September 20, 2021

The Honorable Charles E. Schumer
Majority Leader
United States Senate
322 Hart Senate Office Building
Washington, DC 20510

The Honorable Mitch McConnell
Minority Leader
United States Senate
317 Russell Senate Office Building
Washington, DC 20510

The Honorable Ron Wyden
Chairman
Committee on Finance
United States Senate
219 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Mike Crapo
Ranking Member
Committee on Finance
United States Senate
219 Dirksen Senate Office Building
Washington, DC 20510

Dear Majority Leader Schumer, Minority Leader McConnell, Chairman Wyden, and Ranking Member Crapo,

We, the undersigned organizations, representing individuals living with rare diseases in the U.S., urge you not to include the provision that was reported out of the House Committee on Ways and Means on September 15, 2021,[1] that would undermine the Orphan Drug Act (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 U.S., and the millions of rare disease patients our organizations represent who continue to hope for a therapeutic option that treats their condition.

A rare disease is defined as a disease or condition that affects less than 200,000 people in the United States.[2] 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,[3] 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 138141 of the Ways and Means Committee's portion of the "Build Back Better Act" https://waysandmeans.house.gov/sites/democrats.waysandmeans.house.gov/files/documents/NEAL_032_xml.pdf
[2] Section 526, Federal Food, Drug and Cosmetic Act [21 USC 360bb]
[3] 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.[4] Today, there are 652 drugs approved for 1,006 rare disease conditions.[5] While this is significant progress, there is more work to be done given that millions of Americans with rare diseases still do not have access to an FDA-approved drug for their condition or disease.

The Ways and Means Committee's ODTC provision 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 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.

Congress already took action that seriously undermined the ODTC in 2017 when it slashed the 50% credit to 25%. We urge 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.

If you have any questions or need further information about the Orphan Drug Tax Credit or its benefit to rare disease patients, please contact Heidi Ross, Director of Policy for the National Organization for Rare Disorders, at HRoss@rarediseases.org.

Sincerely,

National Organization for Rare Disorders

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[4] 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

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