IN-DISTRICT LOBBY DAYS
RARE DISEASE LEGISLATIVE ADVOCATES
WEST COAST CONFERENCE

@RareAdvocates

Please silence your cell phones
Welcome

Klane K. White, M.D.
Pediatric Orthopedic Surgeon
Associate Professor of Orthopedics
Seattle Children’s Hospital
Thank you to our sponsors and advocacy partners!
Keynote Address

James Hendricks, M.D.
President
Seattle Children’s Research Institute
Keynote Address

The Honorable Suzan DelBene
United States Representative
Washington State 1st District
Keynote Address

The Honorable Patty Murray, United States Senator (WA)
Keynote Address
The Honorable Fred Upton, United States Representative (MI-6)
Keynote Address
The Honorable Gus Bilirakis, United States Representative (FL-12)
Overview of the EveryLife Foundation for Rare Diseases

Julia Jenkins
Executive Director
EveryLife Foundation for Rare Diseases
The mission of the EveryLife Foundation is to accelerate biotech innovation through science-driven public policy.

We seek to achieve our goals by advocating practical and scientifically-sound policies to increase the predictability of the regulatory process through scientific analysis and dialogue, grassroots support and expert-led workshops.
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.
Our Focus

1) Serve and Support Rare Disease Patients
2) Promote Awareness about Rare Diseases
3) Advance Regulatory Science and Policy
4) Drive Public Policy and Legislative Change
5) Build A Grassroots Advocacy Community
Community Support

RareGiving provides $100,000+ in funding to the community in grants and scholarships to ensure FDA and Congress hear from patients.

RAREARTIST Rare Artist promotes awareness of rare diseases and highlights the artistic talents of the rare community. The 2016 contest will open in on July 21st.

We provide financial support to the North American Metabolic Academy which trains and encourages the next generation of rare disease physicians and scientists.

Rare Affair informs investors about the impact policy changes have on investment potential.

EveryLife Foundation for Rare Diseases
Public and Scientific Policy Initiatives

**Rare Disease Scientific Workshops** convene FDA, NIH, industry and advocacy organizations to build the science to improve the clinical development process for rare diseases. This year’s conference, to be held in Washington, DC on September 13th, will evaluate models for expanded access.

**Community Congress** brings together patient organizations, industry leaders, and other rare disease stakeholder organizations to collaborate on policy solutions and provide insight on prioritizing future initiatives. The annual meeting will be on November 16th in Washington, DC.

The Foundation is advancing pilot **newborn screening** legislation in California (SB 1095) to require the state to screen for a disease once it’s on the federal Recommended Uniform Screening Panel (RUSP).
Rare Disease Legislative Advocates

• Educates patient advocates about how public policy impacts availability and access to treatments.
• Provides resources to patients, caregivers, physicians and others so they can be successful legislative advocates.
• Serves as an online advocacy center and legislative clearinghouse for all rare disease legislation at the state and federal level.
• Builds awareness on Capitol Hill and ensures Congress hears directly from patients and others in the rare community.
Monthly RDLA Webinars

• Any individual or organization is welcome to contribute agenda items, from pending legislation of interest to the rare disease community to new resources to new policy papers.

• Webinars are available online afterwards for anyone who misses them.

http://rareadvocates.org/monthly-meetings/
Advocacy Events

Brings 300+ patients to Washington, DC to learn how to build effective relationships with Congress and partner with federal agencies.

Empowers advocates to meet with their Members of Congress during summer recess. Three regional Legislative Conferences help advocates prepare.

Hosts quarterly briefings to educate Members of Congress and their staff on issues of importance to the rare disease community.

Recognizes advocates and Congressional aides making a difference in DC and state.
Rare Disease Week on Capitol Hill 2016

**When:** February 29th through March 3rd

**Who:** Rare disease patients, caregivers & other advocates including physicians

**What:** Series of events aimed at empowering patients

**Where:** Washington, D.C.

**Cost:** Free for advocates to attend, and we awarded nearly $60k in travel stipends to help offset travel expenses for advocates from 39 states and Puerto Rico.
Rare Disease Week on Capitol Hill 2016

2016 was our fifth and most successful year!

