# 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.

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