

The Basics: CLINICAL TRIALS



What is a Clinical Trial?

A clinical trial is a research study conducted with volunteers to evaluate the safety and efficacy of new treatments and other medical advances. These trials follow structured protocols to ensure reliable and valid results that can inform medical practice and regulatory decisions. Clinical trials involve both patients and healthy volunteers. The pharmaceutical industry is the major funder of clinical trials.

According to a survey commissioned by Research!America, in partnership with the Association of Clinical Research Organizations, sixty-one (61%) percent agree that consideration of clinical trial participation should be a part of regular health care whether patients are healthy or ill, up from 44% in 2017.

Food and Drug Administration's Role in Clinical Trials:

As the safest and fastest way to discover new treatments and improve health, clinical trials follow strict FDA rules to protect participants and achieve meaningful efficacy data. The protocol outlines patient criteria, test schedules, drug dosages, study duration, and research goals.

Before deciding whether to approve a new prescription drug or other medical advance, the FDA evaluates the data from clinical trials to evaluate whether potential new treatments and technologies are safe and effective.



Phases of Clinical Trials

- ✓ **Phase I:** Examines the safety of the product in a very small group of healthy volunteers. Approximately 70% of drugs make it past Phase I, which typically involves 20–80 participants.
- ✓ **Phase II:** Assesses the efficacy and correct dosing in a larger group of individuals. Approximately 33% of drugs make it past this stage, with trials involving 100–300 participants.
- ✓ **Phase III:** Tests the product in a much larger, more diverse population to confirm efficacy, monitor potential side effects, and develop usage guidelines. Up to 30% of drugs make it past this point, with trials generally involving 1,000–3,000 individuals.
- ✓ **Phase IV (Post-Market Safety Monitoring):** continues to assess safety in the first few months to years after a drug's approval.

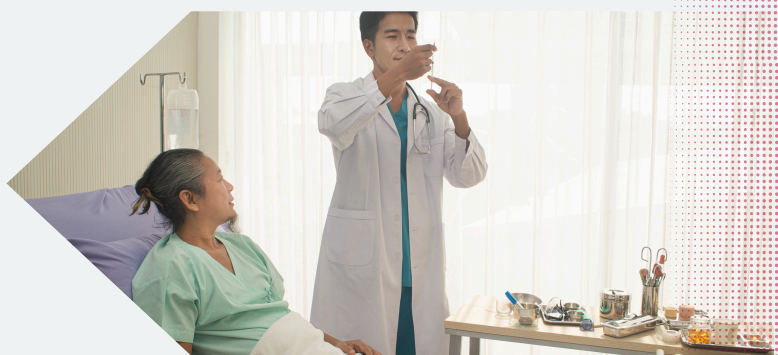
Challenges and Opportunities



Clinical Trial Participation

In 2020, studies showed that 41% of Americans reported not knowing about clinical trials (Yadav et al., 2022). Further, according to a Research!America commissioned survey, only 26% of Americans have participated in or know a family member who has participated in a clinical trial.

The patient and public-private sector research community are working to address challenges impeding participation, which is critical to crossing the research & development "finish line" so that more patients receive the medical advances they need.



Diversity

While people of color make up 30–40% of the U.S. population, recent data indicate they are significantly underrepresented in clinical trials: only 8% of clinical trial participants are Black, 6% of participants are Asian, and 11% of participants are Hispanic. This disparity underscores the ongoing challenge of ensuring diverse participation in medical research, an issue that has worsened since 2019 (Embracing Diversity: The Imperative for Inclusive Clinical Trials, 2023).

Addressing this imbalance is crucial in developing inclusive and effective treatments, as drug interventions can work differently in individuals across various racial or ethnic backgrounds. Studying a diverse population in clinical trials is essential for advancing medical science.



Artificial Intelligence

Artificial intelligence has the potential to revolutionize clinical trials. AI can assist with clinical trial design by answering questions such as: what is the right drug dosage? How many patients are needed to conduct a trial?

AI can also help analyze massive datasets. Studies indicate that AI can help reduce the number of patients and the time needed to complete a clinical trial, leading to a more efficient process and quicker access to life-saving drugs for patients.



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