• 330+ patient advocates registered
• 130+ patient organizations represented
• Leaders from FDA and NIH participated in a panel at the Legislative Conference, Rare Disease Congressional Caucus briefing and Lobby Day breakfast.
Rare Disease Week on Capitol Hill 2016

Monday: Rare Disease Day at NIH
Monday: Cocktail Reception and Film Screening
Tuesday: Legislative Conference
Wednesday: Lobby Day Breakfast
Wednesday: Lobby Day
Thursday: Congressional Caucus Briefing
Thursday: Rare Artist Reception
Please join us in DC in 2017!
Save the dates:
February 27th - March 2nd
Meet your Members of Congress in their local offices during the summer recess (July 18th through September 5th) to discuss your needs and concerns.

Register by July 5th for In-District Lobby Days
5th Annual RareVoice Awards

- The RareVoice Awards recognizes rare disease advocates as well as Congressional and federal agency staff who have taken action to benefit the rare disease community.
- Nominations are open to the public through July 31st. **Nominate someone today!**
- Join us on November 16th in Washington, DC! The event celebrates the community so there is no charge to attend.

http://rareadvocates.org/rarevoice-awards
Our Team

Please feel free to ask questions or provide feedback to any EveryLife Foundation staff member. Vignesh Ganapathy, Max Bronstein and Julia Jenkins are here with us. You’ll hear from Max and Viggy later today.
Why Rare Disease Policy Matters

Dean Suhr – President, MLD Foundation

Leslie Fox – Healthcare Policy and Advocacy Director, Johnson & Johnson; Rare Disease Advocate, Tuberous Sclerosis Alliance
Why Rare Disease Public Policy Matters

Dean Suhr, President – MLD Foundation

Seattle, WA
June 30th, 2016
Where my journey started ... 

- 6 year diagnostic odyssey

- 2 of our 3 children diagnosed with MLD in 1995

Lindy ... 
Diagnosed at 14.
Docs gave us 4-6 yrs.
Just turned 35 and doing very well

Darcee ... 
Passed away at age 10 20 years ago.

Experimental bone marrow transplant – Krivit (MN)
Jclynn, sister was donor
We C.A.R.E.™
Facilitating Compassion
Increasing Awareness
Influencing Research
Promoting Education
... for Metachromatic Leukodystrophy
• 1995 … MLD diagnosis
• 2001 … Foundation formed
• 2007 … first policy work
Agency, Regulatory & Legislative Awareness

NIH Office of Rare Disease

NIH Director Francis Collins

“Educating” on Capitol Hill
Rare Disease Policy History

• 1983 … Orphan Drug Act … NORD Formed

• As the Internet came
  • Many existing organizations struggled to embrace, include, collaborate & adapt
  • Genetic Alliance … became a focal point for rare disease
  • Many new disease organizations blossomed
Rare Disease Policy Today

- Genetic Alliance ... less RD focus, more health ... continue to innovate
- Global Genes (Rare Project) ... awareness, advocacy encouragement
- A few larger disease specific organizations ... generally quite focused
- NORD ... focused *self-driven* policy work
- RDLA (Everylife Foundation) ... *advocacy-driven* policy focus
- Perhaps 2,000 small organizations ... “the trenches”
Why Do YOU Matter?

- **7000+** rare diseases
- **1 in 10** has a rare disease … **30M** Americans
  - **350M** worldwide … world’s 3rd most populous country
- **80%** are **genetic**
- **50%** of patients are **children**
  - **30%** of these children **will not live** to see their **5th birthday**
- **95%** of diseases have no viable therapy
  - **80%** of all rare disease patients are affected by approximately **350** rare diseases

ref: Rare Statistics … [GlobalGenes.org](http://GlobalGenes.org)
Why Does DC or Your State Care?

