WHAT ARE DRUGS?

The medical, nursing, and health professions use the word "drugs" to refer to medicines – substances that can cure or arrest disease, relieve symptoms, ease pain, and provide other benefits. This definition includes essential vitamins and minerals that may be given to correct deficiency diseases.

Powerful drugs often have marked adverse effects. Drugs with less potential to cause harm are sold over the counter in pharmacies and supermarkets. More powerful drugs (those that the Medicines and Healthcare products Regulatory Agency, or MHRA, has ruled cannot be used safely without medical supervision) require a doctor's prescription.

A different use of the word "drugs" refers to those substances on which a person may become dependent. These range from mild stimulants such as caffeine (found in tea and coffee) to powerful agents that alter mood and behaviour. Some addictive drugs have no medical use and cannot be obtained legally.

Where drugs come from

At one time, the only available drugs were substances extracted from plants, or, in some cases, animals. Herbalism, the study and medicinal use of plants, was practised by the Chinese more than 5,000 years ago and is becoming popular in many parts of the world today.

Virtually all the drugs in current use have been developed in the laboratory and are manufactured through various chemical processes. About a quarter of these are derived from plants or other organisms. Most drugs are synthetic chemical copies, but some are still extracted from natural sources. For example, the opioid drugs, including morphine, are made from a species of poppy. Many antibiotics and some anticancer drugs are still of natural origin. The main difference between drugs of plant origin and "herbal medicines" is that drugs have been thoroughly tested to prove that they work and are safe.

Some drugs can now be made through genetic engineering, in which the genes (which control a cell's function) of certain microorganisms are altered, changing the products of cell activity to the desired drug. For example, the hormone insulin can now be manufactured by genetically engineered bacteria. This could eliminate the need to extract insulin from animal pancreas glands, the source until recently, benefiting those people who experience adverse reactions to material derived from animal sources.

Purely synthetic drugs are either modifications of naturally occurring ones, with the aim of increasing effectiveness or safety, or drugs developed after scientific investigation of a disease process with the intention of changing it biochemically.

Developing and marketing new drugs

Pharmaceutical manufacturers find new products in various ways. New drugs are usually developed for one purpose but sometimes a variant is found to be useful for something different.

When a new drug is discovered, the manufacturer often undertakes a programme of molecular tinkering, or elaboration. This refers to investigations into variants of the drug to see if a version can be made that is more effective or has fewer adverse effects. In some cases that experimental process has unexpected results. The elaboration process, for example, transformed some sulpha drugs, which were originally valued for their antibacterial properties, into widely used oral antidiabetics, diuretics, and anticonvulsants.

All new drugs undergo a long, careful test period before they are approved for marketing by the Medicines and Healthcare products Regulatory Agency (MHRA) (see Testing and approving new drugs, below). Once approval has been given, the manufacturer can then market the drug under a brand or trade name. Patent protection gives the manufacturer exclusive rights for 20 years, but this protection starts from when the drug is first patented. The time remaining after MHRA approval can be much less than 20 years.

When patent protection ends, other manufacturers may produce the drug, although they must use a different brand name or the generic name (see How drugs are classified, facing page).

Testing and approving new drugs

Before a drug is cleared by the MHRA, it undergoes a cautious, step-by-step period of testing, often lasting six to ten years. By law, a drug must be both safe and medically effective. Safety is established through various means, including tests on animals and human volunteers. Efficacy is proven through complex tests (including double-blind trials) on groups of healthy and ill patients. The testing is done under government-approved procedures.

The approval process also involves weighing a new drug's risks against its benefits. A dangerous drug whose only potential might be the relief of an ordinary headache undoubtedly would not win approval. Yet an equally toxic drug, effective against cancer, might. Medical judgment is an important part of the approval process.

Developing and testing new drugs
All new drugs undergo a rigorous testing period in the laboratory.

DEVELOPMENT STAGES OF A NEW DRUG OR MEDICINE

Discovery Stage A new chemical undergoes thorough chemical and biological study. If these tests suggest it has promise as a new drug, the process of drug development starts.

Preclinical Studies The first stage of research on a new chemical includes testing on isolated organs and tissues and animal studies. These tests are required before permission can be granted for human clinical trials.

Phase I Studies This is the first stage of testing on human subjects, which usually consist of small groups of healthy volunteers, or sometimes patients. The aim is to assess both the safety of the chemical and how the body deals with it.

Phase II Studies The drug is given to patients to confirm that it is likely to be effective, to decide on a dose for the next phase, and to monitor further for possible unwanted or toxic effects. These studies are short-term (single doses or regular dosing for several weeks).

Phase III Studies Large-scale human studies are carried out to gather sufficient evidence of the drug's efficacy and safety to gain marketing approval. They must be long-term (about a year) double-blind, multi-centre, controlled trials.

Phase IV Studies Once the drug is on the market, further monitoring studies are needed to provide ongoing evidence of its overall effectiveness, safety and usefulness.