Agenda:

- **21st Century Cures Overview** – Max Bronstein, The EveryLife Foundation


- CCM–CARE bill – Amy Akers, Angioma Alliance

- State Level Rare Disease Advisory Council – Patty Weltin, Rare Diseases United Foundation

- Patients Alliance for Drug Safety Protections and information about REMS – Michael Losow

- **RDLA Updates** – Andy Russell, The EveryLife Foundation
21st Century Cures Update

Max G. Bronstein
Senior Director, Government & Public Affairs
EveryLife Foundation for Rare Diseases
Quick History

• Roundtables, whitepapers, and briefings commenced in 2014
• Four legislative drafts have been introduced
• 300+ page bill passed subcommittee markup
• Full committee will vote on full legislative package tomorrow
• Next stop, House Floor
• Senate: Innovation for Healthier Americans
Rare Disease Provisions - IN

• Orphan Product Extensions Now, Accelerating Cures & Treatments (OPEN ACT)
• NIH Funding
• Expanding Hope Act (Priority Review Vouchers)
• Neurological Disease Surveillance
• Compassionate Use Reform & Enhancement Act (Expanded Access)
• Patient Focused Drug Development
• Precision Medicine
Rare Disease Provisions - OUT

• Dormant Therapies Act/MODDERN
• Advancing Care for Exceptional Kids Act (ACE Kids)
• Genetically Targeted Platform Technologies for Rare Diseases
OPEN ACT - Orphan Product Extension Now, Accelerating Cures and Treatments HR 971

- Provision creates a six-month exclusivity extension for drugs repurposed for a rare disease indication
- Modeled after Best Pharmaceuticals for Children Act
- Could double the number of rare disease treatments available to patients
NIH Funding

• Bill includes novel funding mechanism: Innovation Fund
• Allows $2B/year for five years in mandatory funding
• Authorizes significant annual increases for 3 years (funds must be appropriated)
Expanding Hope Act

- Companies that create treatments for rare pediatric diseases are awarded a priority review voucher
- The voucher greatly speeds up the drug approval process and is transferrable
- Passed in FDASIA (2012)
- 21st Century Cures reauthorizes program until 2018
Neurological Disease Surveillance HR 292

- Provision to national data collection system at the CDC
- Critical for monitoring prevalence and progression of neurological diseases
Compassionate Use Reform & Enhancement
CURE Act (Expanded Access) HR 909

• Provisions requiring companies to post expanded access policies online

• FDA is required to issue a guidance
Dormant Therapies Act (MODDERN)

• Was not included in final 21\textsuperscript{st} Century Cures Legislation
• Bill in current form would extend patent life for drugs to treat complex and rare diseases with unmet medical need
• Hopeful that bill could be introduced in the Senate
21st Century Cures Initiative: Priorities for the Rare Disease Community
Thursday May 21, 2015
12:30 pm – 1:30 pm (lunch provided)
Rayburn House Office Building
Room: B318

• Compassionate Use & Reform Enhancement Act (CURE Act), Rep. Michael McCaul (R-TX)
• Dormant Therapies and MODDERN Cures Act, Rep. Leonard Lance (R-NJ)
• Orphan Product Extensions Now, Accelerating Cures & Treatments (OPEN ACT), Rep. Gus Bilirakis (R-FL)
• Overview and Outlook for 21st Century Cures, Steve Usdin, Senior Editor, BioCentury Publications
• Patient Focused Drug Discovery, Kim McCleary, Director of Strategic Initiatives, FasterCures
• Advancing Hope Act/Pediatric Priority Review Voucher, Nancy Goodman, Executive Director, Kids V. Cancer
• Rare Disease Patient Organization Perspective, Paul Melmeyer, Assistant Director of Public Policy, NORD
• NIH Funding Provisions, Ellie Dehoney, Vice President of Policy & Advocacy, Research!America
• Moderator: Max G. Bronstein, Senior Director, Public & Government Affairs, EveryLife Foundation for Rare Diseases
Questions?

