Presented By

RARE

DISEASE WEEK

ON CAPITOL HILL

FEBRUARY 27 - MARCH 2, 2017

PRESENTED BY

RDLA

RAREADVOCATES.ORG
THANK YOU TO OUR 2017 RARE DISEASE WEEK SPONSORS

Presenting Sponsors:

Grassroots Sponsors:

SPECIAL THANKS TO GILEAD FOR UNDERWRITING THE 2017 LEGISLATIVE CONFERENCE BOOKLET
Rare Disease Week 2017 Events

**Monday, February 27th**

**Rare Disease Day at NIH**
NIH Campus
9000 Rockville Pike
Building 10
Bethesda, MD 20892
8:30 am – 4:00 pm

**Rare Disease Documentary Screening and Cocktail Reception**
U.S. Naval Heritage Center
701 Pennsylvania Ave NW
Washington, DC 20004
Featuring the film “Up for Air”
5:30 – 9:30 pm

**Tuesday, February 28th**

**Legislative Conference**
FHI 360
1825 Connecticut Ave NW, 8th Floor
Washington, DC 20009
9:00 am – 5:00 pm

**Wednesday, March 1st**

**Lobby Day Breakfast**
Hyatt Regency Washington
400 New Jersey Ave NW
Washington, DC 20001
7:30 – 8:30 am

**Lobby Day Meetings**
U.S. Senate and House Office Buildings
9:00 am – 5:00 pm

**Rare Artist Reception**
Russell Senate Office Building
Kennedy Caucus Room
Washington, DC 20002
5:00 – 7:00 pm

**Thursday, March 2nd**

**Rare Disease Congressional Caucus Briefing**
Hart Senate Office Building
Room 902
Washington, DC 20002
12:00 – 1:30 pm

**Rare Disease Week Group Photo**
Capitol Steps
First Street NE
Washington DC
2:00 PM
Dear Advocate,

The passage of the 21st Century Cures Act last year was a tremendous victory for the rare disease community. This landmark legislation will bring the most significant changes to rare disease policy since the Orphan Drug Act was signed into law more than 30 years ago, and it could not have happened without your help. It was the tireless resolve of rare disease patients and caregivers from across the country who called, emailed and posted on social media urging Congress to pass the bill who created the momentum necessary to get it done at the very end of the legislative year.

We can take pride in what we accomplished together last year, but we should not be complacent. We have much more work to do. 95% of the more than 7,000 rare diseases still lack a treatment approved by the Food and Drug Administration. By joining us here, you are continuing the fight for public policy that encourages the development and availability of safe, effective and affordable treatments for rare disease patients.

This legislative conference will highlight legislation important to the rare disease community and give you the training and tools to be effective advocates at the state and federal levels. We hope that you will ask tough questions of our experts, and share your expertise with others when you return home. You are the voice of the rare disease community, and key to the success of Rare Disease Week on Capitol Hill.

This booklet includes an overview of the events during Rare Disease Week on Capitol Hill, talking points, maps and other tips to be successful in your Hill meetings. If you need anything during this week, feel free to contact one of us. We are here to help.

Thank you for being an advocate.

Sincerely,

The EveryLife Team

Share your experience!
@RareAdvocates
#Raredc2017
Have a question but don’t know who to ask? The staff of the EveryLife Foundation for rare diseases is here to help! Just look for one of these friendly faces and we’ll point you in the right direction.

**JULIA JENKINS**
Executive Director
Jjenkins@everylifefoundation.org

**CAROL KENNEDY**
Chief Development Officer
CKennedy@everylifefoundation.org

**MAX BRONSTEIN**
Chief Advocacy and Science Policy Officer
Mbronstein@everylifefoundation.org

**STEPHANIE FISCHER**
Senior Director, Patient Engagement and Communications
Sfischer@everylifefoundation.org

**SUE COLTON**
Director of Development
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**GRANT KERBER**
Deputy Director of Communications and Patient Programs
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**VIGNESH GANAPATHY**
Associate Director of Advocacy and Government Relations
Vganapathy@everylifefoundation.org

**LINDSEY CUNDIFF**
Office and Special Events Manager
Lcundiff@everylifefoundation.org

**LISA SCHILL**
RDLa Special Events Coordinator
Lschill@everylifefoundation.org
<table>
<thead>
<tr>
<th>Time</th>
<th>Session Title</th>
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<tr>
<td>8:30 - 9:00 AM</td>
<td><strong>Registration and Breakfast</strong></td>
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<tr>
<td>9:00 - 9:15 AM</td>
<td><strong>Welcome and Overview</strong></td>
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<td>• <strong>Conference Moderator</strong>: Robert Ostrea, Co-Founder, Little Miss Hannah Foundation</td>
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<td>• <strong>EveryLife Foundation Overview</strong>: Stephanie Fischer, Senior Director of Patient Engagement and Communications</td>
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<td>9:15 - 10:00 AM</td>
<td><strong>What to Expect from the New Congress and Administration</strong></td>
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<td>• <strong>How the New Congress and Administration Will Impact Rare Disease Policy</strong>:</td>
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<td>Democratic Perspective: Saul Hernandez, Deputy Chief of Staff, Office of Representative G.K. Butterfield</td>
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<td>Republican Perspective: Stuart Portman, Senior Healthcare Legislative Assistant, Office of Senator Orrin Hatch</td>
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<td>• <strong>Importance of Congressional Committees</strong>: Nick Manetto, Principal, Faegre Baker Daniels Consulting</td>
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<td>10:00 - 10:45 AM</td>
<td><strong>Top Health Policy Issues for 2017</strong></td>
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<td>• <strong>Moderator</strong>: Marilyn Vetter, Vice President of Government and Public Affairs, Horizon Pharma</td>
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<td>• <strong>Prescription Drug User Fee Act (PDUFA) Reauthorization</strong>: Sara Radcliffe, President and CEO, California Life Sciences Association</td>
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<td>• <strong>Affordable Care Act</strong>: Democratic Perspective: Wendell Primus, Senior Policy Advisor on Budget and Health, Office of Leader Nancy Pelosi</td>
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<td>Republican Perspective: Cheryl Jaeger, Principal, Williams &amp; Jensen</td>
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<td>• <strong>Keeping Medicaid and Children’s Health Insurance Program Strong for Children</strong>: James Baumberger, Associate Director of the Department of Federal Affairs, American Academy of Pediatrics</td>
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<td>10:45 - 11:00 AM</td>
<td><strong>Break</strong></td>
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### 11:00 - 11:45 AM
**RARE DISEASE LEGISLATION IN THE QUEUE**
- **Appropriations:** Funding for the National Institutes of Health (NIH) and Food and Drug Administration (FDA): Sara Chang, Director of Policy and Advocacy, Research!America
- **Healthcare Reform:** Paul Melmeyer, Associate Director of Public Policy, National Organization for Rare Disorders
- **Orphan Product Extensions Now, Accelerating Cures and Treatments (OPEN ACT):** Max Bronstein, Chief Advocacy and Science Policy Officer, EveryLife Foundation for Rare Diseases
- **Rebuilding and Expanding the Rare Disease Congressional Caucus:** Cristina Might, Executive Director, Ngly1.org

### 11:45 AM - 12:30 PM
**Meet-and-Greet Lunch**

Presented by [Bio](https://www.biot.org)

### 12:30 - 1:45 PM
**Tricks of the Trade: Preparing for a Successful Meeting**
- **Become an Empowered Advocate: How to Make the Most Impact:** Lisa Schill, Vice President, RASopathies Network USA
- **Congressional Scorecards, Hill Asks and Leave-Behinds:** Vignesh Ganapathy, Associate Director of Advocacy and Government Relations, EveryLife Foundation for Rare Diseases
- **Being an Effective Advocate:** Christopher Kush, CEO and Author, Soapbox

