

Overview of 2018 Wendler et al Right to Try Legislation

This law created a new pathway for people diagnosed with life threatening diseases such as ALS, to gain access to treatments that are in late stage clinical trials, but not yet approved by the Food & Drug Administration (FDA).

The law removes liability on the drug company for any harm done if they choose to provide the treatment and reduces the burden on the FDA to approve each individual request for access. Generally speaking, under this new law, a company and a patient can enter into an individual and private agreement regarding access to a drug still under investigation.

This is an additional pathway for access, not a replacement of expanded access: thus, companies may decide to provide their investigational product via Right to Try and/or via traditional Expanded Access Programs (i.e. Single Patient Compassionate Use IND) or not at all.

Eligible patients living in all 50 states are now offered the same opportunities to access eligible investigational drugs under federal Right to Try legislation. In brief, where the state Right to Try laws conflict with the federal Right to Try law, the federal law trumps. However, it remains to be seen whether provisions of state laws that were not mentioned in the federal law will remain in effect and/or be enforced.

Next Steps?

Now that the President has signed it into law, the next step is for the FDA to implement Right to Try legislation, possibly through the development of systems, tools etc. Many statements have come out from members of Congress, the Secretary of Health and Human Services (HHS), and the FDA, that signal that an implementation plan will be completed within weeks of the President signing the bill into law.

What does Right to Try legislation do?

The law provides certain patients who are not able to participate in late stage clinical trials of experimental treatments which they think may help them combat their disease, with a new way to approach the drug company and ask for access outside of the trial.

This law is similar in many ways to an existing expanded access pathway at the FDA called "single patient compassionate use IND." With that pathway, access to an investigational drug had to be agreed upon, and approved by four parties: the patient, the physician, the drug company and the FDA.

The difference here is that the FDA no longer plays a role in the approval process. Rather, the FDA is directed under the law to monitor access, and determine, when directed by the Secretary of the Health and Human Services, if there are safety issues arising from the investigational drugs that should be considered when (if) the company decides to apply to the

FDA for marketing authorization (aka, approval for sale and use). Under the new law, the patient, the company and the physician still must agree upon, and approve access to the investigational drug.

If access to the treatment is granted, patients may be charged a fee by the company for access to that treatment. It is currently under debate whether that fee is limited (as it is under current Expanded Access Programs) to the cost of the treatment or if it can include a “profit margin” for the company.

What does Right to Try legislation not do?

The law does not require companies to offer their potential treatments outside of trials, no matter how badly patients want them to. It’s still 100% up the company whether they want to provide access.

The law does not require a company be equitable with access or have the same arrangements for access with each patient they provide access to.

This new law does not require that a company have a separate Right to Try policy publicly stated on their website. This means that there is no way to know each company’s policy regarding RTT unless that company proactively chooses to make that policy known.

Who can take advantage of Right to Try legislation?

Anyone who is certified by a physician to 1) have given informed consent, 2) have been diagnosed as terminally ill due to a condition or disease, such as ALS, 3) have exhausted all existing approved treatments, and to 4) be unable to participate in a clinical trial on the potential treatment.

“Exhausted approved treatment options” likely refers to riluzole and edaravone. This likely does not mean that a patient must be on them - or off of them - to be certified as eligible by a physician. Rather, it most likely means that the physician is certifying that the patient has considered and/or tried everything that is currently approved by the FDA to treat their disease.

“Unable to participate in a clinical trial on the potential treatment” is one of the aspects of the law that need to be “translated” into rules by the FDA. For example: If someone is clinically eligible for the trial but unable to travel to the trial site, does that mean the person is ineligible for Right to Try? Or does it simple mean that a person must not meet the literal enrollment criteria? Some of this is likely going to be left up to the certifying physician to interpret as they consider each individual patient’s overall case.

What drugs are eligible under Right to Try legislation?

Under this law, a drug must meet all of the following prerequisites to be eligible for access:

1. not yet approved by the FDA and
2. been the subject of a completed phase 1 clinical trial and
3. be the subject of an active IND before the FDA or in ongoing trial to determine efficacy and
4. not be the current subject of a clinical trial hold and

5. continue to be in active development or production by the company.

In ALS trials right now, there are *at least* two compounds that would very likely meet these prerequisites at this time: 1) [NurOwn](#) is a stem cell approach, the aim of which is to provide neurotrophic support and combat inflammation occurring within the CNS; 2) [levosimendan \(ODM-109\)](#) is a small molecule that sensitizes muscles to calcium, to enable longer term contractibility even as motor axons die back and away from the muscle.

Both of these compounds passed the phase 1 clinical trial stage and are currently in a trial with the primary basis of making a claim for efficacy. However, it is not known how either company will react to requests from patients for access under the RTT law. Brainstorm Cell Therapeutics (NurOwn) held a public discussion on the topic, gaining feedback from its shareholders and patients alike, a recording of which is available online for a limited time [here](#).

How will ALS TDI provide information about drugs available under Right to Try legislation?

Once ALS TDI is able to verify a company's RTT policy for a given drug that meets the eligibility criteria for RTT, we will add that information to our clinical trial listing online [here](#).

ALS TDI is educating people with ALS about the law, including talking about it in a number of webinars e.g. upcoming webinar on June 13th (register [here](#).)

ALS TDI would like to remind people that Right to Try does not replace other Expanded Access Programs such as Single Patient Compassionate Use programs at the FDA. Companies may choose to offer access to experimental medications under those programs instead of Right to Try. For more information on EAPs visit the websites of [FDA](#) here and the [Regan-Udall Foundation](#).

What about health insurance?

Patients exploring experimental treatments in an effort to combat their disease are unlikely to be eligible to access hospice coverage from their health insurer at the same time. It is unclear what, if any other limitations to health insurance coverage may exist when a person accesses a treatment under the Right to Try law.

Patients should check with their health insurance provider to learn about their individual plan's coverage relative to Right to Try.

Helpful Links

- [Wendler Right to Try Act of 2018](#)
- [ALS TDI Statement following passage of RTT law](#)

Disclaimer: The experimental products named in this fact sheet are not FDA approved. By naming them here, ALS TDI does not endorse their use as a treatment for ALS. This fact sheet is intended for educational purposes only and should not be used by anyone as a legal interpretation of a law, or for giving medical advice. ALS TDI advises anyone seeking access to medications under RTT to consult their physician, attorney and health insurance provider for their respective interpretations of the law.