

How New Therapies Get Tested in Clinical Trials



CLINICAL TRIALS PHASES



DURATION OF STUDY



NUMBER OF PEOPLE ENROLLED*



STUDY PURPOSE



PERCENT OF TREATMENTS MOVING TO NEXT PHASE



SEVERAL MONTHS

~10-30
 SMALL NUMBER
 of people with hemophilia

SAFETY DOSE RANGE

±70%
 OF STUDIES ARE SUCCESSFUL
 and move to Phase 2



SEVERAL MONTHS TO 2 YEARS

~10-50
 SLIGHTLY MORE
 people with hemophilia

SAFETY EFFICACY DOSE SELECTION

±30%
 OF STUDIES ARE SUCCESSFUL
 and move to Phase 3



1 TO 4 YEARS

~50-150
 LARGER GROUP
 of people with hemophilia

SAFETY EFFICACY

±25%
 OF STUDIES ARE SUCCESSFUL
 and move to Phase 4

IF SUCCESSFUL

DATA FROM CLINICAL TRIAL SUBMITTED TO REGULATORY AGENCY
 (e.g., FDA, EMA)

REGULATORY APPROVAL



SEVERAL YEARS

MORE THAN 100
 People with hemophilia enrolled

LONG TERM FOLLOW-UP

NEW THERAPY
 available to people with hemophilia

*The number of people with hemophilia enrolled in clinical trials is smaller than in other disease states because hemophilia is a rare disease.