Congress should incentivize drug makers and innovators to “repurpose” already approved drugs for life-threatening rare diseases and pediatric cancers. Economic incentives are imperative to fostering the development of hundreds of safe, effective and affordable rare disease treatments within the next five years.

**Issue:** Despite advances made by the *Orphan Drug Act*, 95 percent of the 7,000 rare diseases still have no FDA-approved treatment. Biopharmaceutical companies seldom consider repurposing already approved therapies to treat rare diseases because there is no incentive for them to do so.

**Solution:** Modeled on the incentive programs in the *Best Pharmaceuticals for Children Act* (BPCA), the OPEN ACT establishes an “Orphan Product Exclusivity Extension,” which would provide an additional six months of market exclusivity for the drug being repurposed for rare disease treatment when the sponsor company establishes that the repurposed therapy is designated to treat a rare disease and obtains an approved rare disease indication from FDA on the drug label.

**Background:** Scientific literature shows that a single targeted drug is likely to have multiple therapeutic uses, and that biopharmaceutical companies “repurpose” drugs for the treatment of different diseases to increase market potential. Repurposing drugs is faster, cheaper, and presents fewer risks than traditional drug development.

For complex rare diseases with small patient populations, the current economic model of drug development often lacks financial viability because of limited market potential. The provision of economic incentives has a proven track record of encouraging industry stakeholders to invest in the development of drugs for diseases with unmet need. For example, the *Orphan Drug Act* provides incentives for industry to develop treatments for rare diseases which has led to numerous new lifesaving therapies. However, with less than 20 new orphan drugs approved each year, additional incentives are necessary to facilitate the development of new treatments for the more than 30 million Americans suffering from rare diseases.

**Outcomes:** The OPEN ACT would leverage the investment already made by biopharmaceutical companies into the development of approved therapies by providing an economic incentive to explore ways to bring more treatments for rare diseases to the marketplace through the process of repurposing drugs, resulting in:

- A surge in biotech investment, new jobs, and grants to research universities to conduct clinical trials.
- Potentially hundreds of well tested therapies approved and on the label for rare disease patients in the next five years. Many of these drugs would be priced at major market drug prices, thus bringing down the average cost of rare disease drugs.
- Fewer rare disease patients using untested and potentially ineffective drugs off–label.

*To co-sponsor, please contact:* Tom Power, Office of Rep. Bilirakis (R-FL): thomas.power@mail.house.gov or Saul Hernandez, Office of Rep. Butterfield (D-NC), saul.hernandez@mail.house.gov. For the Senate, contact: Matthew Richardson matthew.richardson@mail.house.gov for Sen. Hatch (R-UT) or Sarah Smith Sarah_Smith@klobuchar.senate.gov for Sen. Klobuchar (D-MN).
More than 155 Supporting Patient Organizations (partial list):

National MPS Society
With Purpose
National PKU Alliance
Taylon’s Tale
RASopathies Network USA
Kids v Cancer
Let Them Be Little X2 Inc.
Info and Resources for Idiopathic Pulmonary Hemosiderosis (IPH-NET)
Noah's Hope
Mary Payton's Miracle Foundation
HopeBridget Foundation
Batten Disease Support & Research Association
Cure Sanfilippo Foundation
Beyond Batten Disease Foundation
Drew's Hope Scientific Research Foundation
International Pemphigus and Pemphigoid Foundation (IPPF)
Cure AHC
Autoinflammatory Alliance
MLD Foundation
Fabry Support & Information Group
Children's PKU Network
FMD Chat
National Tay-Sachs & Allied Diseases Association (NTSAD)
Little Miss Hannah Foundation
Rare Disease United Foundation
Global Genes Project
Fibromuscular Dysplasia Society of America (FMDSA)
Lymphatic Malformation Institute
Mastocytosis Society
EB Research Partnership
BRBN Alliance
Jonah's Just Begun
Abigail Alliance for Better Access to Developmental Drugs
Hannah's Hope Fund
GNE Myopathy International
The Ryan Foundation
Organic Acidemia Association
Cardio-Facio-Cutaneous International
NGLY1.org
Gwendolyn Strong Foundation
POMC Island One boy an Ocean of friends
Gene Giraffe Project
International FOP Association
Aware of Angels
CADDASIL
GT23 FOUNDATION
Desmoid Tumor Research Foundation (DTRF)
The Association for Glycogen Storage Disease
Gene Spotlight Inc.
Amyloidosis Foundation
Hereditary Neuropathy Foundation
Relapsing Polychondritis
Klippel-Feil Syndrome Freedom
CureDuchenne
Prader-Willi Syndrome Association
EveryLife Foundation for Rare Diseases
Bert's Big Adventure
Parent Project Muscular Dystrophy
Sarcoma Foundation of America
The Nicholas Conor Institute
Luck2Tuck Foundation
Team Sanfilippo Foundation
The Rally Foundation for Childhood Cancer Research
CARES Foundation, Inc.
Help Extinguish Hunter Syndrome
Sephardic Health Organization for Referral & Education
Hunter Syndrome Research Coalition
The Kortney Rose Foundation
Saving Case & Friends
Phelan-McDermid Syndrome Foundation
The Children's Medical Research Foundation, Inc.
Cure SMA
Narcolepsy Network
Celiac Support Association
Caleb's Crusade Against Childhood Cancer
International Waldenstrom's Macroglobulinemia Foundation (IWMF)
PKD Foundation
EDSers United Foundation
Choroideremia Research Foundation, Inc.
Genetic Alliance
The Life Raft Group
The Will Luthecke Foundation
Angioma Alliance
Smashing Walnuts Foundation
Castleman Disease Collaborative Network/Castleman's Awareness & Research Effort
The GIST Cancer Awareness Foundation
The Truth 365
The Arms Wide Open Childhood Cancer Foundation
Sophia's Fund
Journey4ACure
Princesses on a Mission, Inc.
Noah's Light Foundation
Pediatric Cancer Foundation
West Virginia Kids Cancer Crusaders, Inc.
Bear Necessities Cancer Foundation
A Kids' Brain Tumor Cure
RARE Science, Inc.
ISMRD (the International Advocate for Glycoprotein Storage Diseases)
Hermansky-Pudlak Syndrome Network Inc.
Run4Rare
A-T Children's Project
The Global Foundation for Peroxisomal Disorders
The Adult Polyglucosan Body Disease Research Foundation (APBDRF)
Alexa Nawrocki Pediatric Cancer Foundation
Beckwith-Wiedemann Children's Foundation International
The Brooke Healey Foundation
Talia's Legacy Children's Cancer Foundation
The Rare Childhood Cancer Advocacy Group
Alex's Army Childhood Cancer Foundation
The Catherine Elizabeth Blair Memorial Foundation
Stillbrave Childhood Cancer Foundation
Cures Within Reach
ALL4Trey
Team Sabrina
Sofia's Hope, Inc.
Delaine’s Battle
Joey's Wings Foundation
The Bozeman 3
Team Ashley Bragg
Coles vs Cancer
Dominick One in a Million
Samuel Szabo Foundation
Wilms Tumor Survivor Group
Aiden's Army
Sofia's Hope, Inc.
Mikey's Way Foundation
Team Serena
Supporting Our Cancer Kids