Welcome

Robert Ostrea
Parent Advocate
Co-Founder, Little Miss Hannah Foundation
Thank You!

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Family Room
Upstairs in Balcony E
Sponsored by DOHMEN
LIFE SCIENCE SERVICES
LITTLE MISS HANNAH FOUNDATION

To enhance the quality of life for children diagnosed with rare, life-limiting, and complex medical conditions in Southern Nevada

www.littlemisshannah.org
Hannah’s Legacy
Little Miss Hannah Foundation

• Established December 2011 in memory of Hannah and in honor of Ethan and Abigail Ostrea, Hannah’s big brother and sister

• The Ostrea family and close friends formed the LMHF to give hope and support to other families of children who have received a life-limiting rare diagnosis and those families with children receiving hospice or palliative care.

• Became a 501(c)(3) nonprofit charity organization in July, 2012.

**Our Mission:** To enhance the quality of life for children diagnosed with rare, life-limiting, and complex medical conditions

www.littlemisshannah.org
Rare Disease

• 1 in 10 … 30 million Americans affected

• Over 7,000 different rare diseases
  • 83% of rare diseases have less than 6,000 US patients

• Over 50% of the diseases affect children
  • 30% of children will not live to see their 5th birthday

• Average diagnostic odyssey is 7 years
The Medical Science

• Only 289 of the 7,000 rare diseases have an FDA approved treatment
  • That’s less than 5%

• The science exists to treat many of these diseases

• Development of treatments is challenging
  • Difficult to get investment with small populations
  • Complicated regulatory environment
Progress?

• Over 500 new rare disease treatments developed since the passage of the Orphan Drug Act 30 years ago
  • **Only 8 rare disease drugs approved by the FDA in 2016**, down by 15 approved in 2015.

• **Innovation Gap:**
  • Many of the new treatments are for diseases that already have treatments available
  • In 2016, the FDA approved just 22 novel new medicines, a six-year low and half of the 45 approved in 2015.

• **WE MUST DO BETTER**
  • Public policy is needed to close the innovation gap
152 Organizations. 365 Registered Advocates

Our 6th Year on the Hill Together!
Your Conference Booklet

• Overview of the Week’s Events

• Conference Agenda

• Speaker Bio’s

• Maps – Metro, Capitol Hill & Event Locations (*end of book*)

• Overview of Each Bill/Legislative Ask (*pgs 33-39*)
  • Talking Points & Advocacy Tips for Your Hill Meetings
<table>
<thead>
<tr>
<th>Time</th>
<th>Session Topic</th>
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<tbody>
<tr>
<td>9:05 – 9:15am</td>
<td>Overview of the EveryLife Foundation for Rare Diseases</td>
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<tr>
<td>9:15 - 10:00am</td>
<td>What to Expect from the New Congress and Administration</td>
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<td>10:00 -10:45am</td>
<td>Top Health Policy Issues in 2017</td>
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<td>10:45 -11:00am</td>
<td>Break</td>
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<td>11:00 -11:45am</td>
<td>Rare Disease Legislation in the Queue</td>
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<tr>
<td>11:45 -12:30pm</td>
<td>Meet and Greet Lunch</td>
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12:30 -1:45pm Tricks of the Trade: Preparing for a Successful Meeting
1:45 -2:00pm Scenes from the Hill: Your License to Advocate
2:00 -2:30pm Continue the Progress Long After the Meeting: Ways to Stay Engaged
2:30 -3:15pm How to Engage with Federal Agencies
3:15 - 3:30pm Snack Break to Move to Breakout Sessions
3:30-4:15pm     Breakout Sessions - Pick One

   Refine Your Skills for Successful Meetings on the Hill – VISTA
   Rare Disease Issues State by State - ACADEMY
   Understanding the Clinical Drug Development Process – ANGLE
   Advocacy for Young Adults – BALCONY D (upstairs)

4:15-4:30 pm     Regroup in Academy Hall
4:30–5:00 pm     Closing Remarks
Participate to win a $50 Amazon Gift Card!

@RareAdvocates
#RareDC2017
Thank You!!!!

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