2018 Hunter’s Hope Medical Symposium

2018 Medical Symposium

Leukodystrophy Care Network

Leukodystrophies | Newborn Screening | Research

July 16 - 19, 2018

Holiday Valley Resort – Ellicottville, NY
# Schedule Overview

## Monday July 16, 2018

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>4:00pm</td>
<td>Registration Opens – Tamarack Club Lobby</td>
</tr>
<tr>
<td>7:15pm</td>
<td>Welcome Dinner – Sunrise, Holiday Valley Lodge</td>
</tr>
</tbody>
</table>

## Tuesday July 17, 2018

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>7:00 – 8:00am</td>
<td>Breakfast – Sunrise, Holiday Valley Lodge</td>
</tr>
<tr>
<td><strong>Session I</strong></td>
<td>Leukodystrophy Newborn Screening</td>
</tr>
<tr>
<td>8:00am – 12:00pm</td>
<td>Fairway, 2nd Floor, Holiday Valley Lodge</td>
</tr>
<tr>
<td>12:00 – 12:55pm</td>
<td>Lunch - Sunrise, Holiday Valley Lodge</td>
</tr>
<tr>
<td><strong>Session II</strong></td>
<td>Standards of Care for Leukodystrophy NBS</td>
</tr>
<tr>
<td>12:55 – 5:00pm</td>
<td>Fairway, 2nd Floor, Holiday Valley Lodge</td>
</tr>
<tr>
<td>6:30pm</td>
<td>Dinner - Sunrise, Holiday Valley Lodge</td>
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</tbody>
</table>

## Wednesday July 18, 2018

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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</thead>
<tbody>
<tr>
<td>8:00 – 9:00am</td>
<td>Breakfast – Sunrise, Holiday Valley Lodge</td>
</tr>
<tr>
<td><strong>Session III</strong></td>
<td>Leukodystrophy Care Network (LCN)</td>
</tr>
<tr>
<td>9:00am – 4:15pm</td>
<td>Fairway, 2nd Floor, Holiday Valley Lodge</td>
</tr>
<tr>
<td>12:15pm</td>
<td>Lunch - Sunrise, Holiday Valley Lodge</td>
</tr>
<tr>
<td>7:00pm</td>
<td>20th Anniversary Celebration Dinner -Sunrise, Holiday Valley Lodge</td>
</tr>
</tbody>
</table>

*LCN Care Coordinator Meeting – 4:30pm – 6:00pm, Birdie - Holiday Valley Lodge

## Thursday July 19, 2018

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>8:00 – 9:30am</td>
<td>Breakfast - Sunrise, Holiday Valley Lodge</td>
</tr>
<tr>
<td><strong>Session IV</strong></td>
<td>Presentations to Leukodystrophy Families</td>
</tr>
<tr>
<td>9:30am – 2:30pm</td>
<td>Fairway, 2nd Floor, Holiday Valley Lodge</td>
</tr>
<tr>
<td>11:45 – 12:45pm</td>
<td>Lunch - Sunrise, Holiday Valley Lodge</td>
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**Save the Date!**

2019 Hunter’s Hope Medical Symposium

July 22-25, 2019
## 2018 Hunter’s Hope Medical Symposium

**Tuesday, July 17th**

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Location</th>
</tr>
</thead>
<tbody>
<tr>
<td>7:00am</td>
<td>Breakfast</td>
<td>Sunrise, HV Lodge</td>
</tr>
</tbody>
</table>

