

Appendix A: Glossary

NOTE: The National Institutes of Health's MedlinePlus Web site (www.medlineplus.gov/) features a medical dictionary and medical encyclopedia that can be used to look up unfamiliar terms and information about medical conditions free of charge.

The U.S. Department of Health and Human Service's Agency for Healthcare Research and Quality (AHRQ) has posted a very useful glossary written for consumers of terms used in health and drug research. This glossary can be found at www.effectivehealthcare.ahrq.gov/tools.cfm?tooltype=glossary&alpha=A.

A

adherence The degree to which a patient follows his assigned treatment regimen.

adverse effect The terms adverse effect, adverse drug reaction, adverse drug event, and side effect are often used interchangeably. The precise definition of an adverse drug reaction is "a response to a drug which is noxious and unintended and which occurs at doses normally used in man for prophylaxis, diagnosis, or therapy of disease or for the modification of physiologic function."

animal study A safety study done in laboratory animals before a new drug can be tested in human subjects. These studies are the first steps in determining the acute toxicity, cancer causing potential, and provide information about the potential for the agent to cause birth defects.

B

Beers criteria This is sometimes referred to as the Beers List and is a compilation of medications that are generally considered inappropriate when prescribed to older adults because these medications may pose more risks than benefits.

benefit The documented or expected positive outcome resulting from the use of a medication.

benefit-harm balance A comparison between the documented or expected positive result of drug treatment to the medication's known adverse effects. This comparison is important in the decision to prescribe or take a medication.

bias A situation that produces study results that depart from the true values in a consistent direction.

biochemical marker The results of laboratory tests on the blood or other tissues that are used to substitute for a clinically meaningful or important outcome (e.g. cholesterol for heart attack).

blockbuster This is a term used by the investment community to describe a medication that sells more than \$1.0 billion per year. Blockbusters are not necessarily important therapeutic advances.

brand name medication This is the name that is created and owned by the manufacturer of a drug. A brand name is created for marketing purposes so that patients and prescribers will remember that name.

C

clinical trial A research study that tests treatments in human volunteers to determine efficacy and safety. A treatment could be a drug, medical device, or biologic, such as a vaccine, blood product, or gene therapy.

comparative trial A type of clinical trial that directly tests one medication against another medication.

compounded medication These are drugs produced by pharmacies and sometimes referred to as pharmacy compounded medications. These products are not FDA approved for any specific purpose and are not produced in regulated facilities that insure quality, stability, and potency.

co-payment The part of the prescription medication cost that is the responsibility of patient to pay. Typically, the lowest co-payment is for generic drugs and the highest for brand name products.

D

DailyMed This is a Web site sponsored by the FDA and the U.S. National Library of Medicine that provides free of charge to the public the professional product labels, or package inserts, for all drugs marketed in the U.S. The Internet address for DailyMed is www.dailymed.nlm.nih.gov/dailymed/about.cfm.

Declaration of Helsinki A set of ethical principles used in research to ensure the protection of human subjects participating in clinical trials.

dietary supplement The term natural health product and dietary supplement are often used interchangeably. These are products taken by mouth that contain an ingredient intended to supplement the diet. Dietary ingredients may include vitamins, minerals, herbs or other botanicals (plants), amino acids, and substances such as enzymes, organ tissues, glandular products, and metabolites.

direct-to-consumer advertising The promotion of prescription medications directly to patients through the print and broadcast media. Prior to 1997, prescription medications were only advertised directly to health professionals.

disease complication A consequence of chronic disease such as a heart attack, a stroke, or the development of heart or kidney failure.

dose-response relationship This relationship exists if with increasing drug doses the drug effect is more pronounced.

drug-induced complication Sometimes referred to as a drug-induced disease or a serious adverse drug reaction.

drug price This can include retail, wholesale, and negotiated prices. The true cost of a drug is rarely, if ever, known. This most commonly refers to the out-of-pocket retail price paid by the consumer.

E

efficacy A medication's efficacy and effectiveness are commonly used interchangeably. Technically, a medication's efficacy is established in clinical trials and its effectiveness refers to its therapeutic value in the general population.

F

functional food Producers of functional food define it as a food or dietary component that may provide a health benefit beyond basic nutrition. Currently, the FDA has neither a definition nor specific regulatory standards for foods being marketed as functional foods.

G

generic medication These are medications that contain the identical active ingredient as more expensive brand name products; however, they may contain different inactive ingredients. Generic drugs may be sold after a brand name medication loses its patent protection. Generic and brand name medications must meet the same quality standards.

generic substitution This occurs when under state regulations, a pharmacist may substitute, usually in consultation with the consumer, an FDA-approved and recognized generic equivalent medication for a brand name drug.

genetic testing This type of testing may in the future help to predict an individual's response to a medication. Today it is used to predict the effectiveness and the potential for adverse drug reactions for a small number of medications.

