**Adverse drug reaction (ADR):** A negative experience that a participant has during the course of a research study that is determined to be caused by the study drug.

**Adverse event (AE):** Any negative experience that a participant has during the course of a research study. An adverse event may occur suddenly or develop over time. Although undesirable, an adverse event is not always unexpected. If it is determined that the adverse event is caused by the study drug, it is called an adverse drug reaction (ADR).

**Arm:** A group in a randomized trial that receives a particular treatment. Most randomized trials have two arms, but some have three or more arms.

**Approved drug:** A drug that has been approved for marketing in the United States by the Food and Drug Administration (FDA).

**Autonomy:** A person’s capacity to consider alternatives, make choices and act without undue influence or interference from others.

**Benefit:** A desired outcome or advantage.

**Biologic:** A virus, serum, toxin, or similar product used to prevent, treat or cure diseases in humans.

**Blinding:** When the participant and/or the investigator do not know which treatment the participant is receiving as part of the study. In a single-blind study, the participants do not know which treatment they are receiving. In a double-blind study, neither the participants nor the investigator knows which treatment the participant is receiving. Also called “masking.”

**Clinical research coordinator (CRC):** A person who assists the investigator with the daily operations of a study. Other terms for a clinical research coordinator are study coordinator, research nurse, protocol nurse, or data manager.

**Clinical trial:** A research study designed to test the safety and/or effectiveness of drugs, devices, or treatments in humans. Clinical trials can usually be divided into four categories or “phases.”

**Compassionate use:** Providing an experimental treatment to humans before the treatment has received final Food and Drug Administration (FDA) approval. Compassionate use is normally used with very sick individuals who have no other treatment options. In general, case-by-case approval must be obtained from the Food and Drug Administration (FDA) for compassionate use of a drug or other therapy.
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**Consent:** See Informed consent.

**Control group:** A group of study participants who are not treated with the experimental drug, device or treatment. Participants in a control group may receive no therapy, a different therapy, standard therapy, or a placebo.

**Data and Safety Monitoring Board (DSMB):** A group of experts who are not connected to a study that meet periodically and review the research data to ensure the safety of the study participants. Also call a Data and Safety Monitoring Committee (DSMC).

**Device:** A health care product that does not achieve its intended purpose through chemical action or metabolism in the body. Examples of devices are wheelchairs, sutures, orthopedic pins, and crutches.

**Dose-ranging study:** A clinical trial in which two or more doses of a substance are tested against each other in order to determine which dose works best and is least harmful.

**Double-blind study:** A study in which neither the participant nor the investigator knows which treatment the participant is receiving. Also called a “double-masked” study.

**Double-masked study:** See double-blind study.

**Drug:** According to the Food, Drug and Cosmetic Act, drugs are "articles (other than food) intended for the use in the diagnosis, cure, mitigation, treatment, or prevention of disease in man or other animals, or to affect the structure or any function of the body of man or other animals."

**DSMB:** See Data and Safety Monitoring Board.

**DSMC:** See Data and Safety Monitoring Board.

**Efficacy:** An agent’s ability to produce beneficial effects on the duration or course of a disease.

**Eligibility criteria:** Conditions that a person must meet in order to qualify for participation in a study. Eligibility criteria include inclusion criteria and exclusion criteria.

**Ethics Committee:** See Institutional Review Board (IRB).

**Exclusion criteria:** Characteristics that would prevent someone from being eligible to participate in a research study. Exclusion criteria are defined in the study protocol.
**Experimental drug:** A drug that is not approved by the **Food and Drug Administration (FDA)** for use in humans, or for use as a treatment for a particular condition.

**Experimental group:** The group of participants that receive the drug, device, treatment, or intervention that is being studied. In some studies, all participants are in the experimental group. In other studies, called "controlled studies," some participants are assigned to either an experimental group or to a control group.

**FDA:** See **Food and Drug Administration (FDA).**

**Food and Drug Administration (FDA):** The U.S. federal agency that is responsible for enforcing the laws on testing, manufacturing, and using drugs and medical devices. The FDA must approve drugs and devices before they are made available to the public.

**Food, Drug and Cosmetic Act (FD&C Act):** The federal law that states drugs, devices, and biologics must be proven safe and effective before they can be marketed.

**Good Clinical Practices (GCPs):** International ethical and scientific quality standards for the design, conduct, monitoring, recording, auditing, analysis, and reporting of studies. Ensures that the data reported are credible and accurate, and that subjects’ rights and confidentiality are protected.

**Health Insurance Portability and Accountability Act (HIPAA):** A federal law that was passed in 1996. This law includes a privacy rule that creates national standards to protect personal health information.

**Healthy volunteer:** A person who participates in a research study and does not have the specific disease or disorder that is the focus of that study.

**Human subject:** See participant.

**Inclusion criteria:** A list of requirements that a person must meet to be eligible to participate in a research study. Inclusion criteria are defined in the study protocol.

**IND:** See Investigational New Drug (IND).

**Informed consent:** The process of learning the important facts about a study before deciding whether or not to participate. Informed consent begins with a discussion between the researcher and the potential participant. This discussion includes important information about the research study such as the purpose of the study, the procedures involved, and the risks and benefits of participating.
Based on this discussion with the researcher, participants are asked to sign an informed consent document that outlines this important information. Informed consent is an ongoing process.

**Informed consent document:** A written document that outlines the rights of a research participant and important information about a study. Potential participants are asked to sign an informed consent document after discussing the study with a member of the research team and before participation in the study can begin. The informed consent document is not a legal contract.

