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New online navigator helps patients and doctors access experimental treatments

By June S. Wasser

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When approved therapies don't work, or stop working, for people with serious or life-threatening illnesses, it puts them in a difficult position. Some turn to clinical trials that are testing experimental treatments. But many can't do that because they are too sick, don't meet the requirements of the trial, or can't afford to travel to the site of a trial. That doesn't mean they are out of options.

Patients and their doctors can request access to investigational treatments through a national program called single-patient expanded access, also known as compassionate use[1]. It works like this: A physician submits a request to a drug company for access to an investigational medicine. If the drug company agrees, the physician must then get Food and Drug Administration approval[2] before the company can supply him or her with the medicine. The FDA approves more than 99 percent[3] of the compassionate use requests it receives. Through this process, the FDA ensures that the medicine is being administered by a licensed practitioner.

Recent legislation, such as the 21st Century Cures Act[4], and changes in regulations[5] require drug companies to make public whether they provide expanded access and, if they do, their expanded access policies and contact information. The number of companies with publicly posted expanded access policies has more than doubled[6] since the fall of 2016. This information can be hard to find because it is scattered all over the internet.

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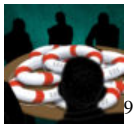
That's why my organization, the Reagan-Udall Foundation for the FDA, has just launched the Expanded Access Navigator[8]. It provides in one place information about the expanded access policies of various companies and the availability of investigational medicines under them. It also helps lead patients and physicians through the request process. The navigator currently focuses on cancer drug manufacturers and resources, but will gradually be expanded to include drugs for other diseases.

Just because a drug is used in a clinical trial doesn't mean it is effective or even safe — that's why it is being tested. Investigational therapies can have serious side effects that could lead to serious complications, hospitalization, reduced quality of life, and even premature death. For people who are terminally ill, such risks may be acceptable. But patients and their physicians must have a meaningful dialogue to weigh the risks and benefits before embarking on treatment with an investigational medicine.

The Expanded Access Navigator is divided into two sections, one for patients and caregivers, the other for physicians.

- The patient and caregiver section explains expanded access, helps users find appropriate clinical trials, and describes how to work with a physician to request access to an investigational medicine.
- The physician section helps clinicians identify investigational treatment options for their patients, and walks them through the process of asking a pharmaceutical company about expanded access for a patient and submitting an expanded access form to the FDA. (The agency has taken steps to streamline the process for physicians, and last year introduced a shortened application form to simplify the necessary paperwork.)

 [9]

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Given the challenges of offering expanded access to investigational drugs, not all companies do it. The company must have enough of the drug on hand to supply both the clinical trial and expanded access request. It must also have resources to manage expanded access requests. Small and emerging companies may find it difficult to manage such challenges.

The last thing that patients with serious or life-threatening illnesses need is a confusing or time-

consuming process for finding and applying for access to investigational drugs that may help them. We hope that the Expanded Access Navigator will let them do this easily and quickly.

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2. https://www.statnews.com/pharmalot/2017/07/12/gao-fda-compassionate-use/
3. http://journals.sagepub.com/doi/abs/10.1177/2168479016656030
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