Clinical trials are prospective biomedical or behavioral research studies on human participants designed to answer specific questions about biomedical or behavioral interventions, including new treatments (such as novel vaccines, drugs, dietary choices, dietary supplements, and medical devices) and known interventions that warrant further study and comparison. Clinical trials generate data on dosage, safety and efficacy. They are conducted only after they have received health authority/ethics committee approval in the country where approval of the therapy is sought. These authorities are responsible for vetting the risk/benefit ratio of the trial—their approval does not mean the therapy is 'safe' or effective, only that the trial may be conducted.

Depending on product type and development stage, investigators initially enroll volunteers or patients into small pilot studies, and subsequently conduct progressively larger scale comparative studies. Clinical trials can vary in size and cost, and they can involve a single research center or multiple centers, in one country or in multiple countries. Clinical study design aims to ensure the scientific validity and reproducibility of the results.

Costs for clinical trials can range into the billions of dollars per approved drug, and the complete trial process to approval may require 7–15 years. The sponsor may be a governmental organization or a pharmaceutical, biotechnology or medical-device company. Certain functions necessary to the trial, such as monitoring and lab work, may be managed by an outsourced partner, such as a contract research organization or a central laboratory. Only 10 percent of all drugs started in human clinical trials become approved drugs.

Trials of drugs

Some clinical trials involve healthy subjects with no pre-existing medical conditions. Other clinical trials pertain to people with specific health conditions who are willing to try an experimental treatment. Pilot experiments are conducted to gain insights for design of the clinical trial to follow.

There are two goals to testing medical treatments: to learn whether they work well enough, called "efficacy", or "effectiveness"; and to learn whether they are safe enough, called "safety". Neither is an absolute criterion; both safety and efficacy are evaluated relative to how the treatment is intended to be used, what other treatments are available, and the severity of the disease or condition. The benefits must outweigh the risks. For example, many drugs to treat cancer have severe side effects that would not be acceptable for an over-the-counter pain

medication, yet the cancer drugs have been approved since they are used under a physician's care and are used for a life-threatening condition.

In the US the elderly constitute 14% of the population, while they consume over one-third of drugs. People over 55 (or a similar cutoff age) are often excluded from trials because their greater health issues and drug use complicate data interpretation, and because they have different physiological capacity than younger people. Children and people with unrelated medical conditions are also frequently excluded. Pregnant women are often excluded due to potential risks to the fetus.

The sponsor designs the trial in coordination with a panel of expert clinical investigators, including what alternative or existing treatments to compare to the new drug and what type(s) of patients might benefit. If the sponsor cannot obtain enough test subjects at one location investigators at other locations are recruited to join the study.

During the trial, investigators recruit subjects with the predetermined characteristics, administer the treatment(s) and collect data on the subjects' health for a defined time period. Data include measurements such as vital signs, concentration of the study drug in the blood or tissues, changes to symptoms, and whether improvement or worsening of the condition targeted by the study drug occurs. The researchers send the data to the trial sponsor, who then analyzes the pooled data using statistical tests.

Examples of clinical trial goals include assessing the safety and relative effectiveness of a medication or device:

- On a specific kind of patient
- At varying dosages
- For a new indication
- Evaluation for improved efficacy in treating a condition as compared to the standard therapy for that condition
- Evaluation of the study drug or device relative to two or more already approved/common interventions for that condition

While most clinical trials test one alternative to the novel intervention, some expand to three or four and may include a placebo.

Except for small, single-location trials, the design and objectives are specified in a document called a clinical trial protocol. The protocol is the trial's "operating manual" and ensures all researchers perform the trial in the same way on similar

subjects and that the data is comparable across all subjects. In 2025 the SPIRIT group released 'SPIRIT 2025', an updated guideline for trial protocols that provides a 34-item minimum checklist, adds a new open-science section, strengthens guidance on reporting harms and intervention/comparator descriptions, and includes a new item on patient and public involvement. As a trial is designed to test hypotheses and rigorously monitor and assess outcomes, it can be seen as an application of the scientific method, specifically the experimental step.

The most common clinical trials evaluate new pharmaceutical products, medical devices, biologics, diagnostic assays, psychological therapies, or other interventions. Clinical trials may be required before a national regulatory authority approves marketing of the innovation.

Trials of devices

Similarly to drugs, manufacturers of medical devices in the United States are required to conduct clinical trials for premarket approval. Device trials may compare a new device to an established therapy, or may compare similar devices to each other. An example of the former in the field of vascular surgery is the Open versus Endovascular Repair (OVER trial) for the treatment of abdominal aortic aneurysm, which compared the older open aortic repair technique to the newer endovascular aneurysm repair device. An example of the latter are clinical trials on mechanical devices used in the management of adult female urinary incontinence.

Trials of procedures

Similarly to drugs, medical or surgical procedures may be subjected to clinical trials, such as comparing different surgical approaches in treatment

of fibroids for subfertility. However, when clinical trials are unethical or logistically impossible in the surgical setting, case-controlled studies will be replaced.

Patient and public involvement

Besides being participants in a clinical trial, members of the public can be actively collaborate with researchers in designing and conducting clinical research. This is known as patient and public involvement (PPI). Public involvement involves a working partnership between patients, caregivers, people with lived experience, and researchers to shape and influence what is researcher and how. PPI can improve the quality of research and make it more relevant and accessible. People with current or past experience of illness can provide a different perspective than professionals and compliment their knowledge. Through their personal knowledge they can identify research topics that are relevant and important to those living with an illness or using a service. They can also help to make the research more grounded in the needs of the specific communities they are part of. Public contributors can also ensure that the research is presented in plain language that is clear to the wider society and the specific groups it is most relevant for.