Baseline data

Demographic information (e.g., age and gender) and other information such as symptoms, medications or measurements on specific tests that is collected from participants at the beginning of a clinical

trial or study.

Breakthrough therapy designation

A U.S. FDA process that speeds the development and review of new therapies that may treat a serious condition if early clinical trial data indicate that the drug may be more efficacious than available therapies.

See also: efficacy; U.S. Food and Drug Administration

Biomarker

A measurable, biological characteristic that can be used to determine the risk, presence or progression of a disease. For example, high blood pressure is a biomarker of potential heart disease.

Blinding

A clinical trial strategy where the researchers and participants do not know which participants are taking placebo (inactive substance) and which are receiving the intervention. In single blinding, only one group (either researchers or participants) knows

which participants are taking placebo or intervention. In double blinding, neither group knows.

See also: intervention; placebo

Arm

A specific group of study participants within a clinical trial. For example, in an interventional trial, one “arm” may

receive the investigational treatment and another “arm” receives placebo.

See also: interventional trial

Clinical Trial Glossary

Adverse event

An unfavorable change in health that can occur during a clinical trial or study or within a certain time period after. These can range from mild (e.g., nausea) to serious or life-threatening (e.g., stroke). This change may or may not be related to the intervention being studied.

See also: intervention

Digital health

A broad scope of health initiatives that include mobile health (devices to track measures such as physical

activity), health information technology, wearables (body sensors to measure movement, sleep, etc.), telemedicine and online studies.

See also: telemedicine; virtual study; wearable

Eligibility criteria

Guidelines for who can and cannot participate in a specific clinical trial. Criteria are comprised of certain characteristics, such as age, gender, time since diagnosis, stage of disease and other medical conditions. Eligibility criteria include both inclusion and exclusion criteria.

See also: exclusion criteria; inclusion criteria

Efficacy

A measure of a drug’s ability to treat a certain condition; efficacy does not reflect tolerability or ease of use. A drug may be very efficacious but be so unpleasant to take that its actual

use is very limited. Efficacy (as well as tolerability and safety) is determined in clinical trials.

See also: tolerability

Disease-modifying therapies Treatments that can prevent, slow, stop or reverse disease progression.

Controlled trial

A type of study in which a new medication or procedure is compared to a standard, called the control. The control may be a placebo (inactive substance)

or the standard of care, which is what medical experts widely use and accept as the proper one.

See also: placebo-controlled

Control volunteer

A person with no known significant health problems who participates in research to test a drug, device or other intervention. These individuals also can contribute to observational studies.

Control volunteers serve as comparisons for patient groups when they are matched on certain characteristics,

such as age and gender.

Clinical studies

Research studies conducted in

human volunteers to better understand the nature of a disease or to evaluate the effect of an intervention (e.g.,

medication, surgical procedure, exercise) on that disease. There are two main types of clinical studies: clinical trials and

observational studies.

Comorbidity

Two or more diseases, such as anxiety and obesity, that occur in the same person at the same time.

Cohort

A group of individuals participating

in clinical research. Cohort studies may follow a large group of people over time, for example, to see who does and doesn’t develop a particular disease and learn about potential causes and risk factors.

Clinical trials

Research studies conducted in

human volunteers that evaluate the effect of an intervention (e.g., medication, surgical procedure, exercise) on symptoms or other features of a disease.

See also: interventional trial

Computed Tomography (CT) scan

A Computed Tomography (CT)

scan, sometimes called CAT scan (for Computed Axial Tomography), uses

x-rays to create two-dimensional images of different regions of the body.

Institutional Review Board (IRB) An independent committee of scientists, doctors and others (usually at least one “non-scientific” person who represents the patient voice) that evaluates and approves each study’s protocol and informed consent document, and monitors ongoing study activities. The Institutional Review Board (IRB) is in place to protect the rights and welfare of people participating in a study.

European Medicines Agency (EMA) A decentralized agency of the European Union (EU) responsible for the scientific evaluation, supervision, safety and monitoring of medicines in the EU.

Neuroregenerative treatment A therapy that stimulates regrowth of dopamine-producing cells in the

brain.

Multicenter trial

A clinical trial performed at more than one medical or research institution.

Magnetic Resonance Imaging (MRI) scan

A Magnetic Resonance Imaging (MRI) scan uses magnetic waves to create detailed pictures of areas inside the body. MRIs are especially useful for imaging the brain, and give clues about structure but not function. Some forms of MRI are being looked at as possible biomarkers.

See also: biomarker

Lumbar puncture (LP)

A lumbar puncture (LP), or spinal tap, is a procedure where a small needle is inserted below the spinal cord between the bones of the lower back to obtain a

small amount of spinal fluid for analysis.

Longitudinal study

A study that follows participants over an extended period of time, often years or decades, and is generally observational in nature. This type of study is particularly useful for evaluating risk factors or progression of a disease.

See also: observational study

Interventional trial

A type of trial in which participants receive an intervention (e.g., drug or surgical procedure) so that researchers can evaluate the effects of the intervention on certain symptoms or other features associated with a disease.

See also: intervention

Intervention

A potential therapy or treatment that is tested in clinical trials. These may include drugs, medical devices or procedures, and they may be

investigational products or therapies that are already available. (See: repurposing.) Interventions also can also include non- invasive approaches, such as exercise or

physical therapy.

Informed consent

A process used to educate potential participants about the possible benefits and risks of a specific clinical trial

or study. Prior to enrolling, all study participants must sign an informed consent document that explains the details of the trial or study and the rights

and responsibilities of the participant.

Inclusion criteria

Factors that need to be met to qualify a person to participate in a clinical trial or study.

