Expanded Access (Treatment Use) of Investigational Agents

Sometimes called "compassionate use", expanded access is a potential pathway for a patient with a serious or immediately life-threatening disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.

Expanded access may be appropriate when all the following apply:

- Patient has a serious or immediately life-threatening disease or condition.
- There is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition.
- Patient enrollment in a clinical trial is not possible.
- Potential patient benefit justifies the potential risks of treatment.
- Providing the investigational medical product will not interfere with investigational trials that could support a medical product's development or marketing approval for the treatment indication.

Investigational drugs, biologics or medical devices have not yet been approved or cleared by FDA and FDA has not found these products to be safe and effective for their specific use. Furthermore, the investigational medical product may, or may not, be effective in the treatment of the condition, and use of the product may cause unexpected serious side effects.

When seeking expanded access to an investigational medical product, it is critical that the patient and his/her licensed physician consider all possible risks. Investigational medical products have not yet been approved or cleared by FDA and FDA has not found these products to be safe and effective for their specific use. Furthermore, the investigational medical product may, or may not, be effective in the treatment of the condition, and use of the product may cause unexpected serious side effects.

Investigational medical products include investigational new drugs and biologics, and investigational devices. Investigational new drug means a new drug or biological drug that is used in a clinical investigation. The term also includes a biological product that is used in vitro for diagnostic purposes. The terms "investigational drug" and "investigational new drug" are deemed to be synonymous. Investigational device means a device, including a transitional device, that is the object of an investigation. This means that these products have not yet been approved by FDA and FDA has not found

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Whenever possible, an investigational medical product should be used as part of a clinical trial. However, if patient enrollment is not possible (e.g., patient ineligibility, lack of ongoing clinical trials), or enrollment in a clinical trial is not feasible (e.g., distance to a trial precludes access), expanded access offers a possible route for gaining access to an investigational medical product.

FDA Resources for detailed information

FDA is committed to increasing awareness about its expanded access process and the procedures for obtaining access to investigational drugs, biologics, and medical devices.

Learn more about expanded access categories for drugs and biologics.

Learn more about expanded access categories for medical devices.

Access for Use at Children's Wisconsin

To help our local clinicians navigate these access pathways, the CW HRPP has created guidance and checklist tools with detailed submission instructions for each regulatory pathway. Each guidance also contains links to FDA pages where you can find more information.

For investigational devices, click here to access Guidance: Individual Patient Expanded Access IDE Applications for patients being treated at Children's Wisconsin.

For investigational drugs/biologics, click here to access Individual Patient Expanded Access IND Applications Checklist for Providers for patients being treated at Children's Wisconsin.