



## **FIVE REASONS WE NEED RIGHT TO TRY**

### **1. Right To Try is needed because most terminal patients cannot participate in clinical trials.**

Fewer than three percent of all cancer patients can enroll in clinical trials, and for other diseases like ALS, it's even lower. And for rare diseases like deadly Duchenne muscular dystrophy, it's lower still. Patients who get into clinical trials must be just sick enough, but not too sick. Most people with a terminal illness are too sick to be selected.

For certain types of patients and diseases, the odds are even worse. In the last 20 years, the FDA approved only three new treatments for childhood cancers. But children under the age of 18 aren't eligible to apply for clinical trials that are designed for adult patients—even teens who are 16 and 17 can't enroll. So, for children with cancer, there are very few trials available. But there are promising treatments being safely used by adults in the U.S. and by children in other countries.

*We believe terminal patients who cannot qualify for a clinical trial should have the same access to promising treatments as the lucky few who get selected for clinical trials.*

### **2. Right To Try is needed because dying people don't have access to promising treatments once clinical trials are over, even if the drug was successful and will be approved.**

For the lucky few terminal patients who get selected for clinical trials, their luck runs out once the trials are over. Once the final phase of a trial is complete, doctors are often no longer allowed to give patients access to the drug. That means that patients who were helped by a promising therapy lose access to it while the FDA is making its final decision about approving the drug.

This was the scenario that happened to Dr. Ebrahim Delpassand and his patients in Texas. He was administering a final phase of a clinical trial for a drug to treat people with terminal pancreatic cancer. The drug has been approved in Europe for decades, but not yet in the U.S. The FDA told him he could no longer provide the treatment to treat his patients after the trial was complete, even though the only thing that was keeping them alive was the treatment he was providing. When Texas's Right To Try law went into effect, he began treating his patients again. He has treated nearly 80 patients, most of whom are still with us today because their state passed Right To Try.

*Right To Try ensures that patients will still have access to treatments that are helping them after clinical trials are over and while they are waiting on final approval from the FDA.*



### **3. Right To Try is needed because the FDA compassionate use process doesn't help enough people.**

The FDA has a program that allows terminal patients to apply for early access to a promising treatment. The application process is complicated, time-consuming, and expensive. Only about 1,200 people a year can make it through the application process. Dr. Razelle Kurzrock explained that when she ran clinical trials at MD Anderson Cancer Center, her department only tried to get compassionate use for about one patient a year. "The fact that we did maybe one a year in our department, which was the largest of its type, probably in the world, I think says it all," she said. "There're only two possibilities: that there was only one patient per year that needed compassionate use, and that's really laughable. Or that there were so many barriers that even at one of the best places in the world and one of the largest departments that did this as their day in and day out job, it was still very challenging."

In 2014, more than 12,000 people in France were using investigational treatments through that government's equivalent program. *If a country with one-fifth the population of the U.S. can help 900 percent more people, the U.S. agency's program clearly isn't working.*

In Australia, doctors are allowed to work directly with drug and device manufacturers to provide investigational treatments to terminal patients without the government's approval. They simply must report to the government at some point that the patient received the drug. No permission slip is required.

### **4. Right To Try is needed because it takes too long for promising treatments to be approved.**

On average, it takes more than ten years for drugs to make it through the clinical trial process and receive approval in the U.S. During trials, drugs must meet benchmarks that demonstrate they are safe and effective. There can be years of testing in between each of these benchmarks. During those years, when we know that promising treatments are safe and effective, millions of Americans facing life-threatening illnesses could be helped.

Some of the most promising drugs that are in the FDA approval pipeline today are already approved in other countries in Europe, Canada, and Japan. In fact, there are 22 pioneering breast cancer treatments in the FDA pipeline today, some of which are already approved and saving lives in Europe.

Even if a drug is approved in other countries for decades and we know it is safe and effective, Americans do not have widespread access to it until it is also approved by the FDA. Americans with the financial resources can travel to other countries for treatments that are approved overseas, but the vast majority of Americans can't afford to do that.

*Right To Try gives Americans who can't afford to travel overseas the ability to access promising treatments before it's too late.*



**5. Right To Try is needed because you shouldn't have to ask the federal government for permission to try to save your own life.**

There is no more fundamental freedom we have than the freedom to try to save our own life, and a dying person should not have to spend his or her time filling out forms and waiting on answers from the FDA or review boards that have never met the patient. If a patient is willing to try a promising treatment, even when he or she understands the risks, a doctor thinks the treatment may help the patient more than anything else on the market today, and a company agrees and is willing to provide the treatment, *the federal government should not get a veto stamp.*