“No, not in a meaningful way,” says Kirstin Matthews, Ph.D.

Since 2014, patient advocates have tried to further increase access to experimental interventions in the United States. Their work is rooted in model legislation created by the Goldwater Institute, which states that terminally ill patients have a “constitutional right to medical autonomy.” As a result, 28 states have passed “Right to Try” laws that aim to give terminally ill patients access to early investigational drugs, before clinical trials are completed. However, Right to Try laws circumvent the U.S. Food and Drug Administration’s own program for expanded access to experimental interventions and highlight a growing conflict between patients and the FDA.

The FDA has two major objectives: (1) “assuring the safety, efficacy and security of human and veterinary drugs” and other medical interventions, and (2) “advancing public health by helping to speed product innovation.” These aims are addressed through research and clinical trials, a sometimes lengthy process intended to assess the safety and efficacy of experimental interventions. However, these two charges can conflict at times as the public pressures the FDA for quick access to new treatments and interventions yet also expects approved treatments to be safe. In the 1970s, the FDA created an “expanded access” policy in which terminally ill patients can access experimental drugs, usually in the final phase (Phase 3) of a trial. The policy is subject to federal regulation and ethical approval by an internal review board, as well as the willingness of pharmaceutical companies to provide the new treatments.

Right to Try laws arguably make the safety and efficacy of unproven drugs secondary to speedy access. It allows terminally ill patients to try experimental drugs after Phase 1 of the clinical trials process is completed. But these patients undergo the treatments without FDA or outside ethical oversight — meaning no one is ensuring that the patients understand the risks associated with the procedures. Right to Try laws focus on protecting physicians rather than patient safety, whereas the FDA’s “expanded access” policy has numerous measures in place to safeguard patients.

Furthermore, Right to Try laws do not really grant access to drugs — it is often the pharmaceutical companies who are blocking access. The FDA estimates that 99 percent of expanded access requests in the past five years were approved. On the other hand, pharmaceutical companies must weigh the benefits of granting access outside of the trial against the risks, such as not having enough drugs for the actual trial participants or, in the case of a non-trial participant, the impact of a negative patient response on the trial results.

Additionally, Right to Try laws can be costly to patients. Some states do not require insurers to cover these new therapies, and move the costs to the patients. In a few states, patients receiving experimental treatments find that Right to Try laws also limit their access to hospice care.

Instead of working against or around the FDA, patients and advocates should work with the agency to improve the expanded access program. In June 2016, in response to public pressure and other critiques, the FDA revised the expanded access application process. It now takes 45 minutes to complete the application compared to the 100 hours previously required. These and other efforts will help increase access to experimental interventions while keeping patient protections in place.
HEALTH POLICY research presents a summary of findings on current health policy issues. It is provided by Vivian Ho, Ph.D., James A. Baker III Institute Chair in Health Economics and director of the Center for Health and Biosciences at Rice University’s Baker Institute for Public Policy, in collaboration with Laura Petersen, M.D., MPH, chief of the Section of Health Services Research in the Department of Medicine at Baylor College of Medicine.

This publication aims to make research results accessible to regional and national health policymakers. The views expressed herein are those of the study authors and do not necessarily represent those of the Baker Institute or of Baylor College of Medicine.

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