LAL-D Patient Association (LAL-D PO)
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LAL-D EXPERT COMMITTEE MEETING

Date: November 20, 2026
Time: 3:30 PM to 8:30 PM
Venue: Hotel PCM Forum Alcalá – C/ de Bulgaria, 2 - 28802 Alcalá de Henares – Madrid

AGENDA

03:30 PM	**Welcome and review of the agenda** Eduardo López - President, LAL-D Patient Organization (LAL-D PO)
03:45 PM	**Development and Validation of Two Caregiver-Reported Outcome Measures for Infantile and Later-Onset LAL-D: The Lysosomal Acid Lipase Deficiency-Health Index 0+ (LALD-HI 0+) & Lysosomal Acid Lipase Deficiency-Health Index 2+ (LALD-HI 2+)** Charlotte Engebrecht, MS-CI - Outcomes Specialist II at the University of Rochester Center for Health + Technology (CHeT)
04:15 PM	**LAL-D: Updates Presented at the 2026 World Symposium** Dr. Javier de las Heras - Head of the Metabolism Unit, Cruces University Hospital (CSUR, MetabERN) Associate Professor, Faculty of Medicine, University of the Basque Country (EHU). BioBizkaia Health Research Institute
04:45 PM	**Enzyme Encapsulation in Complex Vectors** Dr. José Víctor Álvarez González - Miguel Servet Researcher. Health Research Institute of Santiago de Compostela. Inherited Metabolic Disorders Laboratory
05:15 PM	**Phospholipid Nanoparticles for Lipase Deficiency Gene Replacement Therapy** Davide Brambilla - Associate Professor of Drug Delivery and Formulation, School of Pharmaceutical Sciences, University of Geneva, Switzerland. Associate Professor of Drug Delivery and Formulation, Faculty of Pharmacy, Université de Montréal, Canada. Associate Editor, Journal of Controlled Release
05:45 PM	**AAV-LIPA Gene Therapy: Development and Validation in Human Liver Organoids** Dr. Maria Mercadal - Specialist in Pediatric Hepatology and Liver Transplantation, Vall d'Hebron University Hospital
06:15 PM	Coffee Break
06:30 PM	**Two Wolman Disease Cases Presenting with Hemophagocytic Syndrome at Onset** Carlos Cátedra López - Third-Year Resident Pharmacist (FIR), Clinical Analysis Department. La Fe University and Polytechnic Hospital, Valencia
07:00 PM	**Spanish Lysosomal Acid Lipase Deficiency Registry: Organization and Next Steps** Irene Serrano - FEETEG Predoctoral Researcher **Pilot Study of In Silico Predictors for Intronic Variants in Lysosomal Diseases** Dr. Isidro Arévalo - FEETEG Postdoctoral Researcher
07:30 PM	**Proteomic Characterization for the Identification of Liver Fibrosis Biomarkers in Lysosomal Acid Lipase Deficiency (LAL-D)** Sonia Roca – Biotechnologist. MSc in Biochemistry, Molecular Biology and Biomedicine
08:00 PM	**Review of Real-Life Outcomes of Enzyme Replacement Therapy for Lysosomal Acid Lipase Deficiency** Jorge Cebolla, PhD - Postdoctoral Researcher Department of Biochemistry, Molecular and Cell Biology University of Zaragoza
08:30 PM	Closing Remarks and End of the Meeting

Each presentation will last approximately 20 minutes, followed by 10 minutes for questions and discussion.
Once the meeting is over, we will have the opportunity to have dinner together at La Cúpula Restaurant

Join the meeting via Zoom
https://us06web.zoom.us/j/86269392486?pwd=cJRIxdxlYQlCkFzZybKlvb7CFETIFy.1
Meeting ID: 862 6939 2486 Passcode: 385321

Con el apoyo de: ALEXION AstraZeneca Rare Disease, ELPA European Liver Patients' Association, feder

Members of: EURORDIS Rare Diseases Europe, European Reference Network, MetabERN, ELPA, Global Liver Institute, feder, Federación Española del Hígado, Cardio Alianza, FECAMM