

EXPANDED ACCESS PROGRAMS

What is an EAP? What are the different types of EAPs? What other information is important to know about EAPs?



WHAT IS AN EAP?

An expanded access programs (EAPs), sometimes known as “compassionate use,” is a potential pathway for people with a serious or immediate life-threatening disease or condition to access an investigational medical product (e.g., drug, biologic, or device) that is not yet approved by the Food and Drug Administration (FDA) for treatment.

EAPs are intended for people (individuals or groups) who have no other available treatment options due to a lack of FDA approved treatments options and they are not eligible for any existing clinical trials. These programs’ main goal is to help patients with serious diseases or conditions get access to medication.

DIFFERENT TYPES OF EAPS

1. SINGLE PATIENT

These expanded access programs involve treating a single person. Due to lack of treatment options, the medical provider and their patient create a treatment plan. The drug/biologic/device owner must agree to provide treatment to the individual and the treatment plan must be approved by the FDA. The individual must join a registry. Drug sponsors often charge for the product and insurance companies often do not cover the costs, so individuals typically pay out of pocket costs for the drug and any related procedures and appointments.

2. INTERMEDIATE-SIZE POPULATIONS

Intermediate-size populations: If there is a large demand for a therapy that is still in clinical trials due because there are no viable treatments available for numerous patients who have no access to clinical trials, drug sponsors might work with the FDA to create a EAP protocol that can be used to enroll multiple qualified individuals. The main difference between this program and the single-person program is that each individual does not have to get their program approved by the FDA. Like the singular patient EAP, intermediate EAP participants often have to cover the cost of the therapy and any related costs.

3. LARGE PATIENT GROUP

Expanded access for widespread treatment use in larger populations. These programs are often used to continue clinical trial participants on investigational drugs until the drug is approved by the FDA. These EAPs are often used for diseases that impact a large number of people.

What’s important to note here is that these EAPs are not clinical trials, which are meant to determine if a new treatment is safe and effective for a group of patients. Instead they are meant to provide access to a drug for people in need. While ACT for ALS EAPs also provide access to unapproved therapies, they differ from most EAPs in that the law requires there to be a research component.

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