Evaluating the Clinical Trial Option

UNDERSTANDING CLINICAL TRIAL TERMS

INTRODUCTION

A clinical trial is a medical term for a controlled experiment designed to address questions about a condition or disease. It is research or an investigation that explores whether a medical strategy, treatment, or device is **safe** and **effective** (has positive benefits) for humans. The trial is sometimes called a clinical investigation or a clinical study. For another definition of a trial or study visit:

- https://www.clinicaltrials.gov/ct2/about-studies/learn

Terms related to clinical trials are fairly standard across trials, but you should always discuss with medical providers or research coordinators how each term relates to the study you are considering for your child.

You may also encounter terms that are not included on this list. For additional lists and definitions visit:

- https://clinicaltrials.gov/ct2/about-studies/glossary

Clinical Trial Term Glossary

TERM	DEFINITION
Adverse Event (AE)	A negative reaction, side effect or other sign of illness occurring during a clinical trial. Adverse events can range from mild to serious. A Serious Adverse Event (SAE) is one that causes temporary or permanent disability and may result in hospitalization or death. It may not be clear if any adverse event is caused by the treatment itself, so the research team records all AEs regardless of their opinion about whether or not it is related to the treatment.
Arm (see cohort)	Any of the groups in a randomized trial. Most randomized trials have two "arms," but some have three "arms," or even more.

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TERM	DEFINITION
Assent	A child's agreement to participate. Assent is needed in addition to the consent of the parent or guardian when the child is cognitively able to understand the risk/benefit of participating in a trial. It may also be needed for each procedure during a trial.
Baseline	Measurable information gathered from each participant at the start of the trial and used as a point of comparison after treatment or intervention.
Clinical Benefit	Clinical benefit is a favorable effect on a meaningful aspect of how a patient feels (e.g., symptom relief), functions (e.g., improved mobility) or survival as a result of treatment. Clinical benefit may be measured as an improvement or delay in the progression of a disease or condition.
Cohort (see arm)	A group of patients who all receive the same treatment or intervention. For example, in dose-finding trials, a different cohort or group is used at each dose level.
Comorbidity	The presence of two chronic conditions or diseases in a patient.
Confidentiality	The practice of maintaining the private information of all participants including their identity and medical information.
Data & Safety Monitoring Board (DSMB)	Independent group of research experts established by the sponsor who ensure the study is being done safely and meeting performance endpoints. They may recommend the sponsor modify or stop the trial at any point.
Dose Escalation Study	A trial in which a progressive increase in the strength (dose level) of a treatment (e.g., a drug) is given to a group of patients (cohort) to determine its tolerability and maximize its effect.
Effective Dose	The level at which a drug has the ability to produce beneficial or positive effects on the course of a disease.
Efficacy	The performance of an intervention or treatment under ideal and controlled circumstances such as in a clinical trial. Think of it as the ability to get a job done satisfactorily and result in sufficient beneficial effects. It is often interchanged with effectiveness, which is performance in a "real world" setting. OR how well is the treatment working.
Eligibility Criteria (see inclusion & exclusion criteria)	The list of characteristics, things or factors that a participant must meet or be able to do to be in a trial. They are set before the trial begins. They help minimize risks and maximize success of the trial by including participants who are less likely to have complications due to the treatment or intervention. They are sometimes referred to as inclusion and exclusion criteria.

