

## Position on Expanded Access to Investigational Drugs

### Background

Patients seeking preapproval access to investigational drugs often suffer from serious or immediately life-threatening diseases or conditions. These patients may be willing to try an experimental drug for which robust clinical information about efficacy is not yet available. These programs can be referred to by different names; two common ones are “expanded access” and “compassionate use”. Pfizer refers to these collectively as expanded access programs. The process for requesting expanded access to an investigational drug requires several steps. Typically, the treating physician will make a request of the company developing the drug. If the company agrees to supply the investigational drug, the physician will initiate a request with the appropriate regulatory agency to approve the request. In most countries, companies are not allowed to distribute unapproved drugs without formal regulatory approval. In the U.S., a treating physician requests permission from the Food and Drug Administration (FDA). In the European Union, the European Medicines Agency (EMA) provides recommendations, but expanded access programs are implemented by each member state with its own rules and procedures.

Pharmaceutical companies participate voluntarily in these expanded access programs and carefully consider each request. Considerations at Pfizer include: the disease or condition be serious or life-threatening; whether all other approved treatment options have been exhausted; if enrollment in a clinical trial is possible; whether there is a scientific rationale to support that the requested experimental drug may provide benefit and that the potential benefits outweigh the potential risks; and if there is sufficient supply to make the investigational drug available to a patient outside the clinical trial setting.

### Pfizer’s Position

Pfizer’s overarching goal is to protect the best interests of patients by preserving the integrity of clinical trials and the regulatory oversight of expanded access programs, with an emphasis on safety. Clinical trials follow strict scientific standards designed to produce reliable data and results. Where possible, use of an investigational drug within a clinical trial is preferable because clinical trials follow protocols designed to monitor and safeguard patient experiences with a drug, and because trials generate data that may lead to the approval of therapies so that they may be available for wider use. At the same time, Pfizer supports efforts to provide expanded access outside a clinical trial to investigational drugs for patients diagnosed with a serious or life-threatening disease or condition, who have exhausted approved treatment options, and who are not eligible for clinical trials.

- Pfizer supports policies that ensure transparency to patients and their physicians about how to make such requests so that every request is considered equally.
  - Pfizer supports industry guidelines for companies to post policies and provide a single point of contact to make requests.
    - The United States 21st Century Cures Act requires pharmaceutical companies to publicly provide the policies and procedures for processing requests for expanded access. Pfizer follows these requirements and posts information on [Pfizer.com](https://www.pfizer.com).
- Pfizer supports efforts to streamline request processes and, to the extent possible, adopt standardized forms to lessen physician and patient burden.
  - FDA has made significant progress to streamline its application process; to simplify its website; provide factsheets; and streamline the IRB review process.
- Pfizer supports multiple-stakeholder educational efforts for physicians and patients to help them engage with the appropriate governing bodies and companies.
  - Pfizer sponsored the launch of the [Expanded Access Navigator](#), a unique partnership between the Reagan-Udall Foundation for the FDA, patient advocacy organizations, the pharmaceutical industry, and the federal government. This website provides a consolidated directory of industry expanded access programs and provides patients, caregivers, and physicians with information to guide them through the expanded access request process.

## Pfizer's Expanded Access Activities

Pfizer has a record of providing expanded access to Pfizer investigational medicines for qualifying patients via single patient or multi-patient protocols. In 2022, Pfizer received 2,183 requests for expanded access from 72 countries for 34 products, of which approximately 99 percent of requests were granted. Requests span the company's portfolio of products with 77 percent within the oncology therapeutic area

In 2015, Pfizer launched a website, [PfizerCares.com](https://www.pfizercares.com), where healthcare professionals can submit requests for access to Pfizer investigational drugs or unlicensed products on behalf of patients in their care.<sup>1</sup> Patients and others may also use PfizerCares.com to submit questions about expanded access. Pfizer's policy on expanded access describes all the conditions that must be met for the review to proceed and describes the roles and responsibilities of the colleagues who plan, execute, and manage these requests.<sup>2</sup> Requests are reviewed by Pfizer medical experts dedicated to ensuring that requests are handled according to Pfizer policy in a timely manner. Pfizer strives to respond within five business days of receipt of the request and documentation required to evaluate the request. Expanded access requests from the U.S. then go to the FDA for review and approval. Requests originating in other countries are handled according to regulatory requirements and laws of the country where the request originates.

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<sup>1</sup> See: [www.pfizercares.com](https://www.pfizercares.com).

<sup>2</sup> See: [www.pfizer.com/science/clinical-trials/expanded-access](https://www.pfizer.com/science/clinical-trials/expanded-access).