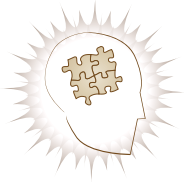
Expanding Expanded Access

An easier application process could make experimental drugs more accessible to patients in need — outside the realm of clinical trials. By DELLANN ELLIOTT MYDLAND

DELLANN ELLIOTT MYDLAND is founder and president of the EndBrainCancer Initiative, whose goal is to provide patients with immediate access to top brain cancer specialists, advanced treatments and clinical trials. She founded the group 14 years ago with her late husband, Christopher Stewart Elliott, who died of glioblastoma in 2002.



IF YOU HAVE A brain cancer or tumor and have questions, or would like to be coached on how to discuss expanded access programs with your doctor, please contact our "Direct Connect" center at EndBrainCancer.org or call us at 425-444-2215. You can also reach our patient navigators online by visiting our website and filling out a patient inquiry form.

"I'M SORRY, BUT THERE isn't anything else I can do for you."

Many patients fear hearing that statement from their doctors if standard treatments for cancer stop working.

But even when those words are spoken, they don't always mark the end of a patient's treatment journey. An option for some may be expanded access programs, which offer promising experimental drugs — outside the structure of clinical trials — to seriously ill people who have exhausted other treatment options. In some cases, drug developers run these "compassionate use" programs, which do not focus on collecting safety or efficacy data, for groups of patients; in others, they are designed to accommodate just one person. Often, developers foot the bill for the treatments.

These programs are rare, but the process of joining them got a bit less complicated in early October, when the Food and Drug Administration (FDA) streamlined the steps doctors must take to enroll patients who meet eligibility requirements by creating a shorter application form, FDA 3926.

An advantage of expanded access programs is that every eligible patient who is enrolled gets the experimental drug in question. This does not always happen with clinical trials, since some participants may be placed in a control group receiving standard-of-care treatment.

Of course, we at the EndBrainCancer Initiative (EndBrainCancer.org) strongly advocate participation in clinical trials. Through our Direct Connect and Clinical Trial Prequalification programs, we often find appropriate studies for people with brain cancer, and help them enroll. However, people with serious illnesses or rare diseases might not qualify for these trials due to specific inclusion or exclusion requirements.

Still, we believe that all patients should have immediate access to advanced treatments for which they are eligible, even if the therapies are still experimental, and sometimes expanded access programs offer more leeway. That's why we encourage patients with cancer to tell their doctors, at the outset of treatment, that they are interested in expanded access programs and would like these options factored into their therapy plans. Patients may not know which drugs, if any, they will later be interested in trying, but they can get the process rolling by putting their doctors on alert and giving them copies of the FDA form.

Admittedly, learning about and enrolling in these programs is not simple.

A patient might find an appropriate one by searching ClinicalTrials.gov and drug company websites, or by checking with the FDA at 855-543-3784. A promising program's sponsor can provide details about joining.

For a drug of interest that offers no expanded access program, a doctor can — after first getting the backing of his or her hospital or independent institutional review board — ask the developer to create one. Once those steps have been taken, the patient can help by contacting the drug's maker and arranging for its representative to meet with the doctor. It's a good idea to act quickly, because the approval process can take as long as two months.

Once the drug maker grants permission, the patient should give the doctor Form FDA 3926, or explain that it can be obtained by visiting tinyurl.com/ycw3w2mx, calling 855-543-3784 or emailing druginfo@fda.hhs.gov.

Luckily, this part of the process can move much faster. In emergency cases, the FDA authorizes submissions in a matter of days, or even over the phone in just hours. Moreover, the FDA ultimately authorizes 99 percent of such requests.

When creating a cancer treatment plan, a doctor should talk to the patient about standard-of-care treatment for his or her disease; any gene mutations that drive that kind of cancer and are treatable with drugs; clinical trials; and expanded access to treatments. The doctor should also provide relevant information about drug safety and efficacy, as well as estimated costs of treatment, diagnostic tests, supportive care and any related travel. If your doctor does not discuss these topics with you, be your own advocate and ask.

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