What Comes First? Priorities for Future Legislative Efforts

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

Rare Disease Caucus:
Molly McDonnell
Legislative Assistant, Office of Representative Leonard Lance (NJ-7)

Orphan Product Extensions Now, Accelerating Cures and Treatments, OPEN Act (H.R. 971/S. 1421):
Saul Hernandez
Deputy Chief of Staff and Legislative Director, Office of Representative G.K. Butterfield (NC-1)

Patient Focused Impact Assessment Act, PFIA Act (S. 1597):
Annie Kennedy
Senior Vice President of Legislation & Public Policy, Parent Project Muscular Dystrophy

Rare Disease Fund Act of 2015 (H.R. 3731):
Scott Hinkle
Legislative Director, Office of Representative Juan Vargas (C-51)
What Comes First? Priorities for Future Legislative Efforts

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Max Bronstein
Senior Director of Public Policy and Government Relations, EveryLife Foundation for Rare Diseases
21st Century Cures: Update & State of Play

Max G. Bronstein
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Senior Director, Advocacy & Science Policy
21st Century Cures Initiative

Reps. Upton (R–MI) & DeGette (D–CO)
The 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.

- **Rare Disease Community 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
  - Expanding Hope Act (Priority Review Vouchers)
  - Neurological Disease Surveillance
  - Compassionate Use Reform & Enhancement Act
  - Patient Focused Drug Development
21st Century Cures in Committee
51–0 Vote
21st Century Cures Floor Vote
Future Majority Whip Max Schill
Special Guest Max Schill, honored for his efforts on the 21st Century Cures Act
Where do we go from here?
01.19.16

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
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
- All bills passed by unanimously by voice vote

March 9th:
- Advancing Hope Act of 2015 (Sens. Casey, Isakson, Brown & Kirk)
- Legislation Supporting Precision Medicine Initiative
So what’s missing?

- No language on Compassionate Use
- How to fund increases for NIH & FDA?
  - Both sides want funding, mechanism is up for debate
- Patient Focused Impact Assessment (PFIA) Act (Sens. Wicker, Klobuchar, Collins, Franken, Isakson, Bennet, Donnelly)
- Orphan Product Extensions Now, Accelerating Cures & Treatments Act (OPEN ACT) (Sens. Hatch & Klobuchar)
- Final hearing scheduled for April 6th
We need to make rare disease the top priority: The Clock is Ticking
One tool for raising our voice & visibility

THANK YOU
TO OUR RARE DISEASE CONGRESSIONAL CAUCUS
CO-CHAIRS:

Senator
Orrin Hatch (R-UT)

Senator
Amy Klobuchar (D-MN)

Representative
Joseph Crowley (D-NY)

Representative
Leonard Lance (R-NJ)
Our Panel Today

- Missing Senate provisions:
  - OPEN ACT
  - Patient Focused Impact Assessment Act
  - April 6\textsuperscript{th} Hearing

- Looking ahead:
  - Rare Disease Fund Act (House bill)

- Raising our Voice:
  - Rare Disease Congressional Caucus
What Comes First? Priorities for Future Legislative Efforts

Rare Disease Caucus:

Molly McDonnell
Legislative Assistant, Office of Representative Leonard Lance (NJ-7)
Molly McDonnell
Legislative Assistant
Congressman Leonard Lance (NJ-07)
• What is a Caucus? •

Forum for **Members of Congress** to...

• **Voice** constituent concerns,
• **Educate** other Members and staff
• **Collaborate** on ideas
• **Facilitate** conversations between relevant stakeholders
• **Build** support for legislation
“The Congressional Rare Disease Caucus is a forum for Members of Congress to voice constituent concerns, collaborate on ideas, facilitate conversations between the medical and patient community and build support for legislation that will improve the lives of people with rare diseases.”
Co-Chairs

• Senate Co-Chairs •

Sen. Orrin Hatch (R-UT)  Sen. Amy Klobuchar (D-MN)
• Briefings •

Precision Medicine

Science Behind Rare Disease Policy

Access to Care & Therapies in the New Healthcare System: A Rare Disease Perspective

Creating Economic Incentives to Spur the Development of Treatments for Ebola and Other Life-Threatening Rare Diseases

Implementation of Rare Disease Provisions in FDASIA

Urgent Healthcare Policy Needs of the Rare Disease Community

21st Century Cures Initiative: Priorities for the Rare Disease Community
The Rare Disease Ecosystem: Fostering Patient Engagement & Driving Biomedical Innovation

