

‘Right to Try’ laws spur debate over dying patients’ access to experimental drugs

By Brady Dennis and Ariana Eunjung Cha, Published: May 16

Colorado, Missouri and Louisiana are poised to become the first states in the nation to give terminally ill patients the right to try experimental drugs without the blessing of the Food and Drug Administration, setting the stage for what could be a lengthy battle over who should decide whether a drug is too risky to try.

Lawmakers in the three states have passed “Right to Try” laws with unanimous votes in recent weeks, after high-profile, social media campaigns in which families of dying patients have pushed for access to unapproved but potentially lifesaving drugs. Colorado’s governor is expected to sign that state’s law Saturday.

Proponents of the measures argue that patients desperate for treatments must navigate a lengthy, cumbersome process to get the FDA to approve early access to experimental drugs and to persuade companies to provide them. The Right to Try laws are intended to cut through some of that red tape by essentially cutting the federal government out of the picture.

“For people who are facing death and have one last hope, they should have a choice to try every possible drug,” said state Rep. Joann Ginal, a Democrat and co-sponsor of the bill in Colorado. Ginal introduced it in part because she witnessed how an experimental treatment helped her older brother, who has a rare blood cancer.

Opponents of the approach call it an ill-advised effort that circumvents federal law, undermines the drug development process and threatens to harm more people than it helps by providing access to medications that haven’t been proven safe and effective.

“The notion is based on the ‘Dallas Buyers Club’ — the idea that you have to get around the indifferent and cruel government to get access to drugs,” said Arthur Caplan, director of the bioethics division at New York University Langone Medical Center, referring to the Oscar-winning movie based on an AIDS patient who smuggled unapproved drugs into Texas during the 1980s.

The reality, Caplan said, is more complicated than singling out the FDA, which approves almost all the requests it receives for “compassionate use” exemptions. He noted that the new legislation does nothing to compel cooperation from drugmakers, who often are reluctant to hand out unapproved drugs, for reasons including high costs, lack of adequate supply and worries over liability.

Frank Burroughs, founder of the Virginia-based Abigail Alliance for Better Access to Experimental Drugs, which has long pushed the FDA to widen access, said people aren't after just any new medication that comes along.

“We’re talking about ‘promising’ drugs,” said Burroughs, whose group has helped the Goldwater Institute, a conservative advocacy organization, push for the state laws. “Patients are much smarter and savvy than they get credit for.”

Burroughs said the FDA simply hasn't moved quickly enough and that people who are out of options are willing to take on more risk than an ordinary person. “The risk-benefit is much different than someone who’s waiting for a new allergy medication or a new toe fungus cream,” he said.

The FDA on Friday declined to take a position on any of the state Right to Try bills. But in a statement, the agency said it is concerned about any efforts that might undermine the “congressionally-mandated authority and agency mission to protect the public from therapies that are not safe and effective.”

FDA regulations allow for access to investigational drugs outside of a clinical trial for patients who have serious or life-threatening illnesses and have no comparable alternatives. While these “compassionate use” exemptions can apply to individuals on a case-by-case basis, the FDA also can grant expanded access for larger groups of patients. However, the agency cannot force a company to provide a drug to patients.

The Right to Try bills aim to provide a streamlined alternative to the FDA process. Instead of having to fill out lengthy and complex paperwork, patients would only need to get an okay from a drug company and a simple prescription or “recommendation” from a doctor to access an unapproved treatment. The drugs involved also must have successfully completed an initial safety trial and moved to the next phase of development.

It’s unclear how many drugmakers might be willing to make use of the state laws at the risk of angering federal regulators. But at least one company plans to take advantage of the new legislation in Colorado.

Neuralstem, based in Germantown, Md., has begun looking for doctors in the state to use its treatment — which involves surgically transplanting neural stem cells in the spinal cord — for Lou Gehrig’s disease. The company’s chief executive, I. Richard Garr, said results of its first trial, involving 15 patients, were promising in slowing down the disease’s progression. “On average, these patients die within two to four years of diagnosis, so our hope is to make this available to everyone as quickly as possible,” Garr said.

Sascha Haverfield, vice president of scientific and regulatory affairs at the Pharmaceutical Research and Manufacturers of America, said companies take expanded use requests seriously and evaluate each case carefully.

Ultimately, Haverfield said it's incumbent on all stakeholders — drugmakers, doctors, the FDA and patients — to figure out the most efficient way to get drugs to those who most need them. But he said it's also important not to undermine the clinical trial process, which can lead to FDA approval. Granting unwarranted expanded access requests not only places “an individual's health ahead of the public's health,” he said, but it also could undermine the regulatory process and hinder a company's ability to make new drugs available to a broader patient population.

For Amy Auden, of Lone Tree, Colo., the decision to publicly push for the new law in her state was deeply personal. Her husband, Nick, died in November after a two-year battle with melanoma. For much of last year, the family tried unsuccessfully to persuade Bristol-Myers Squibb and Merck to give it access to a promising developmental drug for his cancer. *a company*

“Given that there was something on this earth to help Nick, we needed to do everything in our power to try to get it,” said Auden, now a widowed mother of three. “Of course, there was a chance Nick would not have been in the 52 percent of people who are responding to the drug; however, a 52 percent chance at life is better than a zero percent chance at life.”

With the new law, Auden said more families might at least have the hope that hers did not.

“Not a day goes by where it doesn't haunt me,” Auden said. “Those with serious illnesses should not have to fight the illness as well as fight for the right to gain access to lifesaving treatments.”

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