



KEY TERMS: Clinical Trials

Frequently Used Terms

CLINICAL STUDY or CLINICAL TRIAL – Research that involves human participation or the use of human tissues to test safety, accuracy, and effectiveness of various interventions.

INTERVENTION (also called Treatment) – This is what participants will be exposed to and what is being studied in the trial. Interventions can be drugs, surgical techniques, medical devices, procedures, laboratory tests, or other products that are used in the care of patients. Sometimes they include things like new education methods and diet or exercise changes.

INVESTIGATOR (also called Researcher) – A person doing the research. The principal investigator refers to the one in charge of the study.

PARTICIPANT (also called Subject) – A person who agrees to be part of a study.

RESEARCH – A way of gathering information to increase knowledge.

ADVERSE EVENT (AE) – An unexpected change in health of a participant, including abnormal test results, that happens during a clinical study or within a certain period of time after the study has ended that may or may not be caused by the intervention/treatment being studied. These should always be reported to your doctor and/or study coordinator, whether serious (see severe adverse event) or minor so they can track any possible concerns with the review board.

ARM – A specific group of participants that receives a specific intervention according to the trial's protocol. There are both investigational and control arms.

ASSENT – An agreement to participate in a study. It is different than informed consent as it usually does not indicate full understanding of the study. It is often obtained from people under the age of 18 or who are intellectually disadvantaged. Their guardian will usually also need to sign the consent in order to proceed.

BIAS – An abnormal distortion of results that could misrepresent the data outcomes. This could be due to things like flaws in the study design, an analysis based on flawed data, irregularities in how the data was collected, or a patient population that was limited or weighted too heavily in one group or another.



BLINDING (also called Masking) - A strategy used in study design so that either the investigator and/or participants do not know which participants have been assigned which interventions. For example: If two different medications are being studied, blinding would mean all participants are being treated/tested the same way and you do not know which participant is getting the intervention. Types of blinding include open-label blinding, single-blinding, and double-blinding.

CASE CONTROL STUDY – A type of study that does not follow participants over time. Participants are chosen due to a specific outcome (e.g. cancer type, medication use, surgery type, etc.) and data/records/interviews are done to look at the difference in experiences (e.g. number of ultrasounds, side effects, weight changes, etc.). These studies also include the group without the outcome (e.g. no cancer, different cancer/medication/surgery, etc.) to compare the differences chances of having this experience.

CASE REPORT OR CASE SERIES – This usually is simply a report about one particular patient or group of similar patients to show something of interest. No control group is involved, and no special intervention is done beyond usual care.

CLINICAL RESEARCH COORDINATOR (CRC) (also called Study Coordinator) – A person that handles the organization and day-to-day responsibilities of a study and acts as a main study contact for the clinical site/participants.

CLINICAL RESEARCH – Research involving patients or their tissues where an investigator interacts directly with the participants and includes data on that patient. It would not include research done on tissue or other samples that are no longer linked to a living person and his/her medical records.

CLINICAL TRIAL – Research where participants are assigned to one or more interventions to study their effects of medical or behavioral outcomes. The intervention may be drugs, devices, or procedures studied for diagnostic, therapeutic, or prevention and can also include behavioral modifications. Clinical trials are used to determine the safety, effectiveness, and/or accuracy of these interventions.

COHORT- A specific group or subgroup of participants in an observational study.

COMPASSIONATE CARE - See expanded access.

CONFIDENTIALITY – The process of keeping a participant's information private and limiting access to that information to approved persons only.



CONTROL GROUP – A group of participants who share similar characteristics (for example age, cancer type, etc.), but do not receive the intervention. In cancer trials, this group usually receive the current standard-of-care treatment. Changes are measured in both the control group and investigational group to compare the effect of the new drug, medical device, procedure, or prevention technique.

CROSS-SECTIONAL STUDY – A study that gathers data at a particular point in time or over a short time period. The opposite is a longitudinal study.

DATA – A series of recorded observations, measurements, test results or facts about a participant or group of participants.

DATA AND SAFETY MONITORING BOARD (DSMB) – A group of individuals separate from the study investigators to monitor participant safety, data quality and to assess study progress.

DOUBLE-BLINDING – A type of blinding in which the participants and investigators are kept unaware of who is getting which specific intervention.

EFFECTIVENESS (also called Efficacy) – A measure of whether a new intervention works, improves health, limits side effects, and/or prevents disease.

ELIGIBILITY CRITERIA – A list of requirements to be able to participate in or be excluded from a study. These can be listed as inclusion criteria and exclusion criteria.

ENROLLMENT – The process of qualifying and consenting to participate in the study. The "estimated" enrollment is the target number of participants that the researchers need for the study.

