

March 19, 2018

The Honorable Paul Ryan, Speaker  
United States House of Representatives  
H-232, The Capitol  
Washington, D.C. 20515

The Honorable Nancy Pelosi, Minority Leader  
United States House of Representatives  
H-204, The Capitol  
Washington, D.C. 20515

Dear Speaker Ryan and Leader Pelosi:

The undersigned organizations collectively represent millions of patients with serious and life-threatening diseases. We write to express our concern with, and opposition to, the latest version of the *Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act* released on March 10, 2018. While this version of the legislation includes patient safety improvements compared to previous versions of the legislation, we reiterate our concern with creating a secondary pathway for accessing investigational therapies outside of clinical trials that would remove Food and Drug Administration (FDA) approval and consultation, and would not increase access to promising therapies for our patients because it does not address the primary barriers to such access.

FDA's expanded access program, though imperfect, facilitates access to investigational therapies for over a thousand patients facing serious and life-threatening conditions each year. FDA repeatedly approves over 99 percent of requests while sometimes making important dosing and safety improvements to proposed expanded use. Conversely, it is often times the pharmaceutical company that denies access to its investigational therapy outside of its clinical trials for any number of reasons.

We recognize that the latest draft of the legislation incorporates improvements that address some of the patient safety concerns we have consistently raised with prior versions of the legislation. These improvements include a more limited eligibility for this pathway compared to the Senate-passed legislation, more robust informed consent requirements, more frequent and thorough reporting to FDA, and the requirement for manufacturers to provide additional public reporting on the use of this pathway.

However, the alternative pathway in the latest version of the legislation is still less safe for our patients than the current expanded access process. This alternative pathway would allow for a 7-day lag between access to investigational therapies (as well as potential ensuing adverse events) and FDA notification. FDA is also prohibited from halting access to these experimental therapies short of placing a clinical hold on all clinical research on the therapy in question, which is a blunt and disproportionate measure. The legislation would also remove FDA's consultation on dosing, route of administration, dosing schedule, and other important safety measures available under FDA's current expanded access program.

We appreciate the changes that were made to address some of the safety concerns our community has raised with previous legislation, and acknowledge the concerted effort to consider stakeholder perspectives. Our collective organizations remain opposed to the current draft of the legislation. We welcome the opportunity to continue constructive dialogue on ways to improve the ability of patients to genuinely and safely access both approved and unapproved lifesaving therapies.

Sincerely,

A Twist of Fate-ATS  
ADNP Kids Research Foundation  
Adult Polyglucosan Body Disease Research Foundation  
AIDS Action Baltimore  
Alliance for Aging Research  
Alliance of Dedicated Cancer Centers  
American Cancer Society Cancer Action Network  
American Lung Association  
American Society of Clinical Oncology  
American Syringomyelia and Chiari Alliance Project  
Amyloidosis Support Groups  
APS Type 1 Foundation  
Association for Creatine Deficiencies  
Association of American Medical Colleges  
Benign Essential Blepharospasm Research Foundation  
Bonnie J. Addario Lung Cancer Foundation  
Bridge the Gap - SYNGAP Education and Research Foundation  
CancerCare  
Charlotte and Gwenyth Gray Foundation to Cure Batten Disease  
Children's Cardiomyopathy Foundation  
Congenital Hyperinsulinism International  
cureCADASIL  
CurePSP  
Cutaneous Lymphoma Foundation  
Cystic Fibrosis Foundation  
Defeat MSA  
The Desmoid Tumor Research Foundation  
The Disability Rights Legal Center  
Dup15q Alliance  
Dysautonomia Foundation  
Dyskeratosis Congenita Outreach, Inc.  
Equal Access for Rare Disorders  
Fight Colorectal Cancer  
FORCE: Facing Our Risk of Cancer Empowered  
Friedreich's Ataxia Research Alliance (FARA)  
Friends of Cancer Research  
The Global Foundation for Peroxisomal Disorders  
Glut1 Deficiency Foundation  
The Guthy-Jackson Charitable Foundation  
Hemophilia Federation of America  
HLRCC Family Alliance  
Hope for Hypothalamic Hamartomas  
Hyper IgM Foundation, Inc.  
Indian Organization for Rare Disorders  
International Fibrodysplasia Ossificans Progressiva (FOP) Association  
International Myeloma Foundation

International Pemphigus and Pemphigoid Foundation  
International Society for Stem Cell Research  
International Waldenstrom's Macroglobulinemia Foundation (IWMF)  
The Isaac Foundation  
Jack McGovern Coats' Disease Foundation  
The LAM Foundation  
The Leukemia & Lymphoma Society  
Li-Fraumeni Syndrome Association (LFS Association / LFSA)  
LUNGevity Foundation  
Lymphangiomatosis & Gorham's Disease Alliance  
M-CM Network  
Mattie Miracle Cancer Foundation  
MitoAction  
MLD Foundation  
Moebius Syndrome Foundation  
The MSA Awareness Shoe  
Mucopolysaccharidosis Type IV Foundation  
The Myelin Project  
Myotonic Dystrophy Foundation  
National Brain Tumor Society  
National Comprehensive Cancer Network  
National Consumers League  
National Health Council  
National MPS Society  
National Niemann-Pick Disease Foundation  
National Organization for Rare Disorders (NORD)  
National Patient Advocate Foundation  
National PKU Alliance  
National PKU News  
Neurofibromatosis Northeast  
The Oley Foundation  
Operation ASHA  
PCS Partners Seeking a Cure  
Platelet Disorder Support Association  
PRP Alliance, Inc.  
Pulmonary Fibrosis Foundation  
Rare and Undiagnosed Network (RUN)  
Rothmund-Thomson Syndrome Foundation  
Scleroderma Foundation  
The Snyder-Robinson Foundation  
Sofia Sees Hope  
SSADH Association  
Susan G. Komen  
TargetCancer Foundation  
Tarlov Cyst Disease Foundation  
Team Audrey

Treatment Action Group  
The Turner Syndrome Society  
United Leukodystrophy Foundation  
United Mitochondrial Disease Foundation (UMDF)  
Vasculitis Foundation  
Veterans Health Council  
Vietnam Veterans of America  
VHL Alliance  
Wilhelm Foundation  
Worldwide Syringomyelia & Chiari Task Force  
The XLH Network, Inc.

CC: The Honorable Kevin McCarthy, Majority Leader  
The Honorable Steny Hoyer, Minority Whip