

ESLSD 2019

9th European Symposium on Lysosomal Storage Disorders

A Decade of Innovation in the LSD Care: Where we are Today and What is Awaiting us Tomorrow?

November 14th-16th, 2019,
Marriott Auditorium Hotel & Conference Center
Madrid, Spain

Day 1 - Thursday, November 14th, 2019

Time	Session	
18:00 – 18:15	Opening ESLSD 2019, Day 1 Welcome and introductory remarks	2019 ESLSD Scientific Steering Committee Takeda representative
18:15 – 18:55	Keynote Presentation An overview of the lysosome	Andrea Ballabio, IT
18:55 – 19:35	Keynote Presentation Is it enough to target the lysosome in LSDs?	Anthony Futerman, IL
19:45	Welcome Dinner	

Day 2 - Friday, November 15th, 2019

Time	Session		
08:00 – 08:15	Opening ESLSD 2019, Day 2 Welcome and introductory remarks	Timothy Cox, UK Local Host: Guillem Pintos Morell, ES	
08:15 – 08:20	Session 1: The Enzyme	Chair: Aleš Linhart, CZ	
08:20 – 08:50	Keynote Presentation ERT: What do we need and what's on the horizon?	Michael Beck, DE	
08:50 – 09:15	Plenary Presentation Intrathecal delivery of ERT: Successes and limitations in MPS and MLD	Joseph Muenzer, USA	
09:25 – 10:10	Parallel Presentation New biotechnological and nanomedicine strategies for treatment of storage diseases Silvia Muro, USA	Parallel Presentation Extending alternatives for delivery of ERT: Subcutaneous and oral enzyme delivery Atul Mehta, UK	Parallel Presentation Challenges in clinical trials of treatments for neuronopathic lysosomal diseases Elsa Shapiro, USA
10:10 – 10:25	Coffee Break		
10:25 – 10:45	Panel Discussion ERT: Most promising advances and issues still to be addressed	Moderator: Aleš Linhart Discussants: Michael Beck, Joseph Muenzer, Silvia Muro, Atul Mehta, Elsa Shapiro, Guillem Pintos Morell	
10:45 – 10:50	Session 2: The Gene	Chair: Timothy Cox, UK	
10:50 – 11:20	Keynote Presentation Accomplishments and limitations with AAV vector delivered gene replacement therapy	Amit Nathwani, UK	
11:20 – 11:45	Plenary Presentation Engineered stem cell transplantation for treatment of Gaucher disease	Stefan Karlsson, SE	

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Day 2 – Friday, November 15th, 2019 (cont'd)

11:55 – 13:00 <i>Lunch: Hot Topics</i>			
Participants select in advance ONE session Session will last 45 min Lunch will be made available during session	Parallel Presentation Newborn screening for rare diseases: Unmet needs and novel technologies <i>Barbara Burton, USA</i>	Parallel Presentation Goals of treatment in neurodegenerative disease: Reversibility or stability <i>Maurizio Scarpa, IT</i>	Parallel Presentation Gaucher disease and Parkinson <i>Ari Zimran, IL</i>
	Parallel Presentation Developing new therapies for rare diseases: Ethical and practical considerations, involvement of patients <i>Derralynn Hughes, UK</i>	Parallel Presentation Assessing 12 years of data in Fabry disease: Challenges in comparing treatment outcomes <i>Michael West, CA</i>	Parallel Presentation Assessing the benefit of established and emerging therapies for LSDs: Importance of patient-reported outcomes <i>Atul Mehta, UK</i>
13:10 – 13:55	Parallel Presentation The potential of fetal gene therapy in LSDs <i>Simon Waddington, UK</i>	Parallel Presentation Strategies for gene replacement therapy on both sides of the blood-brain barrier <i>Brian Bigger, UK</i>	Parallel Presentation Lentiviral hemopoietic stem-cell gene therapy in early-onset metachromatic leukodystrophy <i>Alessandra Biffi, USA/IT</i>
14:05 – 14:25	Panel Discussion Selecting approaches and patients for gene therapy in LSDs		<i>Moderator: Timothy Cox</i> <i>Discussants: Amit Nathwani, Stefan Karlsson, Simon Waddington, Brian Bigger, Alessandra Biffi</i>
14:25 – 14:35	Introduction to guided poster tour		<i>Elsa Shapiro, USA</i>
14:35 – 15:25 <i>Guided poster tour including Coffee Break</i>			
15:35 – 15:50	Plenary Presentation Oral poster presentation selected by Steering Committee		<i>Selected oral poster presenter</i>
15:50 – 15:55 Session 3: Small Molecules <i>Chair: Elsa Shapiro, USA</i>			
15:55 – 16:25	Keynote Presentation ERT vs SRT for LSDs: Is it OR or AND?		<i>Derralynn Hughes, UK</i>
16:25 – 16:45	Plenary Presentation Chaperones for treatment of LSDs: Current and coming		<i>Giancarlo Parenti, IT</i>
16:55 – 17:40	Parallel Presentation Actions of proteasome inhibitors in storage diseases <i>Yoshikatsu Eto, JP</i>	Parallel Presentation Clinical challenges and future therapeutic approaches for neuronal ceroid lipofuscinosis <i>Angela Schulz, DE</i>	Parallel Presentation Individualized screening for chaperone activity in Gaucher disease using multiple patient derived primary cell lines. <i>Ozlem Göker-Alpan, USA</i>
	Panel Discussion Approaches to employment of small molecules in LSDs		<i>Moderator: Elsa Shapiro</i> <i>Discussants: Derralynn Hughes, Giancarlo Parenti, Yoshikatsu Eto, Angela Schulz, Ozlem Göker-Alpan</i>
18:10 - 18:15	Day 2 closing remarks		<i>Anthony Futerman, IL</i>

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Day 3 – Saturday, November 16th, 2019

Time	Session		
07:40 – 08:10	Meet-the-Expert Session (optional) Delegates cases for discussion on Fabry disease <i>Aleš Linhart, CZ / Michael Beck, DE</i>	Gaucher disease <i>Ari Zimran, IL</i>	Hunter syndrome <i>Barbara Burton, USA</i>
08:20 – 08:30	Opening ESLSD 2019, Day 3 Welcome and introductory remarks	<i>Aleš Linhart, CZ</i>	
08:30 – 08:35	Session 4: Addressing Specific Pathologies with Adjunctive Therapies	Anthony Futerman, IL	
08:35 – 09:10	Keynote Presentation Lysosomal dysfunction: Downstream effects and targets for therapy	<i>Paul Saftig, DE</i>	
09:10 – 09:35	Plenary Presentation Using lysosomal metabolomics to identify treatment targets in storage diseases	<i>Monther Abu-Remaileh, USA</i>	
09:45 – 10:30	Parallel Presentation Directly addressing inflammation and oxidative stress in LSDs <i>Einat Vitner, IL</i>	Parallel Presentation Progranulin and related molecules as potential treatments in Gaucher disease <i>Gregory Pastores, IR</i>	Parallel Presentation mTORC1: A potential treatment target in lysosomal storage diseases? <i>Carmine Settembre, IT</i>
10:30 – 10:45	<i>Coffee Break</i>		
10:45 – 11:10	Panel Discussion Exploring novel interventions in LSDs: Best bets?	<i>Moderator: Anthony Futerman Discussants: Paul Saftig, Monther Abu-Remaileh, Einat Vitner, Gregory Pastores, Carmine Settembre</i>	
11:10 – 11:50	Closing Keynote Presentation	<i>Steven Walkley, USA</i>	
11:50 – 12:00	ESLSD 2019 closing remarks	<i>Timothy Cox, UK</i>	