



June 3, 2022

The Honorable Patty Murray  
Chairwoman  
Committee on Health, Education, Labor & Pensions  
United States Senate  
Washington, D.C. 20510

The Honorable Richard Burr  
Ranking Member  
Committee on Health, Education, Labor & Pensions  
United States Senate  
Washington, D.C. 20510

Dear Chairwoman Murray and Ranking Member Burr,

The 85 undersigned organizations representing patients with rare disorders urge you to incorporate S. 4185, the Retaining Access and Restoring Exclusivity (RARE) Act, as introduced by Senator Tammy Baldwin and Senator Bill Cassidy, into the FDA Safety and Landmark Advancements Act (FDASLA). The RARE Act would clarify the original intent of the Orphan Drug Act (ODA) and codify the Food and Drug Administration's (FDA) long-standing interpretation of that landmark law. Our organizations are deeply concerned that a decision from a recent court case, if not corrected by the enactment of the RARE Act, could hinder continued progress in rare disease drug development. The implications of this case could leave some rare disease patients, including children or those with less common variations of a rare disease, without access to an FDA approved treatment that has been proven to be safe and effective for their specific circumstances and/or condition.

The ODA provides a set of incentives to support research and development into drugs for rare diseases. One of the key incentives is a seven-year term of “exclusivity” for the orphan drug once approved and marketed. The ODA established a two-part process for obtaining orphan drug exclusivity. First, at an early stage in development, a company can request that FDA “designate” the drug as an orphan drug to prevent, diagnose or treat a rare disease or condition. Once a company receives this designation from the FDA, the company can access other ODA incentives, including tax credits for research and clinical testing of the drug. Second, after completing the necessary clinical studies and obtaining FDA approval, the drug is then awarded exclusivity that protects from competition the specific use of the drug that is approved.

In most cases, the orphan designation is intentionally broader than the use ultimately approved. For instance, a drug might be designated for the treatment of Fabry’s disease, a rare lysosomal storage disorder. After conducting studies in the disease, the sponsor may have only obtained data sufficient to support approval for a narrower population than the entire patient population with Fabry’s disease, such as only adults with the disease. Similarly, many orphan drugs being developed for cystic fibrosis (CF) receive orphan designation for the disease broadly, but, after years of continued drug development, may ultimately be approved for use in specific subpopulations of CF patients, such as those with specific mutations.

However, the recent 11th Circuit decision in the case of *Catalyst Pharms., Inc. v. Becerra*, if left unaddressed by Congress, would overturn FDA’s decades-long interpretation of the ODA that the exclusivity protects the “use or indication” ultimately approved. The Court instead held that the rare disease designated at the outset of the drug development process dictates the scope of the orphan drug exclusivity. This decision threatens to undermine 40 years of practice and would incentivize sponsors to seek broader designations for an entire rare disease at the outset, leaving little incentive to continue to study the safety and efficacy of that drug in special populations, like children. More than half of people with rare diseases are children, so the implications of this Court ruling have the potential to be significant.

The RARE Act would maintain the original intent of the ODA, making clear that orphan drug exclusivity is tied to the approved indication, while ensuring proper incentives remain in place to foster robust rare disease drug development. Clarifying the scope of orphan drug exclusivity is critical since rare diseases remain an area with significant unmet needs. Over 90% of the estimated 7,000 known rare diseases still do not have an FDA-approved treatment indicated for the specific rare disease. If the RARE Act is not enacted, there is likely to be fewer orphan drugs approved for special patient populations, an outcome that runs counter to the goal of the ODA and is not in the best interest of the rare disease community.

We urge the HELP Committee to modify FDASLA to include the RARE Act and preserve the intent of this critical ODA incentive that has benefited millions of Americans and their families facing rare disease diagnoses. For more information, please contact Heidi Ross, Vice President of Policy and Regulatory Affairs for the National Organization for Rare Disorders, at [HRoss@rarediseases.org](mailto:HRoss@rarediseases.org).

Thank you for your consideration,

National Organization for Rare Disorders  
Alpha-1 Foundation  
Alport Syndrome Foundation  
ALS Association  
Alternating Hemiplegia of Childhood Foundation  
American Academy of Pediatrics  
American Behcet’s Disease Association (ABDA)  
American Cancer Society Cancer Action Network  
APS Foundation of America, Inc  
Avery’s Hope

CAD Foundation  
Canavan Research Foundation  
CancerCare  
CDH International  
Children’s Cancer Cause  
Children’s PKU Network/ NPKUA  
Cholangiocarcinoma Foundation  
Choroideremia Research Foundation  
Congenital Hyperinsulinism International  
CRMO Foundation

Cure CMD  
CURED Nfp (Campaign Urging Research for Eosinophilic Diseases)  
Cutaneous Lymphoma Foundation  
Cyclic Vomiting Syndrome Association  
Cystic Fibrosis Foundation  
Cystic Fibrosis Research Institute (CFRI)  
Dup15q Alliance  
Epilepsy Foundation  
FACES: The National Craniofacial Association  
FOD FAMILY SUPPORT GROUP  
Foundation for Prader-Willi Research  
Foundation For Sarcoidosis Research (FSR)  
FOXG1 Research Foundation  
Gaucher Community Alliance  
Gorlin Syndrome Alliance  
GRIN2B Foundation  
HCU Network America  
Hydrocephalus Association  
HypoPARathyroidism Association  
Immune Deficiency Foundation  
International Foundation for Gastrointestinal Disorders (IFFGD)  
International Pemphigus Pemphigoid Foundation  
Jamal's Helping Hands, Inc.  
Juju and Friends CLN2 Warrior Foundation  
Mississippi Metabolics Foundation  
MLD Foundation  
Muscular Dystrophy Association  
National Association for Continence  
National Ataxia Foundation  
National Eosinophilia Myalgia Syndrome Network  
National Health Council  
National MALS Foundation

National Niemann-Pick Disease Foundation  
NBIA Disorders Association  
NephCure Kidney International  
Neuromuscular Disease Foundation  
Organic Acidemia Association  
PFIC Network  
Phelan-McDermid Syndrome Foundation  
PRISMS  
Pulmonary Fibrosis Foundation  
Pulmonary Hypertension Association  
Rare Army  
Rare Kids Network  
Recurrent Respiratory Papillomatosis Foundation  
Shwachman-Diamond Syndrome Foundation  
Siegel Rare Neuroimmune Association  
Spina Bifida Association  
STXBP1 Foundation  
Team Telomere, Inc.  
The Association for Frontotemporal Degeneration  
The Bonnell Foundation: Living with Cystic Fibrosis  
The Global Foundation for Peroxisomal Disorders  
The Hermansky-Pudlak Syndrome Network  
The Leukemia & Lymphoma Society  
The Life Raft Group  
The RYR-1 Foundation  
The Snow Foundation for Wolfram Syndrome Research  
TSC Alliance  
Turner Syndrome Society of the United States  
United Porphyrias Association  
Vasculitis Foundation  
VHL Alliance

CC: Members of the Senate Committee on Health, Education, Labor & Pensions