May 21, 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 strong opposition to the *Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act* (S.204).

On March 21st, The House of Representatives passed a version of the Right to Try Act (H.R.5247), that incorporated important patient safeguards such as more robust informed consent and public reporting requirements, additional Food and Drug Administration (FDA) oversight, and a narrower definition of eligibility for this pathway. The Senate version does not include these safeguards and therefore could greatly increase the likelihood of our patients being harmed by unsafe and ineffective experimental therapies. Therefore, this version is substantially worse for patients.

We reiterate our concern with creating a secondary pathway for accessing investigational therapies outside of clinical trials. This pathway removes FDA approval and consultation and would not increase access to promising therapies for our patients because it does not address the primary barriers to 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.

The Senate version of the legislation is less safe than the pathway proposed in the House version and is dangerous compared to the current expanded access process. The Senate's bill would allow unproven therapies to be given to patients without FDA notification for up to a full year and would not establish any standards for informed consent.

Additionally, both versions prohibit FDA from halting access to these experimental therapies short of placing a clinical hold on all clinical research on the therapy in question. Both House and Senate versions 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.

While we did not support the recent House passed version of this legislation, the House legislation includes improved patient safeguards compared to the Senate version. The Senate version would negatively impact patient safety substantially, and our collective organizations are strongly opposed. We appreciate past efforts in the House to consider stakeholder perspectives and desire to continue the dialogue, but returning to the Senate version is simply not the way forward.

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.
Incontinentia Pigmenti International Foundation
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
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
The Oley Foundation
Operation ASHA
Organic Acidemia Association
PSC Partners Seeking a Cure
Platelet Disorder Support Association
PRP Alliance, Inc.
Pulmonary Fibrosis Foundation
Rare and Undiagnosed Network (RUN)
Rothmund-Thomson Syndrome 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 Greg Walden, Chairman, House Committee on Energy and Commerce
The Honorable Frank Pallone, Ranking Member, House Committee on Energy and Commerce