March 22, 2018

The Honorable Mitch McConnell, Majority Leader
United States Senate
S-230, The Capitol
Washington, D.C. 20515

The Honorable Chuck Schumer, Minority Leader
United States Senate
S-221, The Capitol
Washington, D.C. 20515

Dear Leaders McConnell and Schumer:

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 *Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act* (H.R.5247) passed by the House of Representatives on March 21, 2018. While this version of the legislation includes some 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 H.R.5247 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, 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 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.
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
Melorheostosis Association
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
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 Lamar Alexander, Chairman, Senate HELP Committee
The Honorable Patty Murray, Ranking Member, Senate HELP Commitee