Right To Try: Questions Answered

Right To Try allows terminally ill Americans to try medicines that have passed Phase I of the FDA approval process and remain in clinical trials, but are not yet widely available. Right To Try could expand access to potentially life-saving treatments years before patients would normally be able to access them.

In little over a year, Right To Try has become law in 41 states and counting: Alabama, Alaska, Arizona, Arkansas, California, Colorado, Connecticut, Florida, Georgia, Idaho, Illinois, Indiana, Iowa, Kentucky, Louisiana, Maine, Maryland, Michigan, Minnesota, Mississippi, Missouri, Montana, Nebraska, Nevada, New Hampshire, North Carolina, North Dakota, Ohio, Oklahoma, Oregon, Pennsylvania, South Carolina, South Dakota, Tennessee, Texas, Utah, Virginia, Washington, West Virginia, Wisconsin, and Wyoming. It has passed with overwhelming bipartisan support in all 41 states. *In fact, in most states, it has passed through the state legislature unanimously.*

This document answers the common questions about the impact of Right To Try.

Question: What is the Right To Try Act?
Answer: Right To Try is state legislation that allows terminally ill patients to work with their doctors and drug or device manufacturers to access investigational treatments that have passed the FDA's safety testing phase (Phase I), but are not yet widely available.

Question: Why was Right To Try developed?
Answer: More than 1 million Americans die from a terminal illness every year. Many spend years searching for a potential cure, or struggle in vain to get accepted into a clinical trial. Unfortunately, FDA red tape and government regulations restrict access to promising new treatments, and sometimes for those who do get access, it's too late. Fewer than 3 percent of terminally ill patients gain access to investigational treatments through clinical trials. Right To Try was designed to help the other 97 percent.

Question: Right To Try advocates state that the FDA is the key obstacle to patients getting permission to try investigational treatments. In fact, the FDA approves 99% of requests it receives from physicians seeking to try investigational drugs on seriously or terminally ill patients. So why is Right To Try needed?
Answer: Only about 1,000 people make it through the FDA's "compassionate use" application process each year. The process is complicated, time-consuming, and

expensive. The first step in the process requires a doctor to complete an application that the FDA estimates takes 100 hours. After the doctor submits the application to the FDA, the drug manufacturer must also submit lengthy documentation requirements. The FDA then has a month to review the submission and either grant or deny the request, but if there are any questions the one-month clock starts over. After the FDA approves a request, a separate committee not affiliated with the FDA, called an Institutional Review Board, also must approve the patient's use of the drug. The Institutional Review Board can sometime take up to a month to reach a decision. Sadly, there are many documented cases of patients dying while their application is being considered.

Earlier this year, the FDA announced plans to shorten the application, but the other steps will still remain in place unchanged. A shorter application for the first step is helpful, but it only addresses one part of the approval process. And ultimately, it's still an application to the government to ask permission to try to save your own life. If you have a terminal illness, you don't have time for a multi-step government process. If your child is dying and you know there's an investigational medication that is already helping other children survive, a shorter form isn't good enough. We need to remove barriers that limit doctors from providing the care they are trained to give—and this is exactly what Right To Try does.

Question: Right To Try laws may create a sense of false hope for patients that they will get guaranteed access to an investigational drug or device. In fact, under the law, manufacturers aren't compelled to approve a patient's request for access to experimental treatment. Why didn't you require drug companies to help?
Answer: Because these drugs are investigational, it wouldn't be appropriate to force a manufacturer to provide a treatment they aren't ready or prepared to administer to large numbers of people. But manufacturers who are ready should be able to work directly with patients and their doctors to offer treatments that may save someone's life. Right To Try doesn't guarantee that an investigational medication will work; but it guarantees that if there is a promising medication that is working for other people in an FDA-sanctioned clinical trial and the manufacturer would like to provide it to a dying person, the government won't stand in the way.

Question: Right To Try laws require informed consent of the patient. But in some cases, little is known about the requested investigational product. For example, some patients request drugs or devices that have never been tested for efficacy on humans. Couldn't this hurt people?
Answer: The only drugs available under Right To Try must have passed FDA safety testing on humans and be part of the ongoing FDA approval process. Although some of these medicines may have unknown adverse effects, some even severe, all terminal illnesses have one certain adverse effect: death. Right To Try can only be used by patients who have received a terminal diagnosis from licensed medical professionals and they must have exhausted all known treatment options, and in most states the law also requires that they cannot be eligible or able to participate in an on-going clinical

trial. The people Right To Try is designed to help are out of options, and if they want to try a medicine that has been shown to work on other people, they should have that right.

Right To Try laws simply afford terminal patients, unable to participate in restrictive clinical trials, the same access to investigational medications that the FDA is already allowing for clinical trial patients enrolled in Phase II and III tests.

Question: Right to try laws claim to give terminal patients access to unapproved treatments such as investigational drugs, biologics, and devices after these patients have exhausted all approved treatment options. In fact, there is no evidence that Right To Try laws have helped anyone. Why is that?
Answer: While Right To Try laws have been passing swiftly nationwide, they are still new. The first law passed just over a year ago. As with any new policy that makes a significant change to the way things are currently done, it will take time for doctors, patients, and drug manufacturers to learn about Right To Try and what is and is not permissible. The Goldwater Institute has been working with doctors in a handful of Right To Try states to prepare for making treatments available under the law.

Question: The FDA controls drug approval, and it trumps state law. How can states legally do this?
Answer: It is well established that the U.S. Constitution was designed to provide a floor of protection for individual rights, not a ceiling. States may provide additional and greater protections of individual rights—and all of them do. For instance many states protect free speech rights to a greater extent than the U.S. Constitution, others provide greater privacy rights. While the Supreme Court has never addressed Right To Try specifically, it has held that states have great latitude in regulating health and safety, including medical standards, which are primarily and historically protected as a matter of local concern.

The Supreme Court has recognized a state's power to govern the practice of medicine involving terminal patients. For example, in *Gonzales v. Oregon*, the Court upheld the state's "right to die" law, enacted by Oregon voters, over the objections of the U.S. Attorney General, who argued that federal law preempted the state law. Considering the Supreme Court deferred to a state's authority to protect a person's right to die, it would be consistent for the Court to protect a patient's right to try to save his own life.

In *Abigail Alliance v. Von Eschenbach*, a three judge panel found that the due process clause of the 5th Amendment guaranteed terminally ill patients' access to investigational treatments that had passed FDA Phase I safety testing. However, upon a request by the FDA for a rehearing by the full court, that ruling was reversed. That decision is not binding on any other federal court outside the D.C. Circuit. Most important, it did not involve the same scenario presented by Right To Try, including the fact that no state law protecting access to investigational drugs was in place at the time.

Question: Won't Right To Try hurt the clinical trial process?

Answer: Right To Try will not negatively impact the clinical trial process—it may even complement it. Investigational medicines that are available to terminal patients through Right To Try must be part of an on-going clinical trial. If a drug is removed from the clinical trial process, it will no longer be available to patients under Right To Try. Furthermore, in most states in order to be eligible for a drug under Right To Try the patient cannot be eligible for or able to enroll in a clinical trial. Only 3 percent of patients today are enrolled in clinical trials, and the trials often exclude the sickest people. Right To Try will not reduce the number of people participating in trials, but it will allow more patients to access drugs being tested, giving doctors and scientists even more information about safety and effectiveness.

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