[11/22/2021]

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

The Honorable Mitch McConnell
Minority Leader
U.S. Senate
317 Russell Senate Office Building
Washington, DC 20510

The Honorable Ron Wyden
Chairman, Committee on Finance
U.S. Senate
219 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Mike Crapo
Ranking Member, Committee on Finance
U.S. Senate
219 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Bernie Sanders
Chairman, Committee on the Budget
U.S. Senate
624 Dirksen Senate Office Building
Washington DC, 20510

The Honorable Lindsey Graham
Ranking Member, Committee on the Budget
U.S. Senate
624 Dirksen Senate Office Building
Washington, DC 20510

We, the 24 undersigned organizations representing people and families living with and affected by the epilepsies, urge you to remove the provisions related to the Orphan Drug Tax Credit (ODTC) in the *Build Back Better* legislation. This provision would undermine the Orphan Drug Act (ODA) by limiting the availability of the ODTC to only the first approved orphan use of a drug. With 95 percent of individuals who live with a rare disease—including many with rare forms of epilepsy—still waiting for a treatment, we hope you will work to maintain this critical incentive for orphan drug development.

According to the CDC, at least 3.4 million Americans live with epilepsy and seizures, including about 470,000 children. Epilepsy is a medical condition that produces seizures, which affect a variety of mental and physical functions, and often includes serious comorbidities like anxiety, depression, cognitive or behavioral issues, or sleep disorders. Approximately 1 in 26 Americans will develop epilepsy at some point in their lifetime. In the past two decades, our understanding of epilepsy has evolved; epilepsy is now viewed as a spectrum of diseases with different underlying causes and varied experiences. Advances in genetics and mechanistic understanding have accelerated the discovery of many epilepsy-causing gene variants and complex chromosomal anomalies. As a result, there are an increasing number of rare epilepsy diagnoses made in both rare and ultra-rare epilepsies. Many people diagnosed with a rare epilepsy are disproportionately impacted by developmental delays, behavioral disorders, sleep disorders, and other impairments or co-morbidities. Further, they experience disproportionate challenges in daily activities like school, work, driving, relationships, and social interactions. Some people with a rare epilepsy are significantly physically and cognitively disabled and require round the clock care, life-saving equipment, frequent hospital visits, and extensive support and services. Existing treatments for controlling seizures are very seldom able to fully control seizures for those with rare epilepsy diagnoses.

A rare disease is defined as a disease or condition that affects less than 200,000 people in the United States.[1] In 1983, due to the unique challenges associated with developing drugs for small patient

[1] Section 526, Federal Food, Drug and Cosmetic Act [21 USC 360bb]

populations, there were less than 30 available drugs specifically approved for rare diseases. Congress passed the Orphan Drug Act of 1983, 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 originally provided for a 50% credit of qualified clinical testing expenses associated with developing orphan drugs. In 2017 Congress weakened the ODTC, reducing the amount of the tax credit from 50% to 25%.

This provision currently included in the House version of the *Build Back Better* legislation would 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 cannot be understated. Every time an orphan indication is approved by FDA, whether that be on a first-in-class drug or an already-marketed drug, it provides critical and life-saving progress for children and adults with rare disease including the rare epilepsies who did not previously have access to an FDA-approved drug. Even after the 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. And critically important, additional indications added to a drug's label give more people with rare epilepsy assurance that the drug is safe and effective for them.

There are still no standardized clinical guidelines for many rare epilepsy syndromes, no curative treatments other than surgery in a very few diagnoses, few treatments to reduce seizure frequency or improve developmental outcomes and quality of life, and no centralized method for identifying individuals with rare epilepsies to improve surveillance, understanding of population, or to conduct comparative effectiveness research. Many epilepsy medications are prescribed off label. When existing drugs are approved for new indications, physicians gain valuable insight from the clinical trials conducted by the manufacturer about how the medication will work for people with the newly indicated conditions. Often, health insurance providers will not cover, or will create barriers to access, for an off-label prescription—delaying access to treatment which can be life-threatening for people with the epilepsies. New indications help people with the epilepsies not only access the right medication but also have coverage for it.

By all accounts, the ODA has been a resounding success at spurring the development of rare disease drugs.[2] Today, there are 652 drugs approved for 1,006 rare disease conditions.[3] While there has been progress and treatment approvals for a few of the rare, severe epilepsies such as Dravet Syndrome and Lennox-Gastaut Syndrome, there is much more work to be done given that thousands of Americans with rare epilepsies still do not have access to an FDA-approved drug for their condition or disease. There are also many rare epilepsies with promising precision therapies—capitalizing on the future of medicine and the potential for many lives transformed and money saved –in the pipeline that depend on this incentive to advance. The ODTC was already weakened in 2017 when it was lowered from 50% to 25%. **We urge Congress to maintain the ODTC as it stands today so that people with rare epilepsy and their families can maintain hope that new orphan uses of drugs will continue to be pursued.** If you have any questions or concerns, please contact Laura Weidner, the Epilepsy Foundation's Vice President of Government Relations & Advocacy at lweidner@efa.org.

[2] 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

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

Sincerely,
BDSRA (Batten Disease Support and Research Association)
BPAN Warriors
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FamilieSCN2A Foundation
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