ACRO
ASSOCIATION OF CLINICAL RESEARCH ORGANIZATIONS

Right to Try
ACRO Policy Statement

Over the past few years, legislation that would give terminally ill patients access to unapproved treatments, known as "Right-to-Try," has gained momentum in a number of states and now in the U.S. Congress. As the organization representing the world's leading clinical research organizations that each year conduct more than 7,000 clinical trials involving 1.3 million patients in more than 100 countries, ACRO is deeply committed to ensuring treatments are made available to patients in need, to the full extent possible, through the established clinical trials process in order to effectively monitor safety and efficacy. ACRO also supports measures to accelerate the drug discovery, development and approval process without sacrificing patient safety.

As such, ACRO's view is that proposed Right-to-Try legislation is deeply flawed because it:

- contains insufficient patient protections;
- compromises the clinical trial process; and
- undermines the FDA's authority to assess safety and effectiveness;

ACRO has significant concerns that including Right-to-Try provisions in the upcoming user fee reauthorization or any subsequent legislation would be detrimental to patients.

The Food and Drug Administration (FDA) currently administers a program for Expanded Access, also known as "Compassionate Use," which gives patients the ability to access an investigational product, i.e., one that has not been approved by FDA, outside of a clinical trial. Compassionate Use is often used when a patient cannot participate in a clinical trial, because of "exclusion criteria" such as having more than one life-threatening disorder. Unlike Right-to-Try, under Expanded Access, a patient must meet certain conditions to participate, including: that the patient's physician determines that there is no comparable therapy available to diagnose, monitor, or treat the patient's disease or condition; that FDA determines that the probable risk to the patient from the investigational product is not greater than the probable risk from the disease or condition; and that the FDA determines that providing the investigational product will not interfere with the conduct of clinical trials to support marketing approval.

Clinical trials provide the pathway for a thorough assessment of the safety and efficacy of a drug, ensuring that patients and their health care providers have adequate information about a product's risks and benefits. Yet, Right-to-Try efforts may exacerbate the problem of recruitment for those trials. According to the Tufts Center for the Study of Drug Development, 37% of clinical trial sites do not meet enrollment goals and 11% fail to enroll a single patient.

Patient recruitment and retention are two of the biggest challenges to clinical trials today. With Right-to-Try, patients could start taking an investigational therapy without ever pursuing the traditional trial route, making it even more difficult to meet enrollment goals. This would create an especially large recruitment hurdle for rare disease, orphan drug and pediatric trials. Compared to trials for other diseases, rare disease trials – those for diseases affecting less than 200,000 patients in the US -- are even harder to recruit for due to limited patient populations. If even a few rare disease patients were to opt for "Right-to-Try" rather than a clinical trial, for example, this could severely delay or compromise the entire drug development effort, placing the entire patient population at risk.

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In 2015, all but 10 of 1,278 Compassionate Use requests (99.2%) were approved by the FDA. Applicants usually receive responses within four days of applying, and a recently streamlined FDA form takes about 45 minutes to complete.[i] Additionally, average response time for emergency use is one day or less. Provisions in the 21st Century Cures Act will further strengthen the FDA's Expanded Access program by requiring greater clarity on the policies of pharmaceutical companies and enhancing communication.

To further improve the accessibility of information about Expanded Access programs for patients and their providers, the Reagan-Udall Foundation plans to introduce an Expanded Access navigator, a portal of online resources that includes educational content, tools, and a contact directory. The Foundation hopes to promote the use of Compassionate Use through this effort. This portal is expected to launch in June, 2017.

Given the FDA's implementation of its current Compassionate Use policy, it is unclear that Right-to-Try legislation would provide any added benefit for individual patients in terms of access. Such legislation would, however, create potentially significant concerns for patient safety. This legislation could also lead to ethical challenges for future clinical research, weakening the informed consent process by diminishing the safety and risk/benefit data that should be available to every patient.

By circumventing the FDA, Right-to-Try eliminates utilizing the agency's objectivity, expertise and knowledge of treatments in various stages of development, in presenting the best options to the patient. Conversely, under Expanded Access, FDA review can require changes to treatment plans that are most beneficial to patients.

The mission of ACRO member companies is to ensure the safety of human subjects and maintain data integrity in clinical research; however, Right-to-Try places both objectives at risk. Recent federal Right-to-Try proposals would prevent FDA from considering adverse events arising out of the use of a product through the Right-to-Try path. This would undermine patient safety protections not only for the patients accessing the product outside of a clinical trial but also for the patients using the product once it is on the market.

ACRO notes the European Medicines Agency (EMA) has also provided recommendations on Compassionate Use. Similar to FDA's Compassionate Use policy, the EMA recommendation states that such compassionate access to investigational (non-approved) drugs should only be used when a patient is left without other treatment options. ACRO generally supports policies that conform to international norms for patient safety and best practices for clinical research.

ACRO believes that any effort to make investigational therapies available to patients outside of a clinical trial should include proper patient protections and avoid compromising the current FDA safety and effectiveness review process. For these reasons, ACRO does not support Right-to-Try legislation.

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[i] Lurie P. Committee on Homeland Security and Government Affairs Hearing Testimony, U.S. Food and Drug Administration, available at https://www.fda.gov/NewsEvents/Testimony/ucm522044.htm

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