**Institutional Review Board (IRB):** An independent group of medical and non-medical persons who review research studies to ensure that the rights, safety and welfare of human participants are protected. The IRB is also responsible to ensure that the study complies with the institution’s policies and federal regulations. All studies that involve humans must be approved by an IRB before they can begin. Also called an ethics committee.

**Intervention:** A physical procedure used to gather research data (such as a blood test). The term intervention also refers to manipulating a participant or the participant’s surroundings for purposes of research.

**Investigator:** A researcher who is responsible for conducting a study. The researcher with overall responsibility for the study is called the principal investigator. Other researchers on the study are called co-investigators or sub-investigators.

**Investigational New Drug (IND):** A drug that the Food and Drug Administration (FDA) has approved for testing in humans, but has not approved for marketing.

**Investigational New Drug (IND) Application:** The application submitted to the Food and Drug Administration (FDA) requesting permission to test a drug in humans.

**Investigational device:** A medical device that has not yet been approved for marketing by the Food and Drug Administration (FDA).

**IRB:** See Institutional Review Board.

**Masking:** See blinding.

**National Institutes of Health (NIH):** An agency within the U.S. Department of Health and Human Services that conducts and provides funding for research.

**New Drug Application (NDA):** An application that is submitted to the Food and Drug Administration after clinical trials have been completed to request a license to market the drug for a specific purpose.
Open-label study: A study in which the participant, investigator, and research staff are aware of the treatment assigned. In an open-label study, no participants are given placebos. These are usually Phase I or Phase II studies.

Participant: A patient or healthy volunteer who is taking part in a research study.

Patient: A person seeking medical care.

Phase: The term “phase” refers to one of the four stages of testing that a drug must undergo before it can be approved by the Food and Drug Administration (FDA) for marketing. The four phases must be completed in order, and positive results must be obtained before moving to the next phase.

Phase I: The first of four stages of clinical trials in humans. In Phase I studies, the experimental drug is usually given to a small group of healthy volunteers. The purpose of a phase I study is to gather information on safety.

Phase II: Usually the second stage of testing new drugs in humans. A Phase II trial involves a larger number of participants, and the participants usually have the disease for which the experimental drug is being tested. The purpose of this phase is to gather additional information on safety and to see how effective the drug is.

Phase III: The third stage of testing involves large numbers of persons that have the disease for which the experimental drug is being tested. This stage of testing usually compares the experimental drug to the current approved treatment for the disease. An application to approve the experimental drug can be submitted after this stage of testing.

Phase IV: This stage is performed after the drug has been approved by the Food and Drug Administration (FDA). In this stage, studies are done to compare the drug to a competitor, examine the long-term effects of the drug, or to look at the drug in new types of patients.

Pharmacokinetics: The way a drug is absorbed, distributed, metabolized, and excreted in a living organism.

Placebo: An inactive substance that has no treatment value. In a blinded study, it is designed to look like the drug being tested. Although it looks like the active drug, it contains no medicine. Sometimes referred to as a "sugar pill."

Placebo controlled study: A study in which an inactive substance (placebo) is given to one group of participants and the active study drug being tested is given
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to another group. The results obtained in the two groups are compared to see if the study drug is more effective in treating the condition.

**Preclinical research:** Research that tests the safety of a drug in the laboratory or in animals. Preclinical research must occur before a drug can be tested in humans.

**Prevention trials:** Studies that try to find better ways to prevent disease. Examples of prevention methods are medications, vaccines, dietary changes, or lifestyle changes.

**Principal investigator:** The person with overall responsibility for the conduct of a research study.

**Protection of Pupil Rights Amendment (PPRA):** A U.S. Department of Education regulation that requires that surveys, questionnaires and instructional materials for school children be inspected by their parents/guardians.

**Protocol:** A written, detailed plan that describes the goal, design and methods of a research study.

**Randomization:** A method of assigning study participants to a treatment arm by chance so that each participant has an equal chance of being assigned to each arm. Also referred to as random allocation or random assignment.

**Randomized trial:** A study in which participants are assigned to one of two or more treatment arms by chance.

**Recruitment:** The process of enrolling participants into a research study.

**Research:** A systematic investigation designed to develop or contribute to generalizable knowledge.

**Research team:** The group of professionals that are involved in conducting a research study. Teams often consist of a principal investigator, other investigators (called co-investigators or sub-investigators), and clinical research coordinators.

**Risk:** The likelihood of harm or injury occurring as a result of participating in a research study.

**Risk-benefit ratio:** The risks associated with participation in a research study versus the potential benefits of the study.

**Screening trials:** Studies that test the best way to detect specific diseases or health conditions.
**Single-blind study:** See **blinding**.

**Single-masked study:** See **blinding**.

**Sponsor:** The individual, company, institution, or organization that takes responsibility for starting, managing, and sometimes funding a research study.

**Standard treatment:** A treatment that is currently approved, accepted, and considered to be effective for the treatment of a specific disease or condition.

**Subject:** See **participant**.

**Treatment Investigational New Drug (IND) application:** A way that the U.S. Food and Drug Administration (FDA) makes promising new drugs available to seriously ill persons as early in the drug development process as possible. Treatment INDs are made available to persons before general marketing begins, typically during Phase III studies. To be considered for a treatment IND a person cannot be eligible to be in the specific clinical trial.

**Treatment trials:** Studies that test new treatments, new combinations of drugs, or new approaches to surgery or radiation therapy.

**Treatment:** A method of caring for or dealing with a medical condition.

**Vulnerable subjects:** Persons who may be unduly influenced to participate in a study. Examples are children, prisoners, mentally challenged individuals, students, impoverished individuals and persons with incurable diseases.