# Patient Access to Investigational Therapies

Many states across the country are considering so-called "Right to Try" legislation to provide patients with access to investigational therapies before they are approved by FDA. However, for over two decades, FDA has had processes in place to do just that. Expanded access, which is sometimes called "compassionate use," supplements the clinical trials process. FDA believes enrollment in clinical trials remains the best option for patients wishing to gain access to investigational drugs—it assures adequate protection for patients and leads to the collection of data that could eventually result in FDA approval of the investigational therapy, which provides the broadest availability to patients. Patients who are not eligible for a clinical trial because of where they live, their age, or some other disqualifying factor have the option to seek expanded access if they have serious or life-threatening conditions and no comparable or satisfactory alternative is available.

FDA acts quickly in response to expanded access requests and allows almost all of them to proceed. In fact, FDA authorized more than 99 percent of individual patient expanded access requests received in Fiscal Years 2010-14. Emergency requests are often granted immediately over the phone. For non-emergencies, the Agency strives to respond promptly and, in general, does not take longer than 30 days. Moreover, FDA continues to improve its processes. In response to feedback from physicians that the expanded access form was challenging, in February 2015, FDA announced the development of a new draft form for individual patient expanded access that is estimated to take only about 45 minutes to complete.

Expanded access to investigational treatments requires the active involvement and cooperation of parties other than FDA, including drug companies and healthcare providers. FDA can encourage drug companies to offer expanded access to their investigational therapies, but companies may choose not to do so for various reasons, including lack of available drug or a desire to focus their attention on completing the clinical trials necessary to support FDA approval.

**Facts:**

- FDA has a longstanding and well-established process for individual patients to obtain access to investigational therapies—expanded access, which is sometimes called compassionate use.
- FDA allows almost all expanded access requests to proceed: more than 99 percent of individual patient expanded access requests made from 2010-14 were granted.
- FDA responds to individual patient expanded access requests quickly; emergency requests are often granted immediately over the phone. For non-emergencies, the Agency strives to respond promptly and, in general, does not take longer than 30 days.
- FDA is improving expanded access to make it easier to apply; a new form for individual patient expanded access requests is estimated to take physicians only about 45 minutes to complete.

FDA is an important part of the process and helps to ensure patients are adequately protected from unnecessary risk. The independent scientific review provided by FDA is an essential component of patient protection, particularly because one is considering treatments for which safety and efficacy have not been demonstrated.

**Contact Us**
For more information, please contact FDA's Office of Legislation at 301-796-8900, or see FDA's website: http://www.fda.gov/ExpandedAccess.