### 1:45 - 2:00 PM
**Scenes from the Hill: Your License to Advocate**
- **Member of Congress:** Jennifer Bernstein, Executive Vice President, Horizon Government Affairs
- **Legislative Aide:** Molly McDonnell, Principal, Winning Strategies Washington
- **Advocate #1:** Dean Suhr, Co-Founder and President, MLD Foundation
- **Advocate #2:** Pam Mace, Executive Director, Fibromuscular Dysplasia Society of America
- **Advocate #3:** Marc Yale, Executive Director, International Pemphigus and Pemphigoid Foundation
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<th>Time</th>
<th>Agenda Item</th>
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<td>2:00 - 2:30 PM</td>
<td><strong>Continue the Progress Long After the Meeting: Ways to Stay Engaged</strong></td>
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<td>• Follow Up, Do Not Drop Out: Best Practices for Staying in Touch and</td>
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<td>Social Media to Build Momentum: Emily Eckland, Digital and Social Media</td>
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<td>Communications Manager, Eli Lilly &amp; Company</td>
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<td>2:30 - 3:15 PM</td>
<td><strong>How to Engage with Federal Agencies</strong></td>
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<td>• Center for Disease Control (CDC): Annie Kennedy, Senior Vice President</td>
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<td>for Legislation and Public Policy, Parent Project Muscular Dystrophy</td>
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<td></td>
<td>• National Institutes of Health (NIH): Charles Mohan, Jr., CEO Executive</td>
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<td>Director, The United Mitochondrial Disease Foundation</td>
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<td>• Food and Drug Administration (FDA): Ryan Hohman, Vice President of Public</td>
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<td>Affairs, Friends of Cancer Research</td>
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<td>3:15 - 3:30 PM</td>
<td><strong>Snack Break to Move to Breakout Sessions</strong></td>
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**Presented By Vertex**

**Thank You to Doehmen Life Science Services for Sponsoring the Legislative Conference Family Room**

The EveryLife Foundation is providing nearly $80,000 in travel stipends through our Rare Giving program in order to enable rare disease advocates from across the country to participate in Rare Disease Week on Capitol Hill.

If you are a stipend recipient, please see foundation staff set-up near registration today to sign for your check. Please bring your photo ID.
### Breakout Sessions:

**3:30 - 4:15 PM**

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<tr>
<th>Breakout #1</th>
<th>Vista Room</th>
<th>Refine Your Skills for Successful Meetings on the Hill</th>
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<td>• Communications: Chris Smith, President and CEO, SmithSolve</td>
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<td>• Policy: Joel White, Founder and President, Horizon Government Affairs</td>
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<th>Breakout #2</th>
<th>Academy Hall</th>
<th>Rare Disease Issues State by State</th>
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<td>• Newborn Screening: Julia Jenkins, Executive Director, EveryLife Foundation for Rare Diseases</td>
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<td>• Creating a State Rare Disease Caucus: Craig Lincoln Tucker, Vice President of Policy and Public Affairs, Life Sciences Pennsylvania</td>
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<td>• Advocating for Rare Patient Access in Medicaid: Sue Landgraf, Executive Director, Cystic Fibrosis Research, Inc.</td>
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<th>Breakout #3</th>
<th>Angle Room</th>
<th>Understanding the Clinical Drug Development Process</th>
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<td>• Jeffrey Sherman, MD, FACP, Chief Medical Officer and Executive Vice President of Research and Development, Horizon Pharma</td>
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<tr>
<th>Breakout #4</th>
<th>Balcony D, Second Floor</th>
<th>Advocacy for Young Adults</th>
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<td></td>
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<td>• Shira Strongin, Founder, Sick Chicks</td>
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<td>• Emily Muller, Founder, Emily’s Fight</td>
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**Advocacy Tip:** It is important to understand the current political climate in your state legislature before you reach out to potential stakeholders.
### Conference Agenda

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<th>Time</th>
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<tr>
<td>4:15 - 4:30 PM</td>
<td><strong>Regroup in Academy Hall</strong></td>
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<td>4:30 - 5:00 PM</td>
<td><strong>Closing Remarks</strong></td>
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<td>• Logistics for Lobby Day</td>
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<td>• Final Q&amp;A</td>
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<td>5:00 PM</td>
<td><strong>End of Conference</strong></td>
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### Tomorrow:

**Lobby Day Breakfast**

**Wednesday, March 1st**

**Hyatt Regency Washington, CD Room**

**400 New Jersey Avenue NW**

**Washington, DC 20001**

**7:30 – 8:30 AM**

**Featuring Keynote Addresses by:**

- Janet Woodcock, MD, Director, FDA Center for Drug Evaluation and Research
- Representative Gus Bilirakis (R-FL)
- Former Representative Brian Baird (D-WA)

**At the Breakfast, attendees will receive any updates or last-minute changes to their Lobby Day Schedules.**
Robert is a co-founder of the Little Miss Hannah Foundation, whose mission is to help enhance the quality of life for young children diagnosed with life-limiting rare diseases, undiagnosed complex medical needs, and children who have been placed in hospice or palliative care in Southern Nevada. Robert is Dad to Ethan (16), Abigail (12) and Hannah, who lost her battle to Neuronopathic Gaucher’s Disease at the age of three. Robert has been involved in business development and promoting higher education for over fifteen years, working primarily with adult professionals in business, teacher education, information technology and healthcare. Influenced by his personal experience caring for Hannah coupled with his desire to be more effective in advancing the mission of the Little Miss Hannah Foundation, Robert pursued and completed an MBA with an emphasis in Healthcare Management and an MPA with an emphasis in Government in Policy. Robert lives with his wife Carrie and their children in Henderson, NV and continues to be a parent advocate of childhood rare disease and special needs issues.

As a rare disease patient and stroke survivor, Stephanie is passionate about rare disease advocacy. In her role as Senior Director of Patient Engagement and Communications at the EveryLife Foundation for Rare Diseases, she works with patients, caregivers and other rare disease advocates from across the country and appreciates the opportunity to represent the Foundation at roundtables and conferences. Stephanie utilizes the Foundation’s Facebook pages and Twitter handles to engage advocates on public policy, promote the Foundation’s programs and events, and share resources of interest to the rare community. Prior to joining the Foundation, Stephanie spent more than 10 years focused on communication at the Biotechnology Innovation Organization (BIO) and Pharmaceutical Research and Manufacturers of America (PhRMA). She previously worked for Representative Jim Greenwood (R-PA) for nine years until his retirement in 2004.
9:15 - 10:00 AM
WHAT TO EXPECT FROM THE NEW CONGRESSS AND ADMINISTRATION

SAUL HERNANDEZ
DEPUTY CHIEF OF STAFF, OFFICE OF REP. G.K. BUTTERFIELD

Saul serves as Deputy Chief of Staff and Legislative Director to Congressman G. K. Butterfield (D-NC), a member of the powerful House Energy and Commerce Committee and Chairman of the Congressional Black Caucus. Saul has worked on Capitol Hill for over a decade. In addition to supervising a busy legislative staff, Saul manages Congressman Butterfield’s work for all matters under the jurisdiction of the House Energy and Commerce Committee’s Subcommittees on Health and Communications and Technology. He previously managed Congressman Butterfield’s work on the Subcommittee on Commerce, Manufacturing, and Trade when he served as the Subcommittee’s Ranking Member. Saul also helps to manage the day-to-day operations of the Congressional office.

STUART PORTMAN
SENIOR HEALTH LEGISLATIVE ASSISTANT, OFFICE OF SENATOR ORRIN HATCH

Stuart serves as the Senior Healthcare Legislative Assistant for Senator Orrin Hatch (R-UT). In this role, he provides policy recommendations on a variety of health matters ranging from disease research and pharmaceutical development to health coverage for persons with disabilities, among others. Stuart also serves as deputy to the Medicaid adviser for Chairman Hatch, linking the Utah perspective to broader policy debates and working collaboratively on pressing Medicaid issues across the country. Stuart received his Master of Public Health degree specializing in health policy from the Milken Institute School of Public Health at The George Washington University.

ADVOCACY TIP: BECOME AN EXPERT ON AN ISSUE
BY FOLLOWING SOURCES YOU TRUST. IDENTIFY BLOGS,
COLUMNISTS, ORGANIZATIONS – EVEN TWITTER FEEDS!
Nick Manetto
Principal, Faegre Baker Daniels Consulting

Nick is a Principal on the Health and Biosciences team of Faegre Baker Daniels Consulting. Nick focuses a significant amount of this practice serving patient advocacy communities, healthcare providers and research institutions with a focus on children’s health and rare diseases and disorders. His work spans all assets of federal health policy and strategy including both Congress and the executive branch, and he has led or co-led multiple efforts that have resulted in concrete results through legislation enacted into law or through other means. Before joining Faegre, Nick spent some time on Capitol Hill where he worked on a number of medical research and public health projects. Before coming to Washington, he spent three years as a newspaper reporter at a daily in New Jersey. A resident of Herndon, Virginia, Nick and his wife Carrie have four young children.