- 1 in 10 … that’s a lot of “affected” voters
- parents, grandparents, neighbors, teachers, bosses, pastors, etc.
- passionate
- Advocate/Family stories, connections, did I mention children?
- Rare Disease policy affects everyone
Can a 501c3 Work on Policy?

• Yes!
  • Most of what we do is Awareness & Education
  • Your ask is a very small percentage of your time, $$, and work

Note: you may not endorse a candidate
Is Policy Difficult or Time Consuming?

• **NO!**
  
  • *Usually someone else is doing the preparation and heavy lifting*
  
  • Phone call
  
  • Sign on letter
  
  • Meet with your representative and share your story
  
  • Can meet state & federal reps in your home state
Does Your Voice Matter?

- **YES!** ... only you can speak for your state!

- Phone calls ... hundreds of calls makes a huge impact

- Sign on letters ... dozens of signatories are noticed

- Personal annual & semi-annual meetings build relationships
  - Meet locally and in DC ... small groups and large groups
Our Current Key Policy Initiatives

- Rare Disease Awareness/Education … Rare Disease Day & throughout the year
- **NIH** … NCATS/ORDR advocacy voice, registries, standards, appropriations
- **FDA** … OOP/PFFD patient voice, regulations, educations
- **Capitol Hill** … 21st Century Cures/OPEN Act
- **GINA** … ethics, privacy, registries, open access to research, openNHS
- Encouraging, empowering, & training other advocates
  **RDLA Legislative Conference** during Rare Disease Week
- **Newborn screening** … federal & state public health
Newborn Screening

- Industry/Payors … research, technology, policy, funding/payments, technology (sequencing)
- Federal … ACHDNC/RUSP, Common Rule, ethics, basic science
- State … labs, public health policy, appropriations, uniformity
  - “One & Done” RUSP model legislation in CA – RDLA initiative
- RUSP Roundtable … includes all of above – [NewbornScreening.us](http://NewbornScreening.us)
  - Launched and driven by MLD Foundation
We C.A.R.E.™
Facilitating Compassion
Increasing Awareness
Influencing Research
Promoting Education
... for Metachromatic Leukodystrophy
Sharing the Patient Voice

Accelerate. Empower. Mobilize
What is Tuberous Sclerosis Complex?

- TSC is a Linchpin Disease
- TSC is the #1 Genetic Cause of Epilepsy & Autism
- TSC occurs more frequently than ALS or Cystic Fibrosis (1:6000)
- There is no CURE
TSC Research – benefits others

• NIH Funding
• HHS Funding
• CDMRP – focus of the TS Alliance Advocacy Day
  • Annual Advocacy of Congress to continue
  • $53 million in funding since 2002
  • Established the TSCRP
Tuberous Sclerosis Complex

Vision - To lessen the impact of TSC

Tuberous sclerosis is a genetic disorder that can affect any or all systems of the body. The disorder is characterized by seizures, developmental delays, kidney disease, behavioral problems, and the growth of benign tumors (tubers) on vital organs such as the brain, kidneys, and heart. These tumors typically calcify with age, becoming hard (sclerotic). Children with tuberous sclerosis may have autistic-like symptoms. Tuberous sclerosis affects as many as 25,000 to 50,000 individuals in the United States and about 1 to 2 million individuals worldwide.

Although this disorder can be inherited as an autosomal dominant trait, two-thirds of cases are the result of a spontaneous genetic change on one of two genes, TSC1 or TSC2. The TSC1 gene is located on chromosome 9 and produces the protein hamartin. The TSC2 gene is located on chromosome 16 and produces the protein tuberin. Hamartin and tuberin are believed to act as tumor growth suppressors. Therefore, their dysfunction may underlie the appearance of tumors that characterize tuberous sclerosis. There is currently no cure for this disease; however, surgical intervention and a number of treatments can help affected individuals.
Why does funding TSCRP matter to Congress?
Dear Northwest Congressional Delegates:

My name is Kelley Fox and I have Tuberous Sclerosis Complex. I am almost 22 years old, and have been diagnosed with the disease since I was three months old. I want you to know my story, and tell you why it is so important to continue funding research for Tuberous Sclerosis Complex (TSC).

