

# Clinical Trial Facts for People With Blood Diseases & Disorders

Clinical trials are medical research studies that use volunteers to help find safe, effective ways to prevent, detect, or treat diseases. The National Heart, Lung, and Blood Institute leads and supports many clinical studies, including those aimed at preventing, diagnosing, and treating blood diseases and disorders.

## Clinical Trial Phases

Researchers conduct clinical trials in four phases:



### Phase 1

A small group of people (20–80) test the medicine or treatment to help researchers begin learning about its safety, side effects, and ideal dosage.



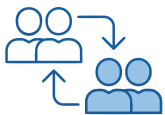
### Phase 2

A larger group of people (100–300) help researchers determine the medicine's effectiveness and allows them to further study its safety.



### Phase 3

An even larger group of participants (1,000–3,000) test the treatment, allowing researchers to confirm its effectiveness and safety, monitor side effects, and compare it with standard or similar treatments or a placebo (an inactive product with no treatment value).



### Phase 4

After the U.S. Food and Drug Administration (FDA) approves the medicine or treatment for public use, researchers track its safety in the general population, seeking more information about its benefits and best uses.



## Questions To Ask Your Doctor and the Research Staff

If you have a blood disease or disorder and are interested in participating in a clinical trial, talk to your healthcare provider to see if you're eligible. Learn as much as possible about the study by asking questions such as these:

- What is the purpose of the study?
- What are the possible short- and long-term benefits for me?
- What are the short- and long-term risks and possible side effects of the medicine I'm testing?
- How do the medical tests in the study compare with those I would get outside of the trial?
- Where will I get my medical care?
- How could being in this study affect my daily life?
- Will I have to pay for any part of the trial?

Children, adults, patients, healthy volunteers, and people from diverse ethnic and racial backgrounds can and are encouraged to participate in clinical trials.

Learn more about the importance of clinical trials, including their benefits and risks, at [nhlbi.nih.gov/research/clinical-trials/how-studies-work](https://nhlbi.nih.gov/research/clinical-trials/how-studies-work)