THURSDAY, MARCH 3RD AT CONGRESSIONAL VISITORS CENTER AUDITORIUM
Congressional Co-Chair Statements: 12:00pm – 12:30pm
Lunch Briefing: 12:30pm – 1:30pm

- Moderator: Julie Anne Smith, CEO, Raptor Pharmaceuticals
- Janet Woodcock, M.D., Director, Center for Drug Evaluation (CDER), at the Food and Drug Administration (FDA) – “The Role of the FDA and CDER in the Drug Development Process”
- Emil Kakkis, M.D. PhD, President, EveryLife Foundation for Rare Diseases & CEO, Ultragenyx Inc. – “Developing Medicines for Rare & Ultra Rare Diseases”
- Rakesh Marwah, M.D., Investment Professional, Palo Alto Investors, Clinical Faculty, Stanford University – “Incentives Driving Biomedical Investment & Innovation”
- David Fajgenbaum, M.D., M.B.A., M.Sc., Executive Director, Castleman Disease Collaborative Network, Assistant Professor of Medicine & Associate Director, Orphan Disease Center, University of Pennsylvania – “Patient/Physician/Researcher Perspective: Driving Research & Innovation for Castleman Disease”
- Brett Felter, J.D., Assistant Attorney General, Maryland Office of the Attorney General & Becker Muscular Dystrophy Patient – “Patient Perspective: How Policy Impacts Development of Rare Disease Therapies”
Membership

100+

AL • CA • CO • CT • FL • GA • IA
IN • KY • KS • IL • MA • MD • MI
MN • NC • NE • NJ • NV • NY
OH • OR • PA • TN • TX • VA
WA • WI • WV
Join the Rare Disease Caucus!
What Comes First? Priorities for Future Legislative Efforts

Orphan Product Extensions Now, Accelerating Cures and Treatments, OPEN Act (H.R. 971/S. 1421):

Saul Hernandez
Deputy Chief of Staff and Legislative Director, Office of Representative G.K. Butterfield (NC–1)
The ‘OPEN’ Act

The Orphan Product Extension
Now Accelerating Cures and Treatments Act
H.R. 971

Saul Hernandez
Deputy Chief of Staff and Legislative Director
Congressman G. K. Butterfield (NC-01)
95% of rare diseases have no FDA-approved treatment

Rare disease patients are often prescribed medications off-label

Many drugs that are already FDA-approved may be effective for rare diseases, but there is little clinical data available

Leverage existing science and therapies to bring treatments to rare disease patients
OPEN Act Overview

☑ Provides an additional six months of market exclusivity for therapies that are repurposed for use in rare disease applications

☑ Includes major-market drugs, orphan drugs, and biologics

☑ Must obtain a rare disease indication (on the label) from FDA by demonstrating safety and efficacy in rare disease population

☑ Included in the House-passed 21st Century Cures Act
Kayla’s Story

- Diagnosed with Neonatal Onset Multisystem Inflammatory Disease (NOMID) by doctors at NIH
  - Causes headaches, seizures, and intellectual disabilities

- NIH doctors identified an existing drug developed to treat arthritis as a potential treatment option
  - Therapy was effective in treating disease

- In 2013, FDA approved existing arthritis drug to treat NOMID
OPEN Act is Important

- 95% of rare diseases have no FDA-approved treatment

- Rare disease patients are often prescribed medications off-label

- Many drugs that are already FDA-approved may be effective for rare diseases, but there is little clinical data available

- Leverage existing science and therapies to bring treatments to rare disease patients
We Need the OPEN Act

- There are at least 7,000 known rare diseases
  - 95 percent of those diseases have no treatments

- It costs several billion dollars and a decade or more to bring a new drug to market
  - Manufacturers are hesitant to develop new drugs for rare diseases because return on investment would be difficult
Repurposed Drugs Show Promise

- Evidence that existing, already-approved drugs may be able to treat rare diseases

- With no approved treatments, many individuals with a rare disease use existing drugs for a disease for which it was not approved (off label)

  - The effectiveness of off label use of drugs is not widely studied or tracked
    - No scientific data on drug effectiveness or correct dosing

  - Off label use is not reimbursed by insurance
    - Unnecessary barrier to potential breakthrough treatments
The ‘OPEN’ Act Makes Sense

- Instead of a biopharmaceutical company investing billions of dollars and a decade of work to develop a new drug, it makes sense to repurpose an existing drug for treatment of a rare disease.

