

How Do Clinical Trials Work?

Clinical trials help doctors and researchers to answer questions about a new treatment's safety and effectiveness.



Clinical trial process

The United States Food and Drug Administration (FDA) has a strict approval process for new treatments. This process has a series of steps called phases.

These steps include:

- Preclinical trials
- Investigational new drug (IND) application to the FDA
- Phase I clinical trial
- Phase II clinical trial
- Phase III clinical trial
- New drug application (NDA) to the FDA
- Phase IV post-marketing surveillance after FDA approval

Preclinical trials

Preclinical trials are also known as laboratory studies. Researchers do these studies before clinical trials on humans take place to see if the new treatment could harm people.

There are two types of preclinical trials:

- **In vitro preclinical trials:** Researchers grow living target cells, such as cancer cells, in a test tube or lab dish. Researchers expose these target cells to the new treatment to see what happens.
- **In vivo preclinical trials:** The next step tests how the new drug or treatment works in a whole, living organism. Preclinical trials are also known as laboratory studies. Researchers do these preliminary studies before applying to the FDA and before beginning clinical trial phases. These results suggest whether the new treatment is safe to use.

Investigational new drug (IND) application

Preclinical studies give scientists useful information about how a new treatment works. But the new treatment still needs to be tested in humans.

Researchers must submit a detailed application to the FDA before testing the new treatment in people. This application includes the following details:

- Any preclinical study results
- A detailed plan for the clinical trial (study protocol)
- Information about the drug or device manufacturer
- Information about how the new drug or device is made
- A list of all materials or substances in the new drug or device
- Details about the clinical trial team and location of the clinical trial

Study protocols lay out the design of the clinical trial and answer questions, including:

- How long is the study?
- How many people are in the study?
- What are the [eligibility criteria](#) for study participation?
- Can the control group receive the new treatment when the study is over?
- What is the treatment dosage?
- How is the treatment delivered (by mouth, vein, injection into soft tissues)?
- What standardized steps will the surgeon take if the treatment is surgical?
- What data will researchers collect, how they will collect it, and how often they will collect it?
- How will the researchers analyze the data?

The FDA decides whether to allow the clinical trial based on all available information. The first step of the clinical trial process—phase I—begins only after FDA approval.

Four phases of clinical trials

The new treatment progresses through four distinct phases (I-IV) after obtaining FDA approval of the IND. Each phase differs in number of participants, duration, and trial goals.

Phase	# of clinical trial participants	Clinical trial duration	Clinical trial goals	Approximate % of trials that move to next phase
I	20-100 people	Several months	<ul style="list-style-type: none"> determine best way to administer treatment (through the mouth, veins, or soft tissues) determine the safest possible maximum dose determine any serious bad (adverse) effects of the new treatment 	70% of all phase I trials
II	Several hundred	Several months to two years	<ul style="list-style-type: none"> study safety and effectiveness of new treatment using the maximum beneficial dose from phase I trial fine-tune or adjust treatment dose design appropriate phase III clinical trial protocols identify less common side effects of the treatment 	33% of all phase II trials
III	300-3,000 people	One to four years	<ul style="list-style-type: none"> determine if the new treatment works better than or similar to existing FDA-approved treatments for the same condition provide data supporting that new treatment benefits a specific population of people detect rarer or long-term side effects missed in shorter duration phase I and II trials present evidence to support a change in clinical practice regarding best available treatments allow researchers to submit new drug application (NDA) to FDA 	25-30% of all phase III trials
IV	Several thousand people	As many as several years after FDA approval of NDA	<ul style="list-style-type: none"> allow doctors to prescribe the treatment to the public identify long-term effects (both good and bad) of the treatment monitor post-market safety of the treatment provide a more realistic picture of how the treatment works in people with multiple conditions (not restricted by trial's eligibility criteria) 	N/A

The Drug Development Process, U.S. Food & Drug Administration, www.fda.gov/patients/learn-about-drug-and-device-approvals/drug-development-process

Postmarket studies required by the US Food and Drug Administration for new drugs and biologics approved between 2009 and 2012: cross sectional analysis, BMJ, 2018, www.bmj.com/content/361/bmj.k2031

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