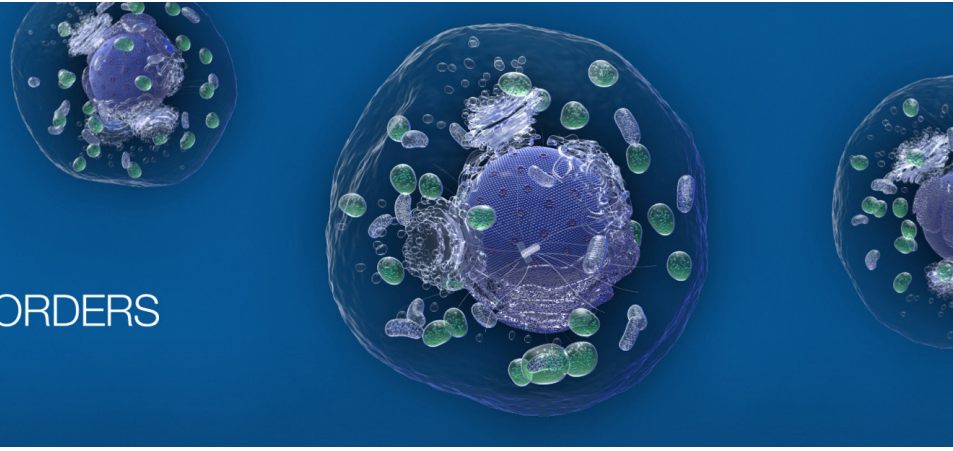
VIENNA, 12th–14th OCTOBER 2017

ESLSD 2017

EUROPEAN SYMPOSIUM ON
LYSOSOMAL STORAGE DISORDERS



Understanding biology and advancing treatments of LSDs

Thursday, 12th October 2017 – Day 1

18:00 – 18:30	Opening ESLSD 2017 – Welcome and introductory remarks
18:30 – 19:15	**Keynote: Ten plus one challenges in diseases of the lysosomal system**

Friday, 13th October 2017 – Day 2

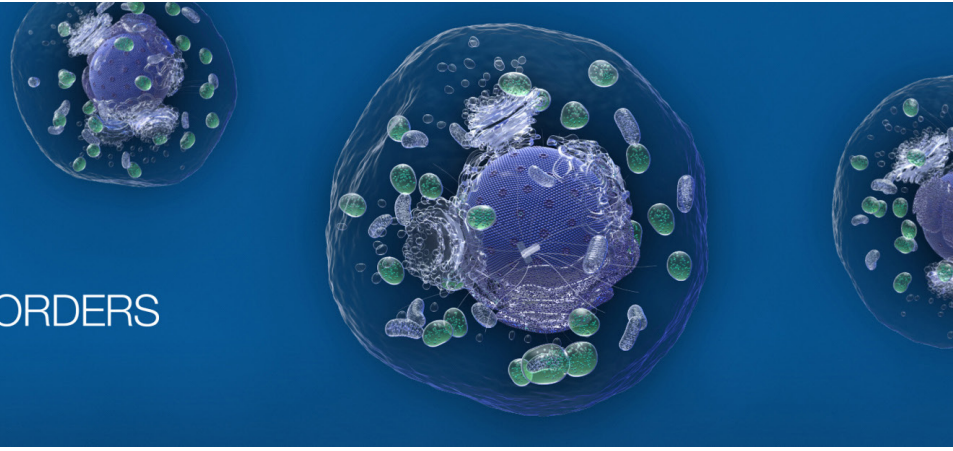
08:30 – 12:25	**Session 1: Pathophysiological pathways in LSDs**
08:30 – 08:40	Welcome day 2
08:40 – 09:20	**Keynote: Autophagy in LSDs: Is there anything new?**
09:20 – 09:50	LSDs: Storage, cellular dysfunction, and damage
09:50 – 10:05	*Coffee break*
	Inflammation in LSDs
10:05 –10:45	**Keynote: Microglia – the cornerstone of neuroinflammation**
10:45 – 11:05	Chronic inflammation in LSDs: Molecular mediators
11:05 – 11.25	Cellular immunology in LSDs
11:25 – 11.45	Sphingolipids – a conspectus in health and disease
11:45 – 12:05	Treating lysosomal and glycogen storage diseases: Normalizing metabolic and immune dysfunction
12:05 – 12:25	Panel discussion and audience Q&A
12:25 – 13:15	*Lunch*
13:15 – 18:00	**Session 2: Biomarkers in LSDs – Diagnosis, prognosis, and patient monitoring**
13:15 – 13:55	**Keynote: Biomarkers and surrogate endpoints: A growing role in drug development**
13:55 – 14:15	Biomarkers across the spectrum of LSDs
14:15 – 14:35	Biomarkers and surrogate endpoints in LSDs: What's the difference and does it matter?
14:35 – 15:00	Panel discussion and audience Q&A
15:00 – 15:30	*Coffee break and move to parallel sessions*
15:30 – 16:30	**Relating biomarkers to comorbidities and clinical endpoints in LSDs (3 parallel sessions)**
I.	Fabry disease: Progressive effects on cardiac structure and function
II.	Molecular and clinical prodrome in Gaucher disease
III.	Early diagnosis of cognitive impairment in MPS II
16:35 – 17:15	**Keynote: Can we predict complications and disease progression in LSDs?**
17:15 – 17:45	Panel discussion and audience Q&A
17:45 – 18:00	Summary and close day 2

This meeting is initiated, organised and sponsored by Shire
C-ANPROM/BEN//0008- based on C-ANPROM/INT//0109
Preparation Date: May 2017 |

VIENNA, 12th–14th OCTOBER 2017

ESLSD 2017

EUROPEAN SYMPOSIUM ON
LYSOSOMAL STORAGE DISORDERS



Understanding biology and advancing treatments of LSDs

Saturday, 14th October 2017 – Day 3

07:30 – 09:10	**Meet-the-Expert sessions (breakfast meeting)** **Detecting, diagnosing, and managing specific complications and comorbidities in LSDs** *Delegates may register for 2 sessions in advance of the meeting.*
I. (07:30 – 08:15)	• Fabry disease • Gaucher disease • MPS II
II. (08:25 – 09:10)	• Fabry disease • Gaucher disease • MPS II

09:15 – 13:00	**Session 3: New directions in LSDs**
09:15 – 09:20	Welcome day 3
09:20 – 09:40	New directions in LSDs: Are we going where we need to?
09:40 – 10:00	Intrathecal delivery of ERT in LSDs: Ready for the clinic?
10:00 – 10:20	LSDs and Parkinson's disease: Genetic links
10:20 – 10:40	Emerging genetic medicines for LSDs
10:40 – 10:55	Panel discussion and audience Q&A
10:55 – 11:05	*Coffee break and move to parallel sessions*
11:10 – 11:55	**Treatment efficacy: A focus on critical complications (3 parallel sessions)**
I.	Effects of therapy on the myocardium in Fabry disease
II.	Pharmacotherapy for Gaucher disease
III.	Addressing comorbidities in patients with MPS II
12:00 – 12:15	Selected oral poster presentation
12:15 – 12:45	**Keynote: Hunter syndrome: 100 years of discovery**
12:45 – 13:00	ESLSD 2017 closing remarks
13:00	*Departures*

This meeting is initiated, organised and sponsored by Shire C-ANPROM/BE//0048C- based on C-ANPROM/INT//0109
Preparation Date: May 2017