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'Right-to-try' laws: A patient's best last chance or false hope?

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(HealthDay)—The Trump administration may have failed in its initial effort to repeal the Affordable Care Act, but some activists hope White House support will prove valuable in changing another piece of federal health care policy.

Vice President Mike Pence campaigned on his support for a federal "Right-to-Try" law that would provide terminally ill patients access to experimental drugs, outside the standard clinical trial process.

Pence met with proponents of the proposal in February, and President Donald Trump has voiced his support for Right to Try.

Advocates say the law is needed because drug companies are reluctant to provide experimental medications to desperate patients, even in the 33 states that have passed their own Right-to-Try legislation.

"We have heard that pharmaceutical companies feel it is just too risky for them to use this path outside of the FDA's approval while they still have a trial ongoing," said Starlee Coleman. She is vice president for communications for the Goldwater Institute, a conservative public policy think tank based in Phoenix.

"This federal legislation would lift that cloud of uncertainty for drug companies, and they would know their [FDA-sanctioned clinical] trial would not be impacted if they chose to help someone through a state Right-to-Try program," she added.

The U.S. Food and Drug Administration (FDA) has a "compassionate use" program that allows access to experimental and unproven drugs, but Right-to-Try advocates argue that that procedure is too daunting and difficult for average patients or doctors to use.

"Imagine what it's like for someone in rural Oregon who's seeing a community oncologist who's never run a clinical trial, never had to contact anyone at the FDA, and doesn't know how this process works," Coleman said. "What do you think the chances are that person is going to get through the process?"

Critics of a federal Right-to-Try law argue that such a measure is unnecessary—the FDA has streamlined its compassionate use process and approves nearly every request it receives.

"The FDA publishes information on how many compassionate use requests it grants, and it's generally in the 99 percent ballpark," said Patricia Zettler, an associate professor with Georgia State University's Center for Law Health and Society and a former FDA lawyer. "For most patients who are requesting access, it should only take their physician 45 minutes to fill out the form. That is what FDA says."

Clinical trials versus compassionate use

But Coleman said that, even if every application receives approval, only about 1,200 patients make it through the FDA compassionate use process each year. And she said she doesn't believe it's because these drugs treat rare diseases involving few patients.

Instead, Right-to-Try advocates contend there's a little known process beneath the FDA's compassionate use program, where doctors who are in the know communicate with agency officials ahead of time to make sure an application will be approved before they file it on their patient's

behalf.

"Doctors who know how to use that system are getting a verbal OK from the FDA before they even submit an application," Coleman said.

Right-to-Try advocates also believe that drug companies are leery of participating in state Right-to-Try programs because they think doing so would slow down or halt the FDA approval process for new medications, particularly if adverse events occur in patients granted use under the program.

A federal Right-to-Try law would prevent the FDA from considering any data outside clinical trials when evaluating a new drug, those advocates say.

Zettler counters that statistics from the FDA compassionate use program show that such a provision isn't necessary. Bad drug outcomes uncovered under the program have resulted in holds for only 0.2 percent of drugs in the approval process, compared with 8 percent for bad outcomes that cropped up during regular clinical trials, she said.

"If there is useful information to be gained from expanded access, it seems a bit counterintuitive to say FDA, you can't use that information," Zettler said. "I think the public would want FDA to use all information available to it, giving the most comprehensive assessment of which drugs should be sold in the United States."

Coleman contends that it makes no sense to consider any data from a drug's use outside of an approved clinical trial.

"If the FDA has directed a company to design a trial to answer certain questions, and we know that clinical trial inclusion restrictions are very tight, then they don't want anybody in the trial who could skew the

results," Coleman said.

The impact on clinical trials a concern

Jack Hoadley is a research professor with Georgetown University's Health Policy Institute. Though not a supporter to Right-to-Try legislation, he agrees with Coleman on the need for rigid guidelines for clinical trials.

"Since the whole principle of clinical trials is that you're doing randomized studies, any patient that comes in under Right to Try is essentially guaranteeing they will get the treatment," Hoadley said. "By necessity, you would have to take them out of the trial results because there's no longer a random element."

Right to Try could undermine the clinical trial process in another way, Hoadley added. Many patients might decide not to bother with the process of inclusion in a clinical trial and simply demand access to an experimental drug through Right to Try.

"These drugs are still going through clinical trials. If there were a lot of people opting out and using Right to Try, you could actually create problems trying to get an adequate number of people in a [clinical] trial," Hoadley said.

"If this ends up making it harder for investigators to get the sample size they need, particularly for diseases that aren't common, that at the very least delays the [drug] company to be able to move forward for full FDA approval. And in the worst case it could drag the trials to a stop," Hoadley added.

There's one financial factor that might make this less likely to happen, Hoadley said.

In clinical trials, pharmaceutical companies provide the drugs at no cost. People using Right to Try would be liable for the cost of the medication, he said.

"You may be responsible for paying for the cost of a drug, unless you can convince a manufacturer to give it to you without cost," Hoadley said. "You don't want to see somebody in a situation where they get their hopes up and then realize it's going to cost $50,000 to receive this experimental drug."

Meanwhile, the debate over Right-to-Try laws shows no sign of slowing down.

On Tuesday, the American Society of Clinical Oncology—the nation's leading cancer doctors—issued a statement saying such laws "lack adequate patient protections and do not remove any of the major barriers patients face in accessing investigational drugs outside of clinical trials."

More information: For more about Right-to-Try initiatives, visit the Goldwater Institute. For a different perspective, read this NPR report.