I had my first seizure at four weeks of age. When I was three months old, the doctor said I had TSC. My mom went to the UW and did research and found that it can be serious, and that I probably wouldn't live past the age of 20. I developed infantile spasms at age of six months, and the standard treatment was ACTH. That did not work and so I had the right rear portion of my brain removed at age one. I continued to have seizures, and my doctor tried many medicines. I was in an NIH study, and helped illustrate how the surgery had a longer term positive benefit. As I got older, my doctors had to watch the other areas that impact the disease – my skin, heart, kidneys and lungs. My kidneys have lots of tumors. Finally, I was out of medications to control my seizures, and so when I was 12 I had the rest of the right side of my brain removed. Because it stopped so many of my seizures, I went from developmentally two with autistic tendencies to how I am today, happy, social, talkative and developmentally six. HUGE improvement.
Leverage Social Media – Keep the connection
Thank you to our sponsors and advocacy partners!
Vignesh Ganapathy
Manager of Advocacy & Government Relations
EveryLife Foundation for Rare Diseases
Goals of In-District Lobby Days

- Help advance rare disease legislation, such as the 21st Century Cures Act
- Strengthen relationships with your Members of Congress
  - View meetings as opportunities to build or sustain a meaningful relationship (meeting with your Member of Congress once a year is not enough).
- Build on the meetings from Lobby Day during Rare Disease Week on Capitol Hill earlier this year
- Allow advocates who can’t travel to D.C. to have an opportunity to meet with their federal elected officials in their local offices

A best-practice of effective advocacy is to make your friends before you need them! We NEED to gain allies who will champion rare disease causes.
Why Relationships Matter

If your Member/Senator has not already arrived at a firm decision on an issue, how much influence might the following advocacy strategies directed to the Washington office have on his/her decision?*

- In-Person Issue Visits from Constituents: 46% (A Lot of Positive Influence), 51% (Some Influence)
- Contact from a Constituent Who Represents Other Constituents: 36% (A Lot of Positive Influence), 60% (Some Influence)
- Individualized Postal Letters: 20% (A Lot of Positive Influence), 70% (Some Influence)
- Individualized Email Messages: 19% (A Lot of Positive Influence), 65% (Some Influence)
- Phone Calls: 14% (A Lot of Positive Influence), 72% (Some Influence)
- Comments During a Telephone Town Hall: 17% (A Lot of Positive Influence), 68% (Some Influence)
- Visit From a Lobbyist: 8% (A Lot of Positive Influence), 74% (Some Influence)
- News Editorial Endorsement of an Issue: 10% (A Lot of Positive Influence), 65% (Some Influence)
- Individualized Faxes: 8% (A Lot of Positive Influence), 62% (Some Influence)
- Form Postal Letters: 1% (A Lot of Positive Influence), 53% (Some Influence)
- Form Email Messages: 1% (A Lot of Positive Influence), 50% (Some Influence)
- Postcards: 1% (A Lot of Positive Influence), 44% (Some Influence)
- Comments on Social Media Sites: 1% (A Lot of Positive Influence), 41% (Some Influence)
- Form Faxes: 0% (A Lot of Positive Influence), 30% (Some Influence)

*A lot of positive influence vs. some influence.
How In-District Lobby Days Works

- Meetings will take place between July 18th and September 5th in your legislators’ district offices.
- Advocacy Associates will schedule the meetings and contact advocates with their schedules.
- RDLA will provide background information on key legislative issues.
- More detailed information, including tips on successful meetings and what to research prior to your meetings, will be provided in a webinar on July 13th at 2pm ET/11am PT.
In-District Lobby Days 2016

The 3rd annual In-District Lobby Days is fast approaching. From July 18th to September 5th, rare disease advocates from across the country will surge into the district offices of their Members of Congress to advocate for legislation benefiting the rare disease community.

