



## Statement for the Record

### **S. 1214, the Retaining Access and Restoring Exclusivity (RARE) Act Senate Committee on Health, Education, Labor and Pensions May 2, 2023**

Chairman Sanders, Ranking Member Cassidy and members of the Health, Education, Labor and Pensions Committee, the National Organization for Rare Disorders (NORD) is pleased to provide this statement for the record in support of S. 1214, the RARE Act, introduced by Senator Tammy Baldwin of Wisconsin and cosponsored by Senator Mike Braun of Indiana.

NORD proudly represents the more than 25 million Americans living with one of the more than 7,000 known rare diseases. NORD was founded 40 years ago, after the passage of the Orphan Drug Act (ODA), to formalize the coalition of patient advocacy groups that were instrumental in passing that landmark law. Our mission has always been and continues to be to improve the health and well-being of people with rare diseases by driving advances in care, research, and policy.

Signed into law in 1983, 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,” or market protection from competition for the orphan drug once it is approved and marketed. The law established a two-part process for obtaining orphan drug exclusivity. First, at an early stage in the drug 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, the company can access key ODA incentives, including tax credits for the research and clinical testing on the drug. Second, after completing the necessary clinical studies and obtaining FDA approval, the drug is then awarded exclusivity that protects the specific use of the drug that is approved.

However, with the recent decision in the case of *Catalyst Pharms., Inc. v. Becerra*, the United States Court of Appeals for the Eleventh Circuit rejected 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 that is *designated* at the outset of the drug development process dictates the scope of the orphan drug exclusivity. NORD believes this is an incorrect interpretation of the statute, and in the absence of a legislative fix, is concerned there would be fewer orphan drugs approved for fewer special patient populations. That is not the goal of the ODA, and it is not in the best interest of the rare disease community.

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Therefore, NORD is supportive of legislation recently introduced by Senator Baldwin and cosponsored by Senator Braun, the Retaining Access and Restoring Exclusivity (RARE) Act (S. 1214), which would clarify that orphan drug exclusivity protects the approved use or indication. This legislation is critical to ensuring proper incentives are in place to continue to foster robust rare disease drug development and is grateful for the inclusion of S. 1214 in today's HELP Committee markup. Additionally, yesterday, NORD and an additional 77 patient organizations serving patients in the rare disease community sent a letter of support for S. 1214, the RARE Act and urging that it be swiftly passed out of the HELP Committee. A copy of this letter is attached at the end of this statement for the record.

Chairman Sanders and Ranking Member Cassidy, thank you for your support of the RARE Act and the opportunity to submit this statement for the record. NORD looks forward to working with the HELP Committee on this critical bill and future legislation to improve access to vital care and treatment for rare disease patients. For more information, please contact Heidi Ross, Vice President of Policy and Regulatory Affairs at [HRoss@rarediseases.org](mailto:HRoss@rarediseases.org) or Karin Hoelzer, Director of Policy and Regulatory Affairs at [KHoelzer@rarediseases.org](mailto:KHoelzer@rarediseases.org).

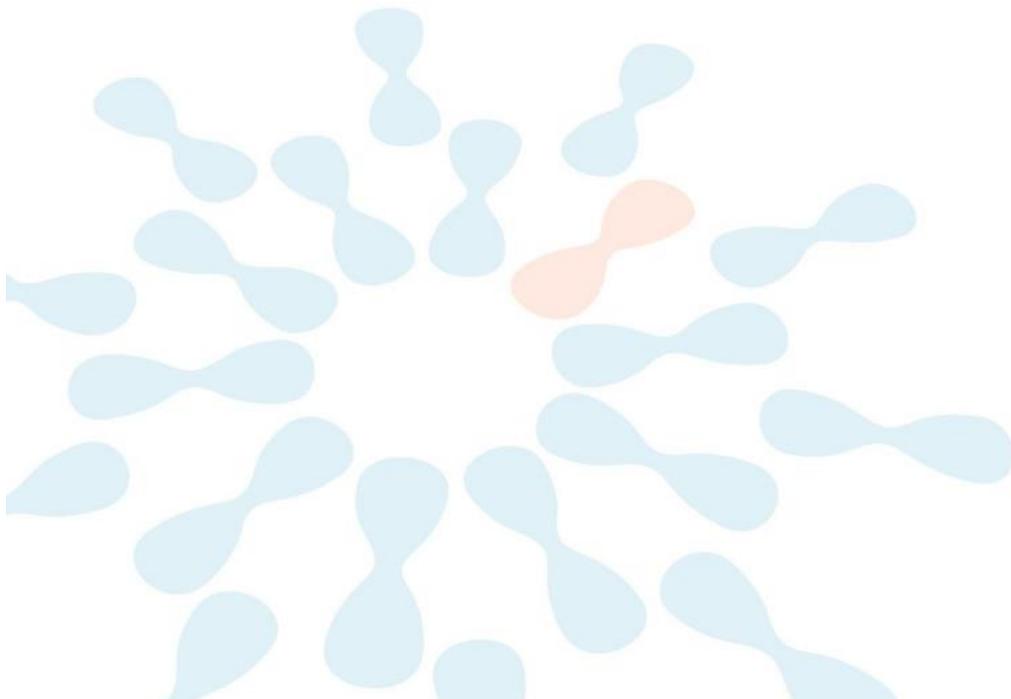
Sincerely,



Heidi Ross, MPH  
Vice President, Policy and Regulatory Affairs  
National Organization for Rare Disorders



