

The Need for Federal Right-to-Try Legislation

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In August of 2017, the Senate [passed](#) by unanimous consent a federal right-to-try bill that would ease restrictions on terminally ill patients gaining access to experimental treatments. Today, the legislation [awaits passage](#) by the House Energy and Commerce Committee – chaired by Representative Greg Walden – before it can advance to a full vote in the House. If the legislation were to advance it would likely pass with overwhelming bipartisan support and give the right to try to thousands of terminally ill patients across the country.

In an [exchange with STAT](#) in December, Chairman Walden addressed his position on right to try, saying, “we’ve been evaluating different state [right to try] laws to see if they’ve even been used ... *if indeed the state laws are not being used very much, then the last thing I want to do is give false hope to families and dying patients* that somehow they’ll have a new access when in fact they may not.”

What this misses, though, is that a lack of efficacy at the state level doesn’t preclude the need for federal legislation; it *underscores* the need for federal legislation. It’s precisely that lack of clarity at the federal level that’s hindering the efficacy of state-level efforts.

WHY “EXPANDED ACCESS” IS NOT ENOUGH

To date, right-to-try laws have been enacted in [38 states](#), and the momentum behind the movement is [credited](#) with finally prompting the U.S. Food and Drug Administration (FDA) to revamp the application process for its expanded access program – commonly known as “compassionate use” – which allows patients to petition the FDA for permission to use an unapproved medical product outside of the context of a clinical trial. That application process previously took an estimated [100 hours](#) for a physician to submit a request for a single patient.

Though reducing the burden of the initial application is a welcome change, it still leaves the rest of the process intact: no change has been made to the documentation requirements from the drug manufacturer, the [up-to-30-day wait](#) for a response from the FDA, or the sign-off needed from a separate committee called the Institutional Review Board. Navigating this process can seem interminable for patients whom, by definition, have little time to spare.

Though the FDA [boasts](#) of granting over 99% of expanded access requests it receives, it only received [1,757 requests](#) for expanded access in total last year, minuscule in comparison to the [596,000](#) Americans who died of cancer in 2015, the 155,000 who died of chronic lower respiratory diseases, or the 111,000 who died of Alzheimer’s. This number is troubling in light of the nearly [26,000 patients](#) receiving experimental treatments in France in 2016 under a similar program, meaning nearly fifteen times as many patients receiving experimental treatments in a country [one-fifth the size](#). Underlying this disparity is the burden placed on patients by the FDA, discouraging them from even submitting a formal application.

In an [article](#) for The Goldwater Institute, Carla Mann Woods, formerly a medical device industry executive, and now a board member of the Alfred E. Mann Institute for Biomedical Engineering at the University of Southern California, put it this way: “In this era of both scientific revolution and information where anyone can find anything on the Internet, ask yourself this: Can you actually believe that only 1,200 dying Americans want to live badly enough to find a legitimately applicable, unapproved therapy and ask to get it?”

The fact that so few compassionate use requests make it to the FDA for final approval is not an indication that terminally ill patients aren’t interested in trying these medications, it’s an indication that the process is broken.

STATE RIGHT-TO-TRY LAWS: OPERATING IN THE COLOSSAL SHADOW OF THE FDA

In the face of federal inaction, states very recently began to pass their own right-to-try laws in an effort to remove barriers for terminally ill patients, with [Colorado leading the way](#) in 2014. The chilling effect from a lack of clear federal guidance, however, undermines state right-to-try laws by leaving open the question of future FDA approval for drugs and biologics in addition to liability.

In a [report](#) released last year by the Government Accountability Office (GAO), drug manufacturers reported a pervasive concern that the FDA is not clear about how data on “unexpected adverse events” that are associated with the use of a drug – which must be reported to the agency – is used in its review of new drug applications and biologics license applications. The fear that an adverse reaction to treatment in an expanded access setting or beyond could result in a clinical hold or impact a final approval decision can contribute to a manufacturer choosing not to grant patients access to their drugs outside of a strictly controlled clinical trial. Furthermore, although state right-to-try laws [remove regulatory barriers on the state level](#), stakeholders can be hesitant to provide access to or administer experimental treatments out of concern for *federal* liability.

Without clear guidelines on how the FDA will respond to adverse effects of experimental treatments and the potential for federal liability, drug manufacturers are taking on significant risk. The net effect is a regulatory regime in which medical professionals and drug manufacturers are disincentivized by federal ambiguity despite overwhelming support at the state level.

REMOVING BARRIERS TO ACCESS IS NOT “GIV[ING] FALSE HOPE”

The purpose of right-to-try legislation is to give terminally ill patients the ability to seek experimental treatments where no other alternative exists. Far from giving “false hope,” this legislation would allow patients and their doctors to decide the best course of action – whether, for instance, trying an amyotrophic lateral sclerosis (ALS) drug that’s [been approved in Japan](#) or a bone cancer drug that’s [been approved in the U.K.](#) – as long as the treatment has passed Phase 1 of FDA approval, remains in clinical trials, and all other treatment options have been exhausted. For patients with life-threatening illnesses, a federal right-to-try law is a last resort.

With states overwhelmingly in favor, Chairman Walden should take the opportunity to advance legislation that would enact clear federal guidelines to remove regulatory barriers to access for patients and establish national standards for pharmaceutical companies, doctors, and patient consent. Advancing this legislation out of the House Energy and Commerce Committee is the last remaining hurdle before it reaches the House floor.

For [Mark Angelo](#), whose diagnosis of pancreatic neuroendocrine cancer was followed by three years of oral chemotherapy that proved to be ineffective, Texas’ passage of right-to-try legislation changed the trajectory of his treatment and wellbeing. After being accepted for peptide receptor radionuclide therapy (PRRT) at Houston’s Excel Diagnostic Center, his tumors shrank while his weight and strength returned over the next two years. Patients like Mark who are undergoing life-threatening illnesses should be able focus their attention on seeking the treatments that are right for them without the added burden and time spent untangling federal rules and paperwork.

Giving someone the right to try is not giving them “false hope” – it’s empowering them to try to save their life. There are thousands of terminally ill patients like Mark Angelo who could benefit from federal legislation. They shouldn’t wait another day longer.

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