Genetic testing

A type of medical test that identifies changes in genetic material. Genetic tests can evaluate a suspected genetic condition to help determine a person’s chance of developing or passing on a genetic disorder.

Genetic counselor

A health professional with expertise in medical genetics and counseling

who provides education and emotional support to people considering or

undergoing genetic testing.

Genetic mutation

A permanent change in the sequence of a gene that can affect health or risk of disease.

Gene

The material of heredity, passed down through the generations from parents to children. These inherited bits of DNA determine many of the body’s traits —

visible features such as eye color, as well

as ones that can’t be seen, including an individual’s risk of a particular disease.

Gaucher disease

A rare condition that causes fatty substances to build up and organs to swell. This disease develops in people who carry two copies of the mutated *GBA* gene. People with this mutation do not produce enough glucocerebrosidase, an enzyme that breaks down a fatty chemical called glucocerebroside.

See also: *GBA*

Exclusion criteria

Factors that prevent a person from participating in a specific clinical trial or study.

Observational studies

A clinical study in which participants’ health and other data is measured, but volunteers do not receive an intervention or drug.

See also: intervention

Study sponsor

The study sponsor is the individual or organization who oversees the study. The sponsor initiates, conducts and is responsible for the research.

Study funder

The study funder provides financial support for research. Funding can come from a variety of individuals or organizations, including foundations, pharmaceutical companies and federal

agencies, such as the National Institutes

of Health.

Statistical significance

A number that refers to whether the study’s results are highly likely to be true or could have occurred purely by chance. Note that statistically significant does not necessarily mean highly important.

Repurposing

Taking an existing drug that has been developed (and typically FDA-approved) for one condition and using it to treat another. Clinical trials are necessary to repurpose, or reposition, a therapy to ensure that it is safe and efficacious in those with a particular disease.

Recruiting

A term used to indicate that a study is open for enrollment and needs participants.

Randomized

A strategy in which participants are assigned to one group in a clinical trial or study by a methodological process that mimics chance. In placebo- controlled interventional trials, one group of participants is randomized to an intervention and another is assigned to placebo.

See also: placebo

Protocol

The written description of a clinical trial or study that describes its objectives, design and methods, as well as inclusion and exclusion criteria.

See also: exclusion criteria; inclusion criteria

Principal investigator

The researcher, often a doctor, who oversees and leads an entire clinical trial or study.

Pre-clinical

Research that is not conducted on humans. Before a drug can enter clinical trials, pre-clinical models must first evaluate its feasibility and safety.

Positron Emission

Tomography (PET) scan

A Positron Emission Tomography (PET) scan is a specialized imaging test that uses a small amount of radioactive medication to study the function of

the brain. For example, researchers are looking to visualize alpha-synuclein protein in the brain with PET scans; this could serve as a biomarker and way to measure the impact of drugs in trials.

See also: alpha-synuclein, biomarker

Placebo effect

A beneficial physical or emotional change that occurs after taking a placebo (inactive substance). This phenomenon is thought to result, at least in part,

from expectations of benefit. (In other words, the more a person believes they will benefit, the more likely it is they will experience benefit.) To separate out this effect from a drug or therapy’s true benefits, clinical trials typically use placebo-controlled designs.

See also: placebo; placebo-controlled

Placebo-controlled

A type of clinical trial in which a group of participants is randomly assigned to receive a placebo (inactive substance) for comparison to the standard of care (control) or intervention.

See also: placebo

Placebo

A substance or device that does

not contain active ingredients but is made to look, feel and taste just like the actual drug or therapy being studied

so that all participants have a similar research experience.

See also: placebo-controlled

Patient-reported outcomes (PROs) Data that is provided directly by participants. Patient-reported outcomes (PROs) complement traditional measures used during in-person clinical trial and study visits to give researchers a more complete picture of disease.

Outcome measure

A test or examination used to measure the effects of an intervention on certain symptoms or other features associated with a disease. Investigators decide on the measures that they are interested

in evaluating before the trial or study begins. Every interventional study has a primary outcome measure, which is most important for evaluating the effect of the intervention. Studies also may include secondary outcome measures, which are not as important but are still of interest

in evaluating the effect.

Open label

Clinical trials in which both investigators and participants know which participants have been assigned the intervention

or placebo.

See also: blinding

New Drug Application (NDA)

A new drug application (NDA) is a formal request from a drug sponsor to the U.S. FDA to ask for approval of a new drug. Data from pre-clinical research and all phases of human clinical trials are submitted as part of the NDA.

See also: U.S. Food and Drug Administration

logo

Wearable

A device that can be worn (e.g., watch, fitness tracker) to capture health-related information, such as movement, sleep or heart rate. Wearable data complements traditional research by providing an objective, continuous window into

the daily experience.

See also: digital health

Virtual study

Studies conducted online or through other digital modalities (e.g., smartphone, telephone, etc.). Virtual studies complement traditional research by allowing participants who might otherwise not be able to participate in traditional clinical trials (e.g., those with transportation or mobility issues) the opportunity to engage in research. These studies also are valuable in providing data outside of the “snapshot” of a face- to-face study visit.

U.S. Food and Drug Administration (FDA)

An agency within the U.S. Department of Health and Human Services, the FDA ensures that human drugs, biological products, medical devices, the food supply, cosmetics and other products are

safe and efficacious for consumers.

Tolerability

The degree to which effects of a drug or therapy can be tolerated by a patient, or how much these effects impact a person’s lifestyle or day-to-day activities.

Telemedicine

A field of medicine that delivers health care through electronic, two-way,

real-time interactive communication between individuals and their physicians

or other providers.

Symptomatic therapy

A treatment that eases the symptoms of a disease but does not address

the underlying disease process.