Karin Hoelzer, DVM, PhD  
Director, Policy and Regulatory Affairs  
National Organization for Rare Disorders





May 1, 2023

The Honorable Bernie Sanders  
 Chairman  
 Committee on Health, Education, Labor &  
 Pensions  
 United States Senate  
 Washington, D.C. 20510

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

Dear Chairman Sanders and Ranking Member Cassidy,

The 78 undersigned organizations representing patients with rare disorders thank you for including S. 1214, the Retaining Access and Restoring Exclusivity (RARE) Act, as introduced by Senator Tammy Baldwin, in the upcoming markup for HELP Committee consideration. 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. In addition, broadening the scope of exclusivity to apply to an entire disease, rather than specific use, could also delay generic competition.

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, could threaten 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. Broadening the scope of exclusivity to apply to an entire disease, rather than specific use, could also delay generic competition.

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 members of the HELP Committee to support the RARE Act and vote to advance this legislation out of Committee to 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) or Karin Hoelzer, Director of Policy and Regulatory Affairs, at [KHoelzer@rarediseases.org](mailto:KHoelzer@rarediseases.org).

Thank you for your consideration,

National Organization for Rare Disorders  
Adrenal Insufficiency United  
Adult Polyglucosan Body Disease Research Foundation (APBDRF)  
Alport Syndrome Foundation  
ALS Association  
Alternating Hemiplegia of Childhood Foundation  
American Behcet's Disease Association (ABDA)  
American Cancer Society Cancer Action Network  
APS Foundation of America, Inc  
Avery's Hope  
Born a Hero, Research Foundation  
CancerCare  
CDH International  
Children's Cancer Cause  
Children's PKU Network  
Cholangiocarcinoma Foundation  
Chondrosarcoma CS Foundation  
Coalition to Cure Calpain 3  
Congenital Hyperinsulinism International  
Cure CMD  
Cure HHT  
CURED (Campaign Urging Research for Eosinophilic Disease  
cutaneous lymphoma foundation  
Cystic Fibrosis Foundation  
Cystic Fibrosis Research Institute  
Dup15q Alliance  
Epilepsy Foundation  
FACES: The National Craniofacial Association

FOD Family Support Group  
Foundation for Sarcoidosis Research  
FOXG1 Research Foundation  
Friedreich's Ataxia Research Alliance (FARA)  
Gaucher Community Alliance  
Glut1 Deficiency Foundation  
Gorlin Syndrome Alliance  
GRIN2B Foundation  
HCU Network America  
Hepatitis B Foundation  
Hypertrophic Olivary Degeneration Association  
International Foundation for Gastrointestinal Disorders  
International Pemphigus Pemphigoid Foundation  
International Waldenstrom's  
Macroglobulinemia Foundation  
Juju and Friends CLN2 Warrior Foundation  
KrabbeConnect  
Lennox-Gastaut Syndrome (LGS) Foundation  
Mississippi Metabolics Foundation  
MLD Foundation  
MSUD Family Support Group  
Muscular Dystrophy Association  
National Ataxia Foundation  
National Health Council  
National MALS Foundation  
National Niemann-Pick Disease Foundation  
National PKU News  
NBIA Disorders Association  
Necrotizing Enterocolitis (NEC) Society

NR2F1 Foundation  
NTM INFO & RESEARCH  
Oral Cancer Foundation  
Organic Acidemia Association  
Phelan-McDermid Syndrome Foundation  
Pulmonary Fibrosis Foundation  
Pulmonary Hypertension Association  
Smith-Kingsmore Syndrome Foundation  
STXBP1 Foundation  
Superior Mesenteric Artery Syndrome Research  
Awareness and Support  
Team Telomere

The Foundation for Casey's Cure, Inc  
The Leukemia & Lymphoma Society  
The Life Raft Group  
The Mast Cell Disease Society (TMS)  
The RYR-1 Foundation  
The Sudden Arrhythmia Death Syndromes  
(SADS) Foundation  
TSC Alliance  
United MSD Foundation  
United Porphyrrias Association  
Vasculitis Foundation  
Yellow Brick Road Project- HNRNPH2 NDD

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