Frequently Asked Questions

What is The ALS Association’s position on Right to Try?
The ALS Association strongly supports initiatives to enable people with ALS to access promising treatments as soon as possible, including prior to FDA approval. We are working with all stakeholders to identify the most effective ways to ensure access to experimental treatments and to truly make a difference on this issue, which is so important to people with ALS. Right to Try law is an example of one approach to address the issue. The Right to Try movement has helped propel preapproval access into the national conversation. The ALS Association believes Right to Try law still needs to break down all the very real barriers that are preventing more people from accessing potential treatments. Therefore, we are aggressively working with key stakeholders, including people with ALS, other national patient organizations, the pharmaceutical industry, Congress, the Reagan-Udall Foundation, and the FDA to identify solutions that will not only bring promising treatments into the hands of patients as soon as possible, but also speed the development and approval of those treatments so that they are available to all patients. Our goal is to make a difference and ensure that people with ALS truly have the right to try promising treatments as soon as possible.

How does the federal-level Right to Try work with state-level Right to Try laws?
A clause within Article VI of the U.S. Constitution dictates that federal law is the "supreme law of the land." For that reason, federal law generally supersedes state law. The FDA is expected to issue guidance in 2018 on the agency’s role in implementing the new federal Right to Try statute.

What is an investigational drug?
An investigational drug is one that is under study but does not yet have permission from the U.S. Food and Drug Administration (FDA) to be legally marketed and sold in the United States.

What are clinical trials and phases?
Clinical trials are research studies that are aimed at evaluating a medical intervention. They are the primary way that researchers find out if a new drug is safe and effective in people. Clinical trials are divided into different stages, called phases. Phase I focuses on safety, Phase II focuses on effectiveness, and Phase III studies different populations and different dosages, using the drug in combination with other drugs.

If I want to try investigational/experimental drugs or therapies, what do I do?
By far, the most common way that patients get investigational drugs is by participating in a clinical trial. Patients who do not meet the eligibility criteria for a clinical trial of an investigational drug may be eligible to receive the drug through expanded access, a program operated by the Food and Drug Administration (FDA), or through Right to Try.

Who is eligible for Right to Try access to investigational drugs?
An “eligible patient” is someone who has been diagnosed with a life-threatening disease or condition and has exhausted approved treatment options and is unable to participate in a clinical trial involving the eligible investigational drug, as certified by a physician.
If I am eligible for a clinical trial of an investigational drug, can I instead choose to access the treatment outside of the trial under Right to Try?
No, eligible patients must have exhausted approved treatment options and be unable to participate in a clinical trial involving the eligible investigational drug.

Will investigational drugs under Right to Try or expanded access be covered by Medicare, Medicaid, the Department of Veterans Affairs, or private insurance?
Medical insurance, of any kind, will not cover investigational drugs or experimental treatments.

What is the difference between expanded access and Right to Try
Under FDA expanded access, patients work with their physician and the FDA to secure access to investigational drugs from the manufacturer. Under Right to Try, patients work with their physicians to directly petition the manufacturer for access to an investigational drug.

Right to Try allows access to experimental treatments after completion of a Phase I clinical trial. What is a Phase I clinical trial?
Phase I clinical trials test a relatively small number of volunteers (up to a few dozen). The main purpose of Phase I trials is to determine the highest dose of a drug that can be given safely without serious side effects. Phase I allows researchers to observe how the drug works within the body and what the body does to the drug. About 70 percent of drugs move on to Phase 2 trials.

Under Right to Try, does a person have to be monitored for drug-drug interactions that may occur?
Right to Try does not currently address monitoring patients for drug-drug interactions, but it does require manufacturer sponsors or sponsors of eligible investigational drugs to provide an annual summary to the FDA detailing the occurrence of any adverse events more broadly.

Who regulates drugs?
The FDA regulates the drug development and approval processes. However, Right to Try provides a different avenue for obtaining experimental drugs outside of these established processes.

Are manufacturers required to provide access to investigational drugs under Right to Try?
No, while Right to Try enables patients to directly petition manufacturers of eligible investigational drugs for access, these manufacturers are not obligated to provide access to their drug for use by the patient.

If a manufacturer provides access to their investigational drug, are they required to furnish it free of cost?
No, if the manufacturer does provide access, neither the manufacturer nor the patient’s health insurance are obligated to cover any cost of the drug. Thus, patients must both convince manufacturers to provide access to their drug and bear the costs for these experimental treatments, which can be costly – particularly given the limited supply.
What drugs are available under Right to Try?
Every manufacturer must make a choice on every drug regarding Right to Try. Eligible patients can apply for access to any “eligible investigational drug” – a drug for which a Phase I clinical trial has been completed, but where the drug has not been approved or licensed for any use. The drug must be the subject of an active investigational new drug application under FDA that is either the subject of a pending New Drug Application (NDA) or Biologics License Application (BLA) or that is under investigation in a clinical trial that is the subject of an Investigational New Drug (IND). The IND must be intended to form the primary basis of a claim of effectiveness supporting FDA approval or licensure, and for which development or production of the drug is ongoing and has not been discontinued by the manufacturer or placed on a clinical hold.

What FDA reporting requirements are there for drugs accessed under Right to Try?
Right to Try has a reporting requirement for manufacturer sponsors of eligible investigational drugs, specifically that sponsors provide annual summaries on any use of such investigational drugs, including:

- The number of doses supplied.
- The number of patients treated.
- The uses for which the drug was made available.
- The occurrence of any adverse events.

How will Right to Try Impact Clinical trials?
It is unclear what impact this law may have on clinical trials. Concerns have been raised that recruitment in trials and subsequent development and approval of a drug could be negatively impacted if patients can instead obtain the experimental drugs outside of a clinical trial.

Under Right to Try, if a person is harmed taking a drug outside of a trial, will that impact the potential approval of the drug? While Right to Try prevents this data from being used unless it is deemed "critical to determining safety," undesirable outcomes might give the FDA pause and impact the approval of drugs.