

Release Date November 2005
Revised January 2008
Revised March 2012

Clinical Trials Patient-Education Brochure



American Society of Transplantation

1120 Route 73, Suite 200
Mount Laurel, NJ 08054
Phone: 856-439-9986
Fax: 856-439-9982
E-mail: info@myAST.org

The information presented and opinions expressed herein are those of the authors and do not necessarily represent the views of the Society.

CLINICAL TRIALS PATIENT-EDUCATION BROCHURE

Introduction

In the past, the practice of medicine advanced by trial and error. Older doctors simply passed on their knowledge and experience to younger doctors. For the most part, this way of teaching and learning worked well. Over the years, medical care improved. At the same time, however, this trial-and-error approach meant some medical practices did not work well, and others were dangerous. Clinical trials have been found to be the best way to make sure new standards of medical care are, in fact, safe and effective. This is especially true for new drugs, devices, or procedures. Successful organ transplantation could not be done today were it not for clinical trials.

Should I take part in a clinical trial?

First of all, you must volunteer in order to be able to join a clinical trial. You cannot be forced to join in any way. A decision to take part is important. It should not be made without full knowledge of what is involved. When you choose to take part in a clinical trial, you may or may not improve your health. You may be one of the first to try a promising new drug or treatment. You will be cared for by a team of dedicated health professionals who are interested in your health. One of the most important reasons to join a clinical trial is to help advance what is known about new treatments.

What is a clinical trial?

A clinical trial is a study that helps test whether a new treatment or medication is safe and effective. Or it can look to see which older treatments produce the best results. In organ transplants, previous studies have been very important in finding the best way to care for your new organ. More trials are needed to see if newer approaches might produce even better results.

There are a number of ways clinical trials are organized and conducted.

Randomized trials

This is when a patient in a study is assigned to one of two or more treatments by chance—like a coin toss—not by choice. Neither the treatment team nor the patient can choose which patient gets which treatment. Yet the care of each patient is similar, regardless of which treatment he or she receives. At the end of the trial, treatment groups are compared. Often, the results of these different treatments are watched as the trial goes along. If one group is doing much better than the other, the trial will be stopped early.

Single- or double-blind studies

In a single-blind study, patients don't know which treatment they are getting, but the treatment team does. In a double-blind study, neither the treatment

team nor the patients know which treatment is being used. If the need arises, it is always possible to find out which treatment the patient is receiving.

Placebo-controlled studies

In some studies, a placebo is used. This is a substance that looks like the real medicine used in a trial but doesn't contain the medicine. Some patients will get the placebo, and others will get the real medicine, but they will not know which they are taking. The placebo lets the study team see if the effect of a new drug is better or no better than usual treatment or no treatment at all.

Observational trials

Patients in the trial receive the same care or treatment as all other patients. The doctors observe and record how patients do over time. They may then compare these results with the results of patients treated in a different time or place.

There are four types of trials:

Phase 1

In phase 1, a new drug or treatment is tested on a small number of volunteers for short periods of time. Researchers look for what dose of the drug works best and record any side effects linked with the drug. They also hope to find out how the body absorbs and reacts to the drug, and how long it stays in the body. Most often, phase 1 trials do not help those who take part, who are often healthy persons. Instead, they give researchers valuable information on what steps to take next.

Phase 2

In a phase 2 trial, a drug with known effects is tested at specific doses to learn how it may be useful. They also look to see what other side effects might be expected. In these studies, patients usually take a medication for a longer period of time. How the body absorbs and reacts to the drug, and how long it stays in the body, are also tested. A phase 2 trial helps researchers learn more about how a treatment improves a condition.

Phase 3 and 4

In phase 3 and 4 studies, a new treatment is compared with a commonly used treatment or with no treatment at all. Some patients in the trial will get the new treatment. Some will get the usual treatment or a placebo. These studies test whether a new treatment is effective and how best to use new treatments in caring for patients. Most clinical trials you will be offered will be phase 3 or 4 studies.

A member of your clinical-trials team will tell you which type of study or trial you can be enrolled in and what treatments you may or may not receive.

What are the risks and benefits of taking part in a clinical trial?

Potential Benefits

- You will be helping others by taking part in medical research.
- You may have access to new research treatments before they are offered to others.
- Often, just being in a clinical trial will result in better health for you, regardless of what treatment group you are placed in. This is because being in a clinical trial most often means you must have closer follow-up than is usual at a transplant center. It is thought that this extra care and attention may improve your health.

