

Clinical Trial Overview

Clinical trials are a method designed to scientifically determine the effectiveness of various treatment regimens in humans. A new drug or treatment enters clinical trials after first being evaluated in research laboratories and on animals. The initial research lays the groundwork, but can't predict exactly how a treatment will work in humans. Clinical trials evaluate investigational drugs in humans that researchers believe may have therapeutic use for patients.

What are the major phases of a clinical trial?

Researchers group treatments into "phases" based on what they know about the treatment and what questions they have left to answer. A new drug or treatment progresses from one phase to the next by building on the findings gathered in the previous phase(s).

- **Phase I:** Determining safe drug doses and/or schedules of a new drug. The treatment is being tested for the first time in people with brain tumors. The main goal is to determine a safe dosage range and identify which side effects the person experiences. The treatment is considered promising for treating brain tumors, but the primary goal of the study is to evaluate safety, not how the tumor responds to treatment. Participants get a specific dose of drug(s) and are carefully observed for side effects.
- **Phase II:** Determining therapeutic efficacy. The primary goal is to examine the treatment's effectiveness and further evaluate its safety. The researcher wants to determine whether the treatment shrinks the tumor and extends the participant's survival.
- **Phase III:** Comparing treatment to an existing, effective standard therapy. The treatment being studied is compared to a standard treatment that is commonly used. A

participant is randomly assigned by a computer to one of the treatments, either the experimental treatment or the standard treatment. The researcher does not know which treatment works best, therefore preventing bias. Comparing the experimental treatment directly to a standard treatment allows researchers to determine which therapy is the better approach.

Who conducts clinical trials?

Clinical trials are conducted by various institutions and groups. Each clinical trial follows a treatment plan or "protocol." These protocols are defined by the researchers initiating the clinical trial, and undergo close scrutiny at each institution or hospital by an Institutional Review Board (IRB). The IRB reviews a clinical trial to be sure it is designed with safeguards to protect the participants and prevent significant risks. The following lists the various groups that may initiate a clinical trial.

- **Institutions:** Individual institutions or hospitals conduct studies of various drugs. The researchers may first try a treatment in the laboratory and then proceed to testing with people who have brain tumors.
- **National Cancer Institute (NCI) Sponsored CNS Consortia:** These are groups of specialized brain tumor centers from around the United States that receive funding to test innovative new treatments for brain tumors. There are currently two consortia that are made up of multiple medical centers.
- **National Cooperative Groups:** These groups consist of both university and community hospitals throughout the United States. Their goal is to test new treatments for efficacy and assess whether the treatment can be done in a community setting.
- **Pharmaceutical Sponsored Multi-Institutional Trials:** Individual pharmaceutical companies often form consortia of hospitals to investigate a drug that they have developed.

Fact Sheet: Clinical Trial Overview

What should I consider when choosing to participate in a clinical trial?

People choose to take part in clinical trials for various reasons, whether to try an innovative treatment, to find hope for a cure, to attempt to feel better, or to assist in moving research forward to help others in the future. There has been an explosion of new treatments for brain tumors. It can be difficult to find information quickly and choose a treatment which may be appropriate. It is hard to be sure what the “best” treatment available is with so many options. Utilize all available resources to make an informed decision.

If you are thinking about participating in a clinical trial, consider which trial is appropriate for you. Factors include: what type of tumor you have (or histological grade), when you were diagnosed and what treatments you have received, and how well you are functioning. Most trials have specific restrictions based on these criteria.

Other factors to consider include who is sponsoring the trial, where it is being conducted, the phase of the study, and the tests, treatments, and time commitment involved. Find out as much as you can about potential side effects and how the study treatment compares with other available options. Practical considerations include cost, distance from home, whether your health insurance will pay for care if you participate, and what other support may be offered, such as free services or reimbursement for travel expenses.

Learning as much as you can before starting a treatment will help you to be prepared for what occurs. There are no bad questions when learning about your treatment options and determining whether a treatment is right for you.

What happens after I select a trial?

The first step of participating is called informed consent. According to the National Cancer Institute, informed consent is a process by which people learn the important facts about the trial to help them decide whether to participate. Informed consent covers the purpose of the study, the potential risks and benefits of participation, as well as the trial’s protocol (or action plan). Giving informed consent means that you fully understand the risks and benefits of the treatment and freely choose to take part in the trial. You will then be asked to sign a consent form. This does not mean that you cannot leave the trial, but only that you understand the protocol. You may choose to leave a clinical trial at any point in the process.

The informed consent process will outline the procedures, tests, and other items involved in your participation. For instance, during the course of the clinical trial, you will be evaluated closely and required to report symptoms that you experience. This may require extra physical examinations, tests, or filling out forms.

Resources

The following organizations can provide you with more information about clinical trials and treatments for brain tumors:

- **National Brain Tumor Society**
Patient Services Line: 800 934 2873
www.brainumor.org
- **National Cancer Institute**
Cancer Information Service: 800 422 6237
www.cancer.gov/clinicaltrials
- **Neuro-oncology Branch**
National Cancer Institute / National Institute of Neurological Disorders and Stroke
New Patient Line: 866 251 9686
- **CureSearch** (pediatric): www.curesearch.org
- **Clinical Trials and Noteworthy Treatments for Brain Tumors:** www.virtualtrials.org
- **National Institutes of Health:**
www.clinicaltrials.gov
- **Coalition of Cancer Cooperative Groups:**
www.cancertrialshelp.org

Adapted from a previous version by Terri Armstrong, RN, MS, NP, CS.

The information in this publication is subject to change. The reader is advised that information obtained from a physician should be considered more up to date and accurate than the information in the publication and that this publication does not and cannot purport to address facts and circumstances particular to any patient. This is something that can only be done by the patient’s physician. Sponsorship of this publication does not imply the National Brain Tumor Society’s endorsement or recommendation of any particular form or forms of therapy, regimen, or behavior. The information in this publication is not meant to be legal advice or replace the advice of an attorney.

Version: 2009