JOURNAL OF CLINICAL RESEARCH BEST PRACTICES
Vol. 14, No. 8, August 2018 "Happy Trials to You"

Right to Try: It's More Complicated Than You Think

By David Vulcano

A dying patient who desperately wants to try an experimental medication cares about speed, cost and convenience. Four years ago, there was only one option for accessing such drugs outside of clinical trials: the FDA's Expanded Access (a.k.a. "Compassionate Use") program. The good news about this program is that the FDA quickly approves almost all requests, and a couple of years ago, the FDA even streamlined the paperwork. The bad news is that there is no requirement that a pharmaceutical company has to provide the drug at a reasonable price or to provide it at all.

Over the past three years, 42 states have passed "Right to Try" laws to make experimental drugs more available to dying patients. These laws have been touted as eliminating "big government" regulations and leaving medical decisions to patients and their physicians. Although, according to the state laws, requests do not have to pass through the FDA's Expanded Access process, there is still no requirement that a pharmaceutical company has to provide the drug at a reasonable price or provide it at all. Even if a manufacturer is willing to provide it, the existence of a state law in seeming contradiction to a federal law is part of the discussion as to why a manufacturer would still require an FDA Expanded Access IND and not just ship under a state Right to Try law.[1]

As a result, there is no verifiable evidence that any state Right to Try law has ever helped a single patient.

On May 30, 2018, after two years of congressional debate, President Trump signed the federal Right to Try law (officially, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, named after four patients).

The good news is that patients now have two options under federal law: the FDA's Expanded Access program or a new process under the federal Right to Try law.

The bad news is that the Right to Try process, depending on the circumstances and the state the patient is located in, is arguably slower and costlier than the Expanded Access process it is intended to circumvent…and there is still no requirement that a pharmaceutical company has to provide the drug at a reasonable price or provide it all.

Even the federal law does not help patients like Bob Bardone. After Missouri became one of the first states to pass a Right to Try law, Mr. Bardone moved from Florida to Missouri to obtain an investigational treatment for ALS. The Missouri state representative who authored the bill had said in a press release, "the Right to Try Act is about guaranteeing the rights of those who are most in need… [It] gives terminally ill patients a chance to continue their fight when they are left with no other options." Nevertheless, after Mr. Bardone and his family moved to Missouri, he still only had the right to *ask* and was not actually given the right to *try*, as the manufacturer still did not provide the drug.

With the new federal law, however, patients, physicians and manufacturers now have two theoretical options for access outside of clinical trials. They must weigh the pros and cons of the choices and, in this case, do so at arguably the most desperate time of one's life. Of course, a patient's choice is moot if the manufacturer is unable or unwilling to provide the product under one or both of the options.

There have been no demonstrated improvements in speed or cost with the Right to Try option, only *more complexity* than Expanded Access in the states with Right to Try laws and

less availability than Expanded Access in some of those states. This is because, in addition to meeting the criteria in the federal Right to Try law, lawyers are reminding us that patients must *also* meet the additional requirements of their state's Right to Try law.

Patients, physicians, care providers, healthcare providers, and manufacturers must now decide which process best fits the needs of a specific patient based on any applicable state laws (see Table 1 below). Even this decision is more complicated than it sounds, since the laws of more than one state might be relevant. Here are some examples:

- A manufacturer might favor Expanded Access over Right to Try in Texas, since Texas forbids the manufacturer from charging patients for the drug. That same manufacturer might want to be paid up front in South Dakota, Ohio, Nebraska or West Virginia, since if the terminally ill patient dies while on the drug, his or her estate is not liable for any associated debts.
- A patient who might want (or want their family member) to sue the manufacturer (even for negligence) will definitely favor Expanded Access to the broad immunity the federal Right to Try law offers manufacturers. However, this may be moot in Tennessee and Oregon, since part of their Right to Try consent form requirements include a liability waiver to essentially not sue.
- A patient might prefer Expanded Access to Right to Try in Colorado, Oklahoma, West Virginia, or Connecticut, since, under their Right to Try laws, their health insurance can deny coverage of claims for non-pre-existing conditions for up to six months after they stop taking the product under Right to Try.
- A patient might find it easier to use Expanded Access in the nine of 41 states whose Right to Try law requires two physicians to certify the illness (which adds to the cost and time), especially in the states that have additive criteria, such as that the second physician cannot be a primary care physician or have certain board certifications.
- Hospitalized patients in seven of 41 states will be unable to get investigational drugs under Right to Try, since their state laws exclude hospitalized patients with a terminal illness from access under Right to Try. Hospitalized patients would, however, have the potential option to discharge themselves against medical advice to obtain the product. This scenario is of particular interest for patients in Oklahoma, West Virginia, and Connecticut, since patients can lose their insurance coverage for up to six months in these states if they obtain a drug under Right to Try.
- Oregon's and Washington's Right to Try laws apply only to residents of those states. State law requires the physician to verify state residency. However, if you are a resident under the age of 18 in those states, you are ineligible for Right to Try under their state law. You would have to either obtain Expanded Access in your home state or go to a state that allows out-of-state minors. For example, they could go to the nearby states of Idaho or California, unless a guarantee of hospice care is important or unless the drug they wanted contained a Schedule 1 Controlled Substance, since California is one of six states that disallow those drugs from Right to Try. In addition, California would, among other things, require IRB oversight similar to that of Expanded Access, as well as a second physician to diagnose the illness and confirm the minor (or authorized representative) is able to provide consent.