Potential Risks

- The new treatment you receive during the study may be more effective, less effective, or the same as standard treatment for you.
- You may have serious or even dangerous side effects from the treatment you are given.
- Because there are risks, being a part of a trial may mean you will need to give more time and attention to your medical care. For example, you may be asked to come to the transplant center more often for follow-up. You may need to have more treatments or more tests or follow a more complicated medication schedule.

Who is looking out for me (the patient)?

The federal government has a law stating each center that performs clinical trials with humans must have an Institutional Review Board (IRB). An IRB is a group of people assigned to review and monitor research. The group includes members of the community. The job of the IRB is to help protect the rights, safety, and welfare of anyone thinking about, or currently taking part in, a clinical trial. They watch over the research plan, the consent process, and how patients are enrolled. They make sure the way the research is carried out is ethical. They check that it follows all laws and standards.

These days, most studies are followed by a Data and Safety Monitoring Board (DSMB). For smaller trials, this is an independent safety officer. A DSMB is made up of scientists who are experts in the field, but who are not researchers in the study. It reviews the data as the study goes along. It knows which treatments subjects are receiving. If results show a treatment does not work or leads to too many side effects, the DSMB can suggest ways to protect patients. It can even stop the study.

Please keep in mind that your entire health-care team (doctors and nurses) is committed to protecting your rights and interests. They want to make sure you have the best possible health care and results.

Informed Consent

Informed consent has been set up to guarantee that you understand the clinical trial, your role in it, and your rights as a patient in the study. It is a process, not a form. If you have a question or concern about your role in the study before or at any time during the trial, ask your health care team. After all, you need to be informed before you can give your consent.

If you are asked to take part in a research study or clinical trial, you have the right to the following:

1. Be told what kind of trial it is and why it is being done.
2. Be told what kind of procedures are to be used as well as given a description of any drug or device to be used.
3. Be given a description of any discomforts and risks to be expected. You must be told whether there will be any financial costs to you or your health insurance company.
4. Be given an explanation of benefits, if any, that you might expect.
5. Be told of procedures, drugs, or devices that will be used for patients who do not take part in the trial. You must also be told how the risks and benefits of such treatments compare with those expected from patients who do take part in the trial.
6. Be told of other treatment choices, if any, that could be offered to patients during and after the trial.
7. Be given a chance to ask any questions about the trial and what will happen in the trial.
8. Be told how new findings will be reported to those in the trial and how these findings could change a person's willingness to be in the trial.
9. Be given a copy of any consent form used in any stage of the trial.
10. Be given the time and the chance to give careful thought to whether to join the trial.
11. Be informed that your consent to take part is voluntary. It must not be due to any kind of force or other influences. Your consent can be withdrawn at any time, and for any reason, without affecting the care that you receive at the transplant center.

Checklist for Considering Clinical Trials

Before you consent to join a clinical trial, it is important for you to have all the information you need to be confident about your decision. Be sure you have answers to all your questions. Discuss them with your family.

These questions might include the following:

- What is the purpose of this trial?
- What would I be expected to do if I enrolled?
- How much time is involved? Will I be compensated for time or travel expenses?
- Will I need more tests or studies compared with the usual care I would receive?
- How will this trial benefit me?
- How will this trial benefit others?
- Are there risks involved in this trial? What are they, and how likely are they to occur?
- How many other people have enrolled in this trial?
- Who is the researcher leading this study? Will it be the doctor I work with regularly?
- Have I discussed joining the trial with those who care about me, such as family and friends?

Special Considerations for Children

For many years, it was thought that children should not be a part of clinical trials. Because of this, almost no drugs, procedures, or devices were proven to be helpful and safe in children. Decisions about use of treatments had to be made from studies done in adults. Children are not small adults. They have different diseases. Their bodies are growing and using energy in different ways. They do not respond to treatments in the same way as adults. As a result, children were often given inappropriate treatments.

Dosing of Medications in Children

Without clinical trials to help decide the best dose of medicines in children, we often give the wrong amount. Drugs can act quite differently in children than in adults. Although it may seem strange, many drugs must be given in larger doses or more often in children to have the same effects seen in adults.

Diseases in Children

Some diseases affect only children. Clinical trials in children give us new knowledge about treatments, procedures, and devices that they may need.

Informed Consent/Assent of the Child

Informed consent is even more important in trials using children. Children must have all the protection that informed consent gives adults. The child must agree to the trial and parents must give their permission. The federal government now requires that children be a part of clinical trials of treatments that will be used for children. If your child is to have a transplant, your doctors and nurses should take the time that is needed to fully explain how clinical trials work for children.