

## Frequently Asked Questions Right-to-Try and Expanded Access to Investigational Therapies

**Q: What is an investigational therapy?**

A: An investigational therapy is a drug, biologic, or medical device that is in development and undergoing testing but has not been approved by the Food and Drug Administration (FDA), since required and rigorous level of testing has not been completed through clinical trials.

**Q. What does “right-to-try” mean, exactly?**

A: “Right-to-Try” (RTT) refers to legislation that aims to speed patient access to investigational therapies that have not been approved by FDA.

**Q: What is the expanded access program?**

A: FDA’s expanded-access program, known as “compassionate use,” allows very ill patients to access investigational treatments outside of clinical trials.

**Q: What is the difference between expanded access and right-to-try?**

A: Both allow patients to apply for access to investigational therapies outside of clinical trials, but FDA’s expanded access program provides guidance on important aspects of patient care, such as appropriate dosing and managing side effects, to treating physicians who would otherwise likely have no clinical experience with the investigational drug a patient might receive. Under expanded access, insurance companies are also required to cover the cost of drugs obtained under compassionate use and the cost of care associated with the drug. Right-to-try laws do not provide these patient protections.

**Q: I understand that FDA oversight has benefits, but doesn’t it also slow down the process?**

A: FDA approves 99.7 percent of all expanded access requests, with many being approved in several days and even quicker for non-emergency cases.

**Q: I’ve heard that doctors’ groups have concerns about right-to-try. What are they?**

A: RTT laws lack important patient safeguards compared to the expanded access program. For example, RTT leaves treating physicians without guidance on critical aspects of patient care, such as appropriate dosing and managing side effects, since doctors likely have no clinical experience with the investigational drug a patient might receive under RTT.

Insurance companies are also not required to cover the cost of drugs obtained under RTT or even the cost of care associated with the drug (as they are in the FDA program). As a result, patients with cancer who receive treatment under RTT assume greater financial risk during one of the most trying times of their life.

**Q: Does right-to-try mean I have the right to receive an experimental drug?**

A: No, because RTT laws do not require drug manufacturers to provide requested drugs. These laws simply allow patients to ask for investigational therapies. Manufacturers only provide the drugs if they choose to do so. A drug company, however, may decide not to provide an investigational drug for any number of reasons. Perhaps it produced only enough of an investigational therapy for a clinical trial or maybe it’s too expensive for a company to provide the drug. A company may also be concerned about negatively affecting clinical trial enrollment if they gave an investigational therapy to someone who is

not in their clinical trial, because clinical trials results are what is needed for FDA's approval of a drug for the patient community as a whole.

**Q: But I (or a loved one) am out of options. Isn't it worth the risk?**

A: Under RTT, patients face significantly more risks than they do in the expanded access program. RTT laws do not provide enough oversight for the drugs that would be given through RTT to provide patients with a good assessment of the potential benefits and potential risks of treatment that should be a part of any cancer treatment decision-making process.

**Q: My request to access an investigational drug through right-to-try was denied by the manufacturer. What should I do now?**

A: First, talk with your doctor. The best way to gain access to an investigational drug is to enroll in a [clinical trial](#). Clinical trials offer hope for many people with cancer, who oftentimes, would like their experience with cancer to help other individuals who might face a cancer diagnosis in the future. If you can participate, you will get the same level of care you would with cancer treatment outside of a clinical trial. Although clinical trials are considered experiments, there is also a chance the treatment will help you.

That said, clinical trials have strict rules that may limit some patients from enrollment. However, a patient may still be able to receive an investigational drug through FDA. Those options include:

- **Expanded access/compassionate use** – Patients may be able to take an investigational drug by enrolling in a large or mid-sized expanded access program through the drug maker. The doctor then submits a request to the FDA, which reviews requests on a case-by-case basis. [Learn more about expanded access/compassionate exemption from the FDA.](#)
- **Individual Patient INDs** – FDA's Individual Patient Investigational New Drug (IND) application allows doctors to request access to a new drug for a patient. Criteria for this includes that the patient is not eligible for any clinical trials and there are no other treatment options. There must be enough data to show that the drug may be effective and has no unreasonable risks.

For more information, visit the [Drug Approval and Labeling](#) page on [Cancer.Net](#).

**Q: Applying for expanded access sounds complicated. Where can I get some help?**

A: [Cancer.Net](#), ASCO's patient information website, offers [online information](#) for patients on navigating the expanded access application process and links to resources from [FDA](#).

The [Reagan-Udall Expanded Access Navigator](#) provides physicians, patients, and caregivers with guidance on the expanded access process.

**For Further Reading:**

- Cancer.Net – [Drug Approval and Labeling](#)
- *The Philadelphia Inquirer*: "[What patients should know about Pa.'s new 'right to try' legislation](#)," by ASCO President Bruce E. Johnson, MD, FASCO, and Marilyn J. Heine, MD, legislative chair of the Pennsylvania Society of Oncology and Hematology (PSOH)