Editorial

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Right to try–a poor solution to a non-problem

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Bringing some order to the plethora of state "Right to Try" laws, the Twenty-first Century Cures Act has increased interest in the role of Right To Try laws in the care of terminally ill patients. Taken at face value, the concept of giving desperate patients the opportunity to make one last effort by permitting them to use investigational drugs seems like a reasonable and humane goal. But it's proponents down play the fact that patients who choose to use an investigational drug won't enjoy the advantage in documented safety and efficacy that are the result of the Food and Drug Administration's rigorous regimen of laboratory studies, animal studies and controlled, carefully monitored trials in patients. These data are then subject to review by a panel of experts prior to a recommendation regarding approving or rejection the sponsors request for FDA clearance to market the product. That's a huge gap.

What are the risks to patients who exercise their right to try? First, the drug will have only been tested through the first element of the FDA testing regimen, the Phase I trial. Phase 1 trials (sometimes described as "first in man," are devoted to testing of a drug on healthy volunteers primarily for dose ranging. Completion of Phase I does not represent a determination of safety or efficacy so the patient may be exposed to unknown hazards. Second the pharmaceutical company may refuse to provide the drug for ongoing use even if it was initially provided to the patient. There may be a cost to obtain the drug and a patient's treatment with an investigational drug may not be covered by insurance.

Third, the pharmaceutical company is protected from liability claims if the unapproved drug proves to be harmful. So what's the big deal? It's that the Right to try legislation is completely unnecessary. The FDA has a well-established program, Expanded Access/ compassionate use, that does all that Right to Try tries to do with far fewer risks to patients. Compassionate use is defined by FDA as "the use outside of a clinical trial of a medical product that has not been approved by the FDA" Of course, detractors may write this off as just another overly complex bureaucratic program that that's slow, unresponsive and difficult to utilize. Others may see this as another form of government intrusion and a threat to patient autonomy.

But such criticism represents a poor understanding of the FDA policy as it currently functions. The request for compassionate use needs to be made by the physician who will treat the patient. FDA has published very complete and helpful guidance on how to apply to any of the Expanded Use programs. FDA provides phone access seven days a week for expedited emergency use requests. Current mean response time is currently reported to be four days. The vast majority of requests are approved. A study by FDA indicated that between January 2005 and December 2014 99.3% of all requests were approved. More recently a review of requests submitted in 2015–2017 for the two FDA offices with the highest number of submissions, CDER and CBER, showed a comparable approval rate.

The facts are clear, FDA's existing Expanded Access/compassionate use program addresses the same problem that has been the subject of intense public interest an extensive political lobbying. The difference is in the degree of safety, based on science that is provided to patients. It is hard to understand why there is so much activity that not only ignores the FDA's programs but also seeks to actively circumvent the FDA in the name of "regulatory relief" which begs the question "to what end" the answer appears to be a long running hostility to all forms of regulation. The FDA is living up to its standard as the world's foremost consumer protection agency. Attempts to weaken the FDA reduce the agency's ability to provide the level of protection the American public deserves.

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Conflicts of interest

The author declares that there is no conflict of interest.