September 13, 2021

The Honorable Richard Neal
Chairman
House Committee on Ways and Means
1102 Longworth House Office Building
Washington, DC 20515

The Honorable Kevin Brady
Ranking Member
House Committee on Ways and Means
1139 Longworth House Office Building
Washington, DC 20515

Dear Chairman Neal and Ranking Member Brady,

On behalf of the 1-in-10 individuals living in the United States with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) urges you to remove Section 138141 of the Manager's Amendment to the "Build Back Better Act" in Committee before this bill advances to the full House for consideration. This provision would undermine the Orphan Drug Act of 1983 (ODA) by limiting the availability of the Orphan Drug Tax Credit (ODTC) to only the first approved orphan use of a new drug. Given that more than 90% of rare diseases lack an FDA-approved drug, this proposal would have a devastating impact on orphan drug development in the US, and the millions of rare disease patients who continue to hope for a therapeutic option that treats their condition.

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. We are committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services. Since its inception in 1983, NORD has maintained independence in its advocacy positions and initiatives, which are formulated by senior staff, the Board of Directors, and NORD's medical advisors; pharmaceutical industry representation in such activities has always been prohibited.

A rare disease is defined as a disease or condition that affects less than 200,000 people in the United States.[1] Given the unique challenges associated with developing drugs for small patient populations, prior to 1983, there was little interest by the pharmaceutical industry in pursuing these therapies. At that time, there were less than 30 available drugs specifically approved for rare diseases. Congress sprung to action and passed the Orphan Drug Act of 1983,[2] which provided a variety of incentives for manufacturers to invest in the research and development of treatments for orphan diseases. One of the critical incentives in the ODA was the ODTC, which

[1]Section 526, Federal Food, Drug and Cosmetic Act [21 USC 360bb]
[2] P.L. 97-414

originally provided for a 50% credit of qualified clinical testing expenses associated with developing orphan drugs.

By all accounts, the ODA has been a resounding success at spurring the development of rare disease drugs.[3] Today, there are 652 drugs approved for 1,006 rare disease conditions.[4] While this is significant progress, there is more work to be done. Dr. Gayatri Rao, the former Director of FDA's Office of Orphan Products, succinctly described the unmet need that persists:

> *My sincere hope is that the interest in rare diseases is sustained. Even though there is this tremendous growth, if we take a step back and think about the larger context, there are more than 7000 rare diseases, and that number is likely much higher. When you think about that larger context versus the number of products that are actually approved to treat those rare diseases, it's a small fraction. There is still a tremendous unmet need out there. I'm hopeful that this interest will continue and will be sustained in the long run.*[5]

Section 138141 of the just-released Manager's Amendment would dramatically curtail the ODTC incentive by limiting its availability to only the first approved orphan use of a new drug. The importance of FDA orphan drug approval for rare disease patients simply cannot be understated. For the millions of rare disease patients without access to an FDA-approved drug, every time an orphan indication is approved by FDA, whether that be on a first-in-class drug or an already-marketed drug, it is critical, and often, life-saving progress. Even after FDA has approved a drug for an orphan indication, there must be appropriate incentives, like the ODTC, to encourage continued development of new orphan uses of a drug. Additional indications added to a drug's label give more rare disease patients assurance that the drug is safe and effective for them.

NORD shares the concern about the high cost of drugs and has issued drug pricing principles[6] to underscore our commitment to addressing it. But as lawmakers consider ways to lower drug costs, it is important to recognize that orphan drugs account for only 11% of the overall drug spend in the United States, according to an IQVIA report commissioned by NORD.[7] Thus, in its efforts to alleviate the burden of high prescription drug costs, Congress should focus on the true

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[3] FDA. John Swann, Ph.D., FDA historian. *The Story Behind the Orphan Drug Act.* (2018). Accessed 9/13/21. https://www.fda.gov/industry/orphan-products-development-events/story-behind-orphan-drug-act

[4] FDA. Orphan Drug Database. Accessed 9/13/21. https://www.accessdata.fda.gov/scripts/opdlisting/oopd/index.cfm

5 Medscape. John J. Whyte, MD, MPH; Gayatri R. Rao, MD, JD. (2016). Orphan Disease R & D Has a Home at FDA. https://www.medscape.com/viewarticle/871115

[6] NORD. Drug Pricing Principles. Accessed 9/13/21. https://rarediseases.org/wp-content/uploads/2019/06/nord-policy-drug-pricing-priniciples-final-2019.pdf

[7] IQVIA. Orphan Drugs in United States (2021). Accessed 9/13/21. https://rarediseases.org/wp-content/uploads/2021/03/orphan-drugs-in-the-united-states-NRD-2020.pdf

drivers of high drug costs, and not attack the incentives necessary for developing products for those who need them the most: rare disease patients.

Congress already took action that seriously undermined the ODTC in 2017 when it slashed the 50% credit to 25%. NORD opposed these devastating cuts then and urges Congress to maintain the ODTC as it stands today, so that rare disease patients can maintain their hope that new orphan uses of drugs will continue to be pursued. Please ensure that Section 138141 is removed in Committee prior to consideration of the Build Back Better Act by the full House.

Sincerely,

Peter L. Saltonstall
President and CEO
National Organization for Rare Disorders

CC: Members of the House Committee on Ways and Means