<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speakers/Topics/Protocols/Details</th>
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<tbody>
<tr>
<td>07:00</td>
<td>Opening and Welcome Address</td>
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<tr>
<td>07:30</td>
<td>Newborn Screening for Spinal Muscular Atrophy &amp; Recommended Uniform Screening Panel in the US: from SCID to SMA, and beyond</td>
<td>Presented by PerkinElmer</td>
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<tr>
<td>08:00</td>
<td>Diagnosing and managing disorders of biogenic amines</td>
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<td>09:00</td>
<td>Interim Results of the Vigilan Observational Study: Clinical Characteristics of L-amino-acid decarboxylase (AADC) deficiency in newborn screening and at-risk subjects with hyperphenylalaninemia</td>
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<td>10:00</td>
<td>Presenting newborn screening outcomes can guide screening strategy</td>
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<tr>
<td>11:00</td>
<td>Presenting newborn screening outcomes can guide screening strategy</td>
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<tr>
<td>13:00</td>
<td>14th International Congress of Inborn Errors of Metabolism 2021 'Transforming Rare Disorders'</td>
<td>21-24 November 2021</td>
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<tr>
<td>14:00</td>
<td>Keynote Address</td>
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<td>15:00</td>
<td>Clinical phenotype and molecular analysis for 16 Cases of Galactosialidosis</td>
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<td>16:00</td>
<td>Overall clinical and molecular insight into Hunter Syndrome</td>
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<td>17:00</td>
<td>Pharmacological assessment of a novel preclinical therapeutic in Niemann-Pick disease type A</td>
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<tr>
<td>18:00</td>
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<td>19:00</td>
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<tr>
<td>24:00</td>
<td>Newborn screening outcomes can guide screening strategy</td>
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# ICIEM 2021 - Congress Program Current as of 20/11/2021

## TUESDAY 23 NOVEMBER 2021

### SESSIONS

#### 08:00 - 09:00

**Online Q&A - New Therapies**
- Chaired by John Christodoulou and Roberto Guigliani

**Online Q&A - Adult Rare**
- Chaired by Aditi Harish and Rebecca Alpigen

**Online Q&A - Paediatric**
- Chaired by Janie Valley and Anjali Tawne

### 09:00 - 11:00

#### SYMPOSIUM A

**The Patient Experience of Research**
- Presented by Maren Sturgis

**The case for early gene therapy for lysosomal storage**
- Presented by Bitty Beaird

**The role of early gene therapy in lysosomal storage disorders**
- Presented by Amanda Sharvers

**The impact of early trials on clinical practice and research in lysosomal storage disorders**
- Chaired by Simon Milunsky

**The impact of early gene therapy on clinical practice and research in lysosomal storage disorders**
- Chaired by Simon Milunsky

**The role of early gene therapy in lysosomal storage disorders**
- Presented by Amanda Sharvers

### 11:00 - 13:00

#### SYMPOSIUM B

**A tale of two Phe states: PKU neurocognitive burden and the Palynziq experience**
- Presented by Krista Doyle

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**A tale of two Phe states: PKU neurocognitive burden and the Palynziq experience**
- Presented by Krista Doyle

### 13:00 - 15:00

#### SYMPOSIUM C

**The impact of early gene therapy on clinical practice and research in lysosomal storage disorders**
- Chaired by Simon Milunsky

**The role of early gene therapy in lysosomal storage disorders**
- Presented by Amanda Sharvers

**The impact of early gene therapy on clinical practice and research in lysosomal storage disorders**
- Chaired by Simon Milunsky

### 15:00 - 17:00

#### SYMPOSIUM D

**The story of the latest genetic therapeutic milestones in Lysosomal Storage Disorders and its global influence on research, changing the face of lysosomal storage disorders management**
- Presented by Almuth Balzer-Kovacs

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### 17:00 - 19:00

#### SYMPOSIUM E

**Awards & Closing Comments**
- Chaired by John Christodoulou and Roberto Guigliani

**Awards & Closing Comments**
- Chaired by John Christodoulou and Roberto Guigliani

**Awards & Closing Comments**
- Chaired by John Christodoulou and Roberto Guigliani

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### 08:00 - 10:00

**SYMPOSIUM A

The Patient Experience of Research**
- Presented by Maren Sturgis

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- Presented by Bitty Beaird

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- Presented by Amanda Sharvers

**The impact of early gene therapy on clinical practice and research in lysosomal storage disorders**
- Chaired by Simon Milunsky

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### 12:00 - 14:00

**SYMPOSIUM B

A tale of two Phe states: PKU neurocognitive burden and the Palynziq experience**
- Presented by Krista Doyle

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- Presented by Krista Doyle

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- Presented by Krista Doyle

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### 14:00 - 16:00

**SYMPOSIUM C

The story of the latest genetic therapeutic milestones in Lysosomal Storage Disorders and its global influence on research, changing the face of lysosomal storage disorders management**
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### 16:00 - 18:00

**SYMPOSIUM D

Awards & Closing Comments**
- Chaired by John Christodoulou and Roberto Guigliani

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- Chaired by John Christodoulou and Roberto Guigliani

**Awards & Closing Comments**
- Chaired by John Christodoulou and Roberto Guigliani

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### 18:00 - 20:00

**Conference Dinner - In person registrations only**
- Presented by Emslie"
### WEDNESDAY 24 NOVEMBER 2021

<table>
<thead>
<tr>
<th>Time</th>
<th>Event Description</th>
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| 07:30-08:00 | Online Q&A - Late breaking news  
Chaired by Kimitoshi Nakamura and Jim Bonham  
07:30  
2:00  
21:30  
14:00 |
| 08:00-08:30 | Online Q&A - Special award presentations  
Chaired by Ute Spiekerkoetter and Shawn McCandless  
08:00  
3:00  
22:00  
15:00 |
| 08:30-09:00 | Online Q&A - Integrated care  
Jose Alderete and Anita Inwood  
08:30  
3:30  
22:30  
15:30 |
| 09:00-09:30 | Society Updates  
SAMD - Gerry Berry  
SLEMPER - Celia Perez de Ferran  
SLEIM - Gaja Salimons and Ute Spiekerkoetter  
ASEM - Veronica Wiley  
Presentation for ICIEM 2025  
SAMD - Kimitoshi Nakamura  
Congress Close  
Kaustuv Bhattacharya  
09:00  
3:30  
23:00  
16:00 |