



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.

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	National Niemann-Pick Disease Foundation
CURED Nfp (Campaign Urging Research for Eosinophilic Diseases)	NBIA Disorders Association
Cutaneous Lymphoma Foundation	NephCure Kidney International
Cyclic Vomiting Syndrome Association	Neuromuscular Disease Foundation
Cystic Fibrosis Foundation	Organic Acidemia Association
Cystic Fibrosis Research Institute (CFRI)	PFIC Network
Dup15q Alliance	Phelan-McDermid Syndrome Foundation
Epilepsy Foundation	PRISMS
FACES: The National Craniofacial Association	Pulmonary Fibrosis Foundation
FOD FAMILY SUPPORT GROUP	Pulmonary Hypertension Association
Foundation for Prader-Willi Research	Rare Army
Foundation For Sarcoidosis Research (FSR)	Rare Kids Network
FOXG1 Research Foundation	Recurrent Respiratory Papillomatosis Foundation
Gaucher Community Alliance	Shwachman-Diamond Syndrome Foundation
Gorlin Syndrome Alliance	Siegel Rare Neuroimmune Association
GRIN2B Foundation	Spina Bifida Association
HCU Network America	STXBP1 Foundation
Hydrocephalus Association	Team Telomere, Inc.
HypoPARathyroidism Association	The Association for Frontotemporal Degeneration
Immune Deficiency Foundation	The Bonnell Foundation: Living with Cystic Fibrosis
International Foundation for Gastrointestinal Disorders (IFFGD)	The Global Foundation for Peroxisomal Disorders
International Pemphigus Pemphigoid Foundation	The Hermansky-Pudlak Syndrome Network
Jamal's Helping Hands, Inc.	The Leukemia & Lymphoma Society
Juju and Friends CLN2 Warrior Foundation	The Life Raft Group
Mississippi Metabolics Foundation	The RYR-1 Foundation
MLD Foundation	The Snow Foundation for Wolfram Syndrome Research
Muscular Dystrophy Association	TSC Alliance
National Association for Continence	Turner Syndrome Society of the United States
National Ataxia Foundation	United Porphyrias Association
National Eosinophilia Myalgia Syndrome Network	Vasculitis Foundation
National Health Council	VHL Alliance
National MALS Foundation	

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