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
Cancer*Care*
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