

#### Speakers:

- |                      |                |
|----------------------|----------------|
| › A. Wiegman         | › M. Shagrani  |
| › B. Greco           | › M. Spada     |
| › C. Dionisi Vici    | › M. Zielonka  |
| › D. Broering        | › P. Mckiernan |
| › D. Debray          | › P. Socha     |
| › D. Martinelli      | › P. Mckiernan |
| › F. Caseiro Alves   | › T. Derks     |
| › J. Willemse        | › U. Herden    |
| › M. Hørby Jørgensen |                |

#### Scientific Committee:

- › Luísa Diogo
- › Isabel Gonçalves
- › ERN Rare-Liver
- › ERN transplantchild
- › MetabERN

#### Organising Committee:

- › Luísa Diogo
- › Isabel Gonçalves
- › Catarina Cunha
- › Sandra Ferreira
- › Sara Ferreira
- › Susana Nobre
- › Ana Mendes
- › João Madaleno
- › Patrícia Couceiro

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## Liver Transplant for Inborn Errors of Metabolism

### Sharing Knowledge Across ERNs



Dolores Faria

**8<sup>th</sup> and 9<sup>th</sup> June 2023**

**Auditorio Hospital Pediátrico › Coimbra › Portugal**

40° 12' 35.2764" N, 8° 24' 50.7564" W



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Thursday 8th June 2023

08:30 › Opening Registration Desk

09:00 › **Welcome Address and Opening Session I Crossing borders within ERNs towards consensus**  
ERNs Coordinators, Representatives: RARE-LIVER, MetabERN, TransplantChild

10:00 › Welcome coffee

## Panel 1

### **Liver Transplant (LT) for Inborn Errors of Metabolism (IEM) - Looking Back, Looking Forward**

**Chairpersons:** E. Furtado, I. Gonçalves, L. Diogo

- 10:30 › **Pediatric Liver Transplant: targeting a 100% immediate survival**  
D. Broering, King Faisal Specialist Hospital & Research Centre, Riyadh, Saudi Arabia
- 10:50 › **Pros / Cons: The Hepatologist View**  
P. Mckiernan, Birmingham Children's Hospital, UK
- 11:10 › **Pros / Cons: The Metabolic Physician View**  
C. Dionisi Vici, Bambino Gesù Children's Research Hospital, Rome, Italy
- 11:30 › **Global Results of Liver Transplant for IEM: High Volume Center**  
M. Shagrani, King Faisal Specialist Hospital and Research Centre, Riyadh, Saudi Arabia
- 11:50 › **Discussion**
- 12:30 › Lunch

## Panel 2

### **Common Indications for LT in IEM - Disease Burden and Timely Decisions**

**Chairpersons:** A. Baker, C. Marbach, C. Pinto

- 13:30 › **Organic Acidurias**  
D. Martinelli, Bambino Gesù Children's Research Hospital, Rome, Italy
- 13:50 › **Urea Cycle Disorders**  
M. Zielonka, University Children's Hospital Heidelberg, Germany
- 14:10 › **Glycogen Storage Diseases**  
T. Derks, University Medical Center Groningen Beatrix Children's Hospital, Netherlands
- 14:30 › **Management of IEM presenting as neonatal acute liver failure**  
M. Hørby Jørgensen, Copenhagen University Hospital, Denmark
- 14:50 › **Discussion**
- 15:30 › Coffee-Break

## Panel 3

### **Optimizing Medical and Surgical Care**

**Chairpersons:** E. Nicastro, P. Sanchez Pinto, C. Cunha

- 16:00 › **Challenging Indications for LT (Neonatal IEM, Mitochondrial, Lysosomal, Glycosylation Disorders)**  
D. Debray, Necker-Enfants Malades Hospital, Paris, France
- 16:20 › **Maximizing Organ Donation for IEM (Domino, Auxiliary, Living Donor, Allocation Systems)**  
M. Spada, Bambino Gesù Children's Research Hospital, Rome, Italy
- 16:40 › **Are Metabolic Patients at Different Risks After LT? The Need for Modified Protocols**  
U. Herden, University Medical Center Hamburg-Eppendorf, Hamburg, Germany
- 17:10 › **Discussion**
- 20:00 › Conference Dinner

Friday 9 th June 2023

## Panel 4

### **Combined Medical and LT Options - A New Era?**

**Chairpersons:** J. Madaleno, C. Mayer, M. Shagrani

- 09:00 › **Familial Homozygous Hypercholesterolemia**  
A. Wiegman, Amsterdam University Medical Center, Amsterdam, Netherlands
- 09:20 › **Acute Wilson Disease: To Transplant or To be Patient**  
P. Socha, The Children's Memorial Health Institute, Warsaw, Poland
- 09:40 › **Management of Glycogen Storage Disease Patients with Nodular Lesions**  
F. Caseiro Alves, Radiology, Coimbra, Portugal
- 10:00 › **Discussion**
- 10:40 › Coffee-Break

## Panel 5

### **Quality of Life (QoL) Long Term Results, Patient Perception, Unmet Needs**

**Chairpersons:** Sandra Ferreira, S. Bond, A. Mendes

- 11:10 › **Metabolic and LT Team: A Shared Journey**  
P. Mckiernan, Birmingham Children's Hospital, UK
- 11:30 › **LT for IEM and QoL: The MetabQoL 1.0 Questionnaire**  
B. Greco, Bambino Gesù Children's Research Hospital, Rome, Italy
- 11:50 › **Quality of Life: Patients Testimony**  
Patients and Families
- 12:10 › **Unmet Needs for Patients and Families**  
J. Willemse, Patient advocacy Group (Adult Patient Lead), RARE-LIVER, Netherlands
- 12:30 › **Discussion**
- 13:10 › **Closing Remarks**

(Lectures - 20 minutes talk; Discussion at the end of each panel)