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AMERICAN SOCIETY OF ® TRANSPLANTATION

Clinical trials patient-education brochure

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WHAT IS A CLINICAL TRIAL?

A clinical trial is a study that tests if a new treatment is safe and effective (works well). A clinical trial can also compare a new treatment to older treatments to see which helps people the most. Doctors use clinical trials to test new drugs, medical devices, and procedures. People who take part in clinical trials try a treatment, and researchers collect data to see how well it works.

The current success of organ transplants is due to previous clinical trials, which has allowed doctors to find ways to improve care for organ transplant recipients. New trials can help us find even better ways.

SHOULD I TAKE PART IN A CLINICAL TRIAL?

You must volunteer to take part in a clinical trial – you can't be forced to take part. Your decision to take part is important. You should know that:

- Your doctors and the healthcare team should fully explain the purpose of the clinical trial to you and answer any questions you have. Don't take part unless you fully understand what is involved
- When you take part in a clinical trial, your health may or may not improve
- Your doctors and healthcare team will continue to care for you whether or not you decide to take part in a clinical trial
- You can decide to stop taking part in a clinical trial at any time

You may be one of the first to try a 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 them understand whether a new treatment has benefits (good effects) and/or risks (bad effects) compared to standard treatment

ARE THERE DIFFERENT WAYS TO DO CLINICAL TRIALS?

Yes. Healthcare teams use different ways to carryout clinical trials.

Observational trials

All of the patients in an observational trial get the standard (usual) treatment for their health problem – not a new treatment. The study team carefully observes and records how the patients do over time. Then, the team may compare the results with the results of patients treated for that condition in a different time or place.

Controlled trials

In a controlled trial is a study, patients are divided into different groups:

- Some patients (the experimental group) get a new, treatment
- Some patients (the control group) get the standard treatment

This lets the team directly compare the results of the new and standard treatments. A trial may have more than one experimental group, which lets the team to compare several new combinations of treatments or different doses (amounts) of a medicine.

A controlled trial lets the healthcare team find out if the new drug is better, worse, or the same than the standard treatment (or no treatment at all).

Randomized trials

In a randomized clinical trial, patients are assigned to get one treatment or another by chance (like tossing a coin). For example, some patients may be randomly assigned to the experimental group (get the new treatment), while others are assigned to the control group (get the standard treatment). Neither the healthcare team nor the patient can choose which treatment a patient will get.

The care of all patients is similar, other than the specific treatment being tested. The team will check the results of the different treatments as the trial goes along. At the end of the trial, they'll compare the results from each assigned group.

If patients in one group are doing much better than the others while the trial is still going on, the healthcare team will stop the trial early.

Single- or double-blind trials

Blinded trials help remove the effects of any expectations ("bias") by the patient or the healthcare team:

- In a single-blind trial, patients don't know which treatment they are getting, but the healthcare team does know
- In a double-blind trial, neither the healthcare team nor the patients know which treatment they are getting

If the need arises, such as when a patient has an allergic reaction to a treatment, it's always possible to find out which treatment the patient is getting.

Placebo-controlled trials

A placebo-controlled trial is a type of trial where one group of patients is treated with a placebo. A placebo looks just like the experimental medicine being tested in the trial, but doesn't actually contain the medicine.

In placebo-controlled trials, patients in the experimental group get the new medicine and patients in the control group get a placebo. None of the patients know if they are getting the new treatment or a placebo. In this way, the study is either single- or double-blinded.

ARE THERE DIFFERENT TYPES OF CLINICAL TRIALS?

There are 4 types, or phases, of clinical trials. New treatments must go through trials at each phase, starting with phase 1.

- Phase 1 – **Phase 1 trials find out if a new treatment is safe in humans.**
  - In phase 1, a healthcare team tests a new treatment on a small number of volunteers for short periods of time to find out its safety. They record any side effects, how the body absorbs it, and how long it stays in the body.
  - Phase 1 trials usually use healthy volunteers who don't have a condition. Usually the first trial participants get a very low dose and if side effects are minor, the next trial may give a higher dose. This helps doctors find a dose that's likely to work without too many side effects.
- Phase 2 – **Phase 2 trials find out if the treatment actually works.**
  - If phase 1 trials show a treatment is safe, phase 2 trials will test its effectiveness (how well it works) to improve a condition. Phase 2 studies use volunteers who actually have the medical condition that the treatment intends to treat. These trials often include a control group that gets either a placebo or the standard treatment.
  - Phase 2 trials usually include more patients, and they take the treatment for a longer period of time than phase 1 trials. This helps doctors find side effects that are less common or take time to happen. They may also test different dose amounts to see if this improves the treatment's effectiveness without causing too many side effects.
- Phase 3 and 4 trials – **Phase 3 and 4 trials get more information on a treatment's safety and effectiveness compared to other available treatments.**
  - Phase 3 and 4 trials gather more information about a treatment's safety and effectiveness. They study more patients for longer times and may include other types of patients (such as different ages, different levels of organ function, or who have another health condition than the condition being studied).
  - Phase 3 and 4 trials may test different doses, lengths of treatment, or different combinations of treatments to find out more information about its effectiveness and side effects.

A member of your healthcare team will tell you which trial types are available at your medical center and will give you more information about what treatments you may get.

WHAT ARE THE BENEFITS AND RISKS OF TAKING PART IN A CLINICAL TRIAL?

