

CONTENTS

Ethics of Access

BY ELIZABETH WHITTINGTON

Terminal cancer patients whose tumors don't respond to standard treatment have limited options, especially those with aggressive cancers that have become resistant to various treatment regimens. Typically, these patients don't qualify for clinical trials.

Is it ethical to place these patients in phase I trials with an experimental compound that has never been tested in humans and may cause more harm or undue suffering? Or is it unethical to deny these patients access to investigational agents that may have the potential—even if it's only small—to prolong their lives?

Certain unapproved drugs are available outside clinical studies to a limited number of terminally ill patients through expanded access programs, but they may have the same criteria as clinical trials, including type and stage of disease, or lengthy processes to acquire them. With expanded access, investigational drugs—ones that are reasonably safe, have shown activity in late-phase trials, and have an approval decision pending—are made available to patients with few treatment options.

In compassionate use, an investigational drug is offered to an individual patient rather than a group. The drug maker and the Food and Drug Administration approve uses on a case-by-case basis. Decisions are based on the availability of other treatments as well as efficacy, potential toxicity, and availability of the investigational drug.

A limited number of patients are eligible to receive investigational drugs in emergency situations. It is up to the drug company to choose whether or not to charge patients, and while many of the larger companies have set a precedence of providing these drugs for free, smaller companies may not be able to afford free distribution.

A recently proposed rule by the FDA clarifies that companies can charge terminal cancer patients with no other medical options for these drugs. But because most insurance companies do not cover experimental treatments, patients would pay for them out-of-pocket with no guarantee it would help them.

The Abigail Alliance for Better Access to Developmental Drugs, an organization that advocates availability of unapproved drugs for patients who have exhausted all other options, filed a lawsuit against the FDA claiming terminally ill patients have a constitutional right to unapproved drugs that have shown promise in phase I clinical trials.

Not so, says the American Society of Clinical Oncology and the National Coalition

for Cancer Survivorship, which publicly sided with the FDA on the lawsuit. The nonprofit groups say early-phase trials do not provide enough information on safety or efficacy to justify making an investigational drug commercially available. Access to unapproved agents that have not been through the full process of clinical trials and reviews may put the patient at risk for unknown side effects, resulting in a greater risk of harm than benefit, they say, and may decrease clinical trial participation or possibly sideline a promising drug if results appear to be negative outside of a clinical trial setting.

Oral arguments were heard before the U.S. Court of Appeals for the District of Columbia Circuit in March, and a ruling is expected as early as August.