Innovation with Integrity: Inside Clinical Trials

What Is a Clinical Trial?

Clinical trials are research studies performed in people that are aimed at evaluating a medical, surgical, or behavioral intervention. They are the primary way to understand if a new treatment, such as an investigative treatment, diet, or medical device (such as a pacemaker) is safe and effective in people. A clinical trial may learn if a new treatment is more effective and/or has less side effects than the standard or current treatment(s).

Clinical Trials Are Conducted in Phases:

Phase 1: **Is it safe?**

- Focus: **Safety and dosage**
- Conducted with a small number of healthy volunteers
- Researchers test how the treatment interacts with the body
- Identifies the safest dose and any side effects

(Takes several months)

Phase 2: **Does it work?**

- Focus: **Effectiveness and side effects**
- Tests treatment in people with the condition being studied
- Collects data on how well it works and its safety profile

(Takes several months - 2 years)

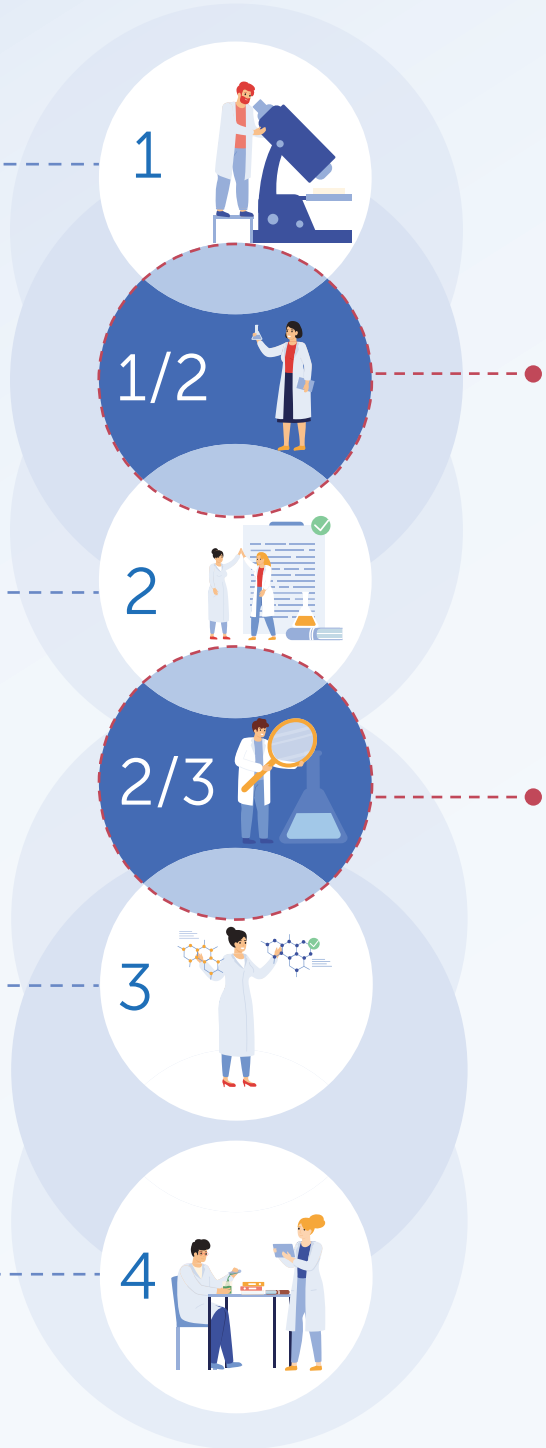
Phase 3: **Is it better than what's available?**

- Focus: **Effectiveness**
- Compares new treatment to the current standard of care
- Involves hundreds to thousands of participants with the condition being studied

(Typically lasts 1–4 years)

Phase 4: **What else do we need to know?**

- Focus: **Long-term safety**
- Monitors how the treatment works in the real world
- Collects additional information on long-term safety, benefits, and quality of life



Phase 1/2: **Safety + Early Effectiveness**

- Focus: **Safety and early signs of effectiveness**
- Combines the goals of Phases 1 and 2
- Starts with small doses to find the highest dose that is safe
- Once safety is confirmed, more participants are added
- Looks for early signs of effectiveness
- Runs continuously with ongoing safety monitoring

Phase 2/3: **Refining + Confirming**

- Focus: **Performance and safety**
- Combines Phases 2 and 3 into one streamlined process
- Tests multiple doses to find the most effective and safest one
- Removes doses that cause too many side effects and/or that lack efficacy
- Expands testing with more participants
- Confirms both safety and effectiveness without breaks between phases

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