

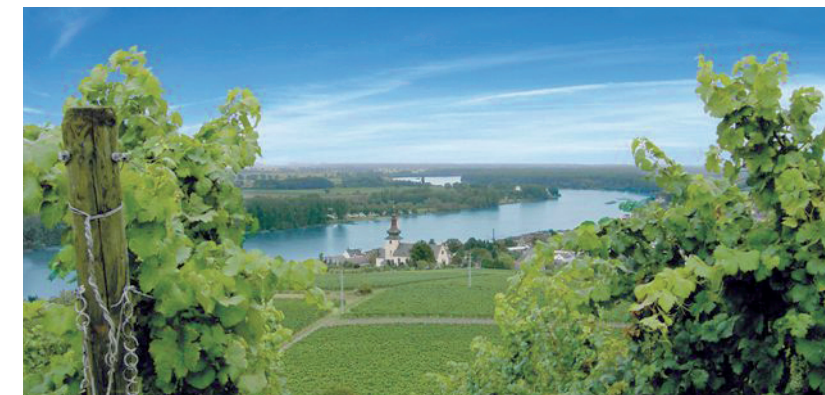


**BEST WESTERN Wein- und Parkhotel Nierstein**

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12<sup>th</sup> International Postgraduate Course on  
**Lysosomal Storage Diseases**

Nierstein (Mainz), Germany  
June 3 - 7, 2013



Arrangement by Prof. Michael Beck,  
Pediatric Department  
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.



12<sup>th</sup> International Postgraduate Course on  
Lysosomal Storage Disorders (LSDs)

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 course:

- Cell biology of lysosomes
- Epidemiology
- Genetics
- Diagnosis
- Fabry Disease
- Gaucher Disease
- MPS syndromes

- Ceroid-ipofuscinoses
- Other LSDs
- Patient 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**  
Pediatric Department, Johannes Gutenberg-University of Mainz  
Phone: +49 6131/ 17-2389/17-5754,  
Email: michael.beck@unimedizin-mainz.de

Questions regarding logistics can be addressed to:

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

FACULTY

**Prof. Michael Beck**  
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Johannes Gutenberg-University of  
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**Dr. David J. Begley**  
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King's College London,  
UNITED KINGDOM

**Prof. Andreas Gal**  
Institute for Human Genetics,  
University of Hamburg,  
GERMANY

**Prof. Roberto Giugliani**  
Medical Genetics Service,  
Hospital de Clinicas de Porto Alegre,  
BRAZIL

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**Dr. Eugen Mengel**  
Pediatric Department,  
University Medical Center,  
Johannes Gutenberg-University of  
Mainz, GERMANY

**Prof. Ans van der Ploeg, MD, PhD**  
Center for Lysosomal and Metabolic  
Diseases, Erasmus MC University  
Medical Center,  
NETHERLANDS

**Prof. Paul Saftig**  
Biochemical Institute,  
Christian-Albrechts-Universität Kiel,  
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**Prof. Konrad Sandhoff**  
LIMES, c/o Kekulé-Institut f.  
Organische Chemie und Biochemie  
Rheinische Friedrich-Wilhelms-  
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**Prof. M. Scarpa, MD, PhD**  
Dept. of Paediatrics,  
University of Padova,  
ITALY

**Prof. Kurt Ullrich**  
Children's Hospital  
University of Hamburg,  
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**Dr. Marie T. Vanier**  
Laboratoire Fondation Gillet Mérieux,  
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**Dr. Catharina Whybra**  
Pediatric Department,  
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Johannes Gutenberg-University of  
Mainz, GERMANY

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Nierstein (Mainz) June 3 - 7, 2013

Sunday, June 2

Arrivals – all participants and lecturers will be met at the Frankfurt airport. Dinner will be arranged at the hotel.

Monday, June 3

08.15 – 8.45 Course introduction  
*M. Beck*  
General overview of LSDs

08.45 – 09.30 Cell biology of lysosomes  
*V. Gieselmann*

09.30 – 10.15 Pathophysiology in LSDs  
*A. van der Ploeg*

10.15 – 10.30 Coffee break

10.30 – 11.15 Genetics of LSDs  
*A. Gal*

11.15 - 12.00 Defects of the lysosomal membrane  
*P. Saftig*

12.00 – 13.15 LUNCH

13.15 – 14.00 Ceroid-Lipofuscinosis  
*H.H. Goebel*

14.00 – 15.00 The blood brain barrier in LSDs  
*D. Begley*

15.00 - 15.15 Coffee break

15.15 – 17.45 Group work: case reports  
The next day all cases will be presented

18.30 – Welcome Dinner

Tuesday, June 4

08.30 -10.00 Sphingolipids, sphingolipidoses  
and the lipid phase problem  
*K. Sandhoff*

10.00 – 11.00 Fabry disease: Clinical manifestation  
*N. Karabul*

11.00 - 11.15 Coffee break

11.15 – 12.15 Gaucher disease non-neuronopathic form:  
Clinical manifestation and treatment  
*E. Mengel*

12.15 – 13.15 LUNCH

13.15 – 14.00 Stem cell transplantation in LSDs  
*K. Ullrich*

14.00 – 15.15 Pathophysiology of neurodegeneration  
in LSDs  
*M. Scarpa*

15.15 – 15.45 Coffee break

15.45 - 17.30 Presentation and discussion of  
case reports from group work  
5 min for each case !

17.30 - 17.45 Coffee break

17.45 - 19.30 Case reports cont.

20.00 Dinner

Wednesday, June 5

08.30 - 09.15 CNS manifestation of Gaucher disease  
*E. Mengel*

9.15 - 10.00 Leukodystrophies  
*M. Vanier*

10.00 - 10.15 Coffee break

10.15 - 11.00 Neurodegeneration in LSD:  
Treatment today and tomorrow  
*M. Scarpa*

11.00 - 12.00 Pompe disease  
*A. van der Ploeg*

12.00– 12.30 Pre- and postnatal presentation of LSDs  
*C. Whybra*

13.00 Lunch  
Afternoon at free disposal  
(Optional participation at excursion)

Thursday, June 6

08.30 – 10:00 Mucopolysaccharidoses (MPS) and  
glycoproteinoses  
*R. Giugliani*

10.00 - 10:15 Coffee break

10.15 – 11.30 Sphingolipidoses  
*M. Vanier*

11.30 – 12.30 Treatment of mucopolysaccharidoses  
*C. Lampe*

12.30 – 13.45 LUNCH

13.45 – 14.30 Principles for laboratory diagnosis in LSDs  
*M. Vanier, M. Beck*

14.30 – 15.00 Newborn screening  
*R. Giugliani*

15.00 - 15.45 Cardiac manifestations in LSDs  
*C. Kampmann*

15.45 – 16.00 Coffee break

16.00 – 17.30 Role of patient support groups in LSDs  
*C. Lavery*

19.00 - FAREWELL DINNER

Friday, June 7

Departures  
Optional visit to Villa Metabolica/Department of Pediatrics in  
Mainz (09.00-11.00) before departure

Name

Position

Department

Hospital

Hospital address (for correspondence)

Phone

Fax

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 May 3, 2013 – to:

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

Applicants will be informed by Professor Beck about the outcome before May 13, 2013.

