City of Hope

What You Should Know About

CLINICAL TRIALS

Clinical trials are **carefully controlled** and **federally monitored** research studies on **drugs, procedures** or **other treatments** that must pass rigorous testing before they are granted government approval.

How do clinical trials work?

THE GOAL

To determine whether:

- **Existing treatments** can be used in new ways
- **New treatments** work better than standard therapies

THE RESEARCH

Researchers study whether the treatments:

- Are effective in treating cancer
- Work better than existing therapies used to treat a particular cancer
- Are safe for patients
- Cause side effects, and if so:
  - Identify side effects
  - Measure how severe they are

THE PROCESS

Before a clinical trial can be conducted on people, the research is tested thoroughly in a laboratory, on **cells** and **animals.**

If the U.S. Food and Drug Administration (FDA) grants permission to conduct the study on people, **the clinical trial stage is generally performed in four steps, or phases.**

	PHASE I	PHASE II	PHASE III	PHASE IV
GOAL	To determine the safest maximum dose	To determine safety and effectiveness	To determine whether the new treatment works better than existing treatments	To measure long-term safety and effectiveness, once FDA-approved for market
LENGTH	Several months	Several months to 2 years	1 to 4 years	Several years
AVG. SIZE	**10 to 30** people	**30 to 120** patients	**More than 300** patients	**Thousands** of patients
SUCCESS RATE	About **70% of drugs** move to Phase II	About **33% of drugs** move to Phase III	About **25-30% of drugs** move to approval	APPROVED
DETAILS	Slowly increases doses over time Determines how the treatment should be delivered (i.e. orally, intravenously)	Gives different treatment protocols to different patients (i.e. varying by dose or combination of therapies) Measures the treatment's impact on a certain cancer	May divide patients into two groups: 1. Control group (getting the standard treatment) 2. Study group (getting the new treatment) Are typically randomized (doctors do not choose which patients get which treatments)	Studies long-term results Determines cost-effectiveness Collects information on side effects

What about placebos?

While common in other areas of research, placebos (inactive drugs or treatments) are very rarely used in cancer treatment clinical trials, and only when no standard treatment exists. In all other cases, rather than a placebo, control group patients receive the standard treatment for comparison.

Who qualifies for clinical trials?

Patient participants are volunteers—they enroll in clinical trials only if they choose to, and they may decide not to participate at any time during the trial. Clinical trials are only available to patients who meet certain criteria, which vary widely from trial to trial. For those who do qualify, deciding whether to participate is a personal decision made between those patients and their doctors.

Who looks out for the patient?

A clinical trial must meet a set of rigorous standards to protect participating patients and ensure the study's results are accurate and comprehensive.

The trial must follow an **established process** detailing eligibility, timelines, measurement criteria and enrollment.

Each trial is led by a **doctor** who collaborates with a research team of nurses, researchers and other health care clinicians.

An **Institutional Review Board (IRB)** monitors the trial to makes sure it is safe and ethical. The IRB is an independent committee of professionals, including doctors, nurses, lawyers, patient advocates and other community leaders.

The **FDA** oversees clinical trials involving new drugs or new drug uses and evaluates the accuracy of the records involved.

What are the benefits and risks?

Participating in a clinical trial may help patients by:

- Offering access to treatments that may not have otherwise been available to them
- Providing the support of a team of cancer experts who are closely monitoring their progress and overall health
- Delivering personalized, expert care specific to their disease
- Providing comfort in knowing that their participation may benefit other cancer patients

Risks of participating in a clinical trial may include:

- The treatment may cause unexpected side effects.
- Patients may experience side effects that are worse than those experienced with standard treatments.
- The treatment may not work on the cancer, even if it works for other patients.
- The treatment may not result in better outcomes than standard treatments.

Clinical trials by the numbers

patient
The smallest **number of patients** needed for a clinical trial

(Also called N=1, or "the N of 1," single-participant trials are a growing trend in the field of genomic medicine, which analyzes the unique mutations that may be driving a patient's tumor, in search of a treatment designed to target those abnormalities.)

years
How long a drug is **studied,** on average, before it makes it to the clinical trials stage

years
How long it takes, on average, from the time a cancer drug enters clinical trials until it's **approved**

55 cancer drugs
The number of cancer drugs the FDA **approved** in 2021, following extensive clinical trials

"At Cancer Treatment Centers of America® (CTCA), we believe very strongly in clinical trials as an option for our patients. We've learned many of the things that are now standard of care from clinical trials, and I believe that is important to see if there are any potential clinical trials for a patient."

- Pamela Crilley, DO, Chair of the Department of Medical Oncology at CTCA®

SOURCES
U.S. National Institutes of Health, National Cancer Institute, U.S. Food and Drug Administration, American Society of Clinical Oncology, American Cancer Society

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