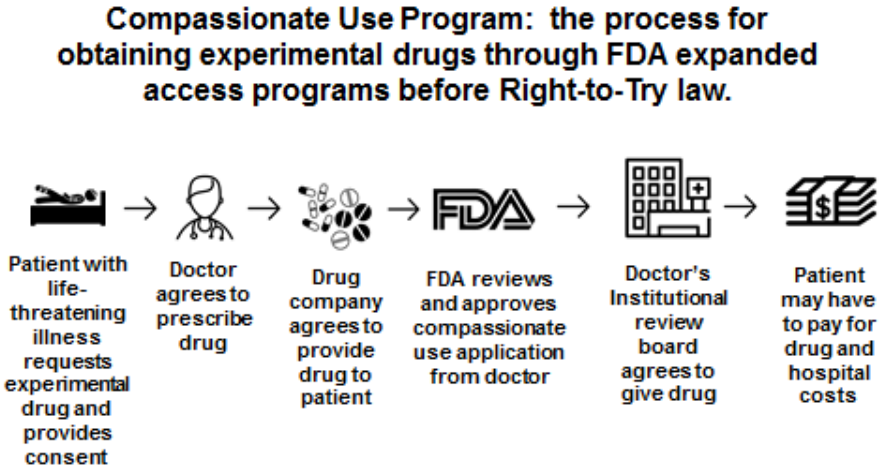
Right-to-Try: the Pros, Cons, and Unknowns

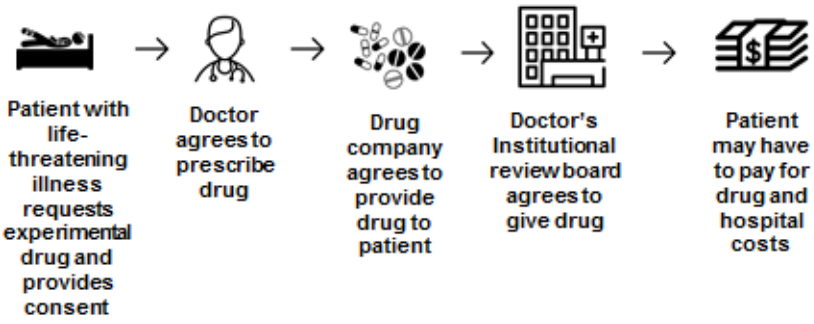
By Christina Fournier, MD

The federal “Right- to-Try” bill was signed into law on May 30, 2018. This legislation allows patients with a terminal illness, such as ALS, to seek out drug treatments that have successfully completed Phase 1 clinical trial safety testing, but are not yet FDA-approved, and therefore the effectiveness is unknown. Prior to passage of this legislation, unapproved drugs have only been available to terminally ill patients with FDA approval through the compassionate use program, detailed below.



From 2005-2014, 10,939 requests for expanded access of investigational drugs were submitted to the FDA, and 99% of these requests were granted. It is unknown how many patients sought investigational drugs during that time period but did not have applications filed due to lack of permission from the physician or drug company. Under the new Right-to-Try legislation, the step of seeking FDA approval is removed from the process, but the other steps remain the same, as shown below.

Right-to-Try: The Process for a Patient to Obtain Experimental Drugs Under New Federal Right-To-Try Law



Under new Right-to-Try laws, drug companies must still notify the FDA when a patient is given non-approved medications, and they must also report any adverse events that occur in Right-to-Try patients. Patients must still find a physician that agrees to prescribe and manage the drug and must still get permission from the drug company to provide the therapy under these new laws.

Advocates for Right-to-Try are hopeful that by removing the need for FDA permission, the sickest patients will have faster access to experimental therapies. Supporters of the legislation note that terminal patients often do not qualify for clinical trials and will not survive long enough for new drug treatments to come to market. Right-to-Try proponents believe that although there are risks associated with unproven treatments, a patient who has exhausted standard treatment options should have the right to decide to whether or not they seek out alternatives.

Critics of Right-to-Try are concerned that without sufficient FDA oversight, patients could be harmed by drugs where the patients and prescribers are unaware of drug risks. Right-to-Try opponents note that without FDA oversight, the most vulnerable patients may be taken advantage of by unethical providers seeking profit. Opponents also note that Right-to-Try could be giving false hope to patients by giving access to drugs that may not help and could be harmful.

It is unknown how much the new Right-to-Try laws will actually expand access to non-approved therapies. Experts on both sides of the issue note that the Right-to-Try law in its current form does not actually give patients the right to receive experimental therapies, only the right to ask for them, and the impact of this law may be modest. Pharmaceutical companies are expected in many cases to be reluctant to provide non-approved drugs to patients outside of clinical trial process as complications in these sicker patients could hinder the FDA-approval of their drug and pose an unnecessary liability for the company. For patients with ALS, the impact of the new law remains uncertain.