



**Statement for the Record
National Organization for Rare Disorders**

**Markup of H.R. 7667, the Food and Drug Amendments of 2022
Subcommittee on Health
Committee on Energy and Commerce
May 11, 2022**

Chairwoman Eshoo, Ranking Member Guthrie and Members of the Health Subcommittee, the National Organization for Rare Disorders (NORD) appreciates the opportunity to provide this statement for the record on today's markup of H.R. 7667, Food and Drug Amendments of 2022.

Founded in 1983, NORD represents over 300 different rare disease patient organizations and the 25-30 million Americans living with a rare disease. We are committed to identifying, treating, and curing rare disorders through programs of education, policy, research, and patient services.

It is critically important that Congress reauthorize the Prescription Drug User Fee Act (PDUFA), and the other user fee programs before their current authorization expires and NORD appreciates the bipartisan work of this Committee on H.R. 7667. In particular, NORD would like to highlight two sections of H.R. 7667 that are of great importance to the rare disease community.

Section 804: Post-approval studies and program integrity for accelerated approval drugs

The accelerated approval pathway has proven to be a vital tool in bringing safe and effective treatments to patients with rare disorders. Many facets of rare diseases make them particularly difficult to study in clinical trials targeting direct clinical benefit. For example, the number of patients with a rare condition can be small and heterogeneous, with highly diverse clinical manifestations and a long timeframe for disease progression. Furthermore, there is often a lack of prior clinical studies and a limited number of clinical investigators and treatment centers knowledgeable about a given rare disorder. This makes accelerated approval, and the ability to use surrogate endpoints in the approval process, a particularly important tool for the development of treatments for rare diseases.¹

However, there has been mounting criticism from a variety of stakeholders who have raised concerns about drugs approved under the accelerated approval pathway. NORD is encouraged that this Committee's leadership has included the provisions in Section 804 that will help to alleviate some stakeholder concerns, support continued utilization of the pathway, and enable robust patient access to these FDA approved treatments. Consistent with what was recommended in a [letter to Congressional leaders](#) signed by 91 patient organizations last month, NORD is pleased that sponsors will be required to submit more robust and frequent reports on the progress being made on their post-market confirmatory trials. NORD is also grateful that the bill explicitly allows real world evidence to be used in post-market studies to verify and describe the

¹ U.S. Food & Drug Admin., CDER Drug and Biologic Accelerated Approvals Based on a Surrogate Endpoint (Jan. 14, 2021), <https://www.fda.gov/media/88907/download>

effect on the clinical benefit of the drug, as well as the required report to the Secretary describing the circumstances under which real world evidence was used to support the post-approval studies required as part of an accelerated approval. Additionally, NORD is supportive of the provisions that improve the process to support the timely withdrawal of a product that fails to conduct the post-approval study with due diligence or when the post-market study fails to verify the anticipated clinical benefit.

Finally, NORD also supports the establishment of the Rare Disease Endpoint Advancement (RDEA) Pilot, which will provide critical resources to address novel endpoint development, a long-standing obstacle for rare disease drug development. The RDEA program will provide selected sponsors with an opportunity for earlier structured, repeated interactions with FDA to help in the evaluation and development of appropriate novel endpoints. It is critical that the learnings from this program ultimately be made available so that future rare disease drug applications can build on the progress made within the program.

FDA approval is only the first step to a patient obtaining access to a treatment. True access is achieved when patients can get their treatments prescribed and affordably covered by their health program or insurer. NORD thanks the Energy and Commerce Committee for their commitment to ensuring this pathway remains a viable way for patients to obtain access to FDA approved treatments for their rare condition at the earliest possible time.

Sec. 811. Clarifying application of exclusive approval, certification, or licensure for drugs designated for rare diseases or conditions

NORD is grateful for the inclusion of the language in Section 811 of H.R. 7667 which will protect the intent of the Orphan Drug Act (ODA) and ensure proper incentives are in place to continue to foster robust rare disease drug development.

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 other 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 11th Circuit Court 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. NORD is grateful to see Congress working to correct and clarify the law as part of this year's reauthorization of PDUFA.

Conclusion

Chairwoman Eshoo and Ranking Member Guthrie, thank you for the opportunity to submit this statement for the record and for your efforts to ensure the FDA has the resources and oversight it needs to review and approve new and innovative therapies for rare disease patients. NORD looks forward to working with you and your staff to see the reauthorization of the relevant user fee acts signed into law in a timely manner and effectively implemented to benefit the rare disease community. For more information, please contact Heidi Ross, Vice President of Policy and Regulatory Affairs at HRoss@rarediseases.org.