Interested in meeting with your U.S. Senators and Representative in their district offices during the summer Congressional recess? Rare Disease Legislative Advocates will schedule meetings for you, and help you prepare by providing key background materials. In addition, anyone registered for In-District Lobby Days will receive an invite to a preparatory webinar to be held on July 13th.
Getting Registered

• Enter your personal info on the registration page.

• BE SPECIFIC about the dates that you are unavailable!
  • Advocacy Associates uses this to schedule your appointments.

• We understand that some advocates may have difficulty traveling. Please note how far you are willing to travel for the meetings with your Representatives and Senators.

• VISIT THE CHECK-IN TABLE OUTSIDE TO GET REGISTERED TODAY!
In-District Lobby Days Logistics

- Once you register, we will use the information you provide to:
  - Match advocates to their federal legislators
  - Determine (based on constituency and location) the appropriate offices to approach about scheduling
  - Send meeting request letters (and initiate follow-up calls, if necessary, to schedule meetings)
  - Ensure the meeting time works for both advocates and the legislator

- On our July webinar, Advocacy Associates will help you prepare, with strategies on how to have a successful meeting.
The problem

The ask

Info about you and/or your organization

How this bill would solve the problem listed at the beginning

The One Sheet

Unequal drug pricing allows pharmaceutical companies to charge dramatically higher prices in the United States than almost any country in the developed world. At times, these prices are so high that patients may be forced to choose between medications and other essential needs, such as food or housing. These high drug prices have been shown to increase the risk of health complications and death in patients with rare diseases. Furthermore, many patients are unable to afford the medications they need, leading to potential harm or death.

Under the Affordable Care Act, pharmaceutical companies are required to disclose the actual wholesale acquisition cost (WAC) of their drugs. However, these numbers are often grossly inflated to ensure profits. This bill would require drug manufacturers to disclose the actual acquisition cost (AC) of their drugs to ensure that patients have access to affordable treatments.

Bill Sponsor: Rep. McKinley, David B. (R-WV)

Committees:

- Energy and Commerce
- Budget
- Education and the Workforce

Summary:

- Requires drug manufacturers to disclose the actual acquisition cost (AC) of their drugs to ensure that patients have access to affordable treatments.

Why this bill is important:

- Ensuring access to affordable medications is crucial for patients with rare diseases.
- The bill would provide transparency in drug pricing, allowing patients to make informed decisions about their medical care.

Potential amendments:

- The bill may be amended to include provisions for price control measures or public funding to help offset the cost of medications.

Potential objections:

- Drug manufacturers may argue that disclosing AC would be too costly and could lead to price manipulation.

Key quotes:

- "Access to affordable medications is a basic human right. It is unacceptable that some patients are forced to choose between life-saving treatments and other essential needs." - Rep. McKinley, David B.

Contact: Mark Paden, (202) 523-2716, markpaden@rdla.org

Rare Disease Legislative Advocates (RDLA) is a non-profit advocacy organization dedicated to advocating for the rights of patients with rare diseases. RDLA works to ensure that patients have access to affordable and effective treatments.

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For more information about RDLA and its advocacy efforts, visit www.rdla.org.
Getting to know your Members of Congress

- Go to your legislators’ websites (which you can find at www.house.gov or www.senate.gov) to check out the bios as well as the issue and news sections. Sign up for their newsletters.
- Google your legislators and pay attention to what they are doing in the media. What are they saying? What do they care about?
- Visit Project Vote Smart (VoteSmart.org) to look up a Member’s interest group ratings, speeches and statements, etc.
- Look for things that you may have in common. For example, Rep. Marino from PA has a daughter with a rare disease and is the Co-Chair of the Cystic Fibrosis Caucus.
- Follow your legislators on Twitter and like their pages on Facebook.
How to Advocate for the Rare Disease Community

Max Bronstein – Senior Director of Advocacy and Science Policy, EveryLife Foundation for Rare Diseases

Ann Simons – Legislative and Public Policy Consultant, AES Public Policy
THE OPEN ACT & 21ST CENTURY CURES: ADVOCACY NOW!

