Clinical Trials

Your first steps on the path to understanding clinical trials start here

Participating makes new treatments possible

Voyager THERAPEUTICS

Why Do We Need Clinical Trials?

- To study what can cause a disease
- To study ways to better diagnose a disease
- To study ways to treat a disease or control its symptoms
- To study how comfort and quality of life can be improved
- To study changes in a disease over time

Why Participate in a Clinical Trial?

- To better understand your disease
- To help develop new treatments or better understand current treatments
- To make a difference in the care that people receive in the future

It's important! Clinical trials need participants that represent all individuals that are affected by a disease

How an Investigational Treatment is Studied Before it Can be Used as a Treatment Option

Clinical trials are usually done in phases that build on one another

1 Phase 1 trials

- Include a small number of participants, sometimes people with no disease
- Find the dose or doses that are safe and can be studied more
- Study how the investigational treatment moves through the body (pharmacokinetics)
- Study the effects of the investigational treatment and how it acts on the body (pharmacodynamics)
- May be **controlled** and/or **randomized**

2 Phase 2 trials

- Include a larger number of participants
- Continue to study the safety of the investigational treatment
- Keep studying the effects of the investigational treatment and whether it acts as intended
- May be **controlled** and/or **randomized**

3 Phase 3 trials

- Include an even larger number of participants
- Compare the safety and effects of the investigational treatment to a **placebo**, another treatment, or no treatment
- Are **controlled** and **randomized**

! Two phase 3 trials are usually required for an investigational treatment to be approved as a treatment option

- For diseases with no currently approved treatment or that need more effective treatments the approval process may be shortened, or "fast tracked," to make the treatment available sooner

4 Phase 4 trials

- Take place after the investigational treatment is approved as a treatment option
- Continue studying the treatment as it is used by people with the disease to gather information on long-term benefits and potential safety concerns

i Definitions

Controlled: a clinical trial that includes a comparison (control) group. Participants in the control group may receive a placebo, another treatment, or no treatment

Placebo: a control treatment that is designed to have no effect on the participant, but looks the same and is delivered the same way as the investigational treatment (for example, a "sugar pill")

Randomized: when participants are placed in a trial group based on chance (for example, a coin flip)

Common Questions Answered

Things You May Consider

Who Can Participate?

- Every clinical trial has rules for who can or can't participate. These are called the inclusion (can) and exclusion (can't) criteria
- These rules increase the likelihood of getting higher-quality data and decrease the likelihood of harm to participants

What Are the Possible Risks of Participating?

- The investigational treatment may not work as intended
- The way the treatment is given may have possible risks (for example, a surgical procedure)
- There may be side effects, or side effects that are worse than expected
- In a **controlled** trial, you may not receive the investigational treatment

What May I Be Asked to Do?

- You may be asked to travel to a different clinic or hospital than where you usually see your doctor
- Clinical trial visits may be more frequent than your usual doctor visits
- You may have more tests than you normally do
- The trial may require some uncomfortable tests (for example, taking blood)

Who Conducts Clinical Trials?

- A clinical trial is usually led by a principal investigator (PI), often a medical doctor, and a team that can include other doctors, nurses, and staff
- Trials are funded by one or more sponsors. Sponsors may include:
  - Federal agencies such as the National Institutes of Health (NIH)
  - Companies making new treatments
  - Academic medical centers
  - Patient advocacy groups

What's Involved?

On the Path Toward a Better Understanding of Clinical Trials

- **After you have done your research and spoken with your health care provider and others you trust, you may decide that you want to participate in a clinical trial**
- **It's important to learn about how clinical trials work: who you might interact with along the way, which questions you may want to ask before consenting to participate in a trial, and what you may need to do during a trial**

1 Getting Started: The People Who Work Together on a Clinical Trial Team

- Clinical trial team members you may meet include doctors, nurses, and trial coordinators
- Someone from the clinical trial team, usually the trial coordinator, will speak with you to discuss your interest in joining the trial
- The trial coordinator will also keep up-to-date contact information for you and will schedule check-ins and visits to the trial site
- The clinical trial team will be available during the entire study to answer your questions and discuss your issues or concerns

2 Informed Consent

- An "informed consent form" explains the details of the trial, including its purpose, length, the tests involved, and potential risks and benefits
  - A person who chooses to participate in a trial, or the person who makes medical decisions for them, must sign the informed consent form before participation in a clinical trial can begin
  - The consent form is not a contract – you can stop participating in a trial at any time after signing it, for any reason
  - Signing the informed consent form does not guarantee that you will be part of the trial
- In addition to the informed consent form there may be separate consent forms for specific trial procedures, such as a surgery

Make sure all your questions are answered before signing the informed consent form

Questions You May Want to Ask About Clinical Trials

General Questions About the Clinical Trial:

- What are the goals of the trial?
- How many participants will be in the trial?
- Is this the first clinical trial for this investigational treatment or has it been studied before?
- Who decides if I can or can't participate in the trial?
- How long will I be in the trial?
- What if I have unexpected reactions during the trial?
- Can I participate if English is not my first language?

Questions About Treatments and Tests During the Trial:

- Will every participant receive the investigational treatment? If not, how are different treatments assigned?
- Will I be told that I have received the investigational treatment? Will members of the clinical trial team know? What about my other doctors?
- What are the possible benefits and risks of the investigational treatment?
- Will I have to stop my current treatment? If not, will the investigational treatment change how my current treatment works?
- What tests will I have before I receive treatment? After I receive treatment?
- Will all the tests happen in a medical center, or will some happen at home?

