From the Lab to Your Medicine Cabinet: A Pharmaceutical Drug's Journey
Supplemental Information and Alphabetical Glossary of Terms

Acute Toxicity	Toxicity produced by a pharmaceutical drug when it is administered in one or more doses during a period not exceeding 24 hours.
ADME	Absorption, distribution, metabolism, and excretion.
Adverse Event	Any untoward medical occurrence, including death, in a patient treated with a pharmaceutical product but which does not necessarily have a causal relationship with treatment.
Analog	A compound that is analogous to another. Usually analogs share a common structural scaffold but bear different chemical substituents.
API	Active Pharmaceutical Ingredient – the active ingredient in a drug product.
Arraying	A numbered series of test objects such as activity assays, DNA probes, antibodies, chemical reactions, etc., that are of the same size and/or type.
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Assay Plate	A plate with typically 96, 384, or 1536 similar microliter volume wells arranged in a two-dimensional array for parallel or multiple tests or reactions.
Attrition	Reduction of lead or drug candidate molecules due to failures in preclinical or clinical trials.
AUC	Area under curve is a measure for drug exposure. AUC is the product of the drug concentration in the bloodstream or a specific tissue and a defined time period.
Bioavailability	The rate and extent to which the active ingredient or active moiety is absorbed from a drug product and becomes available at the site of action. For drug products not intended to be absorbed by the bloodstream, bioavailability may be assessed by measurements intended to reflect the rate and extent to which the active ingredient becomes available at the site of action.

Biological	A biological product that is any virus, therapeutic serum, toxin, antitoxin, or analogous product applicable to the prevention, treatment , or cure of diseases or injuries of humans.
Biomarker	A characteristic that may be measured objectively and evaluated as an indicator of normal biologic processes, pathogenic processes, or pharmacologic responses to a therapeutic intervention. A 'valid biomarker' can be measured in an analytical test system with well-established performance characteristics for which there is widespread agreement in the medical and scientific community about the physiologic, toxicologic, pharmacologic, or clinical significance of the results.
CADD	Computer-assisted drug design. Use of computational tools and algorithms for the visualization, design and optimization of leads and drug molecules.
Carcinogenicity	The type of toxicology study intended to detect the potential of a drug to cause cancer in humans. These studies involve chronic dosing of the drug to mice and/or rats for 18 to 24 months. They are also referred to as "oncogenicity studies." They are required for all products intended for chronic administration to patients for more than 6 months or intermittently for recurrent conditions.
CBER	Center for Biologics Evaluation and Research. The branch of FDA responsible for the review of new biologic products, including blood products and vaccines.
CDER	Center for Drug Evaluation and Research. The branch of FDA responsible for the review of new pharmaceutical drugs and some therapeutic biological products such as monoclonal antibodies.
Cell line	Cells which grow and replicate continuously outside the living organism and are maintained in vitro for medical and/or research purposes.
CFR	The Code of Regulations is a codificationof the general and permanent rules published in the Federal Register by the Executive departments and agencies of the US Federal government, such as the FDA.
cGMP	Current Good Manufacturing Practice – the standard for manufacturing quality and control of pharmaceutical drusg and biologics. See also GMP.
Chemical Library	A collection of distinct, defined and characterized molecules or mixtures thereof.

Chemistry Scale-Up	Once a compound has been synthesized in a controlled laboratory environment, the manufacturing process must be scaled-up to produce increasing quantities of active ingredient – initially to support laboratory and animal testing, then to support laboratory and animal testing, then to support clinical testing, and ultimately for commercial manufacture.
Chirality	When screening promising active substances, stereochemically-defined active substances should be studied where possible. New active substances should be checked for steroeogenic centers and chirality. If a new racemate appears promising, both enantiomers should be studied separately as early as possible to assess the relevance of stereoisomerism for effects and fate *in vivo*. See Racemate, Enantiomer and Sterochemistry.
Chromatography	The separation of a mixture of substances by charge, size, or other property by allowing the mixture to partition between a moving phase and a stationary phase.
Class	A gene or protein family/class refers to genes or proteins that have in common one or any number of characteristics such as sequence homology, biological function or mechanism of action, etc.
CMC	Chemistry, Manufacturing and Controls. This refers to the quality information gathered on the active ingredient and the formulated product. It is also referred to as the Chemical and Pharmaceutical Documentation.
CNS	Central nervous system.
COGS	Cost of goods. The cost of producing a certain amount of a dru molecule.
Compound	A defined and discreet molecule. A compound can either be a small molecule, a protein or antibody, or an oligonucleotide.
Compound library	A collection of distinct, defined and characterized molecules or mixtures thereof.
Compound Series	A number of analogs which bear common features and were synthesized to address a specific drug discovery or development question.
Controlled Trials	Design of a clinical trial that includes a balanced and randomized group of test subjects in terms of age and gender (where applicable), a control group following the same test protocol without actually receiving drug (placebo), and an independent review of the trial data. Trials may be open, blind or double-blind.

