September 5, 2017

Dear Members of the United States House of Representatives:

The undersigned groups respectfully urge you to oppose S. 204 — which is deceptively titled the "Right to Try Act of 2017" but should instead be called the "False Hope Act of 2017."

We recognize the desire of patients with terminal illness who have exhausted available treatment options to access experimental medical products that have not been approved or cleared by the Food and Drug Administration (FDA). However, the best way for patients to gain such access is through the FDA's Expanded Access Program, which allows seriously ill patients to receive treatment with experimental medical products while also providing basic safeguards to protect patients' rights and welfare. Importantly, the recently enacted FDA Reauthorization Act of 2017, which renewed the FDA's user fee programs, included responsible bipartisan language intended to enhance the agency's Expanded Access Program.

We are concerned that S. 204, as amended and passed by the U.S. Senate on August 3, 2017, would put countless patients at risk by undermining important FDA safety rules related to the use and oversight of unapproved, experimental medications. Such legislation would expose vulnerable patients to risks of serious harm, including dying earlier and more painfully than they otherwise would have, without appropriate safeguards.

**FDA's Current Expanded Access Program**

Currently, the FDA oversees the use of all experimental drugs and biological products in the U.S. The FDA's Expanded Access Program allows patients across the country to gain access to such products, provided that each patient's doctor believes such access is appropriate and that the manufacturer of the product agrees to provide it for that use.

To protect patients, the FDA and an institutional review board (IRB) must approve each use of an experimental drug or biological product under the Expanded Access Program. As conditions of approval, there must be sufficient evidence of the safety and effectiveness of the experimental drug to support its use in a particular patient, and the probable risk to the patient from the drug must not be greater than the probable risk from the disease or condition. The program further protects patients by requiring a robust informed consent process that is similar to the consent process for a clinical trial, as well as monitoring and reporting of serious adverse events.
The FDA grants 99 percent of all Expanded Access Program requests and, in urgent circumstances, can respond to such requests within one or two days. The agency also recently streamlined the program to require less paperwork. In addition, the 21st Century Cures Act of 2016 included useful provisions that require drug manufacturers to publicly post their expanded access policies and provide points of contact for requests. The potential impact of these streamlining efforts has yet to be fully realized.

It is also important to recognize that many of the experimental products made available through this program ultimately are not shown to be safe and effective in clinical testing and are not approved or cleared by the FDA.

**Undermining Patient Protections While Offering False Hope**

The false-hope legislation passed by the Senate and now being considered by the House would create a dangerous, uncharted pathway for access to experimental drugs and biological products that essentially bypasses the protections of the FDA's Expanded Access Program for patients diagnosed with life-threatening diseases or conditions — a patient population that is much broader than "patients diagnosed with a terminal illness," which was the patient population covered by the original version of S. 204.

Of particular concern, this alternative pathway for accessing experimental drugs and biological products would put vulnerable patients at risk and undermine their rights by:

- ➢ Specifying completion of a single phase I clinical trial as the evidentiary threshold for allowing use of experimental drug products under the legislation. Such a threshold is insufficient for allowing use of an experimental drug outside the context of a clinical trial because initial phase 1 clinical trials often only involve healthy volunteers, typically involve testing of a single dose of an experimental drug, provide no meaningful data on efficacy, and yield only very limited preliminary data on safety.
- ➢ Eliminating the requirements for review and approval by the FDA and an IRB, which help to ensure that proposed uses of experimental drugs do not pose unacceptable risk to patients and that the patients are fully informed of the risks and other key information when their consent is sought.
- ➢ Eliminating the requirements that (a) the consent of the patient be sought only under circumstances that provide the patient with sufficient opportunity to consider whether or not to participate and that minimize the possibility of coercion or undue influence; (b) the information given to the patient when consent is sought be understandable to the patient; and (c) the consent process exclude exculpatory language through which the patient is made to waive or appear to waive any of his or her legal rights, or releases or appears to release the investigator, the sponsor, the institution, or its agents from liability for negligence.
- ➢ Broadly immunizing sponsors, manufacturers, prescribers, and dispensers from liability for any alleged acts or omissions related to eligible experimental drugs, unless the relevant conduct constitutes reckless or willful misconduct, gross negligence, or an intentional tort under applicable state law. This provision would bar suits in a variety of situations in which state law might reasonably impose liability. For example, it would immunize manufacturers from being held accountable for harm caused by contamination of an investigational drug product, which can be serious. It also would bar state-law negligence suits against the physician prescribers; for example, if the physician negligently prescribed an investigational drug that was known to be contraindicated for a particular patient's set of circumstances, but the situation did not arise to "gross negligence." Decisions about liability in such situations are properly based on consideration of the specific facts, and the bill's immunity provision may cause physicians to be less careful in making prescribing decisions for seriously ill patients.
- ➢ Eliminating the requirement that the treating physician report immediately to the manufacturer or sponsor any serious adverse events regardless of whether they are considered drug-related.

In closing, we urge you to oppose S. 204 and any similar false-hope legislation that is introduced in the future. Thank you for considering our views on this important matter.

Sincerely,

Public Citizen
ACTUP New York
Breast Cancer Action
Doctors For America
END AIDS NOW
Government Accountability Project
Health GAP
Jacobs Institute of Women's Health
MedShadow Foundation
National Consumers League
National Physicians Alliance
National Women's Health Network
Richard N. Gottfried, Chair, Committee on Health, New York State Assembly
Social Security Works
The Annie Appleseed Project
The Society for Patient Centered Orthopedics
Treatment Action Group
Washington Advocates for Patient Safety