PLAIN ENGLISH GUIDE

medical research charities group

Unity is our Strength

The Medical Research Charities Group (MRCG) is an umbrella group of medical research and patient support charities, many of which are involved in with rare diseases. The main purpose of these organisations is supporting, promoting and funding medical research to improve health in Ireland and internationally.

In order to support patients and those interested in medical research, MRCG has decided to compile a "Plain English Guide" to common research terms. We plan that this document will be a living document and will be updated and adapted as necessary to be a useful tool for all who may use it.

This guide has brought together information from a variety of sources in a central and accessible resource.

COMMON RESEARCH TERMS

A

Adverse reaction: (Adverse event / side effect) An unwanted effect caused by the administration of drugs. Onset may be sudden or develop over time.

B

Baseline: 1. Information gathered at the beginning of a study from which variations found in the study are measured. 2. A known value or quantity with which an unknown is compared when measured or assessed. 3. The initial time point in a clinical trial, just before a participant starts to receive the experimental treatment which is being tested. At this reference point, measurable values such as those relating to the strength of the immune system, are recorded. Safety and efficacy of a drug are often determined by monitoring changes from the baseline values.

Bias: When a point of view prevents impartial judgment. In medical science it is important to avoid bias affecting the interpretation of data. In clinical studies, bias is controlled by blinding (see Blind below) and randomization.

Bioinformatics: is the field of science in which biology, computer science, and information technology merge to form a single discipline. The ultimate goal of the field is to enable the discovery of new biological insights.

Biotechnology: is the use of biological processes, organisms, or systems to manufacture products usually intended to improve the quality of human life. The earliest biotechnologists were farmers who developed improved species of plants and animals by cross pollenization or cross breeding. In recent years, biotechnology has expanded in sophistication, scope, and applicability.

Blind: A clinical trial is "Blind" if participants are unaware on whether they are in the experimental or control arm of the study; also called masked.

C

Cohort: Refers to a group of subjects who have some defining characteristic in common and who remain part of this group over an extended period of time. The common characteristic in a medical cohort may be a risk factor for a disease or health effect.

Clinical: Pertaining to or founded on observation and treatment of participants, as distinguished from theoretical or basic science.

Clinical investigator: A medical researcher in charge of carrying out a clinical trial's protocol.

Clinical trial: A clinical trial is a research study to answer specific questions about vaccines or new therapies or new ways of using known treatments. Clinical trials are used to determine whether new drugs or treatments are both safe and effective. Carefully conducted clinical trials are the fastest and safest way to find treatments that work in people. Trials are in four phases: Phase I tests a new drug or treatment in a small group; Phase II expands the study to a larger group of people; Phase III expands the study to an even larger group of people; and Phase IV takes place after the drug or treatment has been licensed and marketed.

Compassionate use: A method of providing experimental therapeutics prior to final approval for use in humans. Approval is given in the United States by the Food and Drug Administration (FDA) and in Europe by the European Medicines Evaluation Agency (EMEA). This procedure is used with very sick individuals who have no other treatment options. Often, case-by-case approval must be obtained for "compassionate use" of a drug or therapy.

Contraindication: A specific circumstance when the use of certain treatments could be harmful.

Control group: The standard by which experimental observations are evaluated. In many clinical trials, one group

of patients will be given an experimental drug or treatment, while the control group is given either a standard treatment for the illness or a placebo.

Controlled trials: Clinical trials which include a control group (see above).

D

DNA: Deoxyribonucleic acid (DNA) is the chemical compound that contains the instructions needed to develop and direct the activities of nearly all living organisms. DNA molecules are made of two twisting, paired strands, often referred to as a double helix.

Double blind study: A clinical trial design in which neither the participating individuals nor the study staff know 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; also called double-masked study.