EXCLUSION CRITERIA – Type of eligibility criteria that states the reasons a person would not be allowed to participate in a study. Examples could include pregnant women, people under the age of 19, people with certain medical conditions, or people who have been previously treated with certain medications.

EXPANDED ACCESS (also called Compassionate Care) – Special approval by various groups, including the FDA, to gain access to an experimental intervention when they do not qualify for the specific study (they are excluded) and the product is not yet approved for the general public.

EXPERIMENTAL DRUG/DEVICE – see Investigational drug.

EXPERIMENTAL GROUP/ARM – see Investigational group.



FOOD AND DRUG ADMINISTRATION (FDA) - An agency within the U.S. Department of Health and Human Services that is responsible for protecting the public health by making sure that human and veterinary drugs, vaccines and other biological products, medical devices, the Nation's food supply, cosmetics, dietary supplements, and products that give off radiation are safe, effective, and secure. They approve/disapprove new drugs and devices, however not all medical interventions undergo FDA approval at this time.

INCLUSION CRITERIA - A type of eligibility criteria that states the requirements for a person to participate in a study. Examples would include certain ages, cancer types, or participants on certain medications or with specific genetic test results.

INFORMED CONSENT - A process by which potential and enrolled participants are told about the study and potential risks and benefits of participating. Once this is done, and the patient has had all his/her questions answered, their willingness to participate is shown by signing a consent form that has all the study details. This should not be done only at the beginning of a study, but repeatedly throughout to make sure patients understand their rights. Even after a consent is signed, a person can stop participating at any time (withdraw consent) by notifying the clinical study coordinator.

INSTITUTIONAL REVIEW BOARD (IRB): A committee responsible for protecting people involved in research by reviewing and approving each clinical study. They can also recommend a study be stopped if it is not showing to be effective or is showing harm. Sometimes other monitoring groups (Data and Safety Monitoring Board [DSMB]) are used to monitor specific parts of a study.

INTERVENTIONAL GROUP/ARM – see Investigational group.

INTERVENTIONAL STUDY- A type of study in which participants are put into groups that receive one or more interventions so that researchers can evaluate the effects. The other type of study is Observational.

INVESTIGATIONAL GROUP/ARM (also called Experimental or Interventional Group/Arm) - A group of participants the receives the intervention and is the main focus of the study. This group is usually compared to a control group.

INVESTIGATIONAL DRUG, DEVICE, or TREATMENT – A new medical drug, device, or treatment that has been tested in a lab but has not yet been approved by the FDA to be sold/used by doctors or patients that are not a part of a study.

LITERATURE REVIEW (also called Systematic Review) – A type of study that uses studies already published on a particular topic to summarize what has already been done and learned from past research and/or suggest what needs to be done to account for holes in previous studies/data.



LONGITUDINAL STUDY – A study that gathers data over a period of time. The opposite is a cross-sectional study.

MASKING – see Blinding.

META-ANALYSIS – A type of analysis that combines data from multiple other studies. This can be collected from previously done studies only or can include old and new data.

NCT NUMBER – A unique identifying code given to each study registered with the National Institute of Health. It is an 8-digit number (e.g., NCT00000419) that is linked to its listing on www.clinicaltrials.gov.

OBSERVATIONAL STUDY – A type of study in which participants who have certain characteristics are monitored for outcomes. They may receive diagnostic, therapeutic, or other types of interventions, through their usual care, but the study does not control who receives what. Types of observational studies include registries, case-control, case study/series, and cohort studies.

OPEN-LABEL – A study in which investigators and participants know who is getting which intervention.

OUTCOME – Anything that is measured and reported determine the effectiveness of an intervention. It could be a test result, a diagnosis, a measurement of time, rates of side effects, etc. Primary outcomes are the main focus of the study, and there is usually only one. However, there can be multiple other secondary outcomes as well that are being studied. They are not as important as the primary outcome but are still of interest.

REGISTRY – A type of observational study that collects information about patients' medical conditions and/or treatments they receive as a normal part of their care. This data is often used to try and understand how the disease or intervention works in the real-world (not in a study environment) when there are a lot more variety to patients.

PHASE – A word used to describe the stage the research is at in studying a particular intervention. Each phase has its own purpose and size.

PHASE 1 STUDY – A study focused on the safety of an intervention. It expands on knowledge from earlier phases (sometimes called Phase 0 or early Phase 1) that were done in a laboratory or from animal data. It will look at things like the safest maximum level of a certain intervention, how the drug works in the body, and what side effects are most common/possible. This phase is usually done on less than 100 people and can



include people who are healthy. According to the FDA, approximately 70% of phase 1 studies will go on to phase 2.