Max Bronstein
Senior Director, Advocacy & Science Policy
EveryLife Foundation for Rare Diseases
Rare Disease Landscape

- 7,000 diseases, 95% of which have no FDA-approved tx
- Average 7+ year diagnostic journey, wasting health care dollars and time, meanwhile disease may progress unchecked
- Patients often prescribed medicines off-label:
  - Poor Data
  - Limited efficacy
  - Difficult to get reimbursement
De-risking Rare Disease Drug Development since 1983: The Orphan Drug Act

- **INCENTIVES!!!**
  - Exclusivity
  - Tax Credits
  - User fee exemptions
  - Eligible for fast review at FDA
INCENTIVES WORK

Number of Approved Orphan Products by Year

<table>
<thead>
<tr>
<th>Year</th>
<th>Number of Products</th>
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<td>2012</td>
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<tr>
<td>2013</td>
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<tr>
<td>2014</td>
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500+ Rare Disease Drugs in Development

Medicines in Development
FOR RARE DISEASES

<table>
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<tr>
<th>Disease Category</th>
<th>Number</th>
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<td>Autoimmune Diseases</td>
<td>25</td>
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<tr>
<td>Blood Cancer</td>
<td>82</td>
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<tr>
<td>Blood Disorders</td>
<td>9</td>
</tr>
<tr>
<td>Cancer</td>
<td>151</td>
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<tr>
<td>Cardiovascular Disease</td>
<td>12</td>
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<tr>
<td>Digestive Disorders</td>
<td>10</td>
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<tr>
<td>Eye Disorders</td>
<td>10</td>
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<tr>
<td>Genetic Disorders</td>
<td>148</td>
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<tr>
<td>Growth Disorders</td>
<td>7</td>
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<tr>
<td>Infectious Diseases</td>
<td>31</td>
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<tr>
<td>Neurologic Disorders</td>
<td>38</td>
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<tr>
<td>Respiratory Diseases</td>
<td>11</td>
</tr>
<tr>
<td>Transplantation</td>
<td>13</td>
</tr>
<tr>
<td>Other</td>
<td>49</td>
</tr>
</tbody>
</table>

Note: Some medicines may be in more than one category.
Best Pharmaceuticals for Children Act (BPCA) - 2002

- Problem: 100s of safe and effective medicines, but very few had ever been tested in neonates, infants, and children
- BPCA offered an incentive for conducting clinical trials in children
  - Gain SIX months of exclusivity
- Impact: 426 label changes
A six-month **exclusivity** extension for therapies repurposed for a rare disease indication
- Common->Rare or Rare -> Rare
- Compound is only eligible for 1 extension
- Would stack with BPCA
- Could double the number of rare disease treatments available to patients
OPEN ACT - Champions

Sen. Hatch (R-UT)
Sen. Klobuchar (D-MN)

Rep. Bilirakis (R-FL)
Rep. Butterfield (D-NC)

+ 5 Bipartisan Cosponsors!

+ 19 Bipartisan Cosponsors!
Support for the OPEN ACT

Overwhelming support from patient community:

166 National Patient Orgs and Growing!

Still time to sign-on
The ASK

- For House & Senate:
  - PLEASE Co-Sponsor the OPEN ACT (HR 971/S 1421)
  - See Sample Scripts & One Sheets
  - Questions welcome!
The Energy & Commerce (E&C) Committee spent over 1 year gathering input from stakeholders and released 4 draft bills.

The 21st Century Cures Act (HR 6) includes a variety of provisions of critical importance to the rare disease community.