H

health plan An insurance policy that may or may not include prescription medication coverage.

I

industry sponsorship Payments or donations from manufacturers to fund activities such as clinical trials, continuing medical education, and patient advocacy groups.

informed consent form A statement signed prior to the beginning of a clinical trial by persons volunteering to participate as subjects in the trial. The form provides information about all aspects of the trial that are relevant to an individual's decision to participate. Informed consent is documented by this written, signed and dated form.

interaction When two drugs are given together, the effect of one of the drugs may be increased or decreased. This is referred to as a drug interaction.

Internet A network that links computers all over the world by satellite and telephone, connecting users with service networks such as e-mail and the World Wide Web.

M

maintenance dose The dose of medication, usually constant, that is used long-term to prevent complications of a disease.

managed care organization A managed care organization (MCO) is a health care provider or group of medical service providers who contract with insurance companies or self-insured employers to provide a wide variety of managed health care services to enrolled workers.

- Medicaid** A federal-state program that provides health care, including prescription drugs coverage, for low-income Americans.
- Medicare** A federal health insurance program that provides benefits for hospital and physician services for older Americans. In 2006, the program was expanded to help pay for prescription medications.
- Medication Guide** This provides FDA-regulated drug information written specifically for patients in non-technical language. The agency can require that pharmacists distribute Medication Guides with each new and refill prescription for drugs that present serious public health concerns.
The list of drugs that require Medication Guides can be found on the FDA's Web site at www.fda.gov/cder/Offices/ODS/medication_guides.htm.
- medication review** A review by your primary healthcare providers of all medications, vitamins, minerals, dietary supplements and natural products that you are taking.
- medication switch** In general, there are two types of medication switches. The first is the switching of a more expensive brand name medication to a lower-cost, equivalent generic product. The second involves switching one drug to another within a drug class.
- Medline Plus** This is a Web site operated by the National Library of Medicine (NLM), a part of the National Institutes of Health (NIH), that contains medical and drug information prepared for general audiences. Among other features, the site contains a medical dictionary and a medical encyclopedia.
The Web address is: www.nlm.nih.gov/medlineplus/.
- MedWatch** This is the FDA's safety information and adverse event reporting program that allows consumers and healthcare professionals to voluntarily report serious problems that they suspect are associated with drugs and medical devices.
- me-too drug** A medication that differs chemically in a small way from an established brand name drug. These represent new drugs and thus are entitled to patent protection in spite of having no documented clinical benefit over the older parent drugs. The primary purpose for marketing a me-too drug is financial.

N

- natural health product** See dietary supplement above
- non-inferiority study** A type of clinical trial that directly compares two drugs, for example, a new drug versus a standard treatment. The purpose is to find out if a new drug is as effective and safe as standard treatment.
- non-responder** A person who does not respond to a medication usually in the context of a clinical trial.

O

- off-label use** A medication that is prescribed in a way, or for a use, that has not been approved by the FDA is said to have been prescribed off-label. This not only applies to the drug's use but also the dose, duration of treatment, and age group for which the drug is prescribed. There may, or may not be, evidence of safety or efficacy for an off-label use.
- over-the-counter** Non-prescription drugs and over-the-counter (OTC) drugs are the same. The sale of OTC drugs is not restricted to pharmacies.

P

package insert or professional product label This is the FDA-approved information that must be supported by scientific studies. It is written for health professionals and must accompany every medication that is delivered to a pharmacy. The font size of printed professional labels is small. Much easier to read versions of professional labels can be found on the DailyMed Web site (www.dailymed.nlm.nih.gov/dailymed/about.cfm).

palpitations A rapid beating of the heart when excited by exertion, strong emotion, or disease.

patent protection Patents are awarded by governments to recognize the intellectual innovation of inventors and provide for a period of time of exclusive rights. The basic period of patent exclusivity for a new medication is 20 years. However, there are different types of patents and pharmaceutical manufacturers spend extensive resources to extend their patent protection for as long as possible to limit competition from generic drugs.

pharmacy-compounded medication Traditional pharmacy compounding involves a pharmacist preparing a solution or suspension from an FDA-approved tablet or capsule for patients who have difficulty swallowing. Pharmacy-compounded medications are rarely needed.

phase 1 study These studies are used to determine dosing, document how a drug is metabolized and excreted, and identify acute adverse effects. Usually, a small number of healthy volunteers (between 20 and 80) participate in Phase 1 trials.

