What is an orphan drug?
An orphan drug is a drug intended to treat a rare disease or condition, which is defined by affecting fewer than 200,000 individuals in the United States. They are often not cost-effective to produce because of the small patient population.

The Orphan Drug Act
The Orphan Drug Act of 1983 was passed to provide financial incentives to the industry to develop new treatments for rare diseases. The act allows for FDA to approve orphan drugs that have demonstrated effectiveness and safety, even if they do not meet the standard for approval. This is overseen by the Office of Orphan Product Development at the FDA.

Clinical trials can be an exciting option to participate in cutting-edge research for orphan drugs, but it can be confusing to know where to start. Below are some resources for trusted places to look for trials.

FINDING CLINICAL TRIALS

Why Participate in Clinical Trials?
Clinical trials are an integral part of developing new and effective drugs. They can also help contribute to research to help doctors and researchers better understand the disease and improve future therapies and drugs.

Time and Mental Commitment
Participating in a clinical trial may mean frequent trips to hospitals, complex dosing schedules, or invasive procedures such as blood draws. It can take up time and mental energy, and you may have to withdraw at any time.

Privacy and Safety
Informed consent is a very important part of clinical trials. It is a legal document that describes the purpose of the study, including the risks, benefits, and procedures. Informed consent can be withdrawn at any time. Information about privacy and safety should be disclosed in this informed consent. The research coordinators should be able to answer any questions and provide information about the patient or the patient's results may be shared in publications, with other researchers, or databases. In rare disease populations, this information should be thoroughly protected to prevent disclosure.

Clinical trial, especially for rare diseases therapies, can present its own unique opportunities and challenges. Here are some considerations:

CONSIDERATIONS

Not all clinical trials are the same
Clinical trials are an integral part of drug safety and effectiveness. The main goal is to compare several groups at random, participants may receive a placebo or the treatment. This may not be desirable for patients who are looking to receive a therapy in the trial. Alternative trial designs may exist that allows all recruited patients to receive the new therapy, but it is often difficult to determine if the results may be biased.

Trial Design Matters
Well-controlled trials are generally completed on healthy volunteers. Assesses the safety to look for any side effects. Generally completed on smaller samples, test on larger samples, and effectiveness. Seeks further safety and efficacy. Phase I trials are the smallest and least risky. Phase II trials are the second phase and are designed to test the drug on a larger group of patients to determine the dose and schedule. Phase III trials are the largest and most complex. Phase IV trials are conducted after the drug has been approved to evaluate long-term effects.

Phase I
A small group of healthy volunteers is involved. The drug is tested to assess safety and determine the appropriate dose.

Phase II
A group of volunteers with the disease is involved. The drug is tested to assess its potential effectiveness and side effects.

Phase III
A large group of volunteers with the disease is involved. The drug is tested to compare its effectiveness and side effects with other treatments.

Phase IV
After the drug has been approved, it is tested on a larger group of patients to evaluate long-term effects.

SOURCES

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