Rare Disease Legislation in the Queue

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

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OPEN ACT Update and State of Play in the New Congress

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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

• Impact: 500+ label changes
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
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?