- We can find new uses for existing drugs!

- Repurposing is:
  - Cost-efficient
  - Expedient
  - More likely to gain FDA-approval

- Bottom-line: getting treatments to patients as quickly as possible

- Problem: few incentives for companies to repurpose
How the ‘OPEN’ Act Works

- Modeled after the highly successful Best Pharmaceuticals for Children Act (BCPA)
  - 400 label changes since 1998
- Incentivizes manufacturers to repurpose existing drugs
- Receive an additional six months of market exclusivity
Benefits of the ‘OPEN’ Act

- Result in hundreds of treatments for people living with rare diseases
  - Safe, effective, and affordable drugs

- Surge in biotech investment
  - Immediate creation of new, high skilled, high paying jobs in research and patient engagement
  - Huge benefit to research institutions
We Need Your Help

- Contact your Member of Congress and ask that they cosponsor the OPEN Act – H.R. 971 (standalone bill)

- Call your Senators and encourage action on S.1421 (Senate companion)

- Endorsed by over 150 patient organizations

- Will improve patient outcomes and save lives
What Comes First? Priorities for Future Legislative Efforts

Patient Focused Impact Assessment Act, PFIA Act (S. 1597):

Annie Kennedy
Senior Vice President of Legislation & Public Policy, Parent Project Muscular Dystrophy
Patient Focused Impact Assessment Act
PFIA, S. 1597

Annie Kennedy
Parent Project Muscular Dystrophy
SVP – Legislation & Public Policy
✓ House: 21st Century Cures Act Passes Overwhelmingly (344-77)
Legislation included a number of good ideas (provisions) that we like! But did not include PFIA provision.

- Senate: Medical Innovations package
Currently working it’s way through the Senate – We want to ensure PFIA (S.1597) is passed and included in package.
Take you back...to 2012

Food and Drug Safety and Innovation Act (FDASIA) passed in 2012, signed by President Obama

- Accelerated Approval Expansion
- Breakthrough Therapies Designation
- Patient Focused Drug Development (PFDD) – mandate to create new tools of including patient voice in process of drug development and review
Since then... lots of activity

In the spirit of Patient Focused Drug Development (through the lens of the Duchenne community)....

- Putting Patients First white paper
- PPMD Benefit-risk studies
- Patients are Waiting white paper
- Draft Guidance on Duchenne
- Expansion of Duchenne Connect patient registry
- Heavy engagement with 21st Century Cures

TO DO LIST
1. SO
2. MANY
3. THINGS

Pipeline in 2012: 17 companies
Today: Close to 40 companies working on Duchenne
Senate *Innovation* –

**S. 1597** *Patient Focused Impact Assessment Act*

- This provision or *Patient Focused Assessment Act (PFIA)* would bring transparency to the FDA review process and let us know how reviewers are – or are not – using such valuable information (PFDD tools) when reviewing products.

- Creates an essential feedback loop.

- A natural evolution to the *science of patient input* that we have implemented through Patient Focused Drug Development initiatives.

- Ensures FDA has mechanism for incorporating the voice and perspective of patients, caregivers, and disease experts in review and drug development.
S. 1597 *Patient Focused Impact Assessment* Act

**PDFF Tools:**
Benefit-Risk/ Patient Preference studies  
Guidance for Industry and FDA  
PROs  
Registry data  
Testimony from patient & clinical experts  
And more...
Broad Support From Partners

- Parent Project Muscular Dystrophy
- ALS Association
- Association for Frontotemporal Degeneration (AFTD)
- BCC Nevus Syndrome Life Support Network
- BioNJ
- CARES Foundations
- Charley's Fund
- Coalition Duchenne
- Congenital Hyperinsulinism International
- Cure Sanfilippo Foundation
cureCADASIL/CADASIL Association Inc.
CureCMD
CureSMA
Everylife
Fabry Support & Information Group
Foundation for a Cure
Foundation for Prader-Willi Research
Foundation to Eradicate Duchenne (FED)
Friedreich's Ataxia Research Alliance (FARA)
Genetic Alliance
Global Genes
Hannah's Hope Fund
Hope for Javier
Hydrocephalus Association
ICAN, International Cancer Advocacy Network
Summit Therapeutics
Suneel's Light

