CLINICAL TRIAL TERMINOLOGY

A

**Adverse effect:** An unwanted side effect of treatment.

**Adverse event (also known as “AE”):** An unexpected medical problem that happens during treatment with a drug or other therapy. Adverse events do not have to be caused by the drug or therapy. AEs may be mild, moderate, or severe.

**Adverse reaction:** An unwanted effect caused by the administration of drugs; onset may be sudden or develop over time.

**Arm:** A group or subgroup of participants in a clinical trial.

B

**Blinded trial:** A trial in which patient participants do not know if they are in the experimental or control arm of a study (known as a “single blinded” trial), or in which neither the patient participants nor the study doctors know to what arm patients have been assigned (a “double blinded” trial). The opposite of a blinded study is an open label study. (See also Double-Blind Study.)

C

**Clinical investigator:** See Investigator.

**Clinical trial (also known as “clinical study,” “medical research study,” or “research study”):** Clinical trials are conducted to determine whether investigational drugs are safe and effective. Carefully conducted clinical trials are the fastest and safest way to find potential future treatments that work in people. Investigational drugs go through several phases of clinical trials:

- **Preclinical (Phase 0) trials** are conducted in a laboratory setting using computer models and/or animals or cell-lines to gather evidence that justifies a clinical trial.
- **Phase I trials** are usually conducted with healthy volunteers and emphasize safety. Phase I trials seek to identify the most frequent and serious adverse events of an investigational drug.
- **Phase II trials** gather preliminary information on whether (or not) an investigational drugs works in a group of people who have a particular disease or condition. Phase II trials may use an inactive placebo that allows trial doctors to determine the effectiveness of the investigational study drug.
- **Phase III trials** are conducted to confirm the drug or treatment’s effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the drug or treatment to be used safely. The drug or treatment is given to large groups of people to validate the results.
- **Phase IV trials** are conducted after a drug or treatment has been approved by regulatory agencies (e.g., FDA in the US; EMA in Europe). Phase IV studies are often required by regulatory agencies to gather more information about a drug’s safety and effectiveness.
Control group: In a clinical trial, the group that does not receive the investigational drug being studied. This group is compared to the group that receives the experimental drug to see if it works. Sometimes the control group takes a standard, approved treatment so that the investigational drug can be compared to a current standard of treatment; at other times, particularly when there is no standard treatment, a control group may be given an inactive placebo so researchers can learn if the study drug is having any effect not seen in those people who are taking the inactive placebo.

D
Data and Safety Monitoring Board (also known as Independent Data Monitoring Committee or DSMB): An independent committee composed of community representatives and clinical research experts whose members review data while a clinical trial is in progress to ensure that participants are not exposed to unnecessary risk. A DSMB may recommend that a trial be stopped if there are safety concerns or if the trial objectives have been achieved.

Dose: The amount of medicine taken, or radiation given, at one time.

Double-blind study: A clinical trial design in which neither the trial participants nor the study staff knows which participants are receiving the experimental drug and which are receiving a placebo (or another therapy). Double-blind trials are thought to produce objective results, since the expectations of the doctor and the participant about the experimental drug do not affect the outcome. (See also blinded trial.)

E
Efficacy: The ability of a drug or treatment to produce a beneficial effect.

Eligibility criteria (also known as inclusion/exclusion criteria): Requirements that must be met for an individual to be included in a study. These requirements help make sure that patients in a trial are similar to each other in terms of specific factors such as age, general health, and previous treatment. When all participants meet the same eligibility criteria, it gives researchers greater confidence that results of the study are caused by the intervention being tested and not by other factors. Inclusion and exclusion criteria are not used to reject people personally, but to identify appropriate participants and keep them safe.

Endpoint: A clinical measurement that can be evaluated to determine whether the experimental drug is effective (beneficial). Some examples of endpoints are survival, quality of life improvement, symptom relief, and diminishment or disappearance of a tumor. Endpoints are determined before a study begins. (See also primary endpoint.)

Enrollment: The act of securing participants for a study. Enrollment includes evaluating a participant with respect to the eligibility criteria and reviewing an informed consent document.

Exclusion criteria: Reasons that prevent a person from participating in a trial. (See also eligibility criteria.)
**Experimental compound or agent** (also known as **investigational agent or drug**): A substance that has been tested in a laboratory and has received approval from a regulatory agency (the Food and Drug Administration in the US; the European Medicine Agency in Europe) to be tested in people. A drug may be approved by a regulatory agency for use in one disease but be considered experimental or investigational in other diseases.

**F**

**Follow-up:** The monitoring of a clinical trial participant’s health after the treatment phase has ended.

**G**

**Good clinical practice:** A standard for the design, conduct, performance, monitoring, auditing, recording, analysis, and reporting of clinical trials that provides assurance that the data and reported results are credible and accurate, and that the rights, integrity, and confidentiality of trial subjects are protected.

**I**

**Inclusion criteria:** See **Eligibility criteria**.

**Informed consent:** A process during which a person is given important facts about a medical procedure or treatment, a clinical trial, or genetic testing before deciding whether or not to participate. Informed consent also includes advising a patient or participant when there is new information that may affect his or her decision to continue.

**Informed consent document** (or **form**): Informed consent documents include information about the possible risks, benefits, and limits of a procedure, treatment, trial, or genetic testing. They also describe the rights of the study participants and include details about the study, such as its purpose, duration, required procedures, and key contacts. Every prospective clinical trial participant then decides whether or not to sign the document and to participate in the trial. Informed consent is not a contract, and the participant may withdraw from the trial at any time.

**Investigational agent:** See **experimental agent**.

**Investigator:** A medical researcher responsible for the conduct of a clinical trial at a trial site.

**P**

**Phase I, II, III, of IV trials:** See **Clinical trial**.

**Placebo:** An inactive pill, liquid, or powder that has no treatment value. In clinical trials, an experimental drug is often compared to a placebo to assess the experimental drug’s effectiveness. In cancer clinical trials, a placebo would be given together with standard of care treatment and compared to an experimental drug.
**Primary endpoint:** The main result that is measured at the end of a study to see if a given study treatment worked (e.g., the number of deaths or the difference in survival between the treatment group and the control group). The primary endpoint is decided before the study begins. (See also endpoint.)

**Principal Investigator:** The lead research doctor at each clinical trial site, who oversees other health care professionals who help run the trial.

**Protocol:** A written description of a clinical trial that describes the background and rationale for a trial, what types of people may participate; the schedule of tests, procedures, medications, and dosages; and the length of the study.

**Randomized clinical trial:** A study in which the participants are assigned by chance (i.e., randomly) to separate groups that compare different study treatments. Neither the researchers nor the participants can choose which group. Randomization gives each participant an equal chance of being assigned to any of the groups. Using chance to assign people to groups means that the composition of study groups is objective rather than subjective so that the study treatments they receive can be compared objectively and to reduce bias.

**Recruitment:** The period during which a trial is attempting to identify and enroll participants.

**Side effect:** Any undesired actions or effects of a drug or treatment. These may include headache, nausea, hair loss, skin irritation, or other problems. Experimental drugs must be evaluated for both immediate and long-term side effects.

**Sponsor:** An individual, company, institution, or organization that begins a clinical trial and takes responsibility for the initiation, management, and/or financing of that trial, but who does not actually conduct the clinical trial.

**Standard treatment** (also known as **standard of care**): A treatment currently in wide use and approved by the FDA, considered to be effective in the treatment of a specific disease or condition.

**Trial site:** A location where a clinical trial is being conducted.