February 6, 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 once again express our strong opposition to S.204, the *Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017*, as well as H.R.878, the *Right to Try Act of 2017*. If this legislation is brought to the House floor for a vote, we urge House Leadership and all Members to join the patient community and oppose the legislation.

Our organizations support patient access to unapproved therapies. However, the Right to Try bills currently under consideration in the House do not effectuate policy changes that would afford our patients greater access to promising investigational therapies. Instead, these bills would likely do more harm than good. We would welcome the opportunity to discuss alternative legislative proposals that would improve the ability of patients to genuinely and safely access unapproved therapies.

We do not believe S.204 or H.R.878 would successfully increase access to promising investigational therapies for those in need. Both of these bills remove the Food and Drug Administration (FDA) from the initial approval process for accessing an investigational therapy outside of a clinical trial. Removing FDA from this process is not likely to facilitate increased access to investigational therapies because FDA currently approves 99.7 percent of all expanded access requests submitted by physicians and companies for patients with immediately life-threatening illnesses who cannot participate in clinical trials.[1] In a recently released report examining the current FDA expanded access program, the Government Accountability Office (GAO) found that substantial changes were not needed within the program, aside from greater clarity on the use of adverse event data.[2]

When access to a therapy is denied to a patient, it is generally the company that denies the request, and for reasons that appear to be reasonable, such as a determination that the benefits do not outweigh the risks, an unavailability of sufficient product to offer outside of clinical trials, costs, or concerns about adversely affecting clinical trial enrollment.

Mr. Kenneth I. Moch, President and CEO of Cognition Therapeutics, Inc. and witness at the House Committee on Energy and Commerce's Subcommittee on Health's hearing on accessing investigational drugs, made this point abundantly clear when he remarked in his testimony that, "the argument that

[1] Jarow, Jonathan P., et al. "Expanded access of investigational drugs: the experience of the Center of Drug Evaluation and Research over a 10-year period." *Therapeutic innovation & regulatory science* 50.6 (2016): 705-709.
[2] GAO, "FDA Has Taken Steps to Improve the Expanded Access Program but Should Further Clarify How Adverse Events Data Are Used," July 2017.

Right to Try legislation is going to make more people have access to experimental medicines does not exist in my mind as a drug developer nor in anybody I know, and I can't say it more bluntly than that."[3]

At this same hearing, FDA Commissioner Dr. Scott Gottlieb argued, "I think there is a perception, …that there are certain companies and products that aren't necessarily being offered under the current construct and the Right to Try legislation might provide more of an incentive and an opportunity. …I don't necessarily see that same opportunity because I think the biggest obstacle to offering drugs through expanded access is the supply constraints."[4]

Further, it is important to remember that the current regulatory system for medical products and research in the United States was created as a result of serious patient harm and exploitation that occurred early in the 20th Century. Birth defects resulting from Thalidomide are an example of what happens when drugs are given to humans without proper safety review and approval. While obtaining unapproved therapies outside of a clinical trial is not about research, the products themselves remain experimental and have not been shown to be safe and effective. Clinical research subject protections are in place when experimental products are being tested to ensure the safe and ethical treatment of research participants. Patients seeking expanded access to unapproved therapies outside of clinical trials must be afforded the same ethical standards and protections as patients taking part in clinical trials.

Existing expanded access policies are not without room for improvement. We encourage Congress to review other policy options, and we stand ready to serve as a resource as Congress examines this important issue.

We also ask Congress to provide oversight as FDA moves forward with implementation of relevant provisions enacted within the past several years that improve the expanded access system. These include the requirements within the *21st Century Cures Act* for the public posting of expanded access policies on company websites, and greater clarity from FDA on the use of adverse event data. Several provisions in the *Food and Drug Administration Reauthorization Act* (FDARA) also improve access to investigational therapies, such as the allowance for IRBs to appoint one individual to review applications rather than a fully convened IRB. FDARA also directs FDA to further investigate inclusion/exclusion criteria within clinical trials, a key factor in the number of individuals able to access investigational therapies.

Any further action taken to advance the Right to Try legislation currently under consideration in the House is not in our patients' best interests. As you consider bringing this troubling legislation to the House floor for a vote we ask that you remain mindful of our concerns, and take into consideration the negative impact the policies could have on patients living with life threatening disease.

We welcome the opportunity to work with all members of the House of Representatives to develop alternative legislative proposals that would improve the ability of patients to genuinely and safely access both approved and unapproved innovative, lifesaving therapies.

Sincerely,

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[3] "Examining Patient Access to Investigational Drugs." October 03, 2017. http://docs.house.gov/meetings/IF/IF14/20171003/106461/HHRG-115-IF14-Transcript-20171003.pdf.

[4] Ibid

American Cancer Society Cancer Action Network
American Lung Association
American Society of Clinical Oncology
Asbestos Disease Awareness Organization
Breast Cancer Action
Bridge the Gap -SYNGAP Education and Research Foundation
Chloe's Fight Rare Disease Foundation
Congenital Hyperinsulinism International
Cystic Fibrosis Foundation
Disability Rights Legal Center
Dyskeratosis Congenita Outreach, Inc.
Fight Colorectal Cancer
FORCE: Facing Our Risk of Cancer Empowered
Friedreich's Ataxia Research Alliance
Friends of Cancer Research
International Myeloma Foundation
International Society for Stem Cell Research
The Leukemia & Lymphoma Society
Lung Cancer Alliance
LUNGevity Foundation
M-CM Network
MLD Foundation
Moebius Syndrome Foundation
Myotonic Dystrophy Foundation
National Brain Tumor Society
National Consumers League
National Health Council
National Organization for Rare Disorders (NORD)
National Patient Advocate Foundation
Oncology Nursing Society
The Prevent Cancer Foundation
PSC Partners Seeking a Cure
Pulmonary Fibrosis Foundation
Susan G. Komen
TargetCancer Foundation
United Mitochondrial Disease Foundation
VHL Alliance
Wilkins Parkinson’s Foundation

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