

Your patient requests an investigational therapy: How to respond

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For patients facing life-threatening or life-limiting conditions who have exhausted their approved treatment options and are unable to participate in clinical trials, the Food and Drug Administration's (FDA) expanded access program may be their only way to access investigational therapies. Policy recently adopted by the AMA recommends how to respond to patient requests for unapproved treatments, including when to decline to support an application and when to refer a patient to another physician.

The guidance is based on a 2018 report of the AMA Council on Ethical and Judicial Affairs, which maintains and updates the *AMA Code of Medical Ethics*. Foremost among the report's ethical concerns is informed consent.

"Patients who face serious, life-threatening illnesses for which approved therapies have not been effective or for which there are no approved therapies may be particularly vulnerable to holding out false hope for investigational therapy," the report says. "Promoting truly informed decisions about whether to request expanded access is critical, but can be difficult, both because information about an investigational therapy is often incomplete or difficult to obtain, and because patients may be prone to misinterpreting what information is available."

In addition, not all sponsors shoulder the cost of providing investigational therapies, and insurers for the most part do not cover them, raising the concern that expanded access could favor wealthier and more well-connected patients, the report notes.

What the *Code* says

In Opinion 7.3.10, "Expanded Access to Investigational Therapies," the *Code* explains:

Physicians who care for patients with serious, life-threatening illness for whom standard therapies have failed, are unlikely to be effective or do not exist should determine whether questions about access to investigational therapy through the FDA's expanded access program are likely to arise in their clinical practice. If so, physicians should familiarize themselves with the program to be better able to engage in shared decision-making with patients.

When a patient requests expanded access to an investigational therapy, physicians should:

Assess the patient's individual clinical situation to determine whether an investigational therapy would be appropriate, including:

- Whether there is a satisfactory alternative therapy available to diagnose, monitor or treat the patient's disease or condition.
- The nature of potential risks of the investigational therapy and whether those risks are not unreasonable in the context of the patient's disease or condition.
- Whether the potential benefit to the patient justifies the risks of the investigational therapy.
- Whether the patient meets inclusion criteria for an existing clinical trial of the investigational therapy.

Advise the patient—or parent or guardian if the patient is a minor—as part of the informed-consent process that the investigational therapy has not yet been demonstrated to be effective in treating the patient's condition and may pose as yet unknown risks. Physicians should explain the importance of clinical trials, encourage patients who meet inclusion criteria to participate in an existing trial rather than seek access to investigational therapy through the FDA expanded access program, and direct patients who wish to participate in research to appropriate resources.

Decline to support an application for expanded access to an investigational therapy when:

- The physician judges the treatment with the investigational therapy not to be in the patient's best interest, and explain why.
- The physician does not have appropriate resources and ability to safely supervise the patient's care under expanded access. In such cases, physicians should refer the patient to another physician with whom to discuss possible application for expanded access.

Discuss the implications of expanded access for the patient and family and help them form realistic expectations about what it will mean to be treated with the investigational therapy outside a clinical trial.

Physicians should alert patients:

- To the possibility of financial or other responsibilities associated with receiving an investigational therapy through expanded access.
- To the lack of infrastructure to systematically monitor and evaluate the effects of the investigational therapy outside a clinical trial.
- That they need information about how to contact the manufacturer for guidance if they seek emergency care from a health care professional who is not affiliated with a clinical trial of the investigational therapy.
- That the physician has a responsibility to collect and share clinical information about the patient's course of treatment with the investigational therapy, as well as to report any adverse events that may occur over the course of treatment.
- To the conditions under which the physician would recommend stopping treatment with the investigational therapy.

More help here

The *Code* features additional guidance on special issues in research, including opinions on research in gene therapy and genetic engineering, research with stem cells and safeguards in the use of DNA databanks.