Cytochrome P450	Cytochrome P450s (CYP) are a group of mixed function monooxidases that are primarily responsible for metabolizing drugs or other xenobiotics. Understanding which isoenzymes are involved in the metabolism of a compound in development is very helpful when assessing its potential for drug interactions.
Cytotoxicity	Toxic to cells.
Disease marker	A direct or indirect (surrogate) readout measuring the degree, reversal or progression of a specific disease.
DMF	A Drug Master File is a compilation of information about an active ingredient, excipients or container intended for use in a drug product but supplied to the sponsor by a third party.
Dosage Regimen	Te frequency of administration and dosage of a drug product.
Double Blind	A clinical trial design in which neither the patient nor the investigators or sponsor staff involved in the treatment or clinical evaluation of the subjects are aware of which treatments patients have received (i.e. who received active drug and who received placebo).
Double Dummy	A clinical trial design which is useful when the test drug (or comparator) are presented in a distinctive container or delivery system or has a physical characteristic (i.e. color) which cannot be presented to a patient in a blinded manner without disrupting the product, such that a 'dummy' of each treatment must be prepared.
Dose Response	Knowledge of the relationships among dose, drug concentration in blood, and clinical response (effectiveness and undesirable side effects) is important for the safe and effective use of drugs in individual patients.
Drug Substance	An active ingredient that is intended to furnish pharmacologic activity or other direct effect in the diagnosis, cure, mitigation, treatment or prevention of disease or to affect the structure or any function of the human body.
Drug Product	The finished dosage form in the final container, made according to the prescribed manufacturing method to a defined specification and tested by prescribed analytical methods.
Drug Target	Biological molecule or molecules that are inhibited, activated or modulated by a drug molecule. Drug targets can be any form or combinations of (glyco)proteins, DNA or RNA.

EC50	The EC50 represents the plasma concentration/AUC required for obtaining 50 percent of the maximum effect in vivo.
ED50	The dose of a drug that is pharmacologically effective for 50 percent of the population exposed to the drug or a 50 percent response in a biological system that is exposed to the drug.
Effectiveness	Power or capacity to produce a desired, or in the context of disease, beneficiary effect.
Efficacy	Power or capacity to produce a desired, or in the context of disease, beneficiary effect.
Enantiomer	Stereoisomers are molecules that are identical in atomic constitution and bonding that differ in the three-dimensional arrangement of the atoms .
Excipient	An ingredient, usually inactive or inert, that is added to a drug substance to improve manufacturing or delivery characteristics of the finished product.
Exposure	Exposure measures the interaction/contact of an organism and a compound over a specified time period. The area under the curve (AUC) is an important parameter for exposure. It is a measure of how much of a drug reaches the bloodstream in a set period of time. AUC is calculated by plotting drug blood concentration at various times during a 24-hour or longer period and then measuring the AUC between 0 and 24 hours.
FDA	Food and Drug Administration. US regulatory agency (http://www.fda.gov) responsible for the evaluation and approval of drugs and medical devices.
Formulation	The list of chemicals/substances that are not the therapeutically active ingredient, and their relative amounts, to be used in the preparation of a drug.
Function/Functional	Biological/physiological role of a gene, gene product (protein, enzyme). Functional assays assess the this role.
Genomics	The study of the sequence, structure, and function of the genome.
Genotoxicity	A measure of the potency of adverse effects of a toxin on DNA.
GLP	Good laboratory practices.
GMP	Good manufacturing practices.

