Will 'Right To Try' Bill Actually Help Anyone?

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WASHINGTON -- If a new federal bill is made law, terminally ill patients anywhere in the country would be allowed to request access to experimental treatments that haven't yet received FDA approval -- and deal directly with the companies developing them.

"Patients with terminal diseases ought to have a right to access treatments that have demonstrated a level of safety and could potentially save their lives," said Sen. Ron Johnson (R-Wis.) in a press statement following the Senate's unanimous approval of his bill, "The Trickett Wendler Right To Try Act," a week ago.

The latest draft of Johnson's bill now heads to the House, where similar bills have already been filed.

Critics of the right-to-try movement say it's unsafe, exploitative, and a "smokescreen" for an anarchist agenda.

Others argue that right-to-try laws are redundant, since the FDA already has an "expanded access" pathway (sometimes called "compassionate use") that allows patients to receive investigational treatments.

The American Society of Clinical Oncology (ASCO) made this argument in stating its opposition to right-to-try legislation.

"ASCO supports access to investigational drugs outside of clinical trials, when adequate patient protections are in place," ASCO chief medical officer Richard Schilsky, MD, said in a statement in April. "We don't support right-to-try legislation, however, because these laws ignore key patient protections without actually improving patient access to investigational drugs outside of clinical trials."

Instead, ASCO backs the FDA's expanded-access program.

But right-to-try proponents view the new federal bill as a critical tool for seriously ill patients, allowing them to bypass the FDA's red tape and decide the course of their own care.

"If a patient truly is dying, and there truly is no other remedy, and they want to try for a 'Hail Mary,' and truly understand the risks and benefits, then it is hard not to offer them an opportunity -- assuming the integrity of clinical trials can be maintained. Because, if not, there can be risks to future patients," said Robert Field, JD, PhD, MPH, a professor of law and health policy at the Dornsife School of Public Health at Drexel University in Philadelphia.

The Senate Bill

As passed by the Senate, Johnson's bill requires that, to be eligible for early access, treatments must be under an active Investigational New Drug application and have completed a phase I trial. And it obliges drug companies to submit annual reports to the agency that include adverse events.

A physician must certify that patients have "exhausted approved treatment options and [are] unable to participate in a clinical trial involving the eligible investigational drug," and patients must have "written informed consent" to the referring physician. Also, current FDA regulations limiting what companies can do to promote or market investigational drugs would still apply.

But the bill also includes several protections for drugmakers who participate. They can't be sued except for "reckless or willful misconduct, gross negligence, or an intentional tort," and the FDA can't use clinical outcomes from use of investigational treatments in its regular review process, unless senior FDA staff make a documented determination that "use of such clinical outcome is critical" for judging the product's safety.

The bill does limit what patients can be charged for investigational drugs to companies' actual direct costs of providing them. (But enforcement could be difficult: companies are not required to inform regulators of what they provide to individual patients, nor the costs to patients.)

The legislation already appears to have support from the White House. Vice President Mike Pence, former governor of Indiana, signed his state's right-to-try bill in 2015.

In 2014, Colorado was the first state to pass a right-to-try law. But the movement began long before that amid the HIV epidemic of the 1980s, as portrayed in the film "Dallas Buyers Club."

In more recent years, the Goldwater Institute, a Phoenix-based libertarian think tank, has led the right-to-try movement. "There's no more fundamental freedom than the right to save your own life ... Right to Try will open new paths to treatments for many patients who are currently out of options," said Victor Riches, the institute's president and CEO, in a press statement.

If someone is desperate, "I don't think a person or agency has a right to tell that terminally ill person, 'I'm sorry I don't think I'm going to let you try this' ... It should be up to them," added Jeffrey Singer, MD, a senior fellow at the Cato Institute, another libertarian think tank.

Singer, a general surgeon in private practice in Phoenix, said passing a federal bill would prevent the FDA from simply overriding state laws.

Industry a No-Show

For the legislation to have any real effect, it will naturally require participation by industry. But that is far from assured. The bill doesn't oblige drug companies to make treatments available. And no pharmaceutical companies have come forward to embrace the bill or promise to make drugs available; some have opposed it.