- Do I take this treatment myself or is it given it to me? How many times do I receive treatment? How often?
  - If the treatment is given to me, how is it done? How long will it take?
  - If the treatment requires a procedure, such as surgery, how long will it take me to recover?
- How long after starting treatment will follow-up visits begin? How many visits will there be?
- Will I have to travel to a different medical center for any of my visits?
- Who will oversee my medical care while I am participating in the trial?
- What if I feel as though the treatment I'm receiving isn't working?

Questions About Cost:

- How much will it cost me to participate in the clinical trial?
- Can I participate if I don't have health insurance?
- Will my health insurance cover some or all treatment costs during the trial?
- Will I be reimbursed if there are out-of-pocket expenses?

Questions About the End of the Trial:

- Will the treatment be available to me after I finish the trial?
- Will I receive the results of the trial when it is finished?

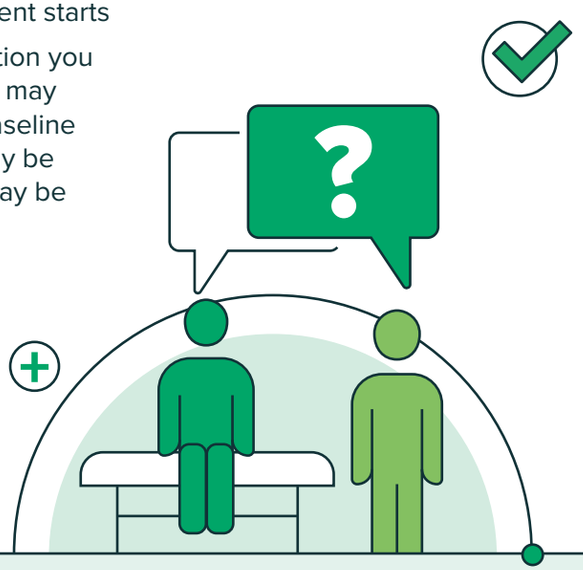
3 What to Expect After Signing the Informed Consent Form

Screening

- Screening is when the clinical trial team determines whether or not you have all of the inclusion and none of the exclusion criteria
- The information collected can include things like demographics (such as age and race), medical history, and current medications
- Some tests may be required and may include a physical exam, a neurological exam, urine and/or a blood samples, a chest X-ray, an electrocardiogram, and questionnaires
- The information may be gathered over one or several visits, depending on the clinical trial

4 Baseline

- The baseline visit is your starting point as an official clinical trial participant and gives a snapshot of your health before treatment starts
- Some of the information you provide at screening may be updated at the baseline visit, certain tests may be repeated, or there may be additional tests



5 Treatment and Follow-up Visits

- Treatment will begin after the baseline tests are completed
- During or after the baseline visit is when your treatment group will be determined
- After starting your assigned treatment, follow-up visits track your progress in the trial
- Tests may be performed during follow-up visits to check the safety and measure any effects of the treatment
  - The number of follow-up visits depends on what kind of data the clinical trial team wants to collect from participants and how long the trial lasts

6 End-of-Study Visit

- The last trial visit often includes tests conducted throughout the trial, as well as during screening or baseline visits
- Your last trial visit doesn't mean that the clinical trial is finished; there may be other participants who have not completed all of their follow-up visits
- This is a good time to ask, "When will the results of the trial will be available?"
- At the end-of-study visit you may be asked if you want to participate in another trial, often called an "extension" trial, which studies the longer-term safety and effects of the investigational treatment

Participants are the most valuable part of any clinical trial!

Your safety and well-being are critical to a successful trial

Protecting the Rights of Clinical Trial Participants

- Institutional Review Board (IRB):
  - Doctors, researchers, and community members who ensure that a clinical trial is well designed, ethical, and that participant rights, safety, and welfare are protected
  - Approves the clinical trial design and informed consent form at each trial site
  - Makes sure that the risk to participants in the clinical trial is minimized and reasonable compared to the potential benefits of the investigational treatment
  - Monitors the trial and makes sure that any side effects or unexpected events are immediately reported to ensure participant safety
- Data and Safety Monitoring Board (DSMB)
  - Also known as Data Monitoring Committee (DMC) or Data and Safety Monitoring Committee (DSMC)
  - Along with the IRB, many clinical trials are closely supervised by a DSMB
  - Made up of experts who are not involved with the clinical trial
  - The DSMB looks at the data as the trial is in progress – they can recommend that a trial be stopped or placed on hold if the treatment is not working, or if there are unexpected risks
  - If trial results are very good, the DSMB can recommend an early stop to reduce testing and stop any control treatments

- Personal and Medical Information
  - It is strictly required that personal and medical information of trial participants be kept confidential during and after the trial
  - Clinical trial data are “de-identified,” so personal information (like your name) cannot be revealed in any reports or publications that present results from the trial

How Do I Find Out More?

Talk to your healthcare provider to learn more about participating in a clinical trial

Reach out to patient advocacy organizations or foundations dedicated to advancing clinical research

Search www.clinicaltrials.gov to learn more about clinical trials that are currently looking for participants

Together, we can discover new treatments

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Additional Resources:

Learn About Clinical Studies. ClinicalTrials.gov website. https://www.clinicaltrials.gov/ct2/about-studies/learn#ClinicalTrials

NIH Clinical Research Trials and You. National Institutes of Health website. https://www.nih.gov/health-information/nih-clinical-research-trials-you/basics

Step 3: Clinical Research. US Food & Drug Administration website. https://www.fda.gov/patients/drug-development-process/step-3-clinical-research

Why Should I Participate in a Clinical Trial? National Institutes of Health website. https://www.nih.gov/health-information/nih-clinical-research-trials-you/why-should-i-participate-clinical-trial