March 12, 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,

ADNP Kids Research Foundation
Alliance for Aging Research
American Cancer Society Cancer Action Network
American Lung Association
American Society of Clinical Oncology
American Syringomyelia and Chiari Alliance Project
Amyloidosis Support Groups
Association for Creatine Deficiencies
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
Cutaneous Lymphoma Foundation
Cystic Fibrosis Foundation
Defeat MSA
The Disability Rights Legal Center
Dup15q Alliance
Dysautonomia Foundation
Equal Access for Rare Disorders
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.
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
The LAM Foundation
The Leukemia & Lymphoma Society
Li-Fraumeni Syndrome Association (LFS Association / LFSA)
LUNGevity Foundation
M-CM Network
Mattie Miracle Cancer Foundation
MitoAction
MLD Foundation

Moebius Syndrome Foundation
The MSA Awareness Shoe
Mucolipidosis 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
PRP Alliance, Inc.
Rare and Undiagnosed Network (RUN)
Scleroderma Foundation
The Snyder-Robinson Foundation
Sofia Sees Hope
SSADH Association
Susan G. Komen
TargetCancer Foundation
Treatment Action Group
The Turner Syndrome Society
United Leukodystrophy Foundation
United Mitochondrial Disease Foundation (UMDF)
Veterans Health Council
Vietnam Veterans of America
VHL Alliance
Worldwide Syringomyelia & Chiari Task Force

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