Rare Disease Legislation in the Queue

Orphan Product Extensions Now, Accelerating Cures and Treatments (OPEN ACT)

Max Bronstein

Chief Advocacy and Science Policy Officer

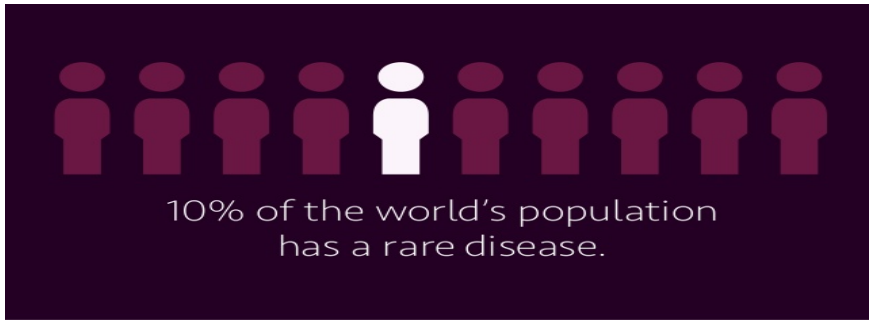
EveryLife Foundation for Rare Diseases

@EveryLifeOrg

OPEN ACT Update and State of Play in the New Congress

Max Bronstein
Chief Advocacy & Science Policy Officer

Rare Disease Landscape



- ~7,000 diseases, 95% of which have no FDA-approved treatment – need for rapid innovation
- Patients often prescribed medicines off-label:
  - Limited safety and efficacy data
  - Difficult to get reimbursement
  - Growing FDA concerns about off-label use
- Rare disease therapies can be costly as they are developed for small patient population

OPEN ACT

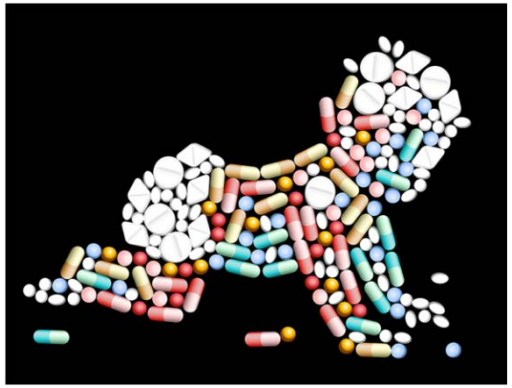
- Goal: dramatically increase the number of therapies available to rare disease treatments
- Mechanism: Repurposing existing therapies for rare indications
  - Repurposing: more cost efficient, rapid development, but underleveraged due to lack of incentives
- Proposed policy change: provide a six month extension of exclusivity for compounds that are repurposed for rare indications
- Exclusivity incentive is critical to spur broader industry repurposing efforts

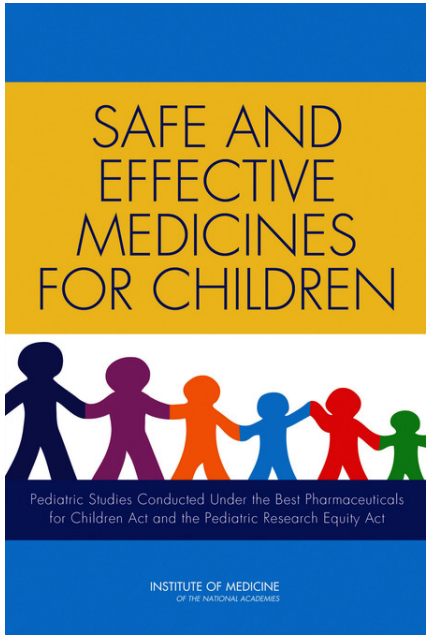
OPEN ACT: Benefits

- Dramatically increase the number of treatments available to rare disease patients
- Generate massive amounts of clinical data needed to transition more therapies from off-label to on-label
  - On-label therapies are more likely to be reimbursed
  - Even failed repurposing trials provide important efficacy data to health care providers and patients
- Repurposed rare disease therapies priced at “major market” level, since the drugs were developed for large population
- ***OPEN COULD DOUBLE THE NUMBER OF LOW-COST THERAPIES AVAILABLE TO RARE DISEASE PATIENTS***

Best Pharmaceuticals for Children Act (BPCA) - 2002

- Problem: hundreds of safe and effective medicines, but very few had ever been tested in neonates, infants, and children
- BPCA offered an incentive for conducting clinical studies in children
  - Gain SIX months of exclusivity – same model for OPEN ACT
- **<u>Impact: 500+ label changes</u>**





Recent History

- Bipartisan co-sponsors in previous Congress & passed in House Cures package in July 2015

- Was not included in final 21st Century Cures package due to objections from two key Democratic senators

Path Forward

- Hoping to have OPEN reintroduced in next two weeks
- Vehicle for OPEN is likely going to be PDUFA
- Timeline: PDUFA committee hearings will take place in March & April
- Congress must pass legislation and send to the President for signature by July 30, 2017 to avoid disruptions at the FDA

everylifefoundation.org/open-act

Max Schill, age 8 of New Jersey, advocates for the OPEN ACT for rare disease patients

Orphan Product Extensions Now ~ Accelerating Cures & Treatments

Bipartisan Legislation Supported by 173 Patient Organizations
including Genetic Alliance, Global Genes, National MPS Society, the National Organization for Rare Disorders (NORD), and the Pediatric Cancer Foundation

How YOU Can Help

- Patient organizations can still sign-on! mbronstein@everylifefoundation.org
- OPEN ACT will be an ‘ASK’ for the Lobby Day during Rare Disease Week on Capitol Hill
- WANTED: patient stories on repurposing treatments or academic articles on repurposing efforts
- Thank you for your advocacy!
- Questions?