

Clinical research versus patient care: Access to experimental treatment

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An outbreak of pandemic disease for which there is no known effective therapy can create a climate of fear and uncertainty that may lead patients, and their physicians, to be willing to take risks they would not consider under normal circumstances. Calls for access to unproven, experimental treatments by individuals who are unable or unwilling to participate in clinical trials of new interventions may proliferate.

Such situations raise concerns about false hope on the part of patients in serious need, and in turn about the quality of decision making and consent as well. Opinion 7.3.10 of the *AMA Code of Medical Ethics*, “Expanded Access to Investigational Therapies,” addresses these critical concerns.

For patients who do not meet the inclusion criteria for ongoing trials or who are unwilling to risk the possibility of being enrolled in the control arm of a study and not receiving the investigational therapy the Food and Drug Administration’s “expanded access” program may offer access to an investigational therapy outside a clinical trial.

In considering an application for expanded access and commonly referred to as compassionate use, a physician should advise the patient, among other things, that:

- the therapy has not yet been demonstrated to be effective in treating the patient’s condition and may pose as yet unknown risks
- the financial cost of the therapy may be the responsibility of the patient
- there may not be appropriate infrastructure to monitor and evaluate the effects of the investigational therapy

Physicians providing treatment under expanded access should inform the patient about the conditions under which the physician would recommend stopping treatment with the investigational therapy.

Physicians should refrain from applying for expanded access on a patient’s behalf when if, in their professional judgment the investigational therapy is not in the patient’s best interest or the physician does not have the resources or ability to safely supervise the patient’s care under expanded access.

“Right to try” laws at the state and federal level permit patients and their physicians to apply directly to the manufacturer, rather than the FDA, for access to a not-yet-approved therapy currently under investigation. However, these laws do not require manufacturers to provide the requested therapy.

Additional ethics guidance in a pandemic

The AMA offers an overview of foundational guidance regarding medical ethics for health care professionals and institutions responding to the COVID-19 pandemic.