

A Closer Look At New Federal 'Right To Try' Law

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(June 1, 2018, 12:17 PM EDT)

The U.S. House of Representatives on May 22 passed the Senate's 2017 version of national "right to try" legislation, which was signed by the president on May 30. This law creates a federal framework for patients to access investigational new drug products outside of clinical trials and outside of the U.S. Food and Drug Administration's already existing expanded access program. The law comes in the wake of a majority of states passing their own right-to-try laws, creating the potential for a conflict between state laws, the FDA's expanded access regulations and federal statutes.

As background, treatment use of investigational drugs is currently subject to multi-tier regulation. The FDA's expanded access program provides a process for FDA approval of such drug uses while state right-to-try laws do not require federal permission. As of May 30, there is also a federal right-to-try law intended to allow patients to access investigational drugs outside of clinical trials and FDA processes. The intersection of these regulatory requirements and laws can be complex. Below we summarize the laws governing the treatment use of investigational drugs and provide areas that clinical trial and medical stakeholders should consider.

FDA's Expanded Access Program

The FDA's expanded access program is outlined in the Federal Food, Drug, and Cosmetic Act, or FDCA, and the FDA's implementing regulations.[1] Through this program, clinical trial sponsors and health care providers, or HCPs, can provide access to investigational drugs for serious diseases or conditions upon FDA approval, provided that certain criteria — which vary depending on the size of the expanded access population — are met, and provided that sponsors and investigators comply with FDA regulatory requirements. With FDA authorization, sponsors may also charge patients for certain costs associated with expanded access investigational drugs. However, sponsors must provide the FDA with supporting cost calculation documentation.[2]

Based upon numbers published by the FDA,[3] the vast majority of requests for expanded access use of investigational drugs are approved by the agency. In fiscal year 2017, the FDA



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received 1,862 requests for expanded access use of biopharmaceutical products and approved 1,831 of these. As reported by the Government Accountability Office,[4] FDA estimates that its efforts have simplified the expanded access process for individual patients, such that on average, the FDA application takes 45 minutes to complete. Per the GAO, the FDA typically responds to emergency investigational new drug, or IND, requests in less than one day (aiming to respond within hours) and all other requests within 30 days. The GAO reported, however, that some stakeholders found the FDA's expanded access forms and website difficult to understand. Further, the GAO noted that there was a lack of clarity regarding how the agency uses adverse event data from expanded access use when reviewing marketing applications, which might discourage sponsor participation.

State Legislative Efforts

Despite the expanded access pathway, many states have enacted right-to-try legislation in recent years. Currently, a majority of states have passed such laws. While there are individual state differences, the basic crux of these laws are that, for patients with terminal diseases who have considered other treatment options and who are unable to participate in a clinical trial, they allow access to investigational drugs without FDA approval, upon recommendation of a physician and agreement of the drug manufacturer.[5] As a general matter, the investigational product must have completed at least a phase one study, the patient must provide informed consent, and there must be some documentation from the physician that the patient meets the state law requirements. Moreover, these laws neither require manufacturers to provide access to the investigational product nor obligate insurers to pay for the product. Under state laws, manufacturers may be able to charge eligible patients for certain costs associated with the investigational product without FDA approval and without the provision of cost documentation to the FDA. If access is provided in accordance with the state law, the treating physician, manufacturer and others in the supply chain are provided with certain legal immunities in the event of patient injury.

While the basic themes in the individual state right-to-try laws are the same, with many versions of the same legislation, there are a number of jurisdictional variations, such as variations on threshold issues, including which patients qualify. These state laws also have been passed against the backdrop of the federal regulation around the use of investigational products, presenting the possibility for federal preemption.

Federal Legislation

The U.S. House of Representatives on May 22 passed S. 204,[6] titled the Trickett Wendler, Frank Mongiello, Jordan McLinn and Matthew Bellina Right to Try Act of 2017, or federal Right to Try Act. This version of the bill, which was previously passed by the Senate in August 2017, was signed into law by the president on May 30. Like the state laws, this federal law aims to permit some patients to access and use certain investigational drugs before they are approved as safe and effective by the FDA, without enrolling in clinical trials and without FDA expanded access approval. Key provisions of this legislation include the following:

- For patients to access investigational drugs under the federal Right to Try Act, they must meet certain prerequisites. They must be diagnosed with a life-threatening disease or condition, have exhausted all approved treatment options, must be unable to participate in a clinical trial involving the investigational drug, and must provide informed consent.

- The particular investigational drug must also meet certain prerequisites to be eligible for use under this law. A phase one clinical trial must be complete; the drug must not be approved for any use by the FDA; either an FDA marketing application must be submitted, or the drug must be under investigation in a clinical trial that is intended to form the primary basis of an efficacy claim and subject to an active IND application; and an active drug development or production program must be ongoing and not discontinued or placed on clinical hold.
- Eligible investigational drugs that are provided to eligible patients are exempt from requirements for FDA approval, INDs and most of the corresponding IND regulations, certain labeling requirements including adequate directions for use, and the FDA's regulations on informed consent and institutional review board, or IRB, approval.
- While not explicitly stated, there is a presumption that patients can be charged for certain costs associated with investigational drugs. These costs, however, are limited to direct costs of making the investigational drug available, as further outlined in the FDA's regulations.
- Manufacturers or sponsors of the investigational drug must provide the FDA with an annual summary of use of the drug under the statute, which must include the number of doses supplied, the number of patients treated, the use for which the product was made available, and any known serious adverse events.
- The FDA is barred from using clinical outcomes associated with use of an investigational drug pursuant to the federal Right to Try Act to delay or adversely impact the review or approval of such product unless the clinical outcome is critical to determining the product's safety or the sponsor requests use of the outcome.
- The law also purports to provide sponsors and manufacturers of investigational products that are supplied pursuant to the law, as well as prescribers, dispensers and other entities, protection from certain liabilities for alleged acts or omissions with respect to the investigational drug and from decisions not to provide access to an investigational drug. This protection, however, is not absolute, as the statute places certain limits on the liability protections.

