



PATIENT PLAYBOOK

Getting a Drug That Isn't Approved Yet

How expanded access and compassionate use actually work

When standard treatment stops working and no clinical trial fits, there is one more pathway. It is called expanded access, or compassionate use. The two terms mean the same thing.

It is real, it is regulated by the FDA, and thousands of people use it every year. It is also poorly explained, which is why most people who could pursue it never hear about it.

The single most important thing to understand

You cannot request expanded access yourself. Your doctor has to.

The FDA requires the request to come from a licensed physician. You can call a company and ask about their program, and you should. But the formal request, the paperwork, and the responsibility for supervising treatment all sit with a doctor who agrees to take it on.

This means your first task is not finding a drug. It is finding a physician willing to pursue one.

What has to happen

Four separate things must all be true. Any one of them can stop the process, which is why it takes time and why persistence matters.

- **A physician agrees to request and supervise it.** Usually your oncologist, but it can be another doctor willing to take on the case.
- **The company agrees to provide the drug.** This is voluntary. No company is required to say yes, and this is where most requests stall.
- **The FDA authorizes it.** In practice the FDA authorizes the large majority of requests it receives, and often within days for emergencies.
- **An institutional review board (IRB) approves it.** For single-patient requests this can be a single IRB chair rather than a full committee meeting.



What the FDA is not

Many people assume the FDA is the obstacle. It usually is not.

The FDA authorizes the overwhelming majority of expanded access requests, and has an emergency process that can respond within 24 hours. The harder step is almost always getting a company to agree and a physician to take it on.

Why companies say no, and what actually helps

Understanding the company's position is not about sympathy. It is about knowing which arguments land.

Providing a drug to one patient outside a trial sits outside what a developer is set up to do. It is not part of their protocol. They are not obligated to offer it. Each request means diverting limited supply, arranging safety monitoring, preparing regulatory paperwork, and accepting risk they did not have to take on. A serious side effect outside a controlled trial can complicate the studies that thousands of other patients are depending on.

Some companies have formal programs and staff to run them. Many do not. And even where willingness exists, the process runs through agreements and approvals that were not built for someone whose disease is moving.

None of that means give up. It means ask precisely.

Step by step

1

Find out whether the company has a policy. It probably does.

Under the 21st Century Cures Act, manufacturers of investigational drugs are required to make their expanded access policies public. Most people do not know this.

The policy usually states whether they consider single-patient requests, how to submit, and roughly how long they take. Look on the company's website under "expanded access," "compassionate use," or "patient access."

The **Reagan-Udall Foundation Expanded Access Navigator** collects many of these policies in one searchable place. It is the single most useful starting point and it is free.

2

Talk to your doctor before you talk to the company

A company will ask, early on, which physician will be requesting and supervising. If you do not have an answer, the conversation ends there.

Ask your oncologist directly: *"If we identified an investigational drug that might fit, would you be willing to submit an expanded access request and supervise treatment?"*

If the answer is no, that is worth knowing early. Some physicians decline because of institutional policy, some because of the administrative load, some because they do not think it is right for you clinically. Ask which, because the first two can sometimes be solved by finding a different doctor and the third is medical advice worth listening to.

3

Make the specific ask, not the general one

"Can you help my father" is hard to act on. A specific request is not.

Include: the exact drug name or trial identifier, the diagnosis and relevant biomarkers, what treatments have already been tried, the name and contact details of the requesting physician, and the treating institution.

Keep it short and factual. The person reading it is often a medical affairs staffer deciding whether to escalate.

4

Expect it to take time, and follow up

Responses can take weeks. Agreements, supply logistics, and shipping arrangements take longer still.

Follow up politely and persistently. Ask for a named contact and a realistic timeline. Ask what would need to be true for a yes. That last question sometimes surfaces a fixable obstacle nobody mentioned.

If the answer is a clear no, ask whether a trial is opening that you might be eligible for, and when.

Right to Try is a different pathway

You may hear about Right to Try, a federal law passed in 2018. It creates a route that bypasses the FDA and the IRB. It applies to drugs that have completed a Phase 1 trial and are still in development.

It sounds simpler, and in one respect it is. But it does not change the part that actually blocks most people: the company still has to agree, and no law compels them to. Removing the FDA from the process removes a

step that was rarely the obstacle.

Both pathways are worth knowing about. Ask your physician which is more practical in your situation.

Cost

Companies may charge for an investigational drug, but only for direct costs, and they need FDA authorization to do it. Many provide it free.

Insurance generally does not cover investigational drugs. It may cover the associated care — the visits, the scans, the monitoring. Ask both the company and your insurer specifically, and get the answer in writing.

Be cautious with anyone who charges a fee to secure access to a drug for you. Expanded access is a regulated pathway, not something that can be bought.

Your notes

Drug or trial identifier I am asking about

Physician who has agreed to request and supervise

Company contact and date of first request

What they said, and what needs to happen next

Reagan-Udall Foundation Expanded Access Navigator: navigator.reaganudall.org · FDA expanded access information: [fda.gov/expanded-access](https://www.fda.gov/expanded-access)

Wondrlink Foundation provides educational information only. We do not provide medical advice, diagnoses, or treatment recommendations, and nothing in this guide should be used in place of guidance from your own doctors. Decisions about your



care belong to you and your treating physicians. Wondrlink Foundation Inc. is a 501(c)(3) nonprofit organization, EIN 33-4493221. Questions? info@wondrlinkfoundation.org · More guides at wondrlinkfoundation.org/blog