
As someone who is considering participation in a gene transfer research study, it's important that you have all the information you need to make an informed decision.

This brochure provides you with some general background on gene transfer—what it is and how it works. It also includes two lists of questions. The first list suggests questions that you may want to ask your health provider or a member of the research team. The second list can serve as a guide to help you decide whether this study is right for you.

What are genes?

Thousands of genes are found in every cell in your body. Each of these genes contains a set of instructions that are read by the cell, allowing your body to make the proteins it needs. However, in many individuals, a gene's instructions aren't written properly, which can result in disease.

What is gene transfer?

Gene transfer is the process of inserting new genes into a person. Many scientists and health care providers think gene transfer might be an effective way to treat certain health problems. However, gene transfer is a relatively new area of science, and so researchers are trying to learn more about it to make sure it's safe to use in people and to see if it works.



How is gene transfer done?

Genes can be inserted into the body a number of ways—for example, through an injection, or for certain types of studies, study participants can breathe them in through the mouth or nose. The genes can be inserted “as is” (naked), or they may be specially coated or inserted into a virus. Genes transferred through a virus rely on the virus's natural ability to insert genetic material into cells. The researchers first remove some genes from the virus so that it is less likely to cause disease. Then they add the new, desirable genes, which they hope will be delivered by the virus to the target cells. Researchers reason that if the genes containing the new instructions reach the target cells, the target cells may be able to read them and start making the proteins that the body needs to treat the disease.

I'm considering participating in a gene transfer study. How do I decide whether to participate?

It's entirely up to you whether you participate in a research study. Before you make a decision, someone from the research team will meet with you to discuss the study in detail. This meeting is an opportunity for you to ask questions, and you're encouraged to do so. In addition to answering your questions, the research team member will give you a written document to take home that describes the study.

It may be a hard decision, but if you ask questions, review the information provided, and think it over carefully, your decision will be an informed one.

Below are some questions you may want to ask your health provider or the research team. You may have other questions in addition to these:

- What is the study trying to find out? Is the study testing the safety or the effectiveness of this type of gene transfer for my disease?



- What do you already know about this gene transfer method for my particular disease? Are there any positive or negative outcomes that I should know about?
- What procedures, tests, or medicines will be administered to me? What are the possible side effects?
- What is expected of me, short-term and long-term, if I decide to participate? How long will I be expected to participate?
- What possible benefits, if any, might I receive by participating? What possible benefits might others receive in the future from my participation?
- What are the risks of harm, short-term and long-term, from participating?
- How will my life be affected if I decide to participate?
- Will I be responsible for any costs?
- Could participating affect my ability to have children? Could participating affect any future children I may have?
- What other options are available if I decide not to participate? What are the risks of harm and possible benefits of these other options?
- If I agree to participate and then change my mind later, whom should I contact? How will it affect treatment for my condition?
- Do any of the research team members have any financial or other conflicts of interest that I should know about?

- Whom should I contact if I have questions before I enroll? After I enroll?
- How and when will I be informed about outcomes related to the study, if at all?
- Who will have access to information about me or my research results?
- What will happen to any biological samples that may be taken from me?

Below are some questions you may want to ask yourself to help decide whether or not participating in a gene transfer study is right for you:

- Do I have any questions for my health provider or the research team that will help me make my decision?
- Do I understand everything that was explained to me?
- Can I make the commitment expected of me as a participant?
- Are there any health or personal considerations that might affect my ability or desire to participate (such as current or past health problems, family medical history, child care needs, or transportation issues)?
- Am I comfortable taking on the risks of participating in this research?
- Are any of the alternative choices better for me?
- Which expenses that are not covered by the study will my health insurance cover? Which expenses will I be responsible for?



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Deciding Whether to Enroll in Gene Transfer Research?

Information for Study Participants

