Glossary

Active substance file holds confidential intellectual property or 'know-how' of the manufacturer of the active substance of a drug.

Advanced Therapeutic Medicinal Products (ATMP) are advanced therapeutic drugs that are based on cell therapy or gene therapy (sometimes in combination with a medical device).

Adverse events are undesired medical occurrences in a patient or clinical investigation subject after the administration of a pharmaceutical product. An adverse event or effect does not necessarily have a causal relationship with the treatment.

Allometric scaling is a prediction method to provide a "sneak peek" at how a product might behave in humans before any clinical studies are conducted.

Bio-distribution is where compounds of interest travel in an experimental animal or human subject.

Biomechanical limitations pertain to the geometric properties of the musculoskeletal system and the stiffness properties of the tissues within this system that affect the movement of an individual.

Cellular and tissue-based products are products containing or consisting of (human) cells or tissues which are intended for implantation, transplantation, infusion or transfer to a human recipient.

Clinical directive is a European Union directive aimed at facilitating the internal market of medicinal products within the European Union.

Comorbidities are the presence of one or more additional conditions often co-occurring with a primary condition

Comparator is the currently accepted standard treatment for the specific clinical problem to be investigated.

Double-blind process is a step in experimental design in which neither the participants nor the experimenters know who is receiving a particular treatment.

Endpoint (in clinical research) is a disease state, symptom, or sign that constitutes one of the target outcomes of the trial or its participants.

Good Clinical Practice (GCP) is an international quality standard, which governments can use to transpose into regulations for clinical trials involving human subjects.

Health Technology Assessment (HTA) is a multidisciplinary process that uses systematic and explicit methods to evaluate the properties and effects of a medical technology.

Hospital Exemption is a pathway (only in Europe) to enable patients to receive an Advanced Therapy Medicinal Product (ATMP) from a clinician under controlled conditions in cases where no centrally authorized medicinal products are available for an indication with a high unmet medical need.

Informed consent is a principle in medical ethics and medical law that consists of sharing sufficient information with a patient so he/she can make a free and well-informed decision about his/her medical care.

Market Approval is the process of reviewing and assessing the evidence to support the use of a medicinal product, such as a drug, in relation to its marketing. This process ends by granting a license to sell the product.

Medical device is any instrument, apparatus, appliance, software, or material used alone or in combination with another device for diagnostic and/or therapeutic uses.

Mode of action describes how a treatment works, what is the underlying mechanism resulting from the exposure of a living organism to a substance.

Notified Bodies are organizations in the European Union designated by a member state to assess the conformity of certain products, before being placed on the EU market, with the applicable essential requirements including a technical dossier and data management systems.

Phase I, human pharmacology (phase) is the first stage of testing in human subjects, designed to test the safety, side effects, dose and formulation method for the drug.

Phase II, therapeutic exploratory (phase) is performed on larger group of human subjects (50–300) to assess how well the drug works and the optimal dose and to continue Phase I safety assessments in a larger group of volunteers and patients.

Phase III, therapeutic confirmatory (phase) also called pivotal trial as definitive assessment of drug efficacy and safety in comparison with a current 'gold standard' treatment.

Phase IV studies are done to assure long-term safety and effectiveness of the drug, vaccine, device or diagnostic test.

Potency assay is the quantitative measure of the presumed biological activity relevant to the outcome, ideally it measures the ability of the product to elicit a specific clinical response.

Preclinical is a stage of research that begins before clinical trials (testing in humans) and during which important feasibility, iterative testing and drug safety data are collected, typically in in vitro/ex vivo models and in vivo laboratory animals.

Protocol is the key document that contains all information on the conduct of the specific trial.

Quality Attributes are measurable or testable properties of a process used to indicate how well the process performs to manufacture a well-defined product.
Quality management system is a collection of processes focused on consistently meeting customer and product requirements and enhancing their satisfaction.
Randomization is a sequence of random variables describing a process whose outcomes do not follow an evolution described by probability distributions.
Randomized Controlled Trials are a form of clinical trial used to control factors not under direct experimental control. Example: Clinical trials that compare the effects of drugs, surgical techniques, medical devices, diagnostic procedures or other medical treatments.
Regenerative medicine deals with the process of replacing, engineering or regenerating human or animal cells, tissues, or organs to restore or establish normal function.
Regulation in the context of marketing a product, harmonizes the assessment and supervision of processes for clinical trials throughout the European Union, via a Clinical Trials Information System.
Staged clinical trials are distinct phases of clinical research in which scientists conduct experiments with a health intervention to obtain sufficient evidence for a process considered effective as a medical treatment.
Standard operating procedures (SOPs) are sets of step-by-step instructions compiled by an organization to help workers carry out routine operations to become user independent, thus robust, and reproducible.
Statistical power, usually expressed in percentages**,** is the probability that the test correctly rejects the null hypothesis when a specific alternative hypothesis is true.
Technical product documentation refers to any document that explains the use, functionality, production or architecture of a product.
Technology Readiness Level(s) **(TLR)** are a method for estimating the technical maturity of a technology during its development.
Tissue engineering is a discipline that uses a combination of cells, engineering, materials methods, and suitable biochemical and physicochemical factors to restore, maintain, improve, or replace different types of biological tissues.