 14th International Postgraduate Course on Lysosomal Storage Disorders

Nierstein (Mainz) May 31 - Ju

Sunday, May 31		
Arrival		
Monday, June 1		
3.30 – 8.45	Course introduction Michael Beck	
3.45 – 9.00	General overview Julia B. Hennermann	
9.00 – 10.15	Cellular pathophysiology of lysosomal storage disorders Volkmar Gieselmann	
0.15 - 10.30	Coffee break	
0.30 – 11.15	Defects of the lysosomal membrane Paul Saftig	
1.15 – 12.15	Genetic principles of lysosomal storage disorders: A practical session on Mendelian genetics and pedigree analysis Andreas Gal	
2.15 - 13.30	Lunch	
3.30 – 14.30	Ceroid lipofuscinosis Hans H. Goebel	
4.30 – 15.15	The Blood brain barrier and lysosomal storage disorders David J. Begley	
5.15 - 15.30	Coffee break	
5.30 – 17.45	Group work: Case reports (Presentation of all case reports on the following day)	
8.30	Welcome Dinner	
Tuesday, June 2		
9.00 – 10.00	Sphingolipids, sphingolipidoses and the lipid phase problem Konrad Sandhoff	
0.00 - 11.00	Sphingolipidoses Marie T. Vanier	
1.00 – 11.15	Coffee break	
1.15 – 12.15	Fabry disease Nesrin Karabul	
2.15 – 13.30	Lunch	

y 31 - June 4, 2015			
15.45 – 17.15	Presentation and discussion of case reports (5 minutes per each case)		
17.15 – 17.30	Coffee break		
17.30 – 19.00	Presentation and discussion of case reports (cont.)		
20.00	Dinner		
Wednesday, June 3			
9.00 - 9.45	Acid lipase deficiency Michael Beck		
9.45 – 11.00	Mucopolysaccharidoses and glycoproteinoses Roberto Giugliani		
11.00 - 11.15	Coffee break		
11.15 – 12.15	Treatment of mucopolysaccharidoses Julia B. Hennermann		
12.15 – 13.00	Stem cell transplantation in lysosomal storage disorders Kurt Ullrich		
13.00	Lunch Free afternoon		
Thursday, June 4			
9.00 – 10.00	Neurodegeneration in lysosomal storage disorders: Treatment today and tomorrow Maurizio Scarpa		
10.00 - 11.00	Leukodystrophies Jutta Gärtner		
11.00 - 11.15	Coffee break		
11.15 – 12.15	Pre- and postnatal presentation of lysosomal storage disorders Catharina Whybra-Trümpler		
12.15 – 13.30	Lunch		
10.00 14.00	Drinianles of laboratory diagnosis in		

13.30 – 14.30 Prinicples of laboratory diagnosis in

14.30 - 15.00 Neonatal screening for lysosomal

storage disorders

Roberto Giugliani

15.00 - 15.45 Cardiac manifestation in lysosomal

storage disorders

storage disorders

Christine Lavery

Farewell Dinner

15.45 - 16.00 Coffee break

20.00

Departures

Friday, June 5

Christoph Kampmann

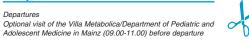
16.00 - 17.00 Role of patient support groups in lysosomal

Adolescent Medicine in Mainz (09.00-11.00) before departure

lysosomal storage disorders

Marie T. Vanier, Michael Beck

Tuesday, June 2		
9.00 – 10.00	Sphingolipids, sphingolipidoses and the lipid phase problem Konrad Sandhoff	
10.00 - 11.00	Sphingolipidoses Marie T. Vanier	
11.00 – 11.15	Coffee break	
11.15 – 12.15	Fabry disease Nesrin Karabul	
12.15 - 13.30	Lunch	
13.30 – 14.30	Gaucher disease Eugen Mengel	
14.30 – 15.30	Pompe disease Ans van der Ploeg	
15.30 – 15.45	Coffee break	



## **Application form**

14th International Postgraduate Course on Lysosomal Storage Disorders May 31 - June 4, 2015

Position Department Hospital Hospital address (for correspondence) Email (please print) Short description of your education/experience Years of training after medical school Clinical research Laboratory research Career intentions Please give a short description of why you would like to attend this course Please give a brief outline of what your five minutes case report will be about

The application form should be sent – not later than April, 29 – to:

IMS GmbH · Sabine Michels Langenbeckstraße 1, D-55131 Mainz, Tel. +49 6131/17-6552. Fax +49 6131/17-6608 ims@um-mainz.de

Name

Applicants will be informed by Professor Beck about the outcome before May 10, 2015.