Reps. Upton (R-MI) & DeGette (D-CO)

• **Foundation Priorities:**
  - Orphan Product Extensions Now, Accelerating Cures & Treatments (OPEN ACT)
  - Improve biomarker qualification
  - Improve FDA’s ability to recruit & retain staff & keep up on the latest science
  - Billions in NIH & hundreds of millions in FDA Funding

• **Foundation supported efforts:**
  - Expanding Hope Act (Priority Review Vouchers)
  - Neurological Disease Surveillance
  - Compassionate Use Reform & Enhancement Act
  - Patient Focused Drug Development
Pushback on the OPEN ACT

- https://youtu.be/jaYqD4yBcLM?t=19m2s
Advocacy WORKS!

- https://www.youtube.com/watch?v=cQuuQHjyZh8&feature=youtu.be&t=19m50s
21st Century Cures in Committee
51-0 Vote
21st Century Cures Floor Vote
July 2015

21ST CENTURY CURES MAKES HISTORY!

YEA: 344
NAY: 77
Chairman Alexander Announces Committee Schedule for “Step by Step” Consideration of Biomedical Innovation Bills

Feb. 9 meeting will be 1st of 3 to produce companion legislation to 21st Century Cures Act already passed by House

“The House has completed its work on the 21st Century Cures Act. The president has announced his support for a precision medicine initiative and a cancer ‘moonshot.’ It is urgent that the Senate finish its work and turn into law these ideas that will help virtually every American.”

WASHINGTON, D.C., Jan. 19 – Chairman Lamar Alexander (R-Tenn.) today announced the Senate health committee will hold its first executive session considering bills on biomedical innovation on Tuesday, Feb. 9. At that committee
Rare Disease Provisions:

February 9th:
- Advancing Targeted Therapies for Rare Diseases Act of 2015 (Sens. Bennet, Burr, Warren, and Hatch)
- Advancing Neurological Diseases Act of 2015 (Sens. Isakson & Murphy) – provision added that would limit surveillance to the five most prevalent diseases

March 9th:
- Advancing Hope Act of 2015 (Sens. Casey, Isakson, Brown & Kirk)
- Legislation Supporting Precision Medicine Initiative
- Patient Focused Impact Assessment (PFIA) Act (Sens. Wicker, Klobuchar, Collins, Franken, Isakson, Bennet, Donnelly)

April 6th:
- Workforce Enhancement at NIH & FDA
Missing Rare Disease Provisions

- No Agreement (yet) on NIH/FDA Funding
- OPEN ACT
- Hopeful that OPEN ACT will be included when package goes to Senate floor
Keeping up the Momentum

I Took Action for #CURESNow
BIT.LY/CURESNOW2016

Congress Blog
The Hill's Forum for Lawmakers and Policy Makers
June 15, 2016, 11:23 am
Passing 'cures' bill means better health possible for virtually every American
By Sen. Lamar Alexander (R-Tenn.)

The Seattle Times
Innovation is needed in the fight against rare diseases
Congress should encourage companies to repurpose existing drugs for rare diseases, potentially doubling the number of treatment options for patients.

By Klane K. White and David Fajgenbaum
Challenges Remain in the Senate

- FUNDING for NIH, FDA, and the OPEN ACT
- Cures package could get bundled with opioid legislation
- Clock is ticking: Summer Recess, Gun Control, Election Politics, Appropriations bills - TIME IS RUNNING OUT

Path Forward:
The ASK

- For House: Please encourage your colleagues in the Senate to bring 21\textsuperscript{st} Century Cures to a floor vote!
- For the Senate: Please bring 21\textsuperscript{st} Century Cures to a floor vote!
- See One Sheets & Script
- Questions Welcome!
FEDERAL APPROPRIATIONS – ADVOCATING FOR NIH & FDA
Total Federal Spending

President’s Proposed $4.1 Trillion Budget by Mandatory and Discretionary Spending and Interest on Federal Debt (FY 2016)

- Interest on Debt: $283.0 billion - 7%
- Discretionary Spending: $1.15 trillion - 28%
- Mandatory Spending: $2.63 trillion - 65%

