January 29, 2020

The Honorable Frank Pallone Jr., Chair
Committee on Energy & Commerce
U.S. House of Representatives
2125 Rayburn House Office Building
Washington, DC 20515

The Honorable Anna Eshoo, Chair
Subcommittee on Health
Committee on Energy & Commerce
U.S. House of Representatives
Washington, DC 20515

The Honorable Greg Walden, Ranking Member
Committee on Energy & Commerce
U.S. House of Representatives
2322 Rayburn House Office Building
Washington, DC 20515

The Honorable Michael Burgess, Ranking Member
Committee on Energy & Commerce
U.S. House of Representatives
2322 Rayburn House Office Building
Washington, DC 20515

Re: Support for H.R. 4712, the Fairness in Orphan Drug Exclusivity Act

Dear Chairman Pallone, Chairwoman Eshoo, Ranking Member Walden, and Ranking Member Burgess:

For 15 years both my passion and job was as the vice president for public policy of the National Organization for Rare Disorders (NORD), which represents the 30 million American patients with rare diseases. Through programs of education, advocacy, research and patient services, NORD seeks to advance medical treatments and medical support services for patients with rare disorders. I remain passionate about the quest to help provide people with rare disorders a better quality and longer life.

One of the most significant and forceful tools that has aided patients with rare diseases is the Orphan Drug Act (ODA). It was passed by Congress in 1983 to provide incentives for the development of newer and better therapies. These incentives have supported the development of hundreds of drugs for rare or orphan diseases since 1983. The law has truly been successful, and the successes have accelerated in the last few years.

In the past few years, fully one-third of new drugs approved by the FDA have been for rare diseases. The total number of orphan indications approved by the FDA jumped from 594 in 2016 to more than 770 in 2018. These approvals reflect not just a commitment by the FDA but also the success of the ODA. It gives companies seven years of exclusivity for drugs with an orphan indication, a 25% tax credit for qualified clinical trials, and the waiver of application fees.

However, more than 90% of the 7,000 rare diseases identified so far don't have any FDA-approved treatments. And insurers, whether private or government-financed, are increasingly making it more challenging for patients to access orphan drugs. The challenges of access will likely become more acute as gene therapy — products that alter a patient's genetic makeup and cure disease — become more available.

Because we have so much more to accomplish, we must make sure the law remains strong. But a loophole exploited by a pharmaceutical company (but recently corrected by the FDA in a one-time act) identified a threat to the integrity of this important law. We must address this loophole so that other companies do not use it in the future to their own financial advantage but to the disadvantage of patients.

H.R. 4712 provides a fix by closing the loophole. I urge you to move this bill through the Congress as quickly as possible.

This loophole was unbeknownst to me until last year when I read a news story about how in 1994 a pharmaceutical company received orphan status for a drug used to treat opioid addiction, even though opioid addiction isn't a rare disease. As I learned, the drug's maker based its application on a rarely used provision in the ODA that permits FDA to grant orphan drug designation when a manufacturer can show that a drug is unlikely to be profitable and will not recover its research and development (R&D) costs.

The law now provides that this orphan drug designation is automatically granted to later products from the same manufacturer that contain the same ingredient if they are shown to be superior to previously-approved drugs containing the same ingredient. The manufacturer thus can gain another seven years of exclusivity.

The loophole is obscure and not well understood. But it does not constitute sound health or public policy for a manufacturer to gain basically a renewal of orphan drug exclusivity without having to demonstrate an inability to recoup its R&D costs.

Over a seven-year period economic circumstances can and often do change, and thus the inability of a manufacturer to be able to recoup its R&D costs can easily disappear. But because the law does not require the manufacturer to again demonstrate that it cannot recoup its R&D costs, a drug could literally make billions due to changing circumstances, and still maintain its orphan designation.

H.R. 4712 addresses this loophole. It would require a company that obtains the economic orphan designation to show after its initial seven-year exclusivity period that it continues to be unable to cover its R&D costs.

H.R. 4712 would only apply to products that received orphan drug designation because the manufacturer has demonstrated an inability to recoup its R&D costs. It would require that for a new (follow on) product to receive orphan designation it would need to show once again – as the original product did – that it would not be able to recoup its R&D costs. It would **NOT** impact orphan products that received designation due to disease prevalence.

While I no longer represent the millions of Americans with an orphan disease, I strongly believe that we must maintain the integrity of the Orphan Drug Act. Drugs that exist thanks to the ODA have saved lives, and future lives depend on this law continuing to work well. We cannot allow mischief and mayhem to stand in the way of the missions of the Law and the incredible outcomes it has produced for people across the country.

H.R. 4712 would do just that.

My work on behalf of patients with rare diseases was the most important thing I have ever done. I appreciate this opportunity to once again represent the best interests of the millions of men, women and children affected by rare diseases. Thank you for your consideration.

Respectfully Submitted,

Diane Edquist Dorman