- Patients who value hospice care would favor Expanded Access in the 20 states whose Right to Try laws allow for the withdrawal of hospice services while on a Right to Try product. Six of those states also include the ability to deny or withdraw in-home care.

Gone are the days when we had a single process for pre-approval access outside clinical trials. Also gone are the days of a single source of guidance on even the federal aspects of the new law pertaining to investigational drugs. In fact, when FDA Commissioner Scott Gottlieb indicated that the FDA was beginning work on the regulations and guidance to support the new law, Senator Ron Johnson, the author of the Senate Right To Try bill, sent Commissioner Gottlieb a letter essentially demanding that the FDA halt its efforts, stating, "This law intends to diminish the FDA's power over people's lives, not increase it."

Subsequently, on June 28, 2018, in an open forum at a Drug Information Association meeting, when a high-ranking official of the FDA was asked how manufacturers should respond to Right to Try requests, the answer was, essentially, that this pathway to access statutorily excluded the FDA, and that manufacturers would have to figure it out for themselves.

It is too soon to tell how pharmaceutical companies will respond to the federal Right to Try law, although one company has found a corollary to the aphorism, "no good deed goes unpunished": "no good response goes unpunished." The CEO of Brainstorm Cell Therapeutics told Bloomberg that the new law would allow it to create a "semi-commercial" business making its unapproved drug available to dying patients under Right to Try. He later mentioned that comparable therapies cost $300,000.[2] He was later criticized for excluding poor individuals from accessing their drug. Days later, the company announced that was discarding its plans to provide the drug under Right to Try altogether (even to those who can afford it), except to one patient, Matthew Bellina, one of the individuals for whom the federal Right to Try law is named. The CEO said he would pay for that drug personally. Mr. Bellina appears to live in Pennsylvania, where the state Right to Try law says his consent form will have to be signed by a witness and he would not be able to get the drug if he is hospitalized.

Conclusion

State and federal lawmakers have created a complex system to give dying patients the right to try experimental drugs, at least in theory. They have left it to the lawyers and judges to sort out the conflicting laws and address unanticipated consequences. In the meantime, patients, physicians, care providers, healthcare providers, and manufacturers will have to make the best of a very confusing situation.

References

1. "The Problem with State 'Right to Try' Legislation," David Vulcano and Norman M. Goldfarb, Journal of Clinical Research Best Practices, April 2015, http://firstclinical.com/journal/2015/1504_Right_to_Try.pdf
2. "The 'Right to Try' Could Cost Dying Patients a Fortune," Michelle Cortez, Bloomberg,6/20/18, https://www.bloomberg.com/news/articles/2018-06-20/the-price-to-try-a-drug-could-be-300-000-for-dying-patient

Table 1. State Right to Try Laws

State	Law Citation
Alabama	Ala. Code. § 22-1-5D.

Alaska	HB43 (recently passed, not yet published)
Arizona	Ariz. Rev. Stat. § 36-11.1.
Arkansas	Ark. Code § 20-2-15.
California	Cal. Health & Safety Code. § 111548.
Colorado	Colo. Rev. Stat. § 25-45-101.
Connecticut	Conn. Gen. Stat. § 20-00-14q.
Florida	Fla. Stat. § 499.0295 (2018).
Georgia	Ga. Code Ann. § 31-52-01.
Idaho	Idaho Code Ann. § 39-94-01.
Illinois	410 Ill. Comp. Stat. § 649/1-90.
Indiana	Ind. Code §16-42-26 (2017).
Iowa	Iowa Code § 144E.1-9 (2018).
Kentucky	Ky. Rev. Stat. Ann. § 217.5401-5408.
Louisiana	La. Stat. Ann. § 40:1169.1-4.
Maine	Me. Stat. tit. 22 § 602-A.
Maryland	Md. Code, Health Gen. Law § 21-2B.
Michigan	Mich. Comp. Laws § 333.26451-26457.
Minnesota	Minn. Stat. §151.375 (2017).
Mississippi	Miss. Code Ann. § 41-131-01.
Missouri	Mo. Rev. Stat. §191.480 (2017).
Montana	Mont. Code Ann. § 50-12-101 (2017).
Nebraska	Neb. Rev. Stat. § 71-9601.
Nevada	Nev. Rev. Stat. § 454.690; § 630.3735.
New Hampshire	N.H. Rev. Stat. Ann. § 126-Z: 1-5 (2016).
North Carolina	N.C. Gen. Stat. § 90-325.1-7 (2015).
North Dakota	N.D. Cent. Code § 23-48-01.
Ohio	Ohio Rev. Code § 4729.89; § 4731.97; § 4745.04.
Oklahoma	Okla. Stat. tit. 63, § 3091.1-6.
Oregon	Or. Rev. Stat. § 127.990 (2017).
Pennsylvania	35. Pa. Cons. Stat. § 10232.1-7. (West 2018).
South Carolina	S.C. Code Ann. § 44-137.
South Dakota	S.D. Codified Laws § 34-51.
Tennessee	Tenn. Code Ann. § 63-6-301.
Texas	Tex. Health & Safety Code. § 6.489.
Utah	Utah Code § 58 85-101.
Virginia	Va. Code Ann. § 54.1-3442.1.
Washington	Wash. Rev. Code § 69 77-010.
West Virginia	W. Va. Code § 16-51-1.

Wisconsin	Wisc. Stat. § 450.137.
Wyoming	Wyo. Stat. Ann. § 35-7-1801.

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