

August 2, 2017

Dear Members of the United States Senate:

The undersigned groups respectfully urge you to oppose efforts to include S. 204 — which is deceptively labeled the “Right to Try Act of 2017” but should instead be called the “False Hope Act of 2017”— as an amendment to the pending legislation to renew the Food and Drug Administration’s (FDA’s) user fee programs.

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 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 drugs, biological products or medical devices while also providing basic safeguards to protect patients’ rights and welfare and maintaining strong incentives for careful clinical testing and timely product development. Importantly, the Senate and House of Representatives bills to renew the user fee programs both include responsible bipartisan language that would enhance the agency’s Expanded Access Program.

We are concerned that false-hope legislation like S. 204 would put countless patients at risk by dramatically undermining the FDA’s role in ensuring that medical products are safe and effective before they become widely used. 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. It also would undermine incentives for companies to swiftly develop life-saving products for FDA approval and impair review of these products by limiting the agency’s access to unfavorable information.

FDA’s Current Expanded Access Program

The FDA’s Expanded Access Program allows patients across the country to gain access to experimental drugs, biological products and medical devices, 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. The program protects patients by requiring informed consent, ethical review by an institutional review board, safety monitoring and the reporting of adverse events to the FDA. It also prevents manufacturers from profiting from the use of experimental products, which helps to maintain incentives to continue rigorous clinical testing aimed at FDA approval.

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. Despite patients’ hopes, there is no evidence that the current Expanded Access Program helps more patients than it harms.

Broadly Attacking Patient Protections While Offering False Hope

Rather than proposing further improvements to the existing program, the false-hope legislation being considered by the Senate would undermine the FDA's fundamental authority to oversee the use of experimental medical products and to ensure they are safe and effective before they become widely used.

The legislation would put vulnerable patients at risk by:

- Offering manufacturers broad rights to sell experimental medical products after only very preliminary clinical testing, when little is known about a product's potential risks, let alone its benefits.
- Eliminating important federal safeguards intended to protect the rights and welfare of patients exposed to such products, including appropriate, fully informed consent; ethical review by an IRB; and safety monitoring.
- Allowing manufacturers to charge high prices for experimental medical products, thus forcing patients to take financial risks for unproven benefits.
- Stripping away legal protections for patients by immunizing manufacturers, doctors and others against liability, even if they failed to exercise reasonable care or inform vulnerable patients about potential risks and benefits of the experimental products.
- Preventing the FDA from enforcing good manufacturing practices or intervening to stop the sale of tainted or otherwise substandard experimental medical products.

The legislation also would slow the development and impair FDA review of new medical products by:

- Reducing incentives to continue rigorous clinical testing in pursuit of FDA approval.
- Discouraging patients from enrolling in placebo-controlled clinical trials by providing them with access to experimental medical products in the general marketplace.
- Prohibiting the agency from considering information about side effects, injuries or deaths in patients treated with experimental medical products under the legislation.

Instead of attacking the FDA's authority to regulate experimental medical products and encouraging false hope for patients, Congress should pass legislation, such as Sec. 610 of H.R. 2430 or Sec. 806 of S. 934, to enhance the agency's Expanded Access Program.

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
AIDS Action Baltimore
Breast Cancer Action
Citizens Commission on Human Rights
Doctors For America
END AIDS NOW
Government Accountability Project

August 2, 2017, Letter to Congress Regarding S. 204

Health GAP

Institute for Safe Medication Practices

Jacobs Institute of Women's Health

National Consumers League

National Women's Health Network

Richard N. Gottfried, Chair, Committee on Health, New York State Assembly

Social Security Works

Society for Patient Centered Orthopedics

Treatment Action Group

Washington Advocates for Patient Safety

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