"While well-intentioned, current 'Right-to-Try' legislation is not in the best interest of patients and is unlikely to help us bring forward innovative, safe, and effective medicines to all patients as quickly as possible," pharma giant Merck & Co. said in a statement issued earlier this year.

Richard Garr, formerly CEO of Neuralstem Inc., which is developing cell therapies and small-molecule drugs for a variety of neurological conditions including spinal cord injury and amyotrophic lateral sclerosis, testified in support of Johnson's bill at a September 2016 hearing.

But Neuralstem's current management disagrees. In a statement sent to *MedPage Today*, the company said, "We feel that providing access to our investigational therapies outside of our ongoing and critical clinical trials may delay or jeopardize the approval of therapies, by reducing the supply of study agents or adversely affecting the data collection process. By focusing on clinical development and seeking regulatory approval, it is our goal to offer our therapies to the largest number of patients as quickly as possible."

The Pharmaceutical Research Manufacturers Association (PhRMA) has issued a series of noncommittal statements about the legislation as it worked its way through the Senate, all of which stopped short of endorsing the bill.

"We appreciated the opportunity to work with Sen. Johnson on the bill and look forward to continuing to work with his office," wrote Andrew Powaleny, director of public affairs for PhRMA, in a recent email to *MedPage Today*. "The revised Right to Try legislation that passed the Senate includes important protections for patient safety and the clinical trial process."

Medical ethicist Arthur Caplan, PhD, of New York University, noted that early access to investigational drugs puts drug companies instead of the FDA into the role of gatekeepers, and negative publicity is among their major concerns.

If one patient has a serious adverse event following an experimental therapy, that could scare off investors, Caplan said.

"As long as you have private sector investment driving drug development, the priority is to get the drug approved and sold and not to start giving it away," he noted.

The record with state-level right-to-try laws also suggests lackluster interest from industry. "It's telling that although 37 states have adopted these laws, when asked to provide examples of success stories, one of the primary groups pushing for their adoption can only provide the testimonies of six patients who received access to experimental medicines through a single physician in a single state," Rachel Sachs, an assistant professor of law at Washington University in St. Louis, told *RAPS* recently.

"The net impact of state right-to-try laws has been absolutely nothing. I don't expect a federal right-to-try law will change that," said Caplan, dismissing Johnson's bill as "a feel-good" exercise.

Redundant, Fraught with Risk

"It's entirely [for] show ... This bill is not going to expedite, accelerate or ease the burden of a single patient getting access to experimental medicine," said Peter Pitts, president and co-founder of the Center for Medicine in the Public Interest, a nonprofit medical issues research group. Pitts is also a former FDA associate commissioner for external relations.

The FDA already approves 99% of the requests for expanded access that it receives, Pitts noted. In fact, in 2016 the agency introduced a streamlined pathway to further accelerate the process, and one report indicated that the agency's average response time is 4 days. An FDA official told *MedPage Today* that emergency requests are usually granted immediately.

"It's not as if the agency is turning people away," Pitts said.

Carolyn Engelhard, MPA, director of the Health Policy Program at the University of Virginia School of Medicine's Department of Public Health Sciences, in Charlottesville, Va., told *MedPage Today* that the FDA's expanded access program already offers the same options as right-to-try but with more "checks and balances."

Engelhard predicts that a federal right-to-try program won't produce a "groundswell of drugs" that couldn't have already been accessed through the expanded use program.

Critics also said the bill's stipulation that drugs must have completed phase I testing does not offer very much assurance of safety.

Most drugs entering phase II trials never make it to market, usually because they turn out to be ineffective or because of safety issues not spotted in phase I. "So it is possible that patients will be taking something of no help, or that creates new health problems," Field said.

Mat Staver, JD, founder and chairman of Liberty Counsel, a self-described "international litigation, education, and policy ministry," said he supported the right-to-try concept. But he stressed that whatever new path develops should be monitored from a cost and availability standpoint, and studied to determine whether people are being exploited and whether the pathway is effective.

Engelhard, meanwhile, called the entire effort a "political smokescreen" for anti-regulation ideologues hoping to get patients believing they can sidestep the FDA and go straight to drug companies for treatments.

"It sounds like it's pro-patient, but by removing the FDA it opens the door for greater risk for fraud and abuse," she said.