Current Composition of Congress

**House of Representatives:**
- 243 Republicans
- 198 Democrats
- Average length of service: 9.4 years

**Senate:**
- 52 Republicans
- 46 Democrats
- 2 Independents
- Average length of service: 10.1 years
MARILYN VETTER  
VICE PRESIDENT OF GOVERNMENT AND PUBLIC AFFAIRS, HORIZON PHARMA

Marilyn is the Vice President of Government and Public Affairs at Horizon Pharma. Marilyn oversees the legislative and political environment for Horizon Pharma as well as the corporation’s civic engagement. She acts as a liaison between the corporation, its employees and government entities and personnel who have an impact on Horizon’s business affairs. With 24 years of experience in the pharmaceutical industry, she brings a depth of knowledge and experience to her position. She has worked in a myriad of capacities, including sales, account management and team leadership roles before becoming a government affairs principal in 2004. Her current position allows her to focus her energies on her interests of patient protections, healthcare policy and the future of the pharmaceutical industry.

SARA RADCLIFFE  
PRESIDENT AND CEO, CALIFORNIA LIFE SCIENCES ASSOCIATION

Sara was appointed the president and chief executive officer of the California Life Sciences Association (CLSA) in December 2014. She formerly served as the Executive Vice President for Health at the Biotechnology Innovation Organization (BIO). Previously, Sara served as Senior Director, Biologics and Biotechnology at the Pharmaceutical Research and Manufacturers of America (PhRMA). She also served in the Alliance and Technology Group at SmithKline Beecham Pharmaceuticals as a Research and Development Policy Analyst, working on evaluation and communication of the promise, ethics, and impact of rapidly-developing technologies in DNA Research. In addition she worked for the Core Services Committee of the New Zealand Ministry of Health. Sara holds a Master of Public Health and a Master of Arts in Philosophy from the Johns Hopkins University, and a Bachelor of Arts from Wellesley College.
Cheryl served as a senior policy advisor for the House Republican leadership, most recently with former Majority Leader Eric Cantor (R-VA). In this role, she led the advancement of healthcare reform legislation to improve patient choice, lower healthcare costs, and expand innovation. Jaeger previously served in the Office of the Majority Whip as a senior advisor to now Senator Roy Blunt (R-MO). Prior to serving as a leadership aide, Jaeger worked as a professional staff member for the Committee on Energy and Commerce. She was the lead staff negotiator of the NIH Reform Act of 2006, the Gabriella Miller Kids First Research Act, and several other pieces of legislation.

**WENDELL PRIMUS**  
SENIOR POLICY ADVISOR ON BUDGET AND HEALTH ISSUES, MINORITY LEADER NANCY PELOSI

Wendell is the Senior Policy Advisor on Budget and Health issues to Minority Leader Nancy Pelosi (D-CA). Prior to this appointment in March 2005, Wendell was the Minority Staff Director at the Joint Economic Committee. Prior to that position, he was the Director of Income Security for the Center on Budget and Policy Priorities in Washington, D.C. He previously served in the Clinton Administration as the Deputy Assistant Secretary for Human Services Policy at the Department of Health and Human Services. In that position, Wendell was primarily responsible for policy development and for the conduct of research and evaluation on issues relating to income assistance, employment and related human services programs. He received his Ph.D. in economics from Iowa State University.

**CHERYL JAEGER**  
PRINCIPAL, WILLIAMS & JENSEN

Cheryl served as a senior policy advisor for the House Republican leadership, most recently with former Majority Leader Eric Cantor (R-VA). In this role, she led the advancement of healthcare reform legislation to improve patient choice, lower healthcare costs, and expand innovation. Jaeger previously served in the Office of the Majority Whip as a senior advisor to now Senator Roy Blunt (R-MO). Prior to serving as a leadership aide, Jaeger worked as a professional staff member for the Committee on Energy and Commerce. She was the lead staff negotiator of the NIH Reform Act of 2006, the Gabriella Miller Kids First Research Act, and several other pieces of legislation.

**ADVOCACY TIP:** **MAKING AN IMPACT ON CAPITOL HILL**  
REQUIRES STRONG RELATIONSHIPS. FOLLOW-UP WITH STAFF AFTER MEETINGS TO BUILD THOSE RELATIONSHIPS.
James Baumberger  
ASSOCIATE DIRECTOR OF THE DEPARTMENT OF FEDERAL AFFAIRS, AMERICAN ACADEMY OF PEDIATRICS

James is an associate director in the American Academy of Pediatrics (AAP) Department of Federal Affairs, based in Washington, DC. He has been at the AAP since 2007. He advocates for the needs of children and adolescents before Congress and the Executive Branch and focuses on a number of policy issues including those related to biomedical research, substance abuse, adolescence, medical workforce, and subspecialty pediatrics. He is also the primary federal affairs representative for the Society for Adolescent Health and Medicine. He has worked extensively on federal tobacco regulation and Food and Drug Administration laws related to pediatric drugs and devices for children. He holds a Master’s in Public Policy from The George Washington University.

“MOST PATIENTS DON’T REALIZE THAT PUBLIC POLICY IMPACTS THE DEVELOPMENT OF THERAPIES AND CURES, ACCESS TO THOSE TREATMENTS AND THEIR AFFORDABILITY. ENSURING RARE DISEASE PATIENTS HAVE AN OPPORTUNITY TO INFLUENCE THESE LIFE AND DEATH POLICY DECISIONS IS RDLA’S TOP PRIORITY.”

- EVERYLIFE FOUNDATION  
EXECUTIVE DIRECTOR, JULIA JENKINS
SARA CHANG
DIRECTOR OF POLICY AND ADVOCACY, RESEARCH!AMERICA

Sara serves as the Director of Policy and Advocacy at Research!America. In her role, Ms. Chang develops and executes policy and advocacy strategies to increase public and policymaker awareness of the health and economic benefits of medical research. Prior to joining Research!America, she was with the Lupus Foundation of America for 10 years, most recently as the Director for Advocacy and Government Relations. During her tenure at the Lupus Foundation of America, Sara advocated to secure funding for lupus research, education, and awareness. Additionally, she provided leadership on patient advocacy priorities for the Foundation. Prior to her work with the Lupus Foundation of America, she worked for Congressman Tom Osborne (R-NE). Sara has a B.S. in Political Science from the University of Nebraska at Kearney. She currently resides in Alexandria with her husband and two sons.

PAUL MELMEYER
ASSOCIATE DIRECTOR OF PUBLIC POLICY, NATIONAL ORGANIZATION FOR RARE DISORDERS

Paul serves as the Associate Director of Public Policy at the National Organization for Rare Disorders (NORD). In this role, Paul engages in federal congressional and regulatory advocacy on behalf of rare disease patients and the organizations that serve them. His overriding mission is to improve the plight of patients with rare diseases and increase incentives for the development of orphan drugs, devices, and diagnostics. Since joining NORD in 2013, Paul has advocated successfully for the passage of various laws and has crafted comments on NORD’s behalf for numerous rules and guidances. Prior to joining NORD, Paul spent time at the Center for American Progress, AARP, and in the Senate.
Max is the Chief Advocacy and Science Policy Officer at the EveryLife Foundation for Rare Diseases. In this role, Max works closely with patient groups, industry, Congress, and key government agencies to accelerate the development of new, life-saving therapies for rare disease patients. Max is the former Director of Science Policy at Research!America and has held various positions in the U.S. government, including at the National Science Foundation, House Science Committee, and the National Institutes of Health. In 2010, Max founded a non-profit organization, the Journal of Science Policy & Governance, to empower students and young scholars to publish their work. He holds a master’s degree in public policy and a certificate in science and technology policy from the Ford School at the University of Michigan. Max earned his BA in biology from Ithaca College, with a minor in writing.

