11th 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:

IMS GmbH, Sabine Michels
Tel: +49-6131/17-6552 • Fax: +49-6131/17-6608
ims@um-mainz.de

FACULTY

Prof. Michael Beck
Pediatric Department,
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. Volkmar Gieselmann
Institute for Physiological Chemistry,
University of Bonn,
GERMANY

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

Prof. Hans H. Goebel
Department of Neuropathology,
Johannes Gutenberg-University
Medical Center, Mainz,
GERMANY

Prof. Christoph Kampmann
Pediatric Department,
University Medical Center,
Johannes Gutenberg-University of Mainz,
GERMANY

Prof. Edwin Kolodny
Department of Neurology, New York
University School of Medicine,
New York, USA

Mrs. Christine Lavery
The Society for Mucopolysaccharide
Diseases, Amersham,
Buckinghamshire,
UNITED KINGDOM

Prof. Atul Mehta
Department of Haematology
Royal Free Hospital London,
UNITED KINGDOM

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

Dr. Arnold Reuser
Department of Clinical Genetics,
Erasmus University, Rotterdam,
THE 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. Gere Sunder-Plassmann
Department of Internal Medicine III
Division of Nephrology and Dialysis,
University of Vienna,
Vienna, AUSTRIA

Prof. Kurt Ullrich
Children's Hospital
University of Hamburg
Hamburg, GERMANY

Dr. Marie T. Vanier
Laboratoire Fondation Gillet Mérieux,
Centre Hospitalier Lyon-Sud,
FRANCE

Dr. Catharina Whybra
Pediatric Department,
Johannes Gutenberg-University
of Mainz,
GERMANY

11th International Postgraduate Course on Lysosomal Storage Diseases

Nierstein (Mainz) June 4 - 8, 2012

Sunday, June 3

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

Monday, June 4

08.15 – 8.45	Course introduction General overview of LSDs *M. Beck*
08.45 – 09.30	Cell biology of lysosomes *V. Gieselmann*
09.30 – 10.15	Pathophysiology in LSDs *A. Reuser*
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	Come together

Tuesday, June 5

08.30 - 09.45	Sphingolipids, sphingolipidoses and the lipid phase problem *K. Sandhoff*
09.45 – 10.15	Fabry disease: Clinical Manifestation *C. Whybra*
10.15 – 10.45	Nephropathy in Fabry disease *G. Sunder-Plassmann*
10.45 - 11.00	Coffee break
11.00 – 11.30	Fabry disease: Neuropathy/CNS manifestation *E. Kolodny*
11.30 – 12.00	Effects of ERT in Fabry disease *A. Mehta*
12.00 – 13.00	LUNCH
13.00 – 14.15	Treatment of neurodegenerative LSDs *E. Kolodny*
14.15 – 15.15	Pompe disease *A. Reuser*
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 6

08.30 - 09.15	Clinical presentation of Gaucher disease in children and adults *E. Mengel*
9.15 - 10.00	CNS manifestation of Gaucher disease *E. Kolodny*
10.00 - 10.15	Coffee break
10.15 - 11.00	Gaucher disease: Neurological complications, genotype-phenotype correlation *E. Kolodny*
11.00 - 11.30	Treatment and monitoring of Gaucher disease *E. Mengel*
11.30 – 12.30	Stem cell transplantation in LSDs *K. Ullrich*
13.00	Lunch Afternoon at free disposal (Optional participation at excursion)

Thursday, June 7

08.30 – 10:00	Mucopolysaccharidoses (MPS) and glycoproteinoses *R. Giugliani*
10.00 - 10:15	Coffee break
10.15 – 11.30	Neurolipidoses – an overview *M. Vanier*
11.30 – 12.30	Enzyme replacement therapy in mucopolysaccharidoses *R. Giugliani*
12.30 – 13.45	LUNCH
13.45 – 14.30	Principles for laboratory diagnosis in LSDs *M. Beck & M. Vanier*
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 8

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

Application form

11th International Postgraduate Course on
Lysosomal Storage Diseases
June 4 – 8, 2012

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 4, 2012 – 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 11, 2012.

VILLA Metabolica

BEST WESTERN Wein- und Parkhotel Nierstein

An der Kaiserlinde 1 · 55283 Nierstein

Telefon: +49 6133 /508 0 · Telefax: +49 6133/508 333

Info@weinhotel.bestwestern.de

www.weinhotel.bestwestern.de

11th International Postgraduate Course on

Lysosomal Storage Diseases

Nierstein (Mainz), Germany
June 4 - 8, 2012

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.

IMS
Gesellschaft für akademischen Forschungstransfer mbH

Shire
Shire Human Genetic Therapies
is a business unit of Sire plc