NORD®
National Organization for Rare Disorders

Clinical Trials Glossary

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Glossary

A

Adverse event: An unfavorable change in the health of a participant, including abnormal laboratory findings, which happens during a clinical study or within a certain amount of time after the study has ended. This change may or may not be caused by the intervention/treatment being studied.[1]

Arm: A group or subgroup of participants in a clinical trial who receive the same treatment or intervention (or no treatment or intervention. Different arms are compared to see how treatments perform.

B

Baseline characteristics: Information collected about participants at the start of a study, such as sex, disease stage, or other health details. This information is used to understand who is in the study and to compare groups.

Blinded Study/Double Blinded Study: A study designed so that one or more groups of participants are receiving the treatment or a placebo. In a single-blind study, participants do not know. In a double-blind study, both participants and researchers do not know, which helps ensure more objective and reliable results.

C

Clinical Outcomes: Measurable changes in symptoms or overall health that result from administering care to patients.

Clinical Research: Research done in clinical trials, testing whether a drug is safe and effective when given to humans.

Clinical Trial: Research to determine how well an investigational therapy works compared to existing standard of care, whether it is safe and effective, and whether the benefits outweigh the risks.

Control Group: A group of people in a clinical trial who do not receive the therapy being tested.

E

Eligibility criteria: The requirements a person must meet to join a clinical study. These include inclusion criteria (who can participate) such as age range or type of disease, and exclusion criteria (who cannot participate), such as health status or location.

Endpoint: An event or outcome that can be measured to determine whether the intervention being studied is beneficial.

I

Indication: A medical condition for which a medicine is used.

Informed Consent: A process in which participants are given information about why the study is being done, exactly what someone participating in the study needs to do, and what the risks and benefits of the study are.

Investigational New Drug (IND) application: An application submitted to the U.S. Food and Drug Administration (FDA) to begin testing a new drug in people This is one step in the move from pre-clinical to clinical research. The application includes data from pre-clinical research, how the drug is manufactured, and the plan for how the study will be conducted safely and ethically.

[1] ICH E6(R2/RD) Good Clinical Practice guidelines and 21 CFR 312.32 FDA regulations for IND safety reporting

Investigational therapy: A drug, biologic, or medical device that is in development and undergoing testing but not yet approved by the FDA.

N

Natural history study: A study that collects information on how a disease would develop or progress over time without intervention.

New Drug Application (NDA): An application submitted to the FDA to review and approve a new drug for use in the U.S. The application includes results from clinical trials and other information to show the drug is safe, effective, and ready to be made and distributed.

O

Observational study: A type of clinical study where researchers observe and collect information about participants' health over time, without assigning a treatment. Participants receive care as they normally would. A patient registry is a type of observational study.[2]

P

Patient-reported outcome (PRO): A measurement based on a report that comes directly from the patient about the status of their health – such as symptoms, daily functioning, or quality of life – without interpretation by a clinician or anyone else.

Phase 1: After experiments are completed in a lab, a small group of healthy volunteers receives the treatment to make sure it is safe and to find the right dose.

Phase 2: With safety information from the first phase, the treatment is then given to a small group of people with the condition to continue checking safety and to see if the treatment works.

Phase 3: Once researchers collect the data from Phases 1 and 2, the treatment is studied in a larger and more diverse group of people to confirm it works and to better understand side effects. In some cases, the treatment will be tested in combination with other treatments. If the results are positive, the treatment may be reviewed by the FDA for possible approval.

Phase 4: Once a treatment is approved and available, the treatment continues to be studied to track long-term safety and see how well it works in everyday, real-world use.

Placebo: A treatment that looks, feels, and tastes the same as the drug being studied but does not contain the actual active drug.

Placebo-controlled trial: A clinical trial in which one group of participants receives the treatment being studied and one group of participants receives a placebo.

Pre-clinical research: Research done in a lab, testing whether a drug is safe and effective when used in cell models and/or animals.

Priority Review: A designation under which the FDA agrees to act on a drug sponsor's application within six months, rather than the ten months required under standard review.

R

Randomization: The process of assigning participants in clinical trials to separate groups by chance, so each group receives a different treatment. Neither the researcher nor the participant chooses which treatment is given.

S

Sponsor: The person, company, or organization responsible for starting, managing, and funding a clinical study. Sponsors are often pharmaceutical or biotechnology companies, but they can also be universities, hospitals, or nonprofit organizations.

Study protocol: A document that outlines how a study will be conducted and how risks to participants will be minimized.

[2] ClinicalTrials.gov