mbronstein@everylifefoundation.org
Pharmaceutical Cost Transparency Act of 2015
California Legislation – AB 463

- Eve Bukowski, Vice President State Government Affairs,
  California Life Sciences Association
Cerebral Cavernous Malformations Clinical Awareness, Research and Education Act of 2015 (CCM-CARE)

Amy Akers, PhD
Chief Scientific Officer
Angioma Alliance
Cerebral Cavernous Malformations (CCM)
Cerebral Cavernous Malformations (CCM)

- Symptoms include: neurological deficits, seizure, stroke or sudden death
- Sporadic vs. familial CCM
- Affects adults and children
- Biological Mechanism beginning to be understood
- No drug treatment; brain surgery remains only option, but not suitable for all patients
ANGIOMA ALLIANCE
because brains shouldn't bleed

Expert Information  Peer Support  Advocacy

Patient Registry  International Scientific Meeting  DNA/Tissue Bank

Genetic Testing  Clinical Centers  Real Treatments

Angioma.org
Unmet Clinical Need

• Need for non-invasive therapy
  – Increased research opportunities
  – Infrastructure & support for clinical trials

• Challenge
  – Advocates can not fund all of these initiatives
  – No current funding mechanism
Purpose
To increase research, education and treatment for CCM.

Key Features
1. Expansion and Coordination of Activities of NIH with respect to CCM Research
2. Centers for Disease Control and Prevention CCM Surveillance and Research Programs
3. FDA CCM Clinical Trial Preparedness and Support Program
CCM Clinical Awareness, Research and Education
Act of 2015 (CCM-CARE)

Key Features

1. Expansion and Coordination of Activities of NIH with respect to CCM Research
   - Coordinate efforts at NIH and provide grant support for research
     - Basic, translational & clinical research
   - Clinical trial Preparedness – Clinical Centers
     - Coordination Centers with current CCM research, expertise, infrastructure & patient population
     - Participation Centers without current CCM research and/or expertise; part of another network (e.g. stroke network) and will complete education program to become CCM participation center
     - Oversight of centers by CCM Consortium composed of advocates, coordinating center leadership & government agencies

2. Centers for Disease Control and Prevention CCM Surveillance and Research Programs
   - Grants for epidemiology research

3. FDA CCM Clinical Trial Preparedness and Support Program
   - Coordination with clinical centers to support IND applications
   - Support for Orphan Product development, where appropriate
Advocate Support

- **Direct Support** – Letters of support
- **Awareness** – Single Disease Legislation necessary where no other funding mechanisms exist or where there is a mismatch between NIH research networks and disease-specific research centers
For more Information

- [www.angioma.org](http://www.angioma.org)
- Amy Akers at [amy.akers@angioma.org](mailto:amy.akers@angioma.org)
State Rare Disease Advisory Council Legislation
What is the purpose behind the Rare Disease Advisory Council legislation?

- This legislation would create a Rare Disease Advisory Council by state made up of public health officials, medical professionals, patient advocates and other stakeholders with the purpose of coordinating efforts for the study, incidence and coordination of rare diseases.

How is the Council appointed?

- This council would be overseen by the Department of Public Health. The Department of Public Health shall appoint the members.
Can anyone file legislation for a Rare Disease Advisory Council?

- Anyone can file the legislation. You do not need to be a member of the Rare Disease United Foundation (RDUF) to file. This legislation has been filed in Rhode Island, Massachusetts, Connecticut, and New Jersey by RDUF. It also has been filed in North Carolina, where it has passed.

Where can I find the legislation and can it be changed?

- A copy of the legislation filed in MA is on our website. A link can be found at the end of the presentation. Each state has made small changes to the language. The MA legislation is a great place to start.
How can I get this legislation filed and passed in my state?

The process of filing legislation in each state is different, but the basic procedure is as follows:

• Find a state representative to file the legislation

• Look for co-sponsors for the legislation.

• Once the legislation if filed, start preparing to testify. The testimony can be given in person when the bill is heard or through submitted written testimony.

