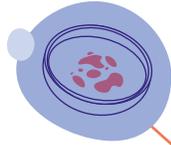


# What Are Clinical Trials?

Clinical trials research the way a drug or treatment will interact with the human body. Each phase of the process is helping answer more questions to better understand the treatment.

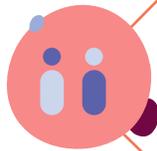


## Preclinical Study

Tests the treatment in cells or animals, looking for positive or negative effects before it can move on to be tested in humans. These studies can take several years to collect enough data.

## IND Application

Researcher submits preclinical study data and possible trial design in an Investigational New Drug application. The U.S. Food and Drug Administration (FDA) carefully reviews to see if it should move on to a clinical trial.

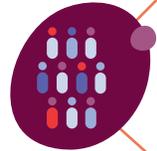
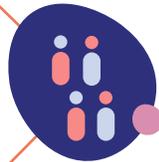


## Phase 1

Tests if the therapy is safe for a small number of participants and can sometimes determine the amount of treatment to be given (dosage).

## Phase 2

Expands the number of participants receiving the treatment to see if benefits continue to outweigh risks.

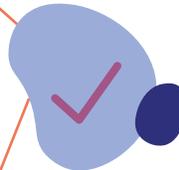


## Phase 3

The longest phase with the most participants, in hopes of showing the therapy has the desired result while being safe.

## FDA Final Approval

Biologics License Application (BLA) is submitted to the FDA if the final phase shows the treatment to be safe and effective. The FDA makes a final decision on whether to approve the treatment for use by anyone who fits the disease criteria, also known as an indication.



## Phase 4

After receiving a treatment in a clinical trial, years of follow-up appointments are required. Researchers need to continue to monitor safety and long-term outcomes.

## Combining Phases

In gene and cell therapy studies, phases 1 and 2 are often combined to a single phase study to test for safety, and efficacy within a smaller number of participants with a disease. This is sometimes done for serious and rare diseases for which there is a clear unmet medical need. Also, the FDA provides various Expedited Pathways to speed up the process for these diseases while still being safe.