Possible 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.
- Being in a clinical trial may result in better health for you, no matter what treatment group you are placed in. Being in a clinical trial often means you get follow-up care that's more detailed than is usual at a transplant center. Doctors think this extra care and attention may improve your health.

Possible risks

- There's no way to know if a new treatment you get during a trial may be more effective, less effective, or the same for you than the standard treatment.

- You may have mild, serious, or even dangerous side effects from the treatment you get in a trial.

Taking part in a trial may mean you'll 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 and tests, or follow a more complex medicine schedule.

Financial incentives – Some trials might offer small amounts of money to help invite people to take part in a trial. However, this should not be an important factor in your decision to take part.

WHO IS LOOKING OUT FOR ME (THE PATIENT)?

Everyone on your healthcare team (doctors and nurses) wants to protect your rights and interests, and make sure you have the best possible healthcare. Your healthcare team will take care of you whether or not you take part in a clinical trial.

If you are interested in taking part in a trial, your team will help you find out which trials are available and if you're a good candidate for any of them.

Rules that help make trials safe for patients

- Any new drug is tested in animals or human cells before being tested in humans. This gives scientists an idea of how the drug works and whether there are major safety concerns that would prevent actual testing in human volunteers.
- Before any new drug or device is used in humans, the Federal Drug Administration (FDA) must approved it as an Investigational New Drug (IND). The FDA reviews everything that is known about the drug and its testing. In many cases the drug may not be brand new, but may have already been approved for use in a different disease.
- By law, each center that performs clinical trials with humans must have an Institutional Review Board (IRB). The IRB:
    - Is an independent group of people assigned to review and monitor research. It includes doctors not involved in your trial, and members of the community.
- Works to help protect the rights, safety, and welfare of anyone who is thinking about, or currently taking part in, a clinical trial. To do this, they:
    - Examine the research plan, the consent process, and how patients are accepted into a trial
    - Make sure the research is carried out in an ethical way
    - Check that the trial design and the way it is carried out follows all laws and meets proper safety and monitoring standards for clinical trials
- Most trials testing a new treatment are followed by a Data and Safety Monitoring Board (DSMB). A DSMB:
    - Is a committee of scientists who are experts in the field, but who are not directly involved in the trial (for smaller trials, the "DSMB" may be an independent safety officer)
    - Reviews the data as the trial goes along and knows which treatments patients are getting
    - Suggests ways to protect patients or can even stop a trial if results show that a treatment does not work or leads to too many side effects

INFORMED CONSENT

Informed consent is a process where you receive an explanation of the clinical trial the potential risks and benefits, your expected role in the trial, and your rights (see below) as a research participant before you agree to participate.

If you have a question or concern about your role before or at any time during the trial, ask your healthcare team. After all, you need to be informed before you can give your consent (agreement).

If you are thinking about taking part in a clinical trial, you have rights.

The healthcare team must tell you:

- What kind of trial it is and why it is being done
- About the experimental device or drug that will be used and what kind of procedures will be involved.
- About procedures, drugs, or devices that will be available to patients who do not take part in the trial. They must also tell you how the risks and benefits of such treatments compare with those expected from patients who do take part in the trial.
- About how the trial might affect any other treatment choices that you could be offered during or after the trial
- How new findings will be reported to patients in the trial and how these findings could change a person's willingness to be in the trial

- Facts about your consent:
  - Your consent to take part is completely 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
  - If you withdraw your consent that this will not affect the care that you get at the transplant center
- If you will be paid for any of your visits and for taking part

The healthcare team must give you:

- A description of any discomforts and risks to be expected. They must also tell you if there will be any financial costs to you or your health insurance company.
- A chance to ask any questions about the trial and what will happen in the trial
- An explanation of the benefits, if any, that you might expect
- A copy of any consent form used in any stage of the trial
- The time and the chance to give careful thought to whether to join the trial

CHECKLIST FOR CONSIDERING TAKING PART IN A CLINICAL TRIAL

Before you agree to join a clinical trial, it's 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 all of the information with your family before you make a decision.

Your questions might include these:

- What is the purpose of this trial?
- What would I be expected to do if I take part?
- How much time is involved? Will I be paid for my time and will travel expenses be paid for?
- Will I need more tests or studies compared with the usual care I would get?
- How will this trial benefit me?
- How will this trial benefit others?
- Are there risks involved in this trial? What are the risks, and how likely are the risks to happen?
- How many other people have enrolled in this trial?
- Who is the researcher leading this study? Will it be one of the doctors I will see regularly, or someone else on the healthcare team?
- Have I discussed joining the trial with those who care about me, such as family and friends?

WHY IT'S IMPORTANT FOR CHILDREN TO TAKE PART IN CLINICAL TRIALS

People used to think children should not take part in clinical trials. Because of this, very few drugs, procedures, or devices were proven to be helpful and safe in children. Doctors had to use studies done in adults to decide about treatments for children – and sometimes the treatments weren't right.

Children need to take part in clinical trials that test:

- Medicines for children so doctors can give them the right strengths and amounts
- Diseases that only happen in children to learn about new treatments, procedures, and devices

Children must give informed consent or agreement

Informed consent is even more important in trials using children. The child must agree to the trial and the 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 will get a new organ, your doctors and nurses should take the time to fully explain how clinical trials work for children.

TO LEARN MORE

- Ask your trial healthcare team any questions or for more information
- Talk with your regular doctor about clinical trials
- Visit the FDA's "Clinical Trials" website (www.clinicaltrials.gov). On that website you can look up trials for the specific condition you have. You can even search for trials you may want to consider taking part in.