Cristina is the proud “mom” of 60 children: 3 biological and 58 with the rare disease NGLY1 deficiency. In September 2012, she started NGLY1.org after her oldest son, Bertrand, became the first person diagnosed with NGLY1 deficiency. As the Executive Director for the nonprofit, Cristina believes that partnership between patients, families, researchers, clinicians and legislators is essential to accelerate science for rare diseases—understanding, treatments and cures. She brings her background as a former tech CEO to run the day-to-day operations of NGLY1.org. Cristina holds a BS in Industrial Design and MBA in Finance and Accounting from Georgia Tech.
Lisa Schill
VICE PRESIDENT, RASOPATHIES NETWORK USA

Lisa is the RDLA Special Events Program Coordinator for the EveryLife Foundation for Rare Diseases. She is a parent advocate who specializes in connecting caregivers, researchers, support organizations, and families to help support patients in the pursuit of advancing treatment options and patient outcomes. Lisa voluntarily serves as the Vice President of the RASopathies Network USA, a non-profit whose mission is to advance research of the RASopathies by bringing together families, clinicians, and scientists. She was a Principal Investigator for the 2015 International Meeting on the Genetic Syndromes on the RAS/MAPk Pathway.

Vignesh Ganapathy
ASSOCIATE DIRECTOR OF ADVOCACY AND GOVERNMENT RELATIONS, EVERYLIFE FOUNDATION

Vignesh serves as the Associate Director of Advocacy and Government Relations at the EveryLife Foundation for Rare Diseases. With his background in law and technology policy, he fosters grassroots patient advocacy and ensures that patients are heard in both state and federal government. Prior to joining the Foundation, Vignesh lobbied for the Civil Justice Association of California to streamline regulatory pathways and foster an entrepreneurial climate in California. He previously staffed the State Assembly Judiciary Committee on technology issues. Vignesh is a graduate of the University of California Santa Cruz, and the McGeorge School of Law, where he studied in a program with Supreme Court Justices Anthony Kennedy and Elena Kagan. He has a deep passion for civic engagement and currently sits on the board of the New Leaders Council – Sacramento.
TRICKS OF THE TRADE: PREPARING FOR A SUCCESSFUL MEETING

CHRISTOPHER KUSH
CEO AND AUTHOR, SOAPBOX

As CEO of Soapbox Consulting, Christopher has trained hundreds of thousands of citizens from all over the United States to effectively influence Congress, state, and local governments. He has helped design sophisticated key-contact networks, Lobby Days, and grassroots training programs for many national associations including the American Cancer Society, Human Rights Campaign, Easter Seals, Goodwill International, United Way Worldwide, and the American Wind Energy Association. Mr. Kush is the author of three books on grassroots organizing including Grassroots Games (ASAE, 2002) and, most recently, The One-Hour Activist (Wiley, 2004). He has appeared on National Public Radio, ABC and Fox-news affiliates, C-SPAN’s “Book TV,” and in U.S. News & World Report, The Los Angeles Times, and on the Sirius Satellite Radio Network.

ADVOCATES ON THE HILL
JENNIFER BERNSTEIN
EXECUTIVE VICE PRESIDENT, HORIZON GOVERNMENT AFFAIRS

Jennifer joined Horizon Government Affairs in December 2008, where she currently serves as Executive Vice President, focusing on legislative and regulatory developments within the pharmaceutical and biotechnology sectors. Jennifer is also a former Capitol Hill staffer, as well as a former staffer for the Pennsylvania House of Representatives. She has worked closely with RDLA since its inception and is personally and professionally committed to advancing the needs of the rare disease community.

MOLLY MCDONNELL
PRINCIPAL, WINNING STRATEGIES WASHINGTON

Molly is a Principal at the lobbying firm Winning Strategies Washington, where she works with clients from across the healthcare sector to creatively tackle their most pressing policy challenges. Prior to joining Winning Strategies, Molly spent five and a half years working for Congressman Leonard Lance (R-NJ), a senior member of the Energy and Commerce Committee. Molly served as Congressman Lance’s Senior Health Policy Advisor working on issues related to the Food and Drug Administration, rare disease, drug development and medical research. Molly also supported the Congressman’s work with the Rare Disease Congressional Caucus, helping to educate, advocate, and develop public policy to improve the lives of patients suffering from rare disease.

“A CHANGE IS BROUGHT ABOUT BECAUSE ORDINARY PEOPLE DO EXTRAORDINARY THINGS.”

-PRESIDENT BARACK OBAMA
SCENES FROM THE HILL:
YOUR LICENSE TO ADVOCATE

1:45 - 2:00 PM

DEAN SUHR
CO-FOUNDER AND PRESIDENT, MLD FOUNDATION

Dean is co-founder and president of the MLD Foundation serving families around the world with metachromatic leukodystrophy, a rare terminal neuro-metabolic condition. Two of his three children have MLD, Darcee passed away at age 10 in 1995 and Lindy (36) is alive and doing better than expected. Dean is active on behalf of MLD, leukodystrophy, lysosomal and rare disease communities with special interests in global health policy (RarePolicy.us), newborn screening (NewbornScreening.us /RUSP Roundtable), registries, diagnostics, consent, privacy, ethics, openNHS, FDA/NIH issues, empowering advocacy organizations, research consortiums (GLIA and LDN/WORLD) and the emerging trend of centers of clinical research excellence.

PAM MACE
EXECUTIVE DIRECTOR, FIBROMUSCULAR DYSPLASIA SOCIETY OF AMERICA

Pam is a registered nurse with more than 20 years of experience. After suffering a life threatening event in 2000 Pam was diagnosed with a rare disease called Fibromuscular Dysplasia (FMD). Since her diagnosis Pam has been very active in the rare disease community and she serves as the Executive Director of the Fibromuscular Dysplasia Society of America. Her accomplishments lead to a very successful awareness campaign and an increase in patients being diagnosed with FMD. Pam was instrumental in the development of the FMD Patient Registry and the formation of the International Research Network for Fibromuscular Dysplasia.

MARC YALE
EXECUTIVE DIRECTOR, INTERNATIONAL PEMPHIGUS AND PEMPHIGOID FOUNDATION

Marc was diagnosed in 2007 with Cicatricial Pemphigoid, a rare autoimmune blistering skin disease. Like others with a rare disease, he experienced delays in diagnosis and difficulty finding a knowledgeable physician. Eventually, Marc lost his vision from the disease. This inspired him to help others with the disease. In 2008, he joined the International Pemphigus and Pemphigoid Foundation (IPPF) as a Peer Health Coach. He works with people to improve their quality of life, and encouraged them become self-advocates. He is a two-time Rare Voice Award nominee, a Global Genes RARE Champion of Hope nominee and a national advocate for rare diseases. Marc currently resides in Ventura, California and is the Executive Director for the IPPF.
EMILY ECKLAND
DIGITAL AND SOCIAL MEDIA COMMUNICATIONS MANAGER, ELI LILLY & COMPANY

Emily is a Digital and Social Media Communications manager at Eli Lilly & Company. Emily focuses on Lilly’s public policy efforts and manages their LillyPad blog and Campaign for Modern Medicines and Lilly Digital Advocacy Institute websites. Prior to joining Lilly, Emily ran strategic partnerships and digital strategy at a cybersecurity non-profit and social media for a Maryland hospital. She also spent six years working as a journalist in D.C.
Chuck is the founder of the United Mitochondrial Disease Foundation. The UMDF initially began in the basement of his home in 1996 and has grown to an international operation represented in every state in the US and over 150 countries providing hope, energy and life to the thousands of people affected by mitochondrial disease. Chuck was hired as the UMDF’s Chief Executive Officer in 2006, where he remains the driving force behind the foundation, helping many families cope with the crisis of mitochondrial disease. Chuck owns a restaurant in Pittsburgh and also taught at a local high school from 1972-1979 and has served as a councilman for his local municipality. Chuck and his wife, Adrienne, lost their 15-year-old daughter Gina to mitochondrial disease in 1995.

