Drug Trial Phases

There are five sequential phases in a drug trial, each with a different purpose and population size. The phases start from small scale Phase 0 trials which discern pharmacological properties in healthy volunteers, followed by Phase I studies were safety and response in patients with the disease of interest is evaluated. In Phase II, efficacy and side effect identification is completed, leading to Phase III trials where the new drug is compared with standard of care or placebo through randomized control trials. Finally, Phase IV trials represent post-marketing studies conducted after a drug is approved for use by the FDA for surveillance and evaluation of pharmacoeconomics.

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Phase 0

Phase 0
Phase (0)
Phase 0 is useful to eliminate drugs that don't measure up to even the most rudimentary requirements, saving time and money. Likewise it smooths the approval process to move on to the next drug trial phases in order to register a new compound.

Pharmacological Properties
Pharmacological Property Registration
The objective of Phase 0 trials is to show whether a drug's pharmacokinetics and pharmacodynamics warrant continued exploration. However, it will not demonstrate any therapeutic effect nor will it replace the dose escalation, safety, and tolerance studies required in Phase I.

Healthy Volunteers
Healthy Volunteer
In Phase 0 the drug is only given to a very small group of healthy volunteers, usually less than 15 individuals, who are administered a microdose, less than 1% of the therapeutic dose of the drug in question, for no more than seven days.

Phase I

Phase I
Phase (1) Wand
In Phase I trials, researchers test an experimental drug or treatment in a small group of people, around 20 to 100 people, for the first time. Around 70% of drugs move to the next phase.

Safety
Ensure Safety Net
The objective is to evaluate the drug's safety, determine a safe dosage range, and identify side effects.

Patients with Disease of Interest
Diseased-guy
Phase I studies are usually conducted in patients with the disease of interest. The goal here is to determine what the drug's most frequent side effects are and, often, how the drug is metabolized and excreted. The number of subjects typically ranges from 20 to 80.

Phase II

Phase II
Phase (2) Tutu
Phase II studies begin if Phase I studies don't reveal unacceptable toxicity. While the emphasis in Phase I is on safety, the emphasis in Phase II is on efficacy. Phase II usually lasts months to 2 years, and 33% of drugs move to the next phase.

Efficacy and Side Effects

Efficacy-elephant vs Cider-effects

Phase II aims to obtain preliminary data on whether the drug works in people who have a certain disease or condition. For controlled trials, patients receiving the drug are compared with similar patients receiving a different treatment, usually an inactive substance (placebo), or a different drug. Safety continues to be evaluated, and short-term side effects are studied. Typically, the number of subjects in Phase II studies ranges from a few dozen to about 300.

Phase III

Phase III

Phase (3) Tree

Phase III studies begin if evidence of efficacy is shown in Phase II. Phase III trials evaluate efficacy using more subjects and comparing the treatment group vs the standard treatment or vs placebo. The number of subjects usually ranges from several hundred to about 3,000 volunteers. Phase III usually lasts 1 to 4 years. Only 25-30% of the drugs move to phase IV.

Comparison with Standard of Care or Placebo

Standard Care-bear and Sugar-pill

The purpose of Phase III is to evaluate how the new medication works in comparison to existing medications for the same condition.

Randomized Control Trial

Randomized Controller

Comparisons are achieved through double-blind randomization of volunteers into two groups where they receive the novel drug vs the comparison existing medication. Double-blind methodology helps to eliminate bias when interpreting results.

Phase IV

Phase IV

Phase (4) Fork

Phase IV trials are post-marketing studies conducted after a drug is approved for use by the FDA. They are usually large studies, including several thousand volunteers with the disease/condition.

Surveillance

Surveillance-camera

The goal of Phase IV trials is to provide additional information including the treatment or drugs risks, benefits, and best use.

Pharmacoeconomics

Pharmaceuticals + Money Bag Prize

These post-marketing requirement and commitment studies are sponsored and conducted after the FDA has approved a product for marketing. The FDA uses these studies to gather additional information about a product's safety, efficacy, or optimal use.