Congress ↔ Autopilot
Congress’s Annual Discretion

Discretionary Spending 2015: $1.11 Trillion

- Military: $598.5 billion - 54%
- Food & Agriculture: $13.1 billion - 1%
- Transportation: $26.3 billion - 2%
- Social Security, Unemployment & Labor: $29.1 billion - 3%
- Science: $29.7 billion - 3%
- Energy & Environment: $39.1 billion - 3%
- International Affairs: $40.9 billion - 4%
- Housing & Community: $63.2 billion - 6%
- Veterans' Benefits: $65.3 billion - 6%
- Medicare & Health: $66 billion - 6%
- Education: $70 billion - 6%
- Government: $72.9 billion - 6%
National Institutes of Health Budget, 1998-2015
budget authority in billions of constant FY 2014 dollars

Source: AAAS data and agency budget documents. Does not include additional $970 million in Opportunity, Growth, and Security Initiative funding. © 2014 AAAS
NIH estimates that it invests $3.7B in rare disease research annually!
FDA Funding

FDA regulates 25% of US economy and all rare disease therapies!
the FDA BUDGET in context

Each year, Americans spend ...

$17 Billion
on video games

$9.1 Billion
on potato chips

$3.36 Billion
on baggage fees

$2.4 Billion
on FDA appropriations
## The Appropriations Process & Timelines

### Annual Budget Process - Timeline

<table>
<thead>
<tr>
<th>Date</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>February</td>
<td>President submits budget proposal to Congress</td>
</tr>
<tr>
<td>April 15</td>
<td>Congress adopts a budget resolution</td>
</tr>
<tr>
<td>May/June</td>
<td>Appropriations Committees Begin Reporting Bills</td>
</tr>
<tr>
<td>Late July</td>
<td>House and Senate finish considering appropriations bills</td>
</tr>
<tr>
<td>Fall/Winter</td>
<td>House and Senate reconcile, send bills to President</td>
</tr>
<tr>
<td>October 1</td>
<td>New Fiscal Year Begins</td>
</tr>
</tbody>
</table>
The Committees – The House

The U.S. House of Representatives
COMMITTEE ON APPROPRIATIONS
Chairman Hal Rogers

"No money shall be drawn from the Treasury but in consequence of Appropriations made by Law" US Constitution Article I, Section 9, Clause 7

Chairman Hal Rodgers (R-KY)  Ranking Member Nita Lowey (D-NY)
House Subcommittees

LABOR, HEALTH AND HUMAN SERVICES, EDUCATION, AND RELATED AGENCIES
Labor, Health and Human Services, Education, and Related Agencies

2358-B Rayburn House Office Building
(202) 225-3598
Chairman: Rep. Tom Cole, Oklahoma

Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

2362-A Rayburn House Office Building
(202) 225-2638
Chairman: Rep. Robert Aderholt, Alabama
The Committees – The Senate

Chairman Thad Cochran (R-MS)

Ranking Member Barbara Mikulski (D-MD)
Senate Subcommittees

Labor, Health and Human Services, Education, and Related Agencies

CHAIRMAN
Roy Blunt

RANKING MEMBER
Patty Murray

Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

CHAIRMAN
Jerry Moran

RANKING MEMBER
Jeff Merkley
Appropriations Complications….

- Deadline: September 30th or we go to Continuing Resolution
- Limited legislative time
- Election Politics….
Time for Advocacy is NOW –

The ASKS:

- Please support robust and mandatory funding for the National Institutes of Health (NIH) and the Food & Drug Administration (FDA)
- See One Sheets & Sample Scripts
- Questions Welcome!
Advocacy Workshop

Kirsten Norgaard
Patient Advocate, President
Adrenal Insufficiency United
Thank you to our sponsors and advocacy partners!

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Global Genes

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MLD Foundation

Seattle Children’s

Jonah’s Just Begun

RDLA

Rare Disease Legislative Advocates