• Find out which committee will be hearing the legislation. If you have people who can testify in person who are constituents of a committee member, it holds more weight.

• Generally, oral testimony is limited to 3 minutes or so. If you can, try not reading your testimony. Committee members are more engaged when people are looking and speaking to them.
What are some problems I might encounter after filing?

- Asking for money for research, etc. makes it difficult for state legislation to pass. Although, there is some cost associated with this bill, it is important to stress that working on the issues surrounding rare diseases will eventually save the state money.

- It might be difficult to find others in your state who can testify. Rare Disease United Foundation has set up Rare Disease Community Facebook groups for each state. Please feel free to use these groups to organize your efforts. A link can be found at the end of the presentation.

What if I decide to change something?

- Legislation can always be amended. Your sponsor should guide you through the process.
What if the legislation does not pass?

- It is very often the case that legislation will not pass on the first go around. Often, legislation takes a few years to pass. It is a very slow process.

What can I do if the bill passes out of committee, but is opposed by the Department of Health?

- This has happened in some of the states where it has already been filed. In North Carolina, the coalition group was able to have the University of North Carolina take on the Council.
State Legislation for the Future

- Legislation that would have dental issues related to rare disease conditions or other illness coded as medical to insure coverage. Many rare diseases negatively affect the teeth. Costly procedures to deal with dental issues are generally not covered by insurance.

- Legislation to introduce rare disease education into health classes in public middle schools. Raising awareness at this level not only creates a generation of people who will already understand the issues surrounding rare, but will create tolerance of those living with a rare disease.
Never doubt that a small group of thoughtful, committed people can change the world. Indeed, it is the only thing that ever has.

MARGARET MEAD

Please remember that your state and federal representatives work for you!

Including caregivers, there are 80 million living rare. Together we are powerful!

Rare Disease United Foundation is available to support you in your efforts!
Resources & Links

Rare Disease United Foundation Website:

http://rarediseaseunited.org

Link to Massachusetts Legislation:


Link to State Facebook Groups:

http://rarediseaseunited.org/state-based-facebook-groups

Email RDUF at:

info@rarediseaseunited.org
REMS & ETASU

Patient Advocacy Concerns With Current Legislative Proposals
2007 the Food and Drug Administration Amendments Act (FDAAA) gave FDA the ability to require important patient safety protections, called Risk Evaluation and Mitigation Strategies (REMS), as a way to approve new medicines for patients with unmet medical needs that carry known safety risks.

Defined by FDA as a “strategy to manage a known or potential serious risk associated with a drug or biological product,” REMS are structured plans to manage specific risks of medicines that are effective but associated with known or potential serious risks (e.g., injury, death) that, without REMS, may outweigh benefits.
In situations where drugs and biologics carry specific, known serious risks, FDA may require highly stringent safety precautions as part of the REMS program. Called “Elements to Assure Safe Use” (ETASU), these carefully planned safety protocols are the most extensive elements of a REMS. ETASU are required rarely and only when the agency determines that due to a medicine’s “inherent toxicity or potential harmfulness,” the medication can only be approved with stringent controls in place to ensure a serious or fatal risk can be avoided by proper use.
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<th>Conditions Impacted</th>
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<td>• Blood disorders</td>
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<td>• Rare cholesterol disorder</td>
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<td>• Dupuytren’s</td>
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<td>• Multiple myeloma</td>
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<td>• Bi-polar disorder</td>
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<tr>
<td>• Crohn's disease and specific bowel disorders</td>
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<td>• Other rare disorders</td>
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Scope of REMS and ETASU

- 2011 FDA authorized 222 REMS programs
  (Since then the agency released 145 less potentially toxic drugs from their REMS requirements)

- January 2015, only 71 products have authorized unique REMS programs
  - 34 are subject to the more restrictive Elements to Assure Safe Use
  - An estimated 25 or fewer, require restricted distribution systems

