Clinical Trial Basics

Clinical trials evaluate the safety and efficacy of investigational medical, surgical, or behavioral interventions for a specific indication. Clinical trials are carefully designed and can take several years to complete. The safety and efficacy of investigational treatments are tested in 4 phases.

Phase	Trial specifics
1	• Generally performed in a small group of healthy participants • Addresses basic questions regarding safety • Can be used to determine the maximum tolerated dose to avoid unacceptable side effects
2	• Performed in a larger group of participants in the intended patient population • Evaluates the efficacy of the investigational therapy • Further defines any potential side effects
3	• Usually large studies performed in the intended patient population • Gathers more data on the safety and efficacy profiles of the investigational therapy • Often directly compares the investigational therapy with a placebo, the current standard of care, or another therapy already available on the market • Phase 3 studies can also be extended post-approval to expand on analyses (eg, additional subpopulations)
	Results from phase 1-3 trials can be submitted for review to the appropriate regulatory agency, such as the US Food and Drug Administration or the European Medicines Agency, for approval
4	• Generally not required for submission to a regulatory authority and occurs after approval • Often observational studies to gather data on long-term safety and efficacy in the general patient population and can identify unexpected side effects

Importance of Clinical Trials

In the past two decades, there has been a paradigm shift from experience-based to evidence-based practice in medicine. Research, including clinical trials, has been the foundation of this evidence-based approach. Clinical trials provide objective scientific data on key aspects of a potential therapy, including:

- How well the investigational therapy works
- Potential side effects and safety concerns associated with the investigational therapy
- How the investigational therapy compares with the current standard-of-care treatment

With this knowledge, healthcare providers are better equipped to give patients the safest and most effective care.

Potential Benefits of Clinical Trial Participation

Participation in clinical trials can directly benefit patients and healthcare providers.

- Patients can receive potentially lifesaving care
- Healthcare providers can offer patients access to possible new therapies and take part in medical advances

Additionally, participation in clinical trials allows patients and healthcare providers to play an active role in driving medical advancements that may lead to earlier diagnoses, better outcomes, and fewer side effects.

Potential Disadvantages of Clinical Trial Participation

Participation in clinical trials may come with several disadvantages for patients and healthcare providers.

- Although clinical trials must undergo a rigorous review process and receive approval from an institutional review board or ethics committee before initiation, there are unknown safety risks involved with taking an investigational medication
- Clinical trial participation requires additional time and resources from healthcare providers

Reasons to Enroll in a Clinical Trial: Discussion Points

- **Many trials face difficulty recruiting the target number of participants.** Advances in technologies involved in the drug development process have led to a dramatic increase in therapies that need to be tested in clinical trials. Encouraging both patients and healthy volunteers to enroll in clinical trials can help with this challenge and support the advancement of new treatment options
- **It may benefit certain patients.** Clinical trials may present a new option to patients who have not responded to or tolerated other therapies. Clinical trials may also provide a treatment option for a patient with a rare disease or condition for which there is no currently approved therapy

? Questions From Healthcare Providers

"Will I lose my patient to the clinical trial investigator?"

Patients who participate in a clinical trial typically continue to see their regular healthcare provider. The healthcare provider can work closely with the research team to ensure that the clinical trial will not interfere with any treatments or medications the participant receives as part of their usual care.

"My patient is really sick. What if they receive a placebo?"

Patients are commonly randomized to different treatment groups and may not receive the investigational therapy. However, patients receive close medical attention and thorough follow-up care when participating in clinical trials.

For additional information regarding Otsuka clinical trials, please refer to your Otsuka Medical Science Liaison.

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