September 21, 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

The Honorable Bernie Sanders
Chairman
Committee on the Budget
United State Senate
624 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Lindsey Graham
Ranking Member
Committee on the Budget
United State Senate
624 Dirksen Senate Office Building
Washington, DC 20510

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

The 87 undersigned organizations representing individuals living with rare diseases in the U.S. urge you not to include the provision 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 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

[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]

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 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
A Cure for Ellie
Acromegaly Community Inc.
Alagille Syndrome Alliance

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[3] P.L. 97-414

[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

ALS Association
American Partnership for Eosinophilic Disorders
American Porphyria Foundation
Angelman Syndrome Foundation
Appendix Cancer Pseudomyxoma Peritonei Research Foundation (ACPMP)
Association for Creatine Deficiencies
Autoimmune Encephalitis Alliance, Inc
Barth Syndrome Foundation
BPAN Warriors
Cauda Equina Foundation, Inc.
CDH International
Children's PKU Network/ NPKUA
Congenital Hyperinsulinism International
CRMO Foundation
Cure CMD
Cure SMA
Cure VCP Disease, Inc.
Cutaneous Lymphoma Foundation
Cystinosis Research Network
DEFEAT MSA ALLIANCE & MSA UNITED CONSORTIUM
Dravet Syndrome Foundation
Dreamsickle Kids Fdn
Dup15q Alliance
Epilepsy Foundation
EveryLife Foundation for Rare Diseases
Fibromuscular Dysplasia Society of America
Foundation for Prader-Willi Research
Friedreich's Ataxia Research Alliance (FARA)
Gaucher Community Alliance
Global DARE Foundation
HCU Network America
Hepatitis B Foundation
Hyper IgM Foundation
Indian Organization for Rare Diseases
International Autoimmune Encephalitis Society
International Pemphigus and Pemphigoid Foundation
International Waldenstrom's Macroglobulinemia Foundation
ISMRD
Lennox-Gastaut Syndrome (LGS) Foundation
LGDA
MitoAction
Multiple System Atrophy Coalition
Muscular Dystrophy Association

Myasthenia Gravis Foundation of America
Myocarditis Foundation
National Brain Tumor Society
National CMV Foundation
National Eosinophilia Myalgia Syndrome Network
National Health Council
National MALS Foundation
National PKU Alliance
National PKU News
National Tay-Sachs & Allied Diseases Association (NTSAD)
NBIA Disorders Association
NTM Info & Research
PFIC Network, Inc.
Phelan-McDermid Syndrome Foundation
Polycystic Kidney Disease Foundation
Rare and Undiagnosed Network (RUN)
Rare Epilepsy Network (REN)
RASopathies Network
Recurrent Respiratory Papillomatosis Foundation
Reflex Sympathetic Dystrophy Syndrome Association
Rett Syndrome Research Trust
Ring14 USA
SATB2 Gene Foundation
SLC6A1 Connect
STXBP1 Foundation
Superficial Siderosis Research Alliance
Syngap Research Fund (SRF)
SYNGAP1 Foundation
Team Telomere
The Guthy-Jackson Charitable Foundation
The LAM Foundation
The RYR-1 Foundation
The Snyder-Robinson Foundation
TSC Alliance
United Leukodystrophy Foundation
Vasculitis Foundation
Vestibular Disorders Association
VHL Alliance
Wilhelm Foundation
Xia-Gibbs Society, Inc