Efficacy: The maximum ability of a drug or treatment to produce a result regardless of dosage. A drug passes efficacy trials if it is effective at the dose tested and against the illness for which it is prescribed. In the procedure mandated by the FDA, Phase II clinical trials gauge efficacy, and Phase III trials confirm it.

E

EMEA: The European Medicines Agency is the regulatory body in Europe which fosters scientific excellence in the evaluation and supervision of medicines, for the benefit of public and animal health.

Empirical: Based on experimental data, not on a theory.

Endpoint: Overall outcome that the protocol is designed to evaluate. Common endpoints are severe toxicity, disease progression, death, remission or cure.

Epidemiology: The branch of medical science that deals with the study of incidence, distribution and control of a disease in a population.

F

FDA: The Food and Drug Administration is the regulatory body in the United Stated with responsibility for protecting the public health by assuring the safety, efficacy, and security of human and veterinary drugs; biological products; medical devices; food supply; cosmetics; and products that emit radiation.

G

Genome: an organism's complete set of DNA is called its genome. Virtually every single cell in the body contains a complete copy of the approximately 3 billion DNA base pairs, or letters, that make up the human genome.

H

Genomics: is the study of an organism's entire genome.

I

Interventions (medical): The act of intervening to improve health or alter the course of disease. Common types of interventions are drug, gene transfer, vaccine, behavior, device, or procedure interventions.

J K L M

Metabolome: represents the collection of all metabolites in a biological organism, which are the end products of its gene expression. Thus, while mRNA gene expression data and proteomic analyses do not tell the whole story of what might be happening in a cell, metabolic profiling can give an instantaneous snapshot of the physiology of that cell.

Metabolomics: is the systematic study of the unique chemical fingerprints that specific cellular processes leave behind - specifically, the study of their small-molecule metabolite profiles.

O

Off label use: A drug prescribed for conditions other than those approved by the FDA/EMEA.

Orphan drugs: An FDA / EMEA category that refers to medications used to treat diseases and conditions that occur rarely. There is little financial incentive for the pharmaceutical industry to develop medications for these diseases or conditions. Orphan drug status, however, gives a manufacturer specific financial incentives to develop and provide such medications.

P

Peer review: Review of a scientific paper or clinical trial by experts in the field. These experts review the paper or trials for scientific merit, participant safety and ethical considerations.

Pharmacokinetics: The processes (in a living organism) of absorption, distribution, metabolism and excretion of a drug or vaccine.

Pharmacoeconomics: refers to the scientific discipline that compares the economic value of one pharmaceutical drug or drug therapy to another. It is a sub-discipline of health economics.

Placebo effect: A physical or emotional change, occurring after a substance is taken or administered, that is not the result of any special property of the substance. The change may be beneficial, reflecting the expectations of the participant and, often, the expectations of the person giving the substance.

Preclinical Trials: Drug trials in which the subjects are non-human, such as animals, bacteria, cells, etc.

Proteome: The term proteome is used to describe the entire complement of proteins expressed by a genome, cell, tissue or organism. PROTEin complement to a genOME - is the large-scale study of proteins, particularly their structures and functions. The proteome of an organism is the set of proteins produced by it during its life. A proteome differs from cell to cell and constantly changes through

its biochemical interactions with the genome and the environment.

Proteomics: is the discipline of studying proteomes.

Protocol: A study plan on which all experimental studies or clinical trials are based. A protocol for clinical trials is carefully designed to safeguard the health of the participants as well as answer specific research questions. A protocol describes what types of people may participate in the trial; the schedule of tests, procedures, medications, and dosages; and the length of the study. While in a clinical trial, participants following a protocol are seen regularly by the research staff to monitor their health and to determine the safety and effectiveness of their treatment.

S

Systems biology: is a field of study in the biosciences, focused on the systematic study of complex interactions in biological systems.

T

Toxicity: An adverse effect produced by a drug that is detrimental to the participant's health.

U V W X Y Z

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For further information, please contact: www.mrcg.ie / info@mrcg.ie