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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 course:

- ✓ Cell biology and pathophysiology 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
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Dr. David J. Begley
Centre for Neuroscience Research,
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Prof. Andreas Gal
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University of Hamburg,
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Center for Child and Adolescent
Health
University Medical Center Göttingen
GERMANY

Prof. Volkmar Gieselmann
Institute for Physiological Chemistry
Rheinische Friedrich-Wilhelms-
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Medical Genetics Service,
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Prof. Ans van der Ploeg
Center for Lysosomal and Metabolic
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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-
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Prof. M. Scarpa, MD, PhD
Dr. Horst Schmidt Kliniken
Wiesbaden
GERMANY

Prof. Kurt Ullrich
International Center for Lysosomal
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University Medical Center Hamburg
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Dr. Marie T. Vanier
Laboratoire Fondation Gillet Mérieux
Lyon-East Hospital
Bron, FRANCE

PD Catharina Whybra-Trümpier
Department of Pediatric and
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University Medical Center,
Johannes Gutenberg-University of
Mainz, GERMANY

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Nierstein (Mainz) May 31 - June 4, 2015

Sunday, May 31

Arrival

Monday, June 1

8.30 – 8.45 Course introduction
Michael Beck

8.45 – 9.00 General overview
Julia B. Hennermann

9.00 – 10.15 Cellular pathophysiology of lysosomal
storage disorders
Volkmar Gieselmann

10.15 – 10.30 Coffee break

10.30 – 11.15 Defects of the lysosomal membrane
Paul Saftig

11.15 – 12.15 Genetic principles of lysosomal storage
disorders:
A practical session on Mendelian genetics
and pedigree analysis
Andreas Gal

12.15 – 13.30 Lunch

13.30 – 14.30 Ceroid lipofuscinosis
Hans H. Goebel

14.30 – 15.15 The Blood brain barrier and lysosomal
storage disorders
David J. Begley

15.15 – 15.30 Coffee break

15.30 – 17.45 Group work: Case reports
(Presentation of all case reports on
the following day)

18.30 Welcome Dinner

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

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-Trümpier

12.15 – 13.30 Lunch

13.30 – 14.30 Principles of laboratory diagnosis in
lysosomal storage disorders
Marie T. Vanier, Michael Beck

14.30 – 15.00 Neonatal screening for lysosomal
storage disorders
Roberto Giugliani

15.00 – 15.45 Cardiac manifestation in lysosomal
storage disorders
Christoph Kampmann

15.45 – 16.00 Coffee break

16.00 – 17.00 Role of patient support groups in lysosomal
storage disorders
Christine Lavery

20.00 Farewell Dinner

Friday, June 5

Departures
Optional visit of the Villa Metabolica/Department of Pediatric and
Adolescent Medicine in Mainz (09.00-11.00) before departure

Application form
14th International Postgraduate Course on
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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 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

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

