

WHAT ARE MEDICINES?

Medicines are compounds which affect the body in a therapeutic way. They may provide something that is deficient in the body (e.g. a vitamin), affect the way cells function (e.g. by increasing or decreasing a cellular activity), or they may kill or inhibit harmful organisms such as bacteria (e.g. antibiotics). Many medicines originally came from plants or animals, but now most medicines are manufactured by chemical processes.

Before a new medicine can be used in humans it has to be tested in the laboratory, including in animals so that scientists fully understand the way the medicine behaves in living bodies. This pre-clinical stage may take 3-4 years before the drug is ready to be tried in human volunteers.

CLINICAL TRIALS

Clinical trials are essential to test whether a treatment or prevention measure is safe and effective for use in people. Trials are carried out following a strict protocol which has to be approved by an independent ethics committee before the trial begins to ensure that it is ethical and the patient's rights will be protected.

A clinical trial often tests a new or experimental treatment against a placebo; an inactive pill, liquid or other substance that resembles the drug being tested but has no treatment value. People who participate in the trial may be divided into groups and some will receive the treatment being tested and others receive the placebo. This is important to decide whether the treatment is truly having an effect or if any changes observed are due to other factors such as the expectations of patients or doctors. When both the researchers and participants involved in the trial are unaware whether they are receiving a placebo or the active treatment it is called a 'double-blind' trial.

Clinical trials usually progress through the following stages:

- **Pilot study**

This is a very small study carried out to see if it would be worth doing a larger study and what problems might arise. A pilot study might last a few weeks to a few months. If the initial results are promising, a drug may go on to the next stages.

- **Phase I**

A small group of around 20-80 people, given the treatment in escalating doses, in order to evaluate its safety and to identify side effects and the best dose to use.

- **Phase II**

The treatment is given to a larger group of people, around 100-300, over a longer period of time (up to 2 years) to further evaluate its safety and look for beneficial effects.

- **Phase III**

Large trial with 100's-1000's of patients taking either the drug or a placebo substance over a long period of time (3-5 years) in order to prove whether the treatment is effective and compare it to other treatments.

- **Phase IV**

This phase is when the drug is in open use on the market and available to a much wider population. As many more people are exposed to the drug over a longer period of time additional information on its side effects, benefits, and optimal use might emerge.

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Many trials are funded by pharmaceutical companies as they need to test their drug products in order to gain a license for the drug to be sold and used by the general public. The cost of developing a new drug through clinical trials is enormous; the average cost for a single new drug from discovery to launch estimated at around \$1.7 billion.

Whilst there are currently very few trials for the ataxias, as scientists identify new potential treatments this will change, and pharmaceutical companies may become increasingly involved in ataxia-related research as they provide the funding to carry out extensive trials and manufacture the eventual product that will be used by patients.

DRUGS FOR RARE DISEASES

Orphan drugs is a term given to medicinal products intended for use in rare diseases, which are those affecting less than 1 in 2000 people or a maximum of 250,000 people in the European Union. The ataxias fall into this category, with the most common, Friedreich's ataxia, thought to affect 1-2 in 50,000 people.

Normally it would not be in the interests of a pharmaceutical company to focus on developing orphan drugs as the small target population for the drug means the product sales will probably not recover their costs in bringing the product to the market. To encourage development, special legislation for orphan drugs (the Orphan Drug Act) has been brought in by governing bodies across the World, which gives companies financial rewards for developing drugs for rare conditions or for children.

The European Parliament has regulations on Orphan Medicinal Products which provide incentives such as reduced licensing fees. A product which has been designated as an orphan drug is eligible for rewards and assistance towards faster development. This is designed to improve treatments for people with rare diseases and make new and potentially beneficial drugs more quickly available to them.

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