Restricted distribution systems are implemented to ensure that a drug or biologic known to cause birth defects, organ damage and other serious or life-threatening events is only acquired and used by patients under carefully controlled conditions.
The proposed legislation would:

• require the “force sale” of drugs subject to REMS requirements for BE testing to nearly anyone – a basement lab scientist or legitimate generic manufacturer without distinction – without any safeguards to ensure these medicines are handled and administered safely

• weaken these drug safety protections and absolve generic manufacturers from liability should a generic version of a medicine known to cause serious risks lead to injury, birth defects, organ failure and even death
Advocating for a Rigorous REMS Program:
The Need for a Patients Alliance for Drug Safety Protections

The Need

Because certain life-saving drugs and biologics carry high risks if not administered and taken appropriately, in 2007 Congress gave the Food and Drug Administration new authority to require Risk Evaluation and Mitigation Strategies (REMS) from manufacturers so these medicines could be made available to patients with unmet medical needs. As a result, millions of Americans with serious diseases (cancers, Crohn’s disease, bowed disorders, chronic obstructive pulmonary disease, HIV, lung conditions, multiple sclerosis, seizures, and schizophrenia) and rare disorders are now being treated effectively with medications that may never have been available without REMS programs to ensure their safe use.

In spite of this track record, eight years have passed since Congress authorized the REMS program and the sense of urgency that led to these requirements has been replaced by misperceptions about the need for REMS controls. That is why collective action is needed to inform and engage policymakers about the meaningful impact of REMS safeguards in advancing patient safety and public health so the protections afforded through the REMS program will remain intact.

Mission Statement

Spearheaded by the Society for Women’s Health Research, the Patients Alliance for Drug Safety Protections is a new alliance of public health, women’s health, men’s health, patient advocacy, health professional and disease organizations working collaboratively to raise awareness of the importance of REMS as a tool to advance patient safety and protect public health.

Guided by the need to prevent harmful exposure to medicines that can cause terrible birth defects, organ damage, and even death when not handled and administered with utmost care, the Alliance will advance the following principles:

- REMS programs ensure continued patient access to innovative, higher-risk medicines
- REMS programs using Elements to Assure Safe Use (ETASU), including those required to control the distribution of medicines carrying high risks, are rare and should not be weakened
- Manufacturers — innovator companies and generic manufacturers — should be held to the same safety requirements when designing and conducting drug studies, including bioequivalence testing
- Legislative policies must recognize that not all manufacturers have the capacity to handle high-risk compounds. Negating the rigorous safeguards now in place through the REMS program is not in the public interest.

All Americans benefit from the drug safety protections REMS makes possible. By providing up-to-date information on the purpose and current use of REMS protocols, it is hoped policymakers will continue to ensure these safeguards are guarded closely.
Members of Congress will return home to their districts to connect with constituents for summer recess August 3rd – September 4th.

This is the perfect opportunity for rare disease advocates to strengthen relationships with Members of Congress or build new relationships if you have not been able to advocate in Washington, DC!

- Registration opens in June!
- Webinar Dates: July 15th and August 5th
- Meetings take place Aug 3rd–Sept 4th
Regional Legislative Conferences

- RDLA is coming to you!

- Before In-District Lobby Days start, RDLA is organizing two regional legislative conferences aimed at helping advocates prepare for meetings with their legislators on rare disease issues.

  - California – UCSF, Mission Bay Campus: July 31st
  - New Jersey – Rutgers: July 20th
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Briefing Sponsored by: Abbvie, Shire, Amgen, Raptor, Alexion, Genzyme, BioMarin, Vertex, Novartis, RDLA, NORD, FasterCures, Polycystic Kidney Disease (PKD) Foundation, National MPS Society, Noah’s Hope, Batten Disease Support & Research Association
Save the date:
November 4, 2015
Arena Stage, Washington DC
Join us next month on June 17th at 1:00 pm EST to discuss the events surrounding the Rare Disease Community.

Agenda is Open!

- Please send potential agenda items to: arussell@everylifefoundation.org