



Bioethics Position Statement: Expanded Access to Investigational Medicines for Treatment Use

Sometimes people may seek access to investigational medicines before they are reviewed and approved by a regulatory authority, such as the U.S. Food and Drug Administration (FDA). There are two ways this can be done – through clinical trials and expanded access.

Lilly's goal, whenever possible, is to encourage patients to participate in clinical trials. Clinical trials are research studies designed to determine if an investigational medicine is safe and effective for patients. Data generated by these studies provide regulatory authorities with essential information needed to evaluate a medicine. Only after reviewing this information will regulators approve a new medicine, allowing it to be widely available.

In rare cases, however, when people don't qualify for clinical trials and have exhausted all available medical options, Lilly may consider providing an investigational medicine for treatment use outside of a clinical trial. This is called 'expanded access'.

There are several challenges with expanded access that require ethical consideration. First, it is unknown in clinical development whether an investigational medicine is safe or effective. Providing an investigational medicine for treatment outside of clinical trial can expose patients to risk with uncertain benefit. Second, it is possible that expanded access could negatively impact ongoing or new clinical trials and subsequently delay the availability of a new medicine for patients.

Because the mission of a pharmaceutical company is to develop safe, effective medicines to benefit patients, Lilly has ethical responsibilities to ensure the quality and integrity of clinical trials and to minimize risks to research participants, current patients, and future patients. These ethical responsibilities require that Lilly:

- Anticipate expanded access requests for our investigational medicines being developed to treat serious and life-threatening diseases;
- Establish medically appropriate and equitable criteria for expanded access.

In general, Lilly authorizes expanded access based on the investigational medicine's phase of development, benefit-risk profile, and probability and timing of regulatory approval. Specifically, expanded access may be approved if all of the following conditions are satisfied:

1. The disease or condition being studied is serious or life threatening;
2. Sufficient efficacy and safety data exist in order for Lilly to make a reasonable assessment of a favorable benefit-risk balance;
3. There are no satisfactory alternative treatment(s) available; and
4. Expanded access will not impede the initiation, conduct, or completion of a clinical study program that is required for regulatory approval of the investigational medicine.

More information about expanded access may be found [here](#).