### Session I and II – Fairway, 2nd Floor Holiday Valley Lodge

<table>
<thead>
<tr>
<th>Time</th>
<th>Session I: Leukodystrophy Newborn Screening and Treatment</th>
<th>Location</th>
</tr>
</thead>
<tbody>
<tr>
<td>8am – 12:00pm</td>
<td>Leukodystrophy Newborn Screening (NBS) – State Updates</td>
<td>Fairway</td>
</tr>
<tr>
<td>8:00 – 10:00am</td>
<td>Joseph Orsini, PhD</td>
<td></td>
</tr>
<tr>
<td>8:00 – 8:20am</td>
<td>New York</td>
<td></td>
</tr>
<tr>
<td>8:20 – 8:35am</td>
<td>Missouri</td>
<td></td>
</tr>
<tr>
<td>8:35 – 8:55am</td>
<td>Kentucky</td>
<td></td>
</tr>
<tr>
<td>8:55 – 9:10am</td>
<td>Ohio</td>
<td></td>
</tr>
<tr>
<td>9:10 – 9:30am</td>
<td>Tennessee</td>
<td></td>
</tr>
<tr>
<td>9:30 – 9:45am</td>
<td>Illinois</td>
<td></td>
</tr>
<tr>
<td>9:45 – 10:00am</td>
<td>MLD NBS</td>
<td></td>
</tr>
<tr>
<td>10:00 – 10:25am</td>
<td>Krabbe NBS Clinical Practice Guidelines (CPGs)</td>
<td></td>
</tr>
<tr>
<td>10:25 – 12:00pm</td>
<td>Leukodystrophy Treatment</td>
<td></td>
</tr>
<tr>
<td>10:25 – 10:55am</td>
<td>Krabbe Disease, MLD and ALD</td>
<td></td>
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<tr>
<td>10:55 – 11:15am</td>
<td>Mediterranean</td>
<td></td>
</tr>
<tr>
<td>11:15 – 11:40am</td>
<td>ALD Clinical Trial</td>
<td></td>
</tr>
<tr>
<td>11:40 – 12:00pm</td>
<td>Family Perspective</td>
<td></td>
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<tr>
<td>12:00 – 12:55pm</td>
<td>Lunch</td>
<td>Sunrise, HV Lodge</td>
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</tbody>
</table>

### Session II: Standards of Care for Leukodystrophy NBS

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Location</th>
</tr>
</thead>
<tbody>
<tr>
<td>12:55 – 5:00pm</td>
<td>Session II: Standards of Care for Leukodystrophy NBS</td>
<td>Fairway</td>
</tr>
<tr>
<td>12:55 – 1:15pm</td>
<td>Joseph Orsini, PhD</td>
<td></td>
</tr>
<tr>
<td>1:15 – 1:30pm</td>
<td>Krabbe NBS CPGs for Late Onset Krabbe Disease (LOKD)</td>
<td></td>
</tr>
<tr>
<td>1:30 – 1:45pm</td>
<td>Psychosine as 2nd Tier Test for Krabbe NBS</td>
<td></td>
</tr>
<tr>
<td>1:45 – 2:05pm</td>
<td>High sensitivity GALC Assay</td>
<td></td>
</tr>
<tr>
<td>2:05 – 2:25pm</td>
<td>LOKD Cases Treated with Hematopoietic Stem Cell Transplantation</td>
<td></td>
</tr>
<tr>
<td>2:25 – 3:25pm</td>
<td>Presentation of LOKD NBS Cases</td>
<td></td>
</tr>
<tr>
<td>3:25 – 5:00pm</td>
<td>Recommended Follow Up for Possible LOKD Cases</td>
<td></td>
</tr>
<tr>
<td>6:30pm</td>
<td>Dinner</td>
<td>Sunrise, HV Lodge</td>
</tr>
<tr>
<td>6:30pm</td>
<td>ALD NBS Follow Up</td>
<td></td>
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**2018 Hunter’s Hope Medical Symposium**
2018 Hunter’s Hope Medical Symposium

Wednesday, July 18th

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<tr>
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<th>Event</th>
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</tr>
</thead>
<tbody>
<tr>
<td>8:00 – 9:00am</td>
<td>Breakfast</td>
<td>Sunrise, HV Lodge</td>
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Session III – Fairway, 2nd Floor Holiday Valley Lodge