According to sense of the Senate, the law does not establish a new entitlement, right or mandate, or otherwise modify any existing entitlement.

Key Takeaways

The federal Right to Try Act will present new opportunities and challenges to the pharmaceutical industry and patient communities. The statute, however, leaves a number of unanswered questions that will need to be resolved, such as:

- How does the statute impact the current patchwork of state laws, which differ from each other and the federal legislation in important ways? The law does not include any express preemption language. Thus, state law preemption will likely be determined on a case-by-case basis.
- How does the statute impact current FDA expanded access regulations, and especially individual patient expanded access? If FDA approval is not required to provide patients with access to investigational drugs, patients and caregivers may prefer to avail themselves of the federal Right to Try Act rather than pursue FDA approval for expanded access use.

- Will companies be required to maintain publicly available policies for the use of investigational drugs under the federal Right to Try Act in the same way that they are currently required to maintain policies for expanded access?[7]
- What constitutes informed consent? While patient informed consent is required under the statute, the statute also exempts right- to-try use of an investigational drug from the FDA's informed consent regulations. Accordingly, the scope of the required informed consent is unclear.
- When is a clinical outcome critical to determining the safety of an investigational drug, such that the FDA may use the clinical outcome in a marketing approval decision?
- What is the extent of the legal protections provided to sponsors, manufacturers, prescribers, dispensers and other health care entities? While the statute provides some protection, it does not provide protection from all potential actions.
- What documentation must be maintained under the federal Right to Try Act, and are there any limitations in the ways regulators may use this documentation? For instance, if patients are charged for investigational drugs provided under the federal Right to Try Act, does documentation need to be maintained evidencing that patients were only charged for the direct costs of the investigational drug, and can this documentation be used by the government, following receipt of FDA marketing approval, for pricing and reimbursement decisions?

While we will wait to see the impact of this law, there are actions that clinical trial sponsors and health care entities, or HCEs, can take. Specifically, sponsors should consider the following action items:

- Sponsors should develop a clear internal policy on their approach to requests for access. This policy should include whether and how access will be provided, and how requests for access will be handled (e.g., who is authorized to handle such requests, how requests should be routed and what responses may be provided). These policies and procedures should account for the geographic location of the requestor, sponsor, patient and manufacturer.
- To the extent access may be provided, sponsors should have a procedure for how access decisions will be made and what information is required. The applicable information may include information on the patient, patient's condition and patient's treatment. Patient-specific information, though, may implicate health privacy requirements. The applicable documentation may include evidence of informed consent and IRB approval (even if IRB approval is not required under law), and agreements between the patient, HCP/HCE and sponsor.
- All persons and entities involved in clinical trial and development programs should be trained on these policies, procedures and requirements.
- Sponsors should also ensure that there is a clear understanding of differences between federal law, the FDA's expanded access regulations and the individual state requirements concerning treatment use of investigational products. States in which clinical trial sites are located may be likely targets for access requests from patients.

HCEs should also consider taking certain steps with regard to the treatment use of investigational products:

- Like sponsors, HCEs should develop clear internal policies and procedures concerning communications about, requests for, approval of and the use of investigational products for treatment purposes. HCEs should also ensure that all applicable persons within the organization are adequately trained on these policies and procedures.
- For those HCEs that have facilities in multiple states, there should be a robust organizational understanding of the laws that apply to the individual facilities. For uniformity, HCEs may want to consider applying the more stringent law. HCEs should also remain cognizant of FDA and federal requirements.
- HCEs should also keep geographical considerations in mind. For instance, treatment decisions may depend on where the facility is located, the patient's home state, where the sponsor is located, and from where the product is shipped.
- To the extent that HCEs will provide treatment use of investigational products, HCEs should also carefully review their internal informed consent forms, or ICFs, and requirements concerning IRB approval. An ICF different from that used in a clinical trial may be appropriate, but should clearly state the risks involved in the use of the investigational product, that the product is investigational, and that the product has not been approved by the FDA.
- Moreover, even if IRB approval is not required under state or federal law, HCEs may want to consider IRB review of the treatment use of the investigational product and any ICF. An internal requirement for IRB review may stretch IRB resources, and thus may impact HCE budgets.

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[1] FFDCA § 561; 21 CFR Part 312, Subpart I.

[2] 21 CFR § 312.8.

[3] www.fda.gov/newsevents/publichealthfocus/expandedaccesscompassionateuse/ucm443572.htm#Expanded_Access

[4] www.gao.gov/assets/690/685729.pdf

[5] See, e.g., Right to Try Act, PL 347, No. 33, Gen. Assemb. (Pa. 2017).

[6] www.congress.gov/115/bills/s204/BILLS-115s204enr.pdf

[7] FFDCA § 561A.