Focused on improving health outcomes for people living with Duchenne muscular dystrophy, Annie’s work includes building strong partnerships with policy makers, federal agencies, Industry, and alliances that can serve as force multipliers in moving Duchenne community priorities forward. Current areas of emphasis include PDUFA VI, implementation of key provisions within 21st Century Cures including the Patient Focused Impact Assessment Act, MD-CARE Act implementation, engagement with the FDA and Industry around regulatory policy and therapeutic pipelines, recent FDA advisory committees for Duchenne products, a national newborn screening program, resources for adults with Duchenne, optimizing clinical trial infrastructure, and drug coverage and access issues. Annie currently serves on the Board of Directors of Cure SMA, as Co-Chair of the National Health Council’s Medical Innovation Action Team, as a Design Team member of the NCATS/ORDR Tool Kit Project and a member of the Secondary Research Review Board for Biogen.
Ryan currently serves as Vice President of Public Affairs at Friends of Cancer Research (Friends). Friends is an advocacy organization based in Washington, DC that drives collaboration among partners from every healthcare sector to power advances in science, policy, and regulation that speed life-saving treatments to patients. During the past 20 years, Friends has been instrumental in the creation and implementation of policies ensuring patients receive the best treatments in the fastest and safest way possible. During his diverse career, Ryan has experienced first-hand the vital need and incredible impact that sustained federal funding of the biomedical field has on physicians, researchers, and scientists and the difficulties many of these communities face when engaging in and navigating the regulatory process. Before joining Friends, Ryan was Director of Corporate and Institutional Partnerships at Georgetown University Medical Center-Lombardi Cancer Center.

**DEPARTMENT OF HEALTH AND HUMAN SERVICES (HHS)**

Secretary Thomas Price, MD

HHS is the U.S. government’s principal agency for protecting the health of all Americans and providing essential human services. HHS is responsible for almost a quarter of all federal outlays and administers more grant dollars than all other federal agencies combined.

[www.hhs.gov](http://www.hhs.gov)

HHS has 11 operating divisions, including eight agencies in the U.S. Public Health Service and three human services agencies. The following will be discussed today.

**FOOD AND DRUG ADMINISTRATION (FDA)**

Acting Commissioner Stephen Ostroff, MD

FDA is responsible for protecting the public health by ensuring the safety, efficacy, and security of human and veterinary drugs, biological products, and medical devices; and by ensuring the safety of our nation’s food supply, cosmetics, and products that emit radiation.

[www.fda.gov](http://www.fda.gov)

**NATIONAL INSTITUTES OF HEALTH (NIH)**

Director Francis Collins, MD, PhD

NIH supports biomedical and behavioral research within the U.S. and abroad, conducts research in its own laboratories and clinics, trains promising young researchers, and promotes collecting and sharing medical knowledge.

[www.nih.gov](http://www.nih.gov)

**CENTERS FOR DISEASE CONTROL AND PREVENTION (CDC)**

Acting Director Anne Schuchat, MD (RADM, USPHS)

CDC protects the public health of the nation by providing leadership and direction in the prevention and control of diseases and other preventable conditions, and responding to public health emergencies.

[www.cdc.gov](http://www.cdc.gov)
BREAKOUT SESSION:
REFINE YOUR SKILLS FOR SUCCESSFUL MEETINGS ON THE HILL

HOW DO YOU TELL YOUR STORY AND COHESIVELY TIE-IN YOUR “ASK” WHEN MEETING WITH A MEMBER OF CONGRESS? IN THIS SESSION, REFINE YOUR ELEVATOR PITCH, FINE-TUNE YOUR LEGISLATIVE TALKING POINTS AND GET YOUR POLICY QUESTIONS ANSWERED.

CHRIS SMITH
PRESIDENT AND CEO, SMITHSOLVE

Chris has more than two decades of experience in healthcare communications, including leadership positions with global pharmaceutical, biotechnology and public relations firms. He founded SmithSolve in 2006 with the conviction that communications is an increasingly powerful tool for all stakeholders in healthcare. Chris specializes in rare disease and helps people understand scientific, medical, clinical, social and economic aspects of orphan drug development. He has experience with dozens of patient communities through programs that include patient advocacy, patient meet ups, media relations, investor events, patient days, clinical trial recruitment, clinical data announcements, product launches, corporate branding, website development and more. SmithSolve is a member of the Global Genes Corporate Alliance and a founding member of the Rare Collective, a group of trusted independent advisors.

JOEL WHITE
FOUNDER AND PRESIDENT, HORIZON GOVERNMENT AFFAIRS

Joel is the President of Horizon Government Affairs (HGA), a full service government affairs consultancy specializing in actionable strategic and tactical advice for navigating the Congressional and regulatory processes. HGA assists clients in their quest to improve health and lower health related costs. Joel is also the Executive Director of the Health IT Now Coalition and is the President of the Council for Affordable Health Coverage, two HGA-managed coalitions. Joel spent twelve years on Capitol Hill as professional staff for the Ways and Means Committee and two Members of Congress. He helped enact nine laws, including the 2002 Trade Act, which created healthcare tax credits, the 2003 law that established the Medicare prescription drug benefit and Health Savings Accounts, the 2005 Deficit Reduction Act, and the 2006 Tax Reform and Health Care Act, which reformed Medicare payment policies.
There is so much legislative work you can do in your own backyard! Learn how the newborn screening state-by-state system can be improved, how to advocate for the creation of a rare disease caucus in your home state, and why experts should be involved in the Medicaid decision-making process.

JULIA JENKINS  
EXECUTIVE DIRECTOR, EVERYLIFE FOUNDATION

Julia served as the Director of Public and Government Relations when the Foundation was created in 2009, and is now the Executive Director. Her background in grassroots organizing, political strategy and legislative advocacy helped unite more than 180 patient organizations under the CureTheProcess Campaign. She worked to ensure ULTRA/FAST was included in the 2012 FDA Safety and Innovation Act (FDASIA). Julia initiated RDLA to serve as a communication platform for rare disease stakeholders to work on legislative issues and to help elevate the voice of rare disease patients on Capitol Hill. Prior to working at the Foundation, Julia worked as a political communications consultant, creating strategic plans to organize communities to support local funding measures. Julia was Legislative Director and registered California state lobbyist for Public Employees Union Local #1 and worked for House Minority Leader Nancy Pelosi.

CRAIG LINCOLN TUCKER  
VICE PRESIDENT OF POLICY AND PUBLIC AFFAIRS, LIFE SCIENCES PENNSYLVANIA

Craig has been a lobbyist in Harrisburg, PA for over 16 years. As a partner at Novak Strategic Advisors, he represented many different industries. In 2014, he sold his shares and joined Life Sciences Pennsylvania. He is responsible for the legislative agenda for the organization in both Harrisburg and Washington, D.C. Life Sciences Pennsylvania is the statewide trade association for the life sciences with over 700 member companies. Craig graduated from Dickinson College with a degree in German and has as Masters in Government Administration from the Fels School of Government at the University of Pennsylvania.
Sue Landgraf
Executive Director, Cystic Fibrosis Research, Inc.

Sue is a UC Berkeley graduate and has been in executive positions at several nonprofits over the past 26 years. She is the Executive Director for Cystic Fibrosis Research, Inc. (CFRI) in Palo Alto, CA. CFRI is dedicated to funding research, providing education and support, and increasing awareness of cystic fibrosis, a genetic disease without a cure. Sue turned to CFRI for help over 26 years ago after her now 32-year-old daughter was diagnosed with CF. Inspired by her daughter, a liver and double lung transplant recipient, UC Berkeley graduate, and married for 14 years, Sue brings her personal passion to work. Her memberships include Rare Disease Legislative Advocates, Global Genes Alliance, founding member of CF Engagement Network, and new member of the American Thoracic Society Public Advisory Roundtable.