phase 2 study These studies include more participants (about 100-300) who have the disease or condition that the experimental medication could potentially treat. In Phase 2 studies, researchers seek to gather further safety data and preliminary evidence of the medication's effects (efficacy). If the Phase 2 studies indicate that the drug may be effective -- and the risks are considered acceptable, given the observed efficacy and the severity of the disease -- the medication moves to Phase 3.

phase 3 trial In this phase, the medication is studied in a larger number of people with the disease of interest (approximately 1,000-3,000). This phase further tests the product's effectiveness, monitors adverse effects and compares the product's effects to a placebo or to a standard treatment, if one is already available.

phase 4 trial A Phase 4 trial is conducted after a medication is on the market (post-approval) to obtain more information about the treatment's long-term risks, benefits, and optimal use, or to test the product in different groups of people, such as children.

placebo An inactive agent often given to the control group in clinical trials.

polypharmacy The use of five or more medications.

post-marketing safety surveillance The monitoring of drug safety by collecting reports of adverse drug effects in the population after a medication has been marketed.

prescription medication A medication that can only be prescribed by licensed health professionals who are authorized by law.

prevention When used in the context of medications, this is a treatment that prevents either the acute or long-term serious consequences of diseases.

product liability Product liability is an area of the law in which manufacturers, producers, distributors, suppliers, retailers, and others who make products available to consumers are held responsible for the injuries those products cause.

professional product label or package insert This is information written specifically for health professionals that is reviewed and approved by the FDA. The information in professional product labels must be based on scientific studies. The printed versions of this information must accompany every medication that is delivered to a pharmacy.

public health Public health is a discipline and practice that focuses on improving the health of the community.

Public Health Advisory This refers to a statement issued by the FDA to announce important safety information about medications, medical devices, or foods.

Q

quality of life An individual's overall well being. This may be related to health status or non-health factors.

R

randomization The process of assigning clinical trial subjects to treatment or control groups using an element of chance, in order to reduce bias.

rebound effect The reappearance of a symptom, sometimes worse, after the use of a medication to relieve that symptom. For example, nasal spray decongestants should not be used for more than several days, because they can cause a "rebound" effect and make the congestion worse.

S

statistically significant Statistics describe the role that chance may play in the results of a clinical study. A statistically significant result is unlikely to be due to chance alone. However, a statistically significant result may not be clinically important and it is possible that a statistically insignificant result is important clinically.

substitution Substitution is a synonym for switching. See medication switches above.

surrogate A laboratory measurement or a physical sign used as a substitute for a clinically meaningful outcome that directly measures disease complications.

survey A analysis of answers to questions of a sample of a population to determine to determine opinions, preferences, or knowledge.

survival benefit The documentation from a clinical trial that a medication (or another intervention) extends, on average, the life of the subjects in the clinical trial.

therapeutic substitution This is frequently done automatically in hospitals where there are large numbers of similar medications in the same pharmacological or therapeutic class. A hospital committee of pharmacists and physicians decides that one drug in a therapeutic class will be dispensed no matter what drug in that class is ordered.

treatment option/alternative A treatment, usually FDA approved, that can be used in place of another treatment.

U

unethical When applied to clinical trials, it usually refers to those trials that do not, for example, address scientifically valid questions; those conducted only for promotional purposes; those in which research subjects were coerced into a trial; or those that were too small to be able to provide a statistically meaningful result.

Appendix B - Abbreviations

ADHD – Attention Deficit Hyperactivity Disorder
AERS – Adverse Event Reporting System
AIDS – Autoimmune Deficiency Syndrome
ASP – Average sales price
AWP – Average wholesale price
BPCA – Best Pharmaceuticals for Children Act
DDMAC – Division of Drug Marketing, Advertising and Communications
DTC – Direct-to-consumer
EMA – European Medicines Agency
FDA – Food and Drug Administration
FDAAA – Food and Drug Administration Amendments Act
FTC – Federal Trade Commission
GAO – Government Accountability Office
GMP – Good Manufacturing Practice
HIV – Human Immunodeficiency Virus
IRB – Institutional Review Board
NCCAM – National Center for Complementary and Alternative Medicine
NDA – New Drug Application
NIH – National Institutes of Health
NLM – National Library of Medicine
NSAID – Non-steroidal anti-inflammatory drug
OTC – Over-the-counter
PDMA – Prescription Drug Marketing Act
PPA – Partnership for Prescription Assistance
PREA – Pediatric Research Equity Act
SSRI – Selective Serotonin Reuptake Inhibitor
VA – Veterans Affairs
VICP – Vaccine Injury Compensation Program
VIPPS – Verified Internet Pharmacy Practice Sites
WHO – World Health Organization