CLINICAL TRIALS & ORPHAN DRUGS

An overview of clinical trials and orphan drugs in the context of the rare disease world

ORPHAN DRUGS

What is an orphan drug?

An **orphan drug** is a drug intended to treat an orphan disease, which is defined to be a rare disease affecting less than 200k individuals in the United States. They are often not cost effective to produce.

The Orphan Drug Act

The **Orphan Drug Act ("ODA")** was passed in 1983 to provide financial incentives to the industry to develop orphan drugs. This is overseen by the FDA's **Office of Orphan Product Development ("OOPD")**. To date, they claim to have 44 FDA approved orphan drugs that were financed through the ODA and OOPD's financial grant program .

CLINICAL TRIALS

Clinical trials are an integral part of drug safety and effectiveness

A **clinical trial** is a study conducted to test the effects of a drug or treatment for a disease. The main goal is to compare several groups and several treatments to determine the efficiency and side effects.

Clinical trials are often lengthy processes and are broken down into multiple phases.

Phase I
Assesses the safety to test on larger samples, generally completed on healthy volunteers.

Phase II
Seeks further safety data, and looks for a biological effect, or proof that the drug is causing an effect.

Phase III
Well-controlled trials that look to provide credible and strong evidence about the desired effect and treatment.

Phase IV
Safety surveillance trials after the drug has been released to the market.

Why Participate in Clinical Trials?

- Clinical trials for orphan drugs may offer rare disease patients the opportunity to receive a new treatment and have additional care from the clinical research team.
- They can also help contribute to research to help doctors and researchers better understand the disease and improve future therapies and drugs.

CONSIDERATIONS

Not all clinical trials are the same

Clinical trial, especially for rare diseases therapies and orphan drugs, can present its own unique opportunities and challenges. Here are some things to think about when considering a clinical trial for an orphan drug:

Trial Design Matters

The gold standard for a clinical trial is a **randomized controlled trial** - this means 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.

Privacy and Safety

While rare, clinical trials may involve risks or harms. In addition, certain 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.

When participating in a clinical trial, patients must provide **informed consent.** Information about privacy and safety should be disclosed in this informed consent. The research coordinators should be able to answer any questions about this consent. Informed consent can be withdrawn at anytime.

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 both **time and mental energy**. In addition, it may also take more time to read and understand the study and informed consent.

Duration for the trial may also vary. Some may require extensive follow-up for long periods of time.

FINDING CLINICAL TRIALS

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.

RGPRS
Our very own RG Patient Resources Services recommends clinical trials whenever possible

Orphanet
Based in Europe, it offers a global database to look up clinical trials for rare diseases

CenterWatch
Centerwatch is a database of domestic and international clinical trials

ClinicalTrials.gov
Clinicaltrials.gov is an NIH database of publicly and privately funded clinical trials in the US

SOURCES

https://www.fda.gov/industry/developing-products-rare-diseases-conditions/about-orphan-products-clinical-trial-grants
https://www.orpha.net/consor/cgi-bin/Education_AboutOrphanDrugs.php?lng=EN&stacage=ST_EDUCATION_EDUCATION_ABOUTORPHANDRUGS_CLINICALTRIALS
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https://pubmed.ncbi.nlm.nih.gov/24014509/
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