



2016 Legislative Conference Agenda

March 1st, 2016

FHI 360 - 1825 Connecticut Ave NW, 8th Floor, Washington, DC 20009

8:15 - 9:00am **Registration & Breakfast**

9:00 - 9:15am **Welcome & Overview**

Dean Suhr, Parent Advocate & President, MLD Foundation

9:15 - 10:00am **Election Madness: Campaign Promises and Legislative Realities**

What to Expect from Congress in Election Year:

Democratic Perspective: Emily Shetty, Consultant, the Stanton Park Group

Republican Perspective: Cheryl Jaeger, Principal, Williams & Jensen

- Impact of Election Year Politics on Legislative Business and Congress
- Importance of Congressional Committees

The Appropriations Process & Federal Support for Medical Research: Caitlin Leach, Director of Policy and Advocacy, Research!America

10:00 - 10:15am **Break**

10:15 - 11:15am **The Promise of Agency Partnership**

- **How Patients Can Engage with Food and Drug Administration (FDA):** Gayatri Rao, M.D., J.D., Director for the Office of Orphan Products Development, FDA
- **Patient Focused Drug Development:** Karen Erickson, Associate Executive Director, Community Engagement, Alpha-1 Foundation
- **Prescription Drug User Fee Act (PDUFA):** Eric Gascho, Vice President of Government Affairs, National Health Council (NHC)
- **How Patients Can Engage with National Institutes of Health (NIH):** Petra Kaufmann, M.D., M.Sc., Director of the Office of Rare Diseases Research and Division of Clinical Innovation, National Center for Advancing Translational Sciences, NIH

11:15 - 11:45am Rare Disease Legislation: A Historical Primer

- **Orphan Drug Act:** Paul Melmeyer, Associate Director of Public Policy, National Organization for Rare Disorders (NORD)
- **Priority Review Vouchers:** Ronald Barteck, Co-Founder/Founding President, Friedreich's Ataxia Research Alliance (FARA)

11:45 - 12:30pm Networking Lunch Break

12:30 - 1:30pm What Comes First? Priorities for Future Legislative Efforts

Moderator: Max Bronstein, Senior Director of Public Policy and Government Relations, EveryLife Foundation for Rare Diseases

- **Rare Disease Caucus:** Molly McDonnell, Legislative Assistant, Office of Representative Leonard Lance (NJ-7)
- **Orphan Product Extensions Now, Accelerating Cures and Treatments, OPEN Act (H.R. 971/S. 1421):** Saul Hernandez, Deputy Chief of Staff and Legislative Director, Office of Representative G.K. Butterfield (NC-1)
- **Patient Focused Impact Assessment Act, PFIA Act (S. 1597):** Annie Kennedy, Senior Vice President of Legislation & Public Policy, Parent Project Muscular Dystrophy
- **Rare Disease Fund Act of 2015 (H.R. 3731):** Scott Hinkle, Legislative Director, Office of Representative Juan Vargas (C-51)

1:30 - 2:10pm Putting Your Best Foot Forward: Tips and Tricks for a Successful Meeting

- **Building a Relationship with Members of Congress:** Lisa Schill, Vice President, RASopathies Network USA
- **Sharing Your Two Minute Personal Story:** Joseph LaMountain, Vice President, Reingold, Inc.
- **Congressional Score Cards, Hill Asks & Leave Behinds:** Vignesh Ganapathy, J.D., Associate Director of Advocacy and Government Relations, EveryLife Foundation for Rare Diseases
- **Being an Effective Advocate:** Stephanie Vance, Owner and Founder of Advocacy Associates

2:10 - 2:30pm Scenes from the Hill: A Mock Legislative Meeting

- **Member of Congress:** Jennifer Bernstein, Executive Vice President, Horizon Government Affairs
- **Legislative Aide:** Taylor Gilliam, Principal, Inside Pitch Government Affairs
- **Advocate #1:** Sarah Kucharski, CEO/Chairman and Founder, FMD Chat
- **Advocate #2:** Robert Ostrea, Co-Founder and Board Member, Little Miss Hannah Foundation
- **Advocate #3:** Jack Whelan, Research Advocate

2:30 - 2:55pm Next Steps: Your Path to Success

- **Continuing Positive Engagement:** Julia Jenkins, Executive Director, EveryLife Foundation for Rare Diseases
- **Effectively Utilizing Media and Social Media:** Steve Weiss, Senior Vice President of Health at Edelman and Board Member, Charcot-Marie-Tooth Association

2:55 - 3:15pm Break: Pick-up packets with Lobby Day schedule and leave-behind materials

3:15 - 3:55pm Breakout Sessions: Multiple Paths to Success: Working with Rare Disease Partners

- **How to Work with Industry:** Brett Kopelan, Executive Director, dEBra of America, and Barbara Wuebbels, RN, MS, Vice President of Patient Advocacy, Audentes Therapeutics
What are the do's and don'ts when trying to working with industry? In this session, learn how to create effective partnerships.
- **Advocacy on Social Media:** Stephanie Fischer, Senior Director of Patient Engagement and Communications, EveryLife Foundation for Rare Diseases
 - *How can you use social media in just 15 minutes a day to raise awareness, make connections and advocate for or against legislation?*
- **Understanding FDA Review Pathways:** Jeff Allen, Ph.D., Executive Director, Friends of Cancer Research
 - *Accelerated Approval, biomarkers, clinical vs. surrogate endpoints, Fast Track designation, orphan designation, and Breakthrough Therapy designation– What does this all mean?*

4:05 - 4:45pm Breakout Sessions: Rare Disease Issues, State by State

- **Modernizing Step Therapy:** Kristin Parde, Deputy Vice President, State Advocacy – Policy, Pharmaceutical Research and Manufacturers of America (PhRMA)
 - *Step Therapy requires that patients try a less costly treatment first before their insurance company will cover a more expensive therapy. What are the implications for patients?*
- **Rare Disease State Advisory Council:** Sharon King, President, Taylor's Tale, and Judith Mayer, Board Member, Taylor's Tale
 - *What is a Rare Disease State Advisory Council? How can you get one in your state?*
- **Newborn Screening:** Max Bronstein, Senior Director of Public Policy and Government Relations, EveryLife Foundation for Rare Diseases
 - *Learn why newborn screening is important, who recommends which tests to perform, and how the current state-by-state system could be improved.*

4:55 - 5:20pm State Meet and Greet

Practice makes perfect! Meet and work as a team to get ready and organized for successful Lobby Day meetings.

5:20 - 5:30pm Closing Remarks

- Logistics for Lobby Day
- Final Q&A