Clinical Trials 101: Understanding the Basics

Educational Resource

What is a clinical trial?

A clinical trial is a **research study** where a person receives an intervention or drug to evaluate the effects of that intervention on a health-related outcome. Clinical trials are required to produce new treatments and medications to prove that a new drug has benefits and acceptable side effects or risks.

How are participants selected for a clinical trial?

Each clinical trial will have inclusion and exclusion criteria for participants. For example, a clinical trial may only be available to people who are under age 18 without a history of seizures. Beyond inclusion and exclusion criteria, there may requirements related to travel to or distances from the site. Ultimately, the decision about whether a person can participate is made by the physician leading the study, the principal investigator (PI).

How do I find and enroll in clinical trials?

How to **find** a trial:

- **Talk** to your Angelman syndrome healthcare team
- **Search** Clinicaltrials.gov
- **Subscribe** to email communications from the Angelman Syndrome Foundation (ASF) and the Foundation for Angelman Syndrome Therapeutics (FAST)

How to **enroll** in a trial:

- Once a trial is published on Clinicaltrials.gov, there will be contact information for the company sponsoring the trial or the trial site.
- Reach out to the provided contact (company or trial site) to ask about eligibility and to learn more about the study.

How do results of clinical trials contribute to the development of new treatments?

Clinical trials collect data throughout the study to better understand the impact and risks of the intervention. This data will be reviewed by the company sponsoring the trial and sometimes an independent monitoring board. If the study drug or intervention shows **improvement on the outcomes** studied and has **low or acceptable risks,** the intervention will continue to move through clinical trial phases leading to consideration of FDA (Food and Drug Administration) or EMA (European Medicines Agency) approval.

Where can I learn more about clinical trials?

- NIH Clinical Research Trials and You
- Clinicaltrials.gov
- ASF - Clinical Trials
- FAST - Clinical Trials
- FDA Basics about Clinical Trials