RARE DISEASE ISSUES AT THE STATE LEVEL

- **Right-to-Try** — Allows for access to investigational drugs before they are approved by FDA
- **EXPEER Act** — Allows for experts to be involved in the Medicaid decision-making process
- **Specialty Tier Co-pay** — Requires patients to pay a percentage of the cost of drugs
- **Step Therapy** — Requires patients to “fail first” on a cheaper therapy
- **Medical Foods** — Provides coverage or financial assistance of medical foods
- **Newborn Screening** — Requires states to screen for life-threatening diseases preventing death and disability
- **Rare Disease State Advisory Councils** — Creates a State-level Advisory Council
Jeff is Chief Medical Officer and Executive Vice President of Research and Development at Horizon Pharma based in Lake Forest, Illinois. He has over 25 years of experience in the biopharmaceutical research and development. Jeff received his bachelor degree in biology from Lake Forest College and medical degree from the Rosalind Franklin University of Medicine and Science/The Chicago Medical School. He completed an internship, residency, and chief medical residency in internal medicine at Northwestern University. Additionally, he completed fellowship training in infectious diseases at the University of California-San Francisco (UCSF) and was a research associate at the Howard Hughes Medical Institute at UCSF in allergy and immunology. Jeff is an Adjunct Assistant Professor of Medicine at the Northwestern University Feinberg School of Medicine and a member of a number of professional societies as well as a Diplomat of the National Board of Medical Examiners and the American Board of Internal Medicine. Jeff is a Past President of the Drug Information Association (DIA) and a former member of the Board of Directors. He also was chairperson of the DIA Annual Meeting, is an inaugural fellow, and serves as the DIA liaison to the FDA Clinical Trial Transformation Initiative (CTTI) Steering Committee. In addition, Jeff serves on the Board of Advisors of the Center for Information and Study on Clinical Research Participation (CISCRP).

“YOU MAY NEVER KNOW WHAT RESULTS COME FROM YOUR ACTION. BUT IF YOU DO NOTHING, THERE WILL BE NO RESULT.”

-MAHATMA GANDHI
SHIRA STRONGIN
DEPUTY VICE PRESIDENT, PHRMA

At 17, Shira is an accomplished, award winning activist and writer who has multiple rare diseases that she does not let stop her. She is the founder of Sick Chicks, an international community that works to empower and unite women with disabilities and illnesses. Her writing has been published in places such as Forbes and she has a passion for speaking on topics such as disability rights, women’s rights, and engaging the young adult community in advocacy. When Shira isn’t lobbying the House and Senate about health policy, she enjoys playing and listening to music, swing dancing, and binge-watching Netflix.

EMILY MULLER
FOUNDER, EMILY’S FIGHT

Emily established herself as a prominent activist in the rare disease community when she launched Emily’s Fight, an awareness campaign, at the age of thirteen. Although she never expected her passion project to gain an international following, she tries to put it to good use. Never afraid to talk about taboo subjects, Emily frequently addresses young adult issues and the mental health effects of serious illness. She has spoken at such events as TEDx, the Global Genes Patient Advocacy Summit, SHINE, and the Starlight Children’s Foundation Midwest Gala. She has also contributed written pieces to The Mighty and To Write Love On Her Arms. When she’s not doing advocacy work, Emily enjoys acting in film projects local to the Chicago area, cult classic movies, and trying (unsuccessfully) to bring back 90s trends.
BACKGROUND: In 2014, the House Energy and Commerce Committee began a bipartisan initiative to improve biomedical innovation in the U.S. This effort would come to be known as the 21st Century Cures initiative and was led by Representatives Fred Upton (R-MI) and Diana DeGette (D-CO). Under their leadership, the Committee convened a wide variety of stakeholders from the biomedical community to provide input on the legislation.

PATIENT ADVOCACY: Throughout the legislative process, rare disease patients and patient organizations engaged with the House and Senate in hearings, action alerts and meetings on Capitol Hill. This two year advocacy campaign had highs and lows, but for many, the bill represented a bipartisan opportunity to make progress in advancing treatments for rare disease patients.

THE VICTORY: After moving through the House with a strong bipartisan vote of 392 – 26 and the Senate with a vote of 94 – 5, the 21st Century Cures Act advanced to the White House. The Act was signed into law by President Obama on December 13th, 2016. Rare disease patient advocates were invited to the White House for the signing ceremony.
HOUSE AND SENATE ASKS:

✓ FUNDING FOR HEALTH RESEARCH AND BIOMEDICAL INNOVATION

✓ ORPHAN PRODUCT EXTENSIONS NOW, ACCELERATING CURES AND TREATMENTS (OPEN ACT)

✓ HEALTHCARE REFORM

✓ RARE DISEASE CONGRESSIONAL CAUCUS
FUNDING FOR HEALTH RESEARCH AND BIOMEDICAL INNOVATION

SUPPORT ROBUST FUNDING FOR THE NATIONAL INSTITUTES OF HEALTH (NIH), THE FOOD AND DRUG ADMINISTRATION (FDA), AND 21ST CENTURY CURES IMPLEMENTATION

NIH and FDA are vital for advancing rare disease research and innovation. Funding for NIH has struggled to keep pace with inflation and is critical for advancing the basic science needed to enhance our understanding of rare diseases. In addition, the NIH Clinical Center and Undiagnosed Disease Program provide life-saving resources for rare disease patients searching for answers.

The FDA is substantially underfunded given the Agency regulates 25% of the national economy. The FDA needs additional funding to fully implement the changes required by the 21st Century Cures Act, and to help improve the drug review and approval process.

HOUSE AND SENATE TALKING POINTS: HELP FUND FEDERAL AGENCIES

✓ We request a $2 billion increase in discretionary funding for the National Institutes of Health (NIH) for fiscal year 2018.
✓ We request a $2.8 billion increase in discretionary funding for the Food and Drug Administration (FDA) in fiscal year 2018.
✓ Ensure that the supplemental funds authorized in the 21st Century Cures Act are provided, including $500M for the NIH Innovation Fund and $60M for the FDA Innovation Fund.
The Orphan Products Extension Now, Accelerating Cures and Treatments (OPEN ACT) is legislation that holds the potential to double the number of treatments available to rare disease patients. The bill would create a six month exclusivity extension for companies that repurpose existing therapies for a rare disease indication. To date, more than 170 patient organizations have signed-on in support of this legislation and the bill has bipartisan sponsorship in both the House and Senate from Representatives Bilirakis (R-FL) and Butterfield (D-NC) and Senators Hatch (R-UT) and Klobuchar (D-MN).

**HOUSE AND SENATE TALKING POINTS: PLEASE CO-SPONSOR THE OPEN ACT**

✓ There are more than 7,000 rare diseases, 95% of which have no FDA-approved treatment. The OPEN ACT could potentially double the number of treatments available to rare disease patients.
✓ Rare-purposed drugs will be priced at major market prices, bringing down the average cost of rare disease drugs.
✓ Fewer rare disease patients will need to use off-label medications which are potentially ineffective. Off-label drugs are often not reimbursed placing a huge financial burden on patients.
Prior to enactment of the Affordable Care Act in 2010, many rare disease patients were unable to access health care coverage due to discriminatory insurance practices, limited Medicaid eligibility, and debilitating cost-sharing requirements. While not perfect, the Affordable Care Act (ACA) reformed these practices by forbidding insurers from discriminating against rare disease patients, outlawing annual and lifetime caps, expanding Medicaid, and improving Medicare Part D coverage for patients who are among the most vulnerable.

Over the past several years, there have been many attempts by Congress to repeal the ACA, but those efforts were mainly symbolic. Now, things are different. With a Republican-controlled Senate, House and White House, efforts to repeal/revamp the ACA are likely to succeed.

**HOUSE AND SENATE TALKING POINTS:** PLEASE ENSURE THAT RARE DISEASE PATIENTS DO NOT LOSE ACCESS TO AFFORDABLE, LIFE-SAVING HEALTH INSURANCE COVERAGE. IN ORDER FOR ANY ACA REPLACEMENT PLAN TO AVOID DETRIMENTAL CONSEQUENCES FOR RARE DISEASE PATIENTS, WE MUST:

✓ Ensure that nobody will be denied affordable health insurance due to a pre-existing medical condition.
✓ Ban all annual and lifetime limits on benefits and coverage.
✓ Retain the maximum out-of-pocket spending limit so that patients don’t face medical bankruptcy.
✓ Ensure that all rare disease patients currently on Medicaid maintain their coverage.
✓ Keep vital programs that allow for in-home care and provide coverage for terminally ill children.
✓ Allow children to remain on their parents’ health plans until age 26.
A caucus is an interest group of Members of Congress who seek to raise awareness about a specific issue on Capitol Hill. The Rare Disease Congressional Caucus was established in the House in 2010 and now encompasses both the House and the Senate. There are more than 100 members in the House, with Representatives Lance (R-NJ) and Butterfield (D-NC) serving as co-chairs. In late 2015, Senators Hatch (R-UT) and Klobuchar (D-MN) agreed to serve as co-chairs, expanding the Caucus into the Senate. The Congressional Rare Disease Caucus provides a vital platform for discussing pressing policy issues and giving rare disease patients a voice in Washington, D.C.