- United Leukodystrophy Foundation
- Team Saij
- Two Smiles One Hope Foundation
- JB's Keys to DMD
- John Owen's Adventure
- Justin Fallon, PhD Brown University
- Kids With Heart
- National Assn for Children's Heart Disorders, Inc
- Life Raft Group
- Little Hercules Foundation
- MLD Foundation
- Marathon Pharmaceuticals
- Muscular Dystrophy Association
- Myotonic Dystrophy Foundation
- National Down Syndrome Society
- National Fragile X Foundation
- National Hydrocephalus Association
- National MPS Society
- National Organization for Rare Disease (NORD)
- National Psoriasis Foundation
- Pediatric Hydrocephalus Foundation
- Phelan-McDermid Syndrome Foundation
- Pietro's Fight
- PKD Foundation
- Powerful Patient
- PXE International (Pseudoxanthoma elasticum)
RASopathies Network USA
Reveragen
Santhera Pharmaceuticals

- Sarepta
“I believe that patient engagement is the 'blockbuster drug of the century' – helping patients live longer, richer, more meaningful lives.”
- Pat Furlong, PPMD President & Founder
S. 1597- *Patient Focused Impact Assessment* Act (PFIA)

**Leads:**
- Senator Roger Wicker (R-MS)
- Senator Amy Klobuchar (D-MN)

**Original cosponsors:**
- Sen. Michael Bennett (D-CO), Sen. Susan Collins (R-ME), Sen. Al Franken (D-MN), Sen. Johnny Isakson (R-GA)

**The Ask:**
Please co-sponsor S. 1597, PFIA.
We must ensure that the tools being created under FDASIA’s PFDD make the regulatory impact they are intended to.
Thank You!

Annie Kennedy  annie@parentprojectmd.org

Ryan Fischer  ryan@parentprojectmd.org
What Comes First? Priorities for Future Legislative Efforts

Rare Disease Fund Act of 2015 (H.R. 3731):

Scott Hinkle
Legislative Director, Office of Representative Juan Vargas (C-51)
H.R. 3731 Rare Disease (RaD) Fund Act

Rep. Juan Vargas (CA-51)
Rep. Tom Rooney (FL-17)

Cure rare diseases by changing the financing
# Movies of 2015

## Most Anticipated Movies

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<tr>
<th>Movie</th>
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<tbody>
<tr>
<td>Star Wars: The Force Awakens</td>
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<tr>
<td>Avengers: Age of Ultron</td>
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<tr>
<td>Mad Max: Fury Road</td>
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<tr>
<td>The Hateful Eight</td>
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<tr>
<td>Mission: Impossible – Rogue Nation</td>
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<tr>
<td>Tomorrowland</td>
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<tr>
<td>Furious 7</td>
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<tr>
<td>Hunger Games: Mockingjay – Part 2</td>
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<td>Inside Out</td>
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<td>The Good Dinosaur</td>
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## Highest Grossing Films

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<tbody>
<tr>
<td>Star Wars: The Force Awakens</td>
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<td>Minions</td>
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<td>Spectre</td>
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<td>Hunger Games: Mockingjay – Part 2</td>
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<tr>
<td>The Martian</td>
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Warner Brothers' Studio Slate

Debt/Equity Financing

Movie Revenues

Investors
- Banks
- Pension Funds
- Mutual Funds
- Life Insurers
- Venture Capitalists
- Angel Investors
Conquering the Valley of Death

• A funding gap between preclinical research and the end of stage 2 of FDA trials

• Venture capital market: $175 – 250 billion

• U.S. bond market: $38 trillion
50% Gov. Guaranteed Bonds and 50% Equity Financing

**RaD Fund (Private Corporation)**

**Investors**
- Banks
- Pension Funds
- Mutual Funds
- Life Insurers
- Venture Capitalists
- Angel Investors

Drug Royalty Revenues
Summary
H.R. 3731 RaD Fund Act

• Creates a $400 million pilot program
• Privately owned and operated fund
• Finances only early-stage rare disease therapeutics
• Government guarantees the bonds (debt)
• Leverages scientific expertise at NIH
Acknowledgments

Special thanks to Dr. Andrew Lo, Dr. Roger Stein, and Jose-Maria Fernandez of MIT for pioneering the research that led to the RaD Fund Act.
Questions?

Staff Contacts
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