Please Support the OPEN ACT (HR 1223)
Orphan Product Extensions Now, Accelerating Cures and 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).

**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 (FDA). 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.
268 Supporting Patient Organizations (partial list):

A Kids’ Brain Tumor Cure
A-T Children's Project
Abigail Alliance for Better Access to Developmental Drugs
Alex’s Army Childhood Cancer Foundation
Alexa Nawrocki Pediatric Cancer Foundation
Amyloidosis Foundation
Amyloidosis Research Consortium
Angioma Alliance
AWARE of Angels
Batten Disease Support & Research Association
Bear Necessities Pediatric Cancer Foundation
Bert's Big Adventure
Beyond Batten Disease Foundation
BRBN Alliance
Bridge the Gap - SYNGAP Education and Research Foundation
Caleb's Crusade Against Childhood Cancer
Cardio-Facio-Cutaneous International
CARES Foundation, Inc.
Castleman Disease Collaborative Network/Castleman's Awareness & Research Effort
Center for Jewish Genetics
Chase After a Cure
Children's Cardiomyopathy Foundation
Children's PKU Network
Choroideremia Research Foundation, Inc.
Cole vs Cancer
Cooley's Anemia Foundation
Cure AHC
Cure HHT
Cure JM Foundation
Cure Sanfilippo Foundation
Cure SMA
CureCADASIL
CureDuchenne
Cures Within Reach
Curing Retinal Blindness Foundation
DC Outreach Inc.
DEFY Foundation
Desmoid Tumor Research Foundation (DTRF)
Dominick One in a Million
Dravet Syndrome Foundation
Drew’s Hope Scientific Research Foundation
EB Research Partnership
EDSers United Foundation
EveryLife Foundation for Rare Diseases
Fabry Support & Information Group
Fibromuscular Dysplasia Society of America (FMDSA)
FMD Chat
Gene Giraffe Project
Gene Spotlight Inc.
Genetic Alliance
Global Genes Project
GNE Myopathy International
Gold Rush Cure Foundation
Grace Science Foundation
Gwendolyn Strong Foundation
Hannah’s Hope Fund
Help Extinguish Hunter Syndrome
Hereditary Neuropathy Foundation
Hermansky-Pudlak Syndrome Network Inc.
Hope4Bridge Foundation
Hunter Syndrome Research Coalition
Info and Resources for Idiopathic Pulmonary Hemosiderosis (IPH-NET)
International FOP Association
International Pemphigus and Pemphigoid Foundation (IPPF)
International Waldenstrom's Macroglobulinemia Foundation (IWMF)
ISMRD (the International Advocate for Glycoprotein Storage Diseases)
Joey's Wings Foundation
Jonah's Just Begun
Journey4ACure
Kids v Cancer
Klippel-Feil Syndrome Freedom
Let Them Be Little X2 Inc.
Little Miss Hannah Foundation
LMSarcoma Direct Research Foundation
Luck2Tuck Foundation
Lymphatic Malformation Institute
Mary Payton's Miracle Foundation
Mastocytosis Society
MDLFoundation
Myotonic Dystrophy Foundation
National Fragile X Foundation
National Leiomysarcoma Foundation
National MPS Society
National Organization for Rare Disorders (NORD)
National Tay-Sachs & Allied Diseases Association (NTSAD)
NGLY1.org
Noah’s Hope
Noah's Light Foundation
OsteoPETrosis Society
Parent Project Muscular Dystrophy
Pediatric Cancer Foundation
Phelan-McDermid Syndrome Foundation
PKD Foundation
POMC Island One boy an Ocean of friends
Prader-Willi Syndrome Association
Princesses on a Mission, Inc.
Pulmonary Fibrosis Advocates
Rare Disease United Foundation
RARE Science, Inc.
RASopathies Network USA
Relapsing Polychondritis
Samuel Szabo Foundation
Sarcoma Foundation of America
Saving Case & Friends
Sephardic Health Organization for Referral & Education
Sickle Cell Warriors, Inc.
Smashing Walnuts Foundation
Sophia's Hope, Inc.
Sophia's Fund
Supporting Our Cancer Kids
Talia’s Legacy Children’s Cancer Foundation
Taylor's Tale
Team Sanfilippo Foundation
Team Serena
The Adult Polyglucosan Body Disease Research Foundation (APBDRF)
The Arms Wide Open Childhood Cancer Foundation
The Association for Glycogen Storage Disease
The Children's Medical Research Foundation, Inc.
The Coalition for Pulmonary Fibrosis
The GIST Cancer Awareness Foundation
The Global Foundation for Peroxisomal Disorders
The Kortney Rose Foundation
The Life Raft Group
The MAGIC Foundation
The Nicholas Conor Institute
The Rally Foundation for Childhood Cancer Research
The Rare Cancer Research Foundation
The Rare Childhood Cancer Advocacy Group

For a complete list of supporters, go to http://everylifefoundation.org/open-act/.