PHASE 2 STUDY- A study focused on determining the effectiveness and/or accuracy of an intervention (asking “does it provide any benefit”) and look more into possible side effects in a larger group of people (up to a few hundred) with a certain condition. According to the FDA, approximately 33% of phase 2 studies will go on to phase 3.

PHASE 3 STUDY– A study used to compare one intervention to another (usually one already approved to treat patients with that condition – standard of care). It is done on a large group of people (100s to 1000s) and continue to monitor side effects and safety. This phase usually includes randomization and is required to get FDA approval. According to the FDA, approximately 25% of phase 3 studies will move on to phase 4.

PHASE 4 STUDY – A study done after FDA approval on 1000s of patients to monitor the intervention safety and effectiveness in the general public (also called post-market monitoring).

PLACEBO – A substance that that does not include any active ingredients (sometimes called a “sugar pill”). It looks the same as and is given in the same way as the intervention being studied and is given to keep someone from knowing they are not being given the intervention. In cancer studies, placebos are not usually used. The participants are almost always given an active treatment, unless there is no current treatment available for that specific condition.

PROSPECTIVE STUDY – A type of study that starts before the primary outcome has been happened. These often start collecting data on a certain day and continue for a period of time until the outcome can be measured on all participants. **EXAMPLE:** If you want to see what side effects a certain medication causes, a prospective study would enroll patients, give them the medication and see what side effects (the outcome) occurred over time. The opposite of this is retrospective.

PROTECTED HEALTH INFORMATION (PHI) – Any information about a person’s health or medical history. This information is protected by law and is required to be kept private and not shared with unauthorized people.

PROTOCOL – This is a written description of a study including what is being studied, when it will be studied, and how the study will be organized, monitored, and reported. Any changes (called amendments) to that protocol after it is approved by the IRB, must be written out and re-approved.

PROTOCOL DEVIATION – Any time a study does not function the way it is described in the protocol, it is considered a deviation and must be documented. Examples include not getting consent, not reporting



adverse events, performing a study intervention that is not approved or at an unapproved site, and not following the steps of the protocol.

QUALITATIVE STUDIES – Studies that collect data from observations, interviews, or verbal interactions with participants, and reports them in a descriptive way with words.

QUANTITATIVE STUDIES – Studies that use measurements and statistics to report results.

RANDOMIZATION – A process of mixing up participants into the investigational group or control group to help reduce bias by having a mixture of different characteristics (age, gender, tumor size, etc.) in each group. Every participant has an equal chance of being assigned to either group. Although most randomization is carried out by a computer, an example of randomization would be flipping a coin to see what group each participant will fall into.

RANDOMIZED CONTROLLED TRIAL (RCT) – A type of study that randomly assigns participants into the investigational group or control group.

REPRODUCIBILITY – The ability of a study's conclusions to be duplicated by others doing similar research. If data is not reproducible, it suggests there was a flaw to the research or intervention or that there was something unique to the population studied that made it work differently in them. Reproducible results increase the trust in the data.

RECRUITMENT – The process of attracting and identifying people to be part of a study.

RETROSPECTIVE STUDY – A type of study that starts after the primary outcome has occurred. It often involves looking back into a patient's medical records for reasons for that outcome. **EXAMPLE:** If you want to understand why a side effect may happen for a medication, this side effect is the outcome that has already happened. A retrospective study would enroll patients with that side effect and see what was different about their medical history, treatment, or dosage. The opposite of a retrospective study is a prospective study.

SAMPLE SIZE – The number of participants in a study. The larger the sample size, the better conclusions that can be estimated.

SCREENING PROCESS – Deciding if someone fits the eligibility criteria for be in a study.

SERIOUS ADVERSE EVENT (SAE)– This is an adverse event that is life-threatening, requires hospitalization, significantly interferes with normal life functions, or could result in death.



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SIDE EFFECT – Any change in health or life activity that occurs while using an intervention. These are usually unpleasant and can be harmful. Examples include increases in blood pressure, feeling tired or sick to your stomach, trouble-breathing, etc.

SINGLE BLINDING – A type of blinding in which only the participant is kept unaware of who is getting which specific treatment, but at least some of the investigators (the doctor, the sponsor, the clinical research coordinator or some combination of those) know who is who.

SPONSOR – A person, company, or organization that provides the money and resources to support a study.

STUDY COORDINATOR – see Clinical research coordinator.

For one-on-one support, contact the Patient Navigator Program.

Email: patients@kidneycancer.org

Call: 1-800-544-3KCA (1-800-544-3522)

www.kidneycancer.org/patient-navigator-program