September 7, 2017

The Honorable Greg Walden, Chairman
2125 Rayburn House Office Building
U.S. House Committee on Energy and Commerce
Washington, DC 20515

The Honorable Frank Pallone, Ranking Member
2471 Rayburn House Office Building
U.S. House Committee on Energy and Commerce
Washington, DC 20515

Dear Chairman Walden and Ranking Member Pallone,

The undersigned organizations collectively represent millions of patients with serious and life-threatening diseases. We write to 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*, currently under consideration in the House Energy and Commerce Committee. We urge the Committee to proceed through regular legislative order to facilitate discussion and consideration of alternative policies that would genuinely increase access to promising investigational therapies for the communities we represent.

Our organizations support patient access to unapproved therapies, but S.204, and H.R.878 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 encourage the Committee to hold hearings to examine these issues more closely, as well as consider other policy options to improve the ability of patients to 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 will not facilitate increased access to investigational therapies because FDA is not a barrier. 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] The Government Accountability Office (GAO) recently released a report examining the current FDA expanded access program, and 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.

It is important to remember that the current regulatory system for medical products in the United States was created as a result of serious patient harm and exploitation that occurred early in the 20th Century.

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[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.

The Tuskegee Syphilis Study, experimentation on humans during WWII, and birth defects resulting from Thalidomide are all examples of what happens when drugs are given to humans without proper safety and efficacy 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 the Committee to examine the predominant reasons why patients interested in access to experimental therapies are ultimately unable to obtain them by enrolling in clinical trials or through the current expanded access process. We also ask the Committee to provide oversight as FDA moves forward with implementation of relevant provisions enacted within the past year 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) will 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.

We are eager to work with the Committee as it considers these proposals, and endeavors to ensure patients gain greater access to investigational therapies. We welcome the opportunity to work with members of the Committee, as well as the sponsors of this legislation, to improve and increase access to both approved and unapproved innovative, lifesaving therapies.

Sincerely,

American Cancer Society Cancer Action Network
Friends of Cancer Research
National Organization for Rare Disorders (NORD)

CC: The Honorable Paul Ryan, Speaker
The Honorable Kevin McCarthy, Majority Leader
The Honorable Nancy Pelosi, Minority Leader
The Honorable Steny Hoyer, Minority Whip