An overview for individuals and caregivers considering participating in a clinical trial

What to Know Before Enrolling in a Clinical Trial

SAREPTA THERAPEUTICS

Clinical Trials: Changing Tomorrow, Starting Today

DID YOU KNOW?

The standards for a treatment to become available are so rigorous that only 14% of treatments that undergo clinical trials are approved by the US Food and Drug Administration (FDA).

Making an informed decision

- This guide was created to give you a better understanding of how clinical trials are developed and what requirements need to be met
- It can help you understand the clinical, practical, and financial considerations when participating in a clinical trial
- It also provides you with important questions that can help you make a more informed decision

Every groundbreaking medicine starts as part of a clinical trial

Before a treatment can be approved for use in the United States, the FDA requires proof showing that it is safe and effective

Clinical trials can provide this proof, in addition to helping us discover new medical knowledge related to diseases and health-related conditions

Clinical trials are controlled experiments to uncover new ways to potentially treat conditions

Eligibility Is Based on Specific Trial Needs

Participating in a clinical trial is a serious commitment and should be discussed with your healthcare provider. Before you sign up, you'll want to learn more to determine whether it's right for you and your family.

Each clinical trial has eligibility requirements, known as inclusion and exclusion criteria, that participants must meet in order to enroll.

Eligibility criteria can include

- Age
- Gender
- The type and stage of disease being studied
- Previous and current treatment history
- Amenability to the clinical trial treatment
- Previous and existing medical conditions
- Results from diagnostic tests

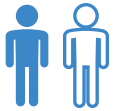
Giving your consent

If you are eligible and decide to participate in a clinical trial, you or your legal guardian will be required to sign an informed consent form. Potential risks and benefits are included on the consent form and should be explained to you by your clinical trial team. Upon providing consent, you may be invited to participate in the clinical trial.

Clinical Trials Can Be Set Up in Different Ways

Single-arm, open-label trials

These trials consist of a single group where all the participants receive the investigational treatment



Randomized, double-blind, placebo-controlled trials

These trials compare the effect of the investigational treatment in one group of participants with a placebo (a pill or liquid that contains no active ingredients) in another group of participants

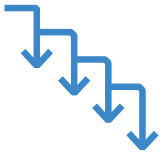
- In a double-blind, placebo-controlled trial, neither the researchers nor the participants will know who is being treated with a placebo or the investigational treatment during the trial
- Using a placebo gives researchers something to compare with the investigational treatment

Head-to-head trials

These trials compare the investigational treatment in one group of participants with an FDA-approved treatment in another group of participants

Clinical trials vary in length

Depending on the nature of the disease, the trial may last from several weeks to many years. Participants are informed of the length of the clinical trial before enrolling.



Traditional clinical trial program

Typically, a clinical trial program has the following phases:

- Research phase confirms that the treatment works in a laboratory environment
- Preclinical phase tests for safety and side effects using animal subjects
- Early clinical phase tests for safety with a small group of human subjects
- Late-stage clinical phase tests that the treatment works with a larger group of human subjects, while providing a better understanding of its safety profile

Accelerated approval program

Accelerated Approval was developed by the FDA to speed the availability of treatments in areas of currently unmet need. There generally will be shorter clinical trials than traditional studies. Treatments granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

Considerations for Clinical Trial Participation

The decision to participate in a clinical trial is personal and should be made in consultation with your healthcare provider. In reaching your decision, consider all perspectives—understanding the potential benefits, as well as the potential drawbacks.

Possible benefits

- Helping to advance possible treatment options for other individuals, present and future, living with the disease
- Access to potentially beneficial treatment
- Feeling that you're taking an active role in your medical care
- Opportunity to receive close medical observation during the clinical trial

Possible drawbacks

- Potential risk of experiencing unknown or serious side effects
- No guarantee that participants will experience any benefit from the treatment under investigation
- Potential assignment to the placebo arm of a clinical trial (if there is one)
- Potential to be excluded from future clinical trials

Learn more about ongoing studies at clinicaltrials.gov.

1 Understand the commitment

- How long will I or my child participate in the clinical trial?
- Does signing a consent form guarantee participation in the trial?
- If I or my child sign the consent form, can we change our mind about participation?
- How much travel would be involved in the clinical trial?
- Are there any costs, such as transportation, that I would be responsible for?
- Does the clinical trial require any inpatient hospital stays?
- How will I or my child be compensated for participating in a clinical trial?
- What evaluations will I or my child undergo during the clinical trial?

3 Understand treatment access when the clinical trial ends

- Will I or my child have access to the investigational treatment after the clinical trial is over?
- Will participation in this clinical trial exclude me or my child from participating in other studies or therapies in the future?

2 Understand how current treatments may be affected

- Will I or my child have to stop current treatment(s) or go through a washout period before starting the clinical trial?
- If I or my child is currently on an approved treatment, should an investigational treatment be considered?
- Will I or my child be able to return to current treatment after the clinical trial; ie, could there be issues related to access?

4 Understand the experience during the clinical trial

- What phase is the clinical trial currently in?
- What safety and efficacy measures have been established with the investigational treatment to date?
- Is there a possibility the disease may progress during the clinical trial (either from being given placebo or in a washout period)?

To learn more about our pipeline, please visit Sarepta.com.

An Informed Discussion Makes for Better Decisions

- Investigational treatments undergo years of rigorous evaluation before they are considered for FDA approval
- Clinical trials bring hope and opportunity to many; however, the potential benefits should be weighed against the possible drawbacks
- Ultimately, participation in a clinical trial is a highly personal decision
- Gather all the facts and make an informed decision with the guidance of your healthcare provider

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