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VILLA Metabolica

16th International Postgraduate Course on

Lysosomal Storage Disorders

Nierstein (Mainz), Germany
June 07 - June 10, 2017

Arrangement by Prof. Julia B. Hennermann
Department of Pediatric and Adolescent Medicine
University Medical Center Mainz

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

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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 pathoyphysiology of lysosomes
- ✓ Genetics
- ✓ Diagnosis and treatment
- ✓ Fabry disease
- ✓ Gaucher disease
- ✓ MPS syndromes
- ✓ Pompe disease
- ✓ Leukodystrophies
- ✓ Other LSDs
- ✓ Patients' organisations

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

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Questions regarding logistics can be addressed to:

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Nierstein (Mainz) June 07 - June 10, 2017

Wednesday, 07 June 2017

13.00 – 14.00	Arrival / Lunch-Snack
14.00 – 14.15	Welcome and Introduction *Julia B. Hennermann*
14.15 – 15.00	LSDs – A clinical overview *Michael Beck*
15.00 – 15.30	Cellular pathophysiology of LSDs – part I *Volkmar Gieselmann*
15.30 – 15.45	*Coffee break*
15.45 – 16.15	Cellular pathophysiology of LSDs – part II *Volkmar Gieselmann*
16.15 – 16.45	Neonatal screening for LSDs *Alberto Burlina*
16.45 – 17.15	*Coffee break*
17.15 – 18.15	Genetic principles of LSDs: A practical session on Mendelian genetics and pedigree analysis *Andreas Gal*
19.30	*Welcome Dinner*

Thursday, 08 June 2017

8.30 – 9.15	Sphingolipidoses *Konrad Sandhoff*
9.15 – 10.00	Fabry disease *Dominique Germain*
10.00 – 10.30	*Coffee break*

Thursday, 08 June 2017 cont.

10.30 – 11.15	Gaucher disease *Eugen Mengel*
11.15 – 12.00	Niemann Pick diseases *Marie T. Vanier*
12.00 – 12.45	Pompe disease *Ans van der Ploeg*
12.45 – 13.45	*Lunch*
13.45 – 15.30	Workshops/Discussion case reports *All*
15.30 – 16.00	*Coffee break*
16.00 – 17.00	Mucopolysaccharidoses and Mucolipidoses *Roberto Giugliani*
17.00 – 17.30	Mannosidoses *Julia B. Hennermann*
17.30 – 18.00	Acid lipase deficiency *Alex Broomfield*
18.00 – 18.30	Eye manifestations in selected LSDs *Susanne Pitz*
19.30	*Meet the Expert – Dinner*

Friday, 09 June 2017

8.30 – 9.15	Mechanisms of neurodegeneration in LSDs *Maurizio Scarpa*
9.15 – 10.00	Neuronal ceroid lipofuscinoses *Alfried Kohlschütter*
10.00 – 10.30	*Coffee break*
10.30 – 11.15	Leukodystrophies *Robert Steinfeld*
11.15 – 12.00	The blood brain barrier and LSDs *David J. Begley*
12.00 – 13.00	*Lunch*
13.00 – 13.45	Practical approach to ERT *Roberto Giugliani*
13.45 – 14.15	Effectiveness of ERT in MPS *Christian Hendriksz*
14.15 – 14.45	Stem cell transplantation in LSDs *Kurt Ullrich*
14.45 – 15.30	New treatment options in LSDs *Julia B. Hennermann*
15.30 – 16.00	*Coffee break*
16.00 – 17.30	Workshops/Discussion case reports *All*
20.00	*Farewell Dinner*

Saturday, 10 June 2017

8.30 – 9.00	Orthopedic management in MPS *Bianca Link*
9.00 – 9.30	Pre- and postnatal presentation of LSDs *Catharina Whybra-Trümpler*
9.30 – 10.15	Defects of lysosomal membrane proteins *Paul Saftig*
10.15 – 10.45	*Coffee break*
10.45 – 11.30	Principles of laboratory diagnosis in LSDs *Marie T. Vanier*
11.30 – 12.00	The role of patient support groups *Sophie Thomas*
12.00 – 13.00	Presentation of Workshop results *All*
13.00 – 13.15	Summary and Farewell *Julia B. Hennermann*
13.15	*Lunch-Snack / Departure*