SUGGESTED TALKING POINTS:

JOIN THE CONGRESSIONAL CAUCUS

✓ IF ALREADY A CAUCUS MEMBER: Thank you so much being part of the Rare Disease Congressional Caucus.
✓ IF NOT A CAUCUS MEMBER: Please join more than 100 of your colleagues in the Rare Disease Congressional Caucus.
✓ FOR ALL MEETINGS: The Caucus is hosting a briefing tomorrow at noon in room 902 of the Hart Senate Office Building. We encourage you and your staff to attend.
Thank you to our Rare Disease Congressional Caucus Co-Chairs

Senator Orrin Hatch (R-UT)
Senator Amy Klobuchar (D-MN)
Representative G.K. Butterfield (D-NC)
Representative Leonard Lance (R-NJ)

The Rare Disease Congressional Caucus is coordinated by RDLA
SENATE:
TOM COTTON (AR)
ORRIN HATCH (UT)
AMY KLOBUCHAR (MN)
JAMES RISCH (ID)
DAVID VITTER (LA)

HOUSE:
MARK AMODEI (NV-2)
LOU BARLETTA (PA-11)
ANDY BARR (KY-6)
AMI BERA (CA-7)
EDDIE BERNICE JOHNSON (TX-30)
DONALD BEYER JR. (VA-8)
GUS BILIRAKIS (FL-12)
MARSHA BLACKBURN (TN-7)
ROD BLUM (IA-1)
SUZANNE BONAMICI (OR-1)
MO BROOKS (AL-5)
JULIA BROWNLEY (CA-26)
VERN BUCHANAN (FL-16)
MICHAEL BURGESS (TX-26)
CHERI BUSTOS (IL-17)
G.K. BUTTERFIELD (NC-1)
MIKE CAPUANO (MA-7)
JOHN CARNEY (DE)
ANDRE CARSON (IN-7)
JOHN CARTER (TX-31)
DAVID CICILLINE (RI-1)
BARBARA COMSTOCK (VA-10)
GERALD CONNOLLY (VA-11)
RYAN COSTELLO (PA-6)
JOSEPH CROWLEY (NY-14)
RODNEY DAVIS (IL-13)
SUZAN DELBENE (WA-1)
JEFF DENHAM (CA-10)
CHARLIE DENT (PA-15)
TED DEUTCH (FL-21)
JOHN DUNCAN, JR. (TN-2)
ELIOT ENGEL (NY-16)
ANNA ESHOO (CA-18)
STEPHEN FINCHER (TN-8)
JEFF FORTENBERRY (NE-1)
JOHN GARAMENDI (CA-3)
GREGG HARPER (MS-3)
DENNY HECK (WA-10)
JAIME HERRERA-BEUTLER (WA-3)
JIM HIMES (CT-4)
JARED HUFFMAN (CA-2)
DARRELL ISSA (CA-49)
LYNN JENKINS (KS-2)
HANK JOHNSON (GA-4)
DAVID P. JOYCE (OH-14)
MARCY KAPTUR (OH-9)
JOSEPH KENNEDY III (MA-4)
PETER KING (NY-2)
DARIN LAHOOD (IL-18)
LEONARD LANCE (NJ-7)
DAN LIPINSKI (IL-3)
FRANK LOBIONDO (NJ-2)
DAVE LOEBSACK (IA-2)
ZOE LOFgren (CA-19)
TOM MACARTHUR (NJ-3)
SEAN PATRICK MALONEY (NY-18)
CAROLYN MALONEY (NY-12)
KENNY MARCHANT (TX-24)
TOM MARINO (PA-10)
DORIS MATSUI (CA-6)
MICHAEL MCCAUL (TX-10)
JIM MCGOVERN (MA-2)
DAVID MCKINLEY (WV-1)
LUKE MESSER (IN-6)
TIM MURPHY (PA-18)

RICHARD NEAL (MA-1)
RICK NOLAN (MN-8)
DONALD NORCROSS (NJ-1)
FRANK PALLONE (NJ-6)
BILL PASCRELL (NJ-9)
ERIK PAULSEN (MN-3)
DONALD PAYNE, JR. (NJ-10)
SCOTT PETERS (CA-52)
COLLIN PETERSON (MN-7)
JARED POLIS (CO-2)
BILL POSEY (FL-8)
MIKE QUIGLEY (IL-5)
ILENA ROS-LEHTINEN (FL-27)
PETER ROSKAM (IL-6)
DAVID ROUZER (NC-7)
LORETTA SANCHEZ (CA-46)
JAN SCHAKOWSKY (IL-9)
DAVID SCOTT (GA-13)
PETE SESSIONS (TX-32)
ALBIO SIRES (NJ-8)
ADAM SMITH (WA-9)
CHRIS SMITH (NJ-4)
STEVE STIVERS (OH-15)
ERIC SWALWELL (CA-15)
GLENN THOMPSON (PA-5)
PAT TIBERI (OH-12)
PAUL TONKO (NY-20)
FRED UPTON (MI-6)
JUAN VARGAS (CA-51)
TIM WALZ (MN-1)
DEBBIE WASSERMAN-SCHULTZ (FL-23)
BONNIE WATSON COLEMAN (NJ-12)
JOE WILSON (SC-2)
ROBERT WITTMAN (VA-1)
JOHN YARMUTH (KY-3)
KEVIN YODER (KS-3)
RARE DISEASE LEGISLATIVE ADVOCATES IN COORDINATION WITH RARE DISEASE CONGRESSIONAL CAUCUS

CO-CHAIRS REPRESENTATIVES LEONARD LANCE (R-NJ) AND G.K. BUTTERFIELD (D-NC) AND
SENATORS ORRIN HATCH (R-UT) AND AMY KLOBUCHAR (D-MN) WELCOME YOU TO:

ADVANCING RARE DISEASE TREATMENTS IN THE ERA OF CURES AND HEALTH CARE REFORM

THURSDAY, MARCH 2ND, 2017
HART SENATE OFFICE BUILDING, ROOM 902

LUNCH BRIEFING: 12:00 – 1:30PM

MODERATOR: CAROLINE KRUSE, EXECUTIVE DIRECTOR, PLATELET DISORDER SUPPORT ASSOCIATION

IMPLEMENTING THE 21ST CENTURY CURES ACT
• FRANK SASINOWSKI, DIRECTOR, HYMAN, PHELPS & MCNAMARA PC

THE PDUFA REAUTHORIZATION PROCESS IN 2017
• ERIC GASCHO, VICE PRESIDENT OF GOVERNMENT AFFAIRS, NATIONAL HEALTH COUNCIL

THE AFFORDABLE CARE ACT REPEAL AND REPLACEMENT
• BETH MCGINN, DIRECTOR OF PUBLIC AFFAIRS AND NEW MEDIA, ARTBA

NEW MODELS FOR RARE DISEASE DRUG DEVELOPMENT
• CLARE THIBODEAUX, PHD, MBA, DIRECTOR OF SCIENTIFIC AFFAIRS, CURES WITHIN REACH

THE ROLE OF INCENTIVES IN DEVELOPING ORPHAN THERAPIES
• SPEAKER TO BE ANNOUNCED
The Scorecard is based on membership in the Rare Disease Congressional Caucus and action taken on legislation important to the rare disease community.

**SCORING CRITERIA AND BILLS:**
Members were given a √ for being a Caucus member, as well as for being an author, original co-sponsor, co-sponsor, or a 'Yes' vote on the bills below.

**RARE DISEASE CONGRESSIONAL CAUCUS MEMBERSHIP:** The bipartisan Caucus is made up of 101 Representatives and five Senators. Points were added if the Representative or Senator was a member of the Rare Disease Congressional Caucus.

