antidote

CHAMP
Coalition for Headache and Migraine Patients

Part two: Clinical trial phases

Clinical trials test whether potential new treatments are safe and effective for patients through carefully-designed research studies.

In order for a new treatment to reach patients, it must successfully pass several clinical trial phases, which involve increasing numbers of participants for each stage. New treatments can't be approved by the Food and Drug Administration (FDA) without volunteers taking part.

What are the phases of clinical trials?

Pre-clinical testing: Before trials start

Before researchers begin testing a potential new treatment in people, extensive research is conducted to decide whether the drug is a good candidate for clinical trials.

Testing includes in vitro (in a test tube or cell culture), in vivo (animal), or computer modeling experiments.

Preclinical research may take anywhere from one to six years.

0
volunteers

1 - 6
years

0

Phase 0 trials

Phase 0 trials are a non-required exploratory phase in which researchers test less than 1% of the dose of the investigational treatment in a very small group of volunteers to understand whether it may work in the body as expected.

less than 20 volunteers

less than 1% dose

1

Phase 1 trials

Phase 1 trials measure the safety – not effectiveness – of a potential treatment. Sometimes, only healthy volunteers participate.

Around 70% of treatments move to the next phase.

20-100 volunteers

Up to **1 year**

2

Phase 2 trials

Phase 2 trials enroll volunteers living with the condition being studied. They measure a potential treatment for effectiveness.

Around 33% of treatments move to the next phase from here.

If researchers want to learn more about an investigational treatment before moving on to Phase 3, they may conduct a Phase 2b trial.

Several hundred volunteers

Up to **2 years**

3

Phase 3 trials

If the drug shows positive results, the sponsor applies to the FDA to have the drug approved. The FDA analyzes data from all previous phases, and if all looks good, the drug is approved to be marketed.

25 to 30% of treatments that enter this phase are approved.

*After a new treatment has been approved, researchers may also conduct Phase 3b trials to learn more about the drug's impact on quality of life, for example.

Hundreds of volunteers

Lasts **several years**

Post-marketing trials

Post-marketing trials take place after a new treatment is approved if the drug developer wants to continue monitoring patients to learn more about the drug's longer-term effects. These studies may also compare the treatment against other already-approved options.

Frequently asked questions?

What about medical device clinical trials?

Medical devices must also be approved for use, but go through a somewhat different process. Devices are tested first for safety and performance in what is called a "pilot" or feasibility study with a small group of patients, typically 10 to 30.

Next, a "pivotal" trial enrolls a larger population of patients, from 150 to 300 participants, to further test safety and effectiveness. Post-approval studies collect long-term information, like post-marketing studies for drug trials do.

How do I find out the phase of a trial I'm interested in?

Whether you're searching for trials on ClinicalTrials.gov or using a clinical trial search tool like Antidote, the trial phase will be included in the listing, along with information about inclusion and exclusion criteria for the trial.

Which phase should I participate in?

Participating in a clinical trial is always a personal decision. Weigh the benefits against the risks when choosing to participate in any trial. For example, a Phase 1 trial is the most likely to offer financial compensation, but also has the most risk because there will have been little to no human testing done before the trial. Whatever your decision, you're always free to leave a clinical trial at any time, for any reason, once you join.

Sources

http://phrma-docs.phrma.org/sites/default/files/pdf/rd_brochure_022307.pdf
http://www.fdareview.org/03_drug_development.php
https://www.fda.gov/forpatients/approvals/drugs/
http://stm.sciencemag.org/content/2/31/31ec76
http://www.psoriasiscouncil.org/docs/chapter_03.pdf