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TERM	DEFINITION
Endpoint	An event or outcome that can be measured objectively to determine whether the intervention/treatment being studied is beneficial. Endpoints are determined before the study begins and are included in the study protocol or design. Some examples of endpoints are survival, improvements in quality of life, relief of symptoms.
Enrollment	The process of registering or entering a patient into a clinical trial. Clinical trials usually enroll a set number of participants.
Exclusion Criteria (see eligibility criteria)	These are the characteristics that if possessed by a patient prevents them from participating in the trial. Or the things a person must NOT have if they are to participate in the trial. These are different for every trial and can be based on things such as age, type or stage of disease, previous treatment history or other medical conditions. They are used to identify appropriate participants for a safe trial.
First-In-Human Study	A trial where the treatment/intervention has previously only been tested in a lab or in animals, not in humans, and is now testing in humans for the first time.
Inclusion Criteria (see eligibility criteria)	These are the reasons why a person CAN participate in a trial or the characteristics or standards that a patient must meet in order to participate. These are different for every trial and can be based on things such as age, type or stage of disease, previous treatment history or other medical conditions. They are used to identify appropriate participants for a trial.
Informed Consent (see Inform Consent Guide for more information)	An agreement the participant or guardian signs when he/she agrees to take part in a clinical trial. It should include details such as the trial's objectives, potential benefits, risks and side effects, time commitment required and alternative therapies available.
Intervention	The treatment, drug or procedure that is being studied in a clinical trial.
Institutional Review Board	An independent committee responsible for reviewing the clinical study protocol to protect the rights of the participants and to ensure it is done is a safe and ethical manner. They monitor the progress of a study and must review it at least once a year.
Investigator	The person responsible for conducting the clinical trial. If a team of researchers and doctors is involved, this person may be referred to as the Principal Investigator (PI). It is most often a doctor.
Observational Study	Non-interventional study where no intervention/treatment is given such as in a Natural History Study.
Open Label Study	A trial in which the patient, physician and study coordinator are informed of the drug/dose being administered and none of the participants are given placebos.

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TERM	DEFINITION
Outcome Measure	An event, experience, or result described in the trial protocol that is used to determine the effect of the intervention/treatment. It is a measurement to show success or benefits of the procedure.
Patient Reported Outcome (PRO)	A health outcome directly reported by the patient (or family caregiver if patient is not able to speak for themselves) who experienced it and not by a clinician who observed or measured it.
Participant/Subject	The patient enrolled in the trial/study.
Phase	The categories that each clinical trial or study fall into based on properties being studied and the number of participants involved. There are five phases: Early Phase I (formerly listed as Phase 0), Phase I, Phase II, Phase III, and Phase IV. Sometimes the phases are combined (e.g. a Phase I/II Trial). For a greater explanation of phases, watch this video • https://youtu.be/kst1Msx9n0I
Pivotal Study	A trial whose purpose is to provide evidence for approval by the FDA or other regulatory agency. It is typically a Phase III clinical trial intended to demonstrate and confirm the safety and efficacy of a treatment.
Placebo	An intervention that looks like the one being tested and is delivered in the same way but that does not contain any active ingredient.
Protocol	The written description of the trial/study that defines who meets the requirements to participate. It includes objectives, design, methods, and relevant statistical and background information. Key information included is: • Number of participants • Who is eligible or inclusion criteria • Tests that will be given and how often • Types of data collected • The trial/study length • Treatment plan
Recruitment	Active efforts by investigators to identify possible patients/participants for the trial or study based on inclusion/exclusion criteria.
Relatedness of an Adverse Effect (AE)	When an AE happens in a clinical trial, the investigators record their opinion about whether or not the AE was caused by the new treatment. This is called "relatedness." It is often not clear if a particular effect is a side effect of the treatment until the trials are completed.

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TERM	DEFINITION
Research or Study Coordinator	Working under the Principal Investigator, the Research Coordinator supports, facilitates and coordinates the daily clinical trial activities.
Safety	In a clinical trial, this refers to the absence of harmful side effects as a result of the treatment/intervention.
Screening	The time after recruitment when potential participants are evaluated to determine if they meet the inclusion criteria.
Sponsor	The individual, company, institution or organization that oversees the study and takes responsibility for the operations, management and financing of the trial/study.
Standard of Care (SOC)	The most widely accepted treatment for a condition.
Termination	The end of a study/trial by the sponsor or by the withdrawal by a regulatory agency before planned completion.
Therapeutic Window	The difference between the minimum and maximum doses that may be given subjects to obtain an adequate clinical response and avoid intolerable toxic effects.
Toxicity	A safety related adverse/negative effect produced by a drug that has a negative impact on the participant's health.

CONCLUSION

Every medical intervention has its own language, and clinical trial is no different. Use this Guide to help your family navigate common terms. And if there is something you don't understand, you should never hesitate to ask the medical team.