GRAS	Generally recognized as safe.
Half Life	The time for the concentration of drug in blood or plasma to decline to half of its original level.
High Throughput Screening	An approach to drug discovery and development where a large number of chemical compounds, possible synthesized as a mixture via combinatorial chemistry, are all evaluated for their activity against an enzyme, receptor or other biomarker of interest in a single assay.
Hit	A compound that shows activity in a primary screen.
IC50	The IC50 represents the concentration of a drug that is required for 50 percent inhibition in an assay.
In silico	Computationally as opposed to in vitro or in vivo.
IND	Investigational new drug.
In vitro	An *in vitro* experiment is conducted in a laboratory and may involve isolated biological tissue, cell cultures or other reagents.
In vivo	An *in vivo* study takes place within a living biological organism or animal or human (as opposed to an *in vitro* study conducted in a laboratory).
LD50	Lethal Dose 50 is the dose of a chemical which kills 50 percent of a sample population. In full reporting, the dose, treatment and observation period should be given. LD50s are strictly only comparable when the age, sex, and nutritional state of the animals is specified.
Lead	A compound with a confirmed activity profile that warrants development.
Lead series	A number of analogous compounds synthesized around a confirmed active—the lead.
Metabolism	The biochemical processes that sustain a living cell or organism. Usually divided into two types: catabolism, the breakdown of complex substances into simple ones, with the release energy; and anabolism, the building up of complex substances from simpler ones, with the absorption or storage of energy. Molecules that are part of these processes are metabolites.
Metabolite	The biochemical processes that sustain a living cell or organism. Usually divided into two types: catabolism, the breakdown of complex substances into simple ones, with the release energy; and anabolism,

	the building up of complex substances from simpler ones, with the absorption or storage of energy. Molecules that are part of these processes are metabolites.
MoA	Mechanism of action.
Molecular Target	Molecule with a function in disease that is targeted with a compound to achieve a therapeutic effect.
MTD	Maximum tolerated dose.
NCE	New chemical entity.
NDA	New drug application.
PD	Pharmacodynamics - The study of drug action on living organisms.
Pharmacology	The study of drugs and their origin, nature, properties and effects upon living organisms.
Pharmacophore	The three-dimensional arrangement of atoms, or groups of atoms, responsible for the biological activity of a drug molecule.
PK	Pharmacokinetics - The study of the action of an organism on a drug. Usually encompasses absorption, metabolism, distribution and excretion (ADME).
Pro-drug	Originates from the term "progenitor drug." A compound that is converted within the body into the active form that has therapeutic effects.
QSAR	Quantitative structure-activity relationship. A model describing how a pharmacophore effects its target.
Racemate	A compound containing a 50:50 mixture of enantiomers (isomers that are mirror images of each other).
Randomized	A process for randomly assigning individual clinical trial patients or volunteers to either treatment or control groups in order to reduce the risk of bias from the sponsor or investigator.
Receptor	A specific protein-binding site on a cell's surface or interior. When compounds bind to receptors, various cellular functions are activated or inhibited.
SAR	(Quantitative) structure-activity relationship. A model describing how a pharmacophore effects its target.

SBDD	Structure-based drug design.
Scale-up	Describes the process of achieving production in a larger scale, i.e., from the gram to kilogram scale or from microliter to liter scale.
Screen	In drug discovery testing large numbers of compounds in order to identify those with particular characteristics.
Screening	In drug discovery testing large numbers of compounds in order to identify those with particular characteristics.
Side Effect	An undesirable effect of a drug.
Small Molecule Drugs	Therapeutic molecules with a molecular weight below 1 kDa. A typical small molecule drug has a molecular weight between 250 and 400 Da.
Stereoisomers	Molecules that are identical in atomic constitution and bonding that differ in the three-dimensional arrangement of the atoms,
Susceptibility	The degree to which an organism is sensitive to a therapeutic or a disease.
Target Class	Systematic or empirical name of a target family, i.e., kinases.
Throughput	Measure for the number of assays or data points per time unit, i.e., 1,000 assays per day.
Toxicology	The study of the toxic effects of substances.