

Demystifying Clinical Trials: What Are They?

If you have a serious disease or medical condition, chances are at some point your doctor, or even just well-meaning acquaintances, will raise the subject of *clinical trials*. That phrase alone may sound a bit intimidating and mysterious: just what is a clinical trial and why should you bother getting involved in one?

The answer is both simple and complex. A clinical trial is a research study related to the physical health of human beings that has a set of scientific rules it must follow. It gets complicated because of the many different types and phases of clinical trials, which are described below. All clinical trials, however, share a common goal: to find new medication, therapies, procedures, or other ways to help treat illness. If a clinical trial proves it's effective and safe, a government agency gives its official stamp of approval for the general public.

"Imagine if clinical trials didn't exist," says [John Chabot](#), *Chief, Division of GI/Endocrine Surgery and Director, Pancreas Center, New York-Presbyterian Hospital/Columbia University Medical Center*. "We wouldn't have chemotherapy or laser surgery or even ibuprofen. Clinical trials are the only way to keep improving the quality of medical care and to turn medical innovation into a day-to-day reality for our patients. It may take a long time, but the effort is more than worthwhile."

Different Types of [Clinical Trials](#)

- **Prevention Trials** test ways to keep people who have never had a disease from getting it or to prevent a disease from returning. Prevention may include medicine, vaccines, supplements, or changes in diet, exercise, sleep, smoking, and so forth.
- **Diagnostic Trials** search for better procedures or tests for diagnosing a disease or disorder.
- **Screening Trials** test the best way to detect certain diseases or health conditions.
- **Treatment Trials** evaluate experimental treatments, new combinations of drugs, or innovative approaches to surgery or radiation therapy.
- **Quality of Life Trials** look for ways to decrease pain and increase well-being and comfort for those with a chronic illness.

Clinical trials usually last for months or even years and involve four main phases, each with its own aim. Hospitals, drug or device companies often conduct larger, later phase trials, whereas early phase trials may be conducted by small groups of physicians or even individuals.

In *Phase 1 trials*, new drugs or treatments are tested on a small number of patients to assess safety, the right dosages, and side effects. The trial group ranges between 20 and 80 people.

Phase II trials test the drug or treatment on a larger number of people (100-300), again for safety and to see how effective it is.

Phase III trials make a dramatic leap in numbers, up to between 1,000 and 3,000 people. The study tries to validate the experimental drug/treatment effectiveness, continue checking side effects, and gather further information about safe usage. Phase III also compares the new approach with more conventional treatment to determine how much benefit may be gained. Once a new treatment completes phase III trials, the Food and Drug Administration (FDA) may approve it to be offered to the public.

In *Phase IV trials*, which are done after the drug or treatment is marketed to consumers, more data on risks, benefits, and best possible use is gathered.