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
<th>Location</th>
</tr>
</thead>
<tbody>
<tr>
<td>9:00am – 4:15pm</td>
<td><strong>Session III: Leukodystrophy Care Network (LCN)</strong></td>
<td>Fairway</td>
</tr>
<tr>
<td>9:00 – 9:15am</td>
<td>Welcome and Introduction</td>
<td>Anna Grantham</td>
</tr>
<tr>
<td>9:15 – 10:15am</td>
<td>Discussion of LCN Center Protocol for Patient Referrals</td>
<td>Catherine Becker, NP</td>
</tr>
<tr>
<td></td>
<td><strong>Leukodystrophy Clinical Practice Guidelines (CPGs) – Overview and Updates</strong></td>
<td></td>
</tr>
<tr>
<td>10:15 – 10:45am</td>
<td>Musculoskeletal</td>
<td>Sally Evans, MD</td>
</tr>
<tr>
<td>10:45 – 11:15am</td>
<td>Treatment</td>
<td>Joanne Kurtzberg, MD</td>
</tr>
<tr>
<td>11:15 – 11:45am</td>
<td>Neurology</td>
<td>Florian Eichler, MD</td>
</tr>
<tr>
<td>11:45 – 12:00pm</td>
<td>Respiratory</td>
<td>Geovanny Perez, MD</td>
</tr>
<tr>
<td>12:00 – 12:15pm</td>
<td>Diagnosis</td>
<td>Maura Ruzhnikov, MD</td>
</tr>
<tr>
<td></td>
<td>Group Photo</td>
<td></td>
</tr>
<tr>
<td>12:15pm – 1:15pm</td>
<td><strong>LUNCH</strong></td>
<td>Fairway</td>
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<thead>
<tr>
<th>Time</th>
<th>Event</th>
<th>Location</th>
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</thead>
<tbody>
<tr>
<td>1:15 – 2:45pm</td>
<td>CPG Breakout Sessions</td>
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<tr>
<td></td>
<td>• Musculoskeletal – Fairway</td>
<td></td>
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<tr>
<td></td>
<td>• Treatment – Bunker, 2nd Floor</td>
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<tr>
<td></td>
<td>• Neurology – Moonshadow, 1st Floor</td>
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</tr>
<tr>
<td></td>
<td>• Respiratory – Morning Star, 1st Floor</td>
<td></td>
</tr>
<tr>
<td></td>
<td>• Diagnosis – Birdie, 2nd Floor</td>
<td></td>
</tr>
<tr>
<td>2:45 – 3:45pm</td>
<td>CPG Breakout Group Updates</td>
<td>CPG Lead Authors</td>
</tr>
<tr>
<td>3:45 – 4:15pm</td>
<td>Group Discussion</td>
<td>Florian Eichler, MD</td>
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<tbody>
<tr>
<td>4:30 – 6:00pm</td>
<td>LCN Care Coordinator Workgroup Meeting</td>
<td>Birdie – HV Lodge</td>
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<thead>
<tr>
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<tbody>
<tr>
<td>7pm</td>
<td>Dinner – Please join us as we celebrate 20 years of Hunter’s Hope and meet our Leukodystrophy families.</td>
<td>Sunrise, HV Lodge</td>
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### Thursday, July 19th

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<tr>
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#### Session IV – Fairway, 2nd Floor Holiday Valley Lodge

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<tr>
<th>Time</th>
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<tbody>
<tr>
<td>9:30 – 2:30pm</td>
<td><strong>Leukodystrophy Care Network (LCN) Presentations to Leukodystrophy Families</strong></td>
<td>Fairway</td>
</tr>
<tr>
<td>9:30 – 9:40am</td>
<td>Welcome &amp; LCN Overview</td>
<td>Jacque Waggoner and Anna Grantham</td>
</tr>
<tr>
<td>9:40 – 9:55am</td>
<td>MLD NBS</td>
<td>Michael Gelb, PhD</td>
</tr>
<tr>
<td>9:55 – 10:10am</td>
<td>Krabbe and ALD Newborn Screening (NBS) Overview</td>
<td>Joseph Orsini, PhD</td>
</tr>
<tr>
<td>10:10 – 10:25am</td>
<td>Krabbe NBS Clinical Practice Guidelines</td>
<td>Jennifer Kwon, MD</td>
</tr>
<tr>
<td>10:25 – 10:40am</td>
<td>Guidelines for ALD NBS</td>
<td>Ali Fatemi, MD</td>
</tr>
<tr>
<td>10:40 – 10:55am</td>
<td>LCN Centers</td>
<td>Stephanie Keller, MD</td>
</tr>
<tr>
<td>10:55 – 11:10pm</td>
<td>LCN Care Coordinators</td>
<td>Tara West, NP</td>
</tr>
<tr>
<td>11:10 – 11:45pm</td>
<td>Treatment</td>
<td>Joanne Kurtzberg, MD</td>
</tr>
</tbody>
</table>

#### 11:45 – 12:45pm | **LUNCH**                  | Fairway         |

<table>
<thead>
<tr>
<th>Time</th>
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<th>Authors</th>
</tr>
</thead>
<tbody>
<tr>
<td>12:45 – 1:45pm</td>
<td>Clinical Practice Guidelines</td>
<td>CPG Lead Authors</td>
</tr>
<tr>
<td></td>
<td>- Musculoskeletal</td>
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<td></td>
<td>- Treatment</td>
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<tr>
<td></td>
<td>- Neurology</td>
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<td></td>
<td>- Respiratory</td>
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<tr>
<td></td>
<td>- Diagnosis</td>
<td></td>
</tr>
<tr>
<td>1:45 – 2:15</td>
<td>Advancements in Leukodystrophies</td>
<td>Amy Waldman, MD</td>
</tr>
<tr>
<td>2:15 – 2:30pm</td>
<td>ALD Clinical Trial</td>
<td>Florian Eichler, MD</td>
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July 22-25, 2019