Cell and Gene Therapy for Rare Disease Clinical Trials
FAQs

Cell and gene therapies hold great promise for some rare diseases. After a rare disease is discovered, research continues to better understand the disease and explore possible treatment options - which can include cell and gene therapies. Clinical trials are an important step in helping scientists understand how well a new therapy may work. But the science is complex. Choosing whether or not to enter a clinical trial as a participant requires careful reflection and informed consent.

What are the possible benefits of being in a clinical trial?

- ✓ You may get access to a new therapy at no cost, sometimes years before it is widely available
- ✓ You will be closely monitored for side effects and your health will be monitored by a team of physicians
- ✓ You will be helping other patients and families living with your rare disease

What are the possible risks?

There is always some risk involved in clinical trial participation. Researchers try to balance the possible benefits and risks of the trial. They also conduct studies to see if the treatment is likely to be safe and effective before testing it on people. It's still possible that:

- You could experience side effects
- The treatment might not work for you, even if it works for others
- You might have a reaction that means you have to stop treatment
- Participating in a clinical trial may take more time than the standard care and may involve additional travel
- You could develop a reaction to the vector that delivers the gene therapy
- You could have additional costs associated with a clinical trial, although some of these costs may exist outside of a clinical trial as well.

How will my rights and safety be protected?

Every clinical trial must be approved and monitored by an Institutional Review Board (IRB) to make sure the risks are as low as possible and that the risks are reasonable in relation to potential benefits.

IRBs usually include doctors, nurses, statisticians, community members, and others who try to make sure that your rights are protected.

The IRB approves a plan, called the protocol, which researchers agree to follow. You will be informed about the protocol before you enroll in the trial and this information will be available to you throughout the trial. You can ask for a copy of the protocol and the informed consent. The FDA must also approve an Investigational New Drug (IND) application.

What is informed consent?

Informed consent is the process of agreeing to be in the clinical trial. The research team will explain the trial's purpose, how long it will take, what will happen, all known potential risks and benefits, and information about the privacy of your medical records. If you want to participate, you will sign an informed consent form. Informed consent is not a one-time event. It continues throughout the trial. You have the opportunity to ask about the trial as it progresses.

Can I quit a clinical trial?

Yes, you can leave at any time, for any reason. Before you leave, you should let the research team know you have decided to withdraw. A clinician will need to follow you, even if you withdraw.

How do I know if I am eligible for the clinical trial?

Every clinical trial has guidelines that determine who can participate. These eligibility and exclusion criteria can include age, gender, stage of the disease, specific genetic change, previous treatment, and other medical conditions. Researchers are careful about screening participants and including only those who fit the criteria. This helps keep you safe and ensures that researchers get the information they need.

How will researchers know if the therapy is working?

In every study there are at least two big sets of data:
1. The first describes what is happening to the whole person. Is the patient experiencing symptoms? Has the patient avoided side effects?
2. The second data set includes the lab tests and other clinical metrics that are used to measure effectiveness of the drug.

How you feel is important, but it may not be enough to prove that the therapy is effective. The FDA wants to see consistency in the effect of the therapy as measured by lab tests or clinical metrics before they can move to the next phase of the research, and invite more people to participate.

What if I participate in the clinical trial and the treatment is not effective?

If the effectiveness of the therapy isn't verified by lab tests or other clinical metrics, it is likely that you will be returned to the standard treatment, even if you are experiencing improved health. Any concerns you have should be discussed with the study coordinator and your physician.

If gene therapy does improve the way my body functions, will the effect last?

Gene therapies effect the way your body functions by introducing normal copies of genes that will produce the right protein in the right amount. If they produce this needed protein over time, and your medical condition improves, the effect is called "durable."

One of the goals of clinical trials is to measure how durable the therapy is. If the response is not long lasting, the trial may not advance to the next phase, or it may stop altogether. This may happen even if some patients are benefitting from the therapy, at least in the short term.

I feel good. Am I done now?

Even if you or your child start(s) to feel better, lab tests and monitoring continue so that researchers can determine whether or not the effect of the treatment is durable. Clinical trials often have long-term follow up with trial participants to see what happens over time.

Will I need more than one treatment?

Many gene therapies are designed to be one-time treatments. But for some diseases, re-dosing may be required. Some clinical trials are designed to answer whether or not re-dosing will be necessary to maintain the effect. Scientists will not know whether or not redosing will be required until the trial is complete. You can request more information about this and other topics during the informed consent process.

What if I develop a reaction to the treatment?

There is a risk of side effects and adverse reactions with any treatment. Because of this, doctors and researchers carefully check study participants for any negative health impact and try to fix it. They also examine whether or not the adverse reaction was caused by the treatment. Stay in touch with the doctor(s) to report anything out of the ordinary.

Most current gene therapies have 2 critical elements:
1. the gene to be introduced
2. the vector (gene delivery vehicle) that helps the new gene get into the cell nucleus.

You may have an immune response to the vector or the new gene. This immune response might make the therapy ineffective, or cause side effects. If this happens, you may be taken out of the study if the problem cannot be fixed.

If this gene therapy doesn't work, can I try another?

It depends. There are a number of factors that will determine whether or not you're eligible for future trials.

With rare disease, the doctor is unlikely to recommend participation unless they feel that it offers the right approach for you. There is a chance that your participation in the trial may prevent you from participating in a future trial. For instance, you could build up resistance to the vector used to carry the DNA.

How long will I need to participate in the study?

Gene and cell therapy studies are intended for people who are willing to participate long-term. Researchers want to be sure that the effect of the gene therapy is durable and that other problems don't arise down the line. Some trials last for years.

What is involved in participating long-term?

You may be required to schedule doctor visits and blood tests that will provide ongoing data for the study. This is true even if you feel better. You might even have emergency visits that you didn't plan. People who participate in clinical studies (called "trial participants") give back to the community through their willingness to commit to a protocol for their whole lives.

Do I have to pay for my treatment in the clinical trial?

No, but you may have to cover some costs related to participation. The treatment and all related visits with healthcare professionals are often paid for by the company that is sponsoring the clinical trial. Health problems connected to trial participation, such as an adverse event, are usually covered as well. This should be listed in the informed consent form, so be sure to ask about this.

You or your insurance company may be responsible for the cost of any treatment, procedures, or tests that are part of your routine care, whether you are participating in a clinical trial or not. Speak with your insurance company before enrolling in a clinical trial to see if you will have any out-of-pocket expenses.

Do I have to pay for travel to the trial site?

Because you will have to be closely monitored by the study coordinator and the team of healthcare professionals working on the trial, you may have to live close to the trial site or plan for regular travel. Some clinical trials will reimburse you for needed travel and lodging, but many will not.

Where can I find out more about clinical trials?

Try ClinicalTrials.gov

CISCRP.gov

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