Please Support the OPEN ACT
Orphan Product Extensions Now ~ Accelerating Cures & Treatments

Congress should incentivize the repurposing of potentially life-saving approved drugs for rare diseases and pediatric cancers. Similar incentives have been critical in the development of new medicines for underserved patient populations and could lead to hundreds of safe, effective and affordable rare disease treatments within the next five years. The OPEN ACT is sponsored in the House (H.R. 1223) by Representatives Bilirakis (R-FL) and Butterfield (D-NC). Senators Hatch (R-UT) and Klobuchar (D-MN) plan to introduce companion legislation shortly.

**Issue:** Despite advances made by the Orphan Drug Act, 95 percent of the 7,000 rare diseases still have no treatment approved by the Food and Drug Administration. Most rare disease patients are prescribed treatments off-label, at times with little clinical evidence and variable effectiveness. As a result, obtaining reimbursement for off-label treatments or procedures can be challenging for patients. Biopharmaceutical companies seldom consider repurposing approved therapies to treat rare diseases because there is little incentive for them to do so.

**Solution:** The OPEN ACT would establish a six-month marketing exclusivity extension, providing an incentive to a sponsor to repurpose an already approved therapy for a rare disease. The sponsor company would need to demonstrate that the repurposed therapy is safe and effective in treating the rare disease and obtain a rare disease indication from FDA on the drug label. The OPEN ACT is modeled on the highly successful Best Pharmaceuticals for Children Act (2002) that has led to more than 500 labeling changes for pediatric populations.

**Background:** Scientific literature shows that a single-targeted drug is likely to have multiple therapeutic uses, and that biopharmaceutical companies can repurpose drugs for the treatment of different diseases. 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. Utilizing targeted, economic incentives has a proven track record of encouraging industry stakeholders to invest in the development of drugs for diseases with unmet need.

**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:

- Potentially hundreds of well-tested therapies approved and on the label for rare disease patients in the next five years.
- Major market drug prices, resulting in a reduction in the average cost of rare disease drugs.
- Fewer rare disease patients using untested and potentially ineffective drugs off–label.
- A surge in biotech investment, new jobs, and grants to research universities to conduct repurposing trials.

To co-sponsor H.R. 1223, please contact Tom Power, office of Rep. Bilirakis (R-FL), at thomas.power@mail.house.gov or Saul Hernandez, office of Rep. Butterfield (D-NC), at saul.hernandez@mail.house.gov. To be named as an original cosponsor on the Senate bill, please contact Stuart Portman, office of Sen. Hatch (R-UT), at stuart.portman@hatch.senate.gov or Rosa Po, office of Sen. Klobuchar (D-MN) at rosa_po@klobuchar.senate.gov.
177 Supporting Patient Organizations (partial list):

Ali's Angels Foundation
RASopathies Network USA
International Pemphigus and Pemphigoid Foundation (IPPF)
Autoinflammatory Alliance
Children's PKU Network
Global Genes Project
GNE Myopathy International
Gwendolyn Strong Foundation
CureDuchenne
EveryLife Foundation for Rare Diseases
The Nicholas Conor Institute
Castleman Disease Collaborative Network/Castleman's Awareness & Research Effort
RARE Science, Inc.
ISMRD (the International Advocate for Glycoprotein Storage Diseases)
Supporting Our Cancer Kids
Gold Rush Cure Foundation
The Coalition for Pulmonary Fibrosis
Mytonic Dystrophy Foundation
National Fragile X Foundation
Cure JM Foundation
Genetic Alliance
The Catherine Elizabeth Blair Memorial Foundation
DC Outreach Inc.
POMC Island One boy an Ocean of friends
International FOP Association
Gene Spotlight Inc.
Prader-Willi Syndrome Association
Phelan-McDermid Syndrome Foundation
Caleb's Crusade Against Childhood Cancer
International Waldenstrom's Macroglobulinemia Foundation (IWMF)
Noah's Light Foundation
Talia's Legacy Children's Cancer Foundation
Joey's Wings Foundation
Sofia's Hope, Inc.
Bert's Big Adventure
The Rally Foundation for Childhood Cancer Research
Sickle Cell Warriors, Inc.
Curing Retinal Blindness Foundation
Noah's Hope
Hope4Bridge Foundation
Knallend Flesh Syndrome Freedom
Hunter Syndrome Research Coalition
The Children's Medical Research Foundation, Inc.
Cure SMA
Bear Necessities Pediatric Cancer Foundation
Cures Within Reach
Aiden's Army
The MAGIC Foundation
Center for Jewish Genetics
Gene Giraffe Project
The Association for Glycogen Storage Disease
Mary Peyton's Miracle Foundation
Lymphatic Malformation Institute
Sarcoma Foundation of America
Team Serena
Cure HHT
National Tay-Sachs & Allied Diseases Association (NTSAD)
Choroideremia Research Foundation, Inc.
Sophia's Fund
Amyloidosis Research Consortium
Amyloidosis Foundation
Relapsing Polychondritis
Pulmonary Fibrosis Advocates
Info and Resources for Idiopathic Pulmonary Hemosiderosis (IPH-NET)
Fabry Support & Information Group
PKD Foundation
Mastocytosis Society
Little Miss Hannah Foundation
Let Them Be Little X2 Inc.
CureCADA
CARES Foundation, Inc.
The Kortney Rose Foundation
EDSers United Foundation
The Life Raft Group
Alexa Nawrocki Pediatric Cancer Foundation
The Brooke Healey Foundation
The Champ's Corner
OsteoPETrosis Society
Children's Cardiomyopathy Foundation
EB Research Partnership
Jonah's Just Begun
Hannah's Hope Fund
Cardio-Facio-Cutaneous International
Hereditary Neuropathy Foundation
Team Sanfilippo Foundation
Seaphardic Health Organization for Referral & Education
The GIST Cancer Awareness Foundation
The Truth 365
The Arms Wide Open Childhood Cancer Foundation
Pediatric Cancer Foundation
A Kids' Brain Tumor Cure
Hermansky-Pudlak Syndrome Network Inc.
The Adult Polyglucosan Body Disease Research Foundation (APBDRF)
Cooley's Anemia Foundation
National MPS Society
Taylor's Tale
Cure AHC
FMD Chat
BRBN Alliance
Princesses on a Mission, Inc.
Cole vs Cancer
The Rare Cancer Research Foundation
Batten Disease Support & Research Association
Fibromuscular Dysplasia Society of America (FMDSA)
Parent Project Muscular Dystrophy
Help Extinguish Hunter Syndrome
Samuel Szabo Foundation
The Global Foundation for Peroxisomal Disorders
LMSarcoma Direct Research Foundation
MLD Foundation
DEFY Foundation
Drew's Hope Scientific Research Foundation
Dominick One in a Million
Rare Disease United Foundation
Cure Sanfilippo Foundation
Chase After a Cure
Saving Case & Friends
Beyond Batten Disease Foundation
The Ryan Foundation
Bridge the Gap - SYNGAP Education and Research Foundation
NGLY1.org
Aware of Angels
Abigail Alliance for Better Access to Developmental Drugs
Angioma Alliance
Smashing Walnuts Foundation
Journey4ACure
The Rare Childhood Cancer Advocacy Group
Alex's Army Childhood Cancer Foundation