**HOUSE BILLS:**
- H.R. 6 – 21st Century Cures Act (initial version)
- H.R. 34 – 21st Century Cures Act (final version)
- H.R. 971 – Orphan Product Extensions Now, Accelerating Cures and Treatments (OPEN ACT)
- H.R. 1537 – Advancing Hope Act (Priority Review Vouchers)
- H.R. 1608 – Lymphedema Treatment Act
- H.R. 3381 – Childhood Cancer STAR Act
- S. 139 – Ensuring Access to Clinical Trials Act of 2015

**SENATE BILLS:**
- S. 139 – Ensuring Access to Clinical Trials Act of 2015
- S. 1878 – Advancing Hope Act (Priority Review Vouchers)
- S. 1421 – Orphan Product Extensions Now, Accelerating Cures and Treatments (OPEN ACT)
- S. 2030 – Advancing Targeted Therapies for Rare Diseases Act of 2016
- S. 2373 – Lymphedema Treatment Act
- H.R. 34 – 21st Century Cures Act (final version)

**HOW TO USE THE SCORECARD:**
- Include the Scorecard in your conversation about rare disease issues.
- If your legislator has a strong score, thank him or her.
- If your legislator has a poor score, ask about the main concerns with the legislation.
- Ask for a commitment for an improved level of support. An easy way to improve a score is to join the Rare Disease Congressional Caucus.

This Scorecard is only a sampling of legislative votes and does not provide the full story. We recognize that Members in leadership positions refrain from co-sponsoring legislation, however their actions can greatly affect passage of rare disease bills. Poor scores are a reflection of the work the rare disease community needs to do to educate Congress on issues that are important to patients.
✓ Arrive on time, but not more than five minutes early.

✓ Share your personal story and explain why a specific issue is important to you.

✓ You don’t have to be an expert on legislation. If you are asked a question that you are not sure how to answer, write it down and be sure to follow-up.

✓ Respect a staff member’s time by limiting meetings to 20 minutes.

✓ Report back to RDLA staff on how the meeting went.

✓ Thank each staff member for agreeing to meet with you.

✓ Make a specific legislative “ask”. You have to give Congress the solutions.

✓ Leave behind a one-pager with a summary of each issue as well as your contact information.

✓ It’s okay to ask for a photo with a Member of Congress. Make it a group shot and do it quickly.

✓ Follow up with a thank you note reinforcing your ask(s).
SOCIAL MEDIA ADVOCACY TIPS

FIRST AND FOREMOST, WHAT IS A HASHTAG?

• On Twitter and Facebook, the pound sign (#) turns any word or group of words that directly follow it into a searchable link. This allows you to organize content and track discussion topics based on those keywords. For instance, if you want to post about Rare Disease Week on Capitol Hill, you would include #RAREDC2017 to join the conversation. You could then click the hashtag to see other posts on Rare Disease Week on Capitol Hill.

HOW DO I ‘MENTION’ SOMEONE ON TWITTER?

• Many Congressional offices have Twitter accounts to keep in touch with constituents. If you know your legislator’s Twitter handle, you can mention him or her in your post about #RAREDC2017.
• If you don’t know your legislator’s Twitter handle, check his or her official website.

BEFORE YOUR MEETING:

• Create a post tagging the Member’s office and the issue you will be talking about, for example:

  “WE ARE EXCITED TO MEET WITH @SENATORJENKINS FOR #RAREDC2017 TO TALK ABOUT WAYS TO BRING MORE TREATMENTS TO #RAREDISEASE PATIENTS.”

This is a good way to introduce yourself and your issue to the staff. This will add a face to the upcoming meeting and will help them remember you.

DURING THE MEETING:

• Ask to take a photo, preferably towards the end of the meeting.
• Jot down any notes that might make for good tweets or quotes on your Facebook page.

AFTER THE MEETING:

• Post your picture with a thank you note on Twitter and Facebook re-emphasizing the ask or any key points you discussed during the meeting, for example:

  “THANK YOU @SENATORJENKINS FOR JOINING THE RARE DISEASE CONGRESSIONAL CAUCUS AND SUPPORTING #RAREDISEASE LEGISLATION! #RAREDC2017”

USE THE HASHTAG #RAREDC2017
Dedicated to accelerating biotech innovation for rare disease treatments through science-driven public policy

We see our Foundation as an agent for change of the drug development system, positioned at the intersection between the patients and families of the rare disease community, science and industry experts, and the policymakers who together can enact change.

We believe:

- No disease is too rare to deserve treatment.
- All new drugs for rare diseases should be safe and effective.
- We could be doing more with the science we already have.

We have the need. We have the science. We have the voices, strength, and capability to accelerate the process. Together, we will see change happen.

www.EveryLifeFoundation.org
The EveryLife Foundation for Rare Diseases and the Offices of Senator Orrin Hatch and Representative Leonard Lance are pleased to invite you to the Rare Artist Reception.

Wednesday, March 1st, 2017
5:00 - 7:00 PM
Kennedy Caucus Room
Russell Senate Office Building
Washington, DC 20002

This event is free and open to the public.

To RSVP, visit www.rareartist.org
Rare on the Road will bring critical education and insights to rare disease patients, advocates and caregivers, while collaborating in an interactive and engaging environment.

Building on the successful 2016 Everylife Foundation regional Legislative Conferences, Rare on the Road will focus on an expanded agenda that includes topics from Capacity Building to the Patients’ Role in Drug Development, including breakout sessions and hands-on workshops.

These regional events will provide patients and advocates the opportunity to engage more broadly with other rare advocates and benefit from connecting with other disease advocacy leaders. Rare on the Road is working to help advocates learn, grow and become independent activists for their rare disease community.
Join us!

Join Global Genes and Everylife Foundation for Rare on the Road

May 13, 2017: TBD, Atlanta, GA
June 5, 2017: Kauffman Foundation Conference Center, Kansas City, MO
July 15, 2017: Shriners Hospital for Children, Portland, OR

GLOBALGENES.ORG/LEADERSHIPTOUR

For more information and for sponsorship questions please contact:

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Rare Disease Legislative Advocates is a collaborative organization designed to support the advocacy of all rare disease patients and organizations. By growing the patient advocacy community and working collectively, we can amplify our many voices to ensure rare disease patients are heard in state and federal government.

RDLA provides many free resources for successful grassroots advocacy.

RDLA Advocacy Events:
- Rare Disease Week on Capitol Hill
- In-District Lobby Days
- Legislative Conferences
- Rare Disease Congressional Caucus briefings
- RareVoice Awards
- Monthly conference calls and webinars

Resources:
- Monthly action alert blasts
- Congressional Scorecards
- Online advocacy tools to contact Congress
- Legislative clearinghouse at RareAdvocates.org
- DC office space
- Consulting on legislative strategies

EMPOWERING THE PATIENT TO BE AN ADVOCATE!

RDLA is a program of the EveryLife Foundation for Rare Diseases, a 501c3 nonprofit.
Online Advocacy Tools at RareAdvocates.org

An action alert is a request for specific action to be taken on a current political issue. Well-designed action alerts are a powerful way to engage Congress and make sure your message is heard by policymakers and the public.

Utilize action alerts to support your legislation including:

✓ **Phone call:** These alerts are reserved for urgent issues and usually need immediate action to save or move a piece of legislation.
✓ **Group sign-on letter:** A letter is circulated in a community to gather organizations to take a position on a piece of legislation or policy.
✓ **Emails to Members of Congress:** RDLA’s online system automatically populates your legislator’s contact information and a draft letter for you to customize once you provide your contact information.

“It’s so easy to use RDLA’s online advocacy tools. The RDLA team uploads our action alert and sends us a link to share with our community. Once the action alert is complete, RDLA provides data on who took action and which legislators were contacted so we can follow-up. These tools really help increase our impact”

- Stephanie Bozarth
  The National MPS Society
Save the Date

RARE V O I C E

Awards

A CELEBRATION TO HONOR ADVOCATES WHO GIVE RARE DISEASE PATIENTS A VOICE ON CAPITOL HILL

PRESENTED BY:

Shire

November 15th, 2017

Arena Stage
Washington, D. C.

KNOW A RARE DISEASE CHAMPION WHO DESERVES RECOGNITION?

NOMINATIONS FOR RAREVOICE AWARDS OPEN ON MAY 15TH, 2017.

WWW.RAREVOICEAWARDS.ORG
EMPOWERING THE PATIENT TO BE AN ADVOCATE

Rare Disease Legislative Advocates

www.RareAdvocates.org

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