

The Right-to-Try Pathway

On May 30, President Trump signed a federal right-to-try law known as the “Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017 S.204.” The new law amends the Food, Drug, and Cosmetics Act to create a second pathway for certain patients to access investigational drugs outside a clinical trial. This new pathway will co-exist with FDA’s existing expanded access program.

While it is too soon to know exactly how this new access route will work, a few things are clear:

- The new law applies only to investigational drugs. It does not apply to medical devices.
- It applies to individual or single patient treatment uses. It does **not** apply to intermediate-size patient populations or widespread treatment access. These types of expanded access continue to follow FDA requirements under FDA’s expanded access program.
- It applies to investigational drugs that:
 - Have completed a Phase 1 clinical trial (defined under [21 CFR 312.21](#)).
 - Have not been approved or licensed by FDA.
 - Are being actively studied in a clinical trial or are included in an application for approval has been filed with FDA.
- It applies only to certain types of patients:
 1. Patients who have been diagnosed with a life-threatening disease or condition. Life-threatening is defined in FDA regulation ([21 CFR 312.81](#)) as:
 - Diseases or conditions where the likelihood of death is high unless the course of the disease is interrupted; and
 - Diseases or conditions with potentially fatal outcomes, where the end point of clinical trial analysis is survival.

2. Patients who have exhausted approved treatment options and are unable to participate in a clinical trial involving an eligible investigational drug (defined above).

In addition:

- A physician who is in good standing with the physician's licensing organization or board and will not be compensated directly by the manufacturer must certify that the two conditions are met.
- Patients or a legally authorized representative of the patient must provide to the physician written informed consent regarding the eligible investigational drug.
- The new law does not require a sponsor/manufacturer, prescriber, dispenser, or other individual entity to provide access to an eligible investigational drug. Sponsors retain their right to deny requests.
- The new law exempts physicians or manufacturers who provide eligible investigational drugs to eligible patients from seeking FDA approval and receiving an IND, and from the requirement for institutional review board (IRB) review and approval.
- The new law exempts a sponsor or manufacturer, prescriber, dispenser, or other individual entity from liability, unless the relevant conduct constitutes reckless or willful misconduct, gross negligence, or an intentional tort under any applicable state law.
- FDA may not use the clinical outcomes associated with these treatment uses to delay or adversely affect the review or approval of the investigational drug unless:
 - FDA determines that the use of the clinical outcome is critical to determining the safety of the investigational drug or the sponsor requests use of the clinical outcome.
- The drug manufacturer or sponsor must submit to FDA an annual summary of any use of an investigational drug under this new right-to-try law.

It is unknown at this point exactly how this new law will be implemented, whether a revision to the code of federal regulations will be required so that FDA may implement the new law, and whether this federal law will preempt state right-to-try laws.

Drug manufacturers and institutions with whom physicians are affiliated can decide to continue to provide expanded access under the existing FDA program. Nothing in the new law prohibits an entity from filing an IND for a single patient expanded access treatment use, or from seeking IRB approval to ensure that a patient's interests are appropriately respected and protected, regardless of whether access is provided under an IND.

The complete text of Senate Bill 204 can be found [here](#).