



**Liver Transplant for Inborn Errors of Metabolism**  
Sharing Knowledge Across ERNs

8<sup>th</sup> and 9<sup>th</sup> June 2023 | Coimbra | Portugal

More information: [ERN.RareLiver@uke.de](mailto:ERN.RareLiver@uke.de)



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**Thursday 8<sup>th</sup> June 2023**

**Welcome Address | Crossing borders within ERNs towards consensus**

ERNs Coordinators / Representatives | RARE-LIVER | MetabERN | TransplantChild

**Panel I**

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

**Pediatric Liver Transplant: targeting a 100% immediate survival**

Dieter Broering, King Faisal Specialist Hospital & Research Centre, Riyadh, Saudi Arabia

**Pros | Cons: The Hepatologist View**

Patrick Mckiernan, Birmingham Children's Hospital, UK

**Pros | Cons: The Metabolic Physician View**

Carlo Dionisi Vici, Bambino Gesù Children's Research Hospital, Rome, Italy

**Global Results of Liver Transplant for IEM: High Volume Center**

Mohammad Shagrani, King Faisal Specialist Hospital and Research Centre, Riyadh, Saudi Arabia

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*Thursday 8<sup>th</sup> June 2023*

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Lunch

## Panel 2

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

#### Organic Acidurias (timing decision and outcome)

Diego Martinelli, Bambino Gesù Children's Research Hospital, Rome, Italy

#### Urea Cycle Disorders

Matthias Zielonka, University Children's Hospital Heidelberg, Germany

#### Glycogen Storage Diseases

Terry Derks, University Medical Center Groningen Beatrix Children's Hospital, Netherlands

#### Management of IEM presenting as neonatal acute liver failure

Marianne Hørby Jørgensen, Copenhagen University Hospital, Denmark

Coffee-Break

## Panel 3

### Optimizing Medical and Surgical Care

#### Challenging Indications for LT (Neonatal IEM, Mitochondrial, Lysosomal, Glycosylation Disorders)

Dominique Debray, Necker-Enfants Malades Hospital, Paris, France

#### Maximizing Organ Donation for IEM (domino, auxiliary, living-donor, allocation systems)

Marco Spada, Bambino Gesù Children's Research Hospital, Rome, Italy

#### Are Metabolic Patients at Different Risks After LT? The Need for Modified Protocols

Uta Herden, University Medical Center Hamburg-Eppendorf, Hamburg, Germany

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*Friday 9<sup>th</sup> June 2023*

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#### Panel 4

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

##### **Familial Homozygous Hypercholesterolemia**

Albert Wiegman, Amsterdam University Medical Center, Amsterdam, Netherlands

##### **Acute Wilson Disease: To Transplant or To be Patient**

Piotr Socha, The Children's Memorial Health Institute, Warsaw, Poland

##### **Management of Glycogen Storage Diseases Patients with Nodular Lesions**

Filipe Caseiro Alves, Radiology, Coimbra, Portugal

Lunch

#### Panel 5

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

##### **Metabolic and LT Team: A Shared Journey**

Patrick Mckiernan, Birmingham Children's Hospital, UK

##### **LT for IEM and QoL: The MetabQoL 1.0 Questionnaire**

Greco Benedetta, Bambino Gesù Children's Research Hospital, Rome, Italy

##### **Quality of Life: Patients Testimony**

Patients and Families

##### **Unmet Needs for Patients and Families**

José Willemse, Patient advocacy Group (Adult Patient Lead), RARE-LIVER, Netherlands

**Closing Remarks | Local Committee**