The Association for Frontotemporal Degeneration
Opening the gateway to help and a cure

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FTD Clinical Drug Trials

Understanding and participating in emerging trials of potentially disease- modifying treatments

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The goal of this session is to educate and empower our patients and families as they face increasing opportunities to participate in research that will lead to the first approved drugs for FTD. Research is not the same as medical treatment and there is no guarantee that an *individual's* condition will improve. Participating in research studies contributes to scientific understanding of FTD and helps society through this increased knowledge.

As we enter a promising new phase in FTD research there is good reason for much hope. But decades of drug development for other diseases demonstrates that the path to effective treatments will most likely be neither swift nor straight. Because FTD is a relatively rare disorder there will be a limited number of clinical sites involved in any one trial, meaning that **a patient may have to travel to participate**. The current lack of biomarkers means that we still do not know for sure what underlying disease process may be causing a person's symptoms; this can greatly complicate design of a clinical trial. Finally, it is important to understand that, in order to have the best chance at producing definitive results, a trial will be designed to **include** patients who display a specific set of symptoms and characteristics—and **exclude** those who do not fit this profile. If you do not qualify for a trial based on these exclusion criteria it does not mean that you have done anything wrong, that your doctor cares any less about your care, nor that you should give up hope. You may well qualify for the next trial.

You and your family are your own best advocate. Getting informed about a research study and asking any questions you have will help ensure that you can take on the role as a partner in the research study, rather than a subject.

Questions to Ask (from www.clinicaltrials.gov)

Anyone interested in participating in a clinical study should know as much as possible about the study and feel comfortable asking the research team questions about the study, the related procedures, and any expenses. The following questions might be helpful during such a discussion. Answers to some of these questions are provided in the informed consent document. Many of these questions are specific to clinical trials, but some also apply to observational studies.

- What is being studied?

- Why do researchers believe the intervention being tested might be effective? Why might it not be effective? Has it been tested before?
- What are the possible interventions that I might receive during the trial?
- How will it be determined which interventions I receive (for example, by chance)?
- Who will know which intervention I receive during the trial? Will I know? Will members of the research team know?
- How do the possible risks, side effects, and benefits of this trial compare with those of my current treatment?
- What will I have to do? What will my study partner have to do?
- What tests and procedures are involved?
- How often will I have to visit the hospital or clinic?
- Will hospitalization be required?
- How long will the study last?
- Who will pay for my participation?
- Will I be reimbursed for other expenses?
- What type of long-term follow-up care is part of this trial?
- If I benefit from the intervention, will I be allowed to continue receiving it after the trial ends?
- Will results of the study be provided to me?
- Who will oversee my medical care while I am in the trial?
- What are my options if I am injured during the study?

Important Terms (http://www.clinicaltrials.gov/ct2/about-studies/glossary)

Clinical Trial-- A clinical trial tests the safety and effectiveness of new medical approaches in humans. Clinical trials may also be called: clinical studies, medical research, or drug trials.

There are five phases of clinical trials, as designated by the US FDA:

> **Phase 0:** Exploratory study involving very limited human exposure to the drug, with no therapeutic or diagnostic goals.
>
> **Phase 1:** Studies that are usually conducted with healthy volunteers and that emphasize safety. The goal is to find out what the drug's most frequent and serious adverse events are and, often, how the drug is metabolized and excreted.
>
> **Phase 2:** Studies that gather preliminary data on effectiveness (whether the drug works

in people who have a certain disease or condition). For example, participants receiving the drug may be compared with similar participants receiving a different treatment, usually an inactive substance (called a placebo) or a different drug. Safety continues to be evaluated, and short-term adverse events are studied.

Phase 3: Studies that gather more information about safety and effectiveness by studying different populations and different dosages and by using the drug in combination with other drugs.

Phase 4: Studies occurring after FDA has approved a drug for marketing. These studies gather additional information about a drug's safety, efficacy, or optimal use.

Disease-modifying treatment – A therapeutic intervention aimed at slowing or reversing the underlying disease process (e.g. lowering cholesterol or decreasing lung inflammation in asthma). At present there are no approved disease-modifying treatments for any of the FTD disorders.

Inclusion criteria/ Exclusion criteria--The factors that allow someone to participate in a clinical study are called inclusion criteria, and the factors that disqualify someone from participating are called exclusion criteria. These are based on things such as age, gender, the type and stage of a disease, previous treatment history, and other medical conditions. If an individual does not qualify, it does not mean something is wrong with the person. There might be other studies that are more appropriate.

Informed consent-- The goal of the informed consent process is to protect participants. All the important information about the study must be given to the potential participant in a written document that is clear and easy to understand and the participant must have the opportunity to ask questions. Generally, a person or his legal representative must sign an informed consent document to enroll in a study.

Interventional research—A research study in which participants receive a specific intervention, such as a drug or change in diet. The goal of an interventional study is to compare outcomes to that of a group that does not receive the intervention. Clinical trials are interventional studies.

Observational research--An observational study tests hypotheses or measures certain outcomes using observational methods without any intervention or experimentation. Many FTD patients have participated in observational studies which are informing our understanding about the natural course of the disease.

Orphan disease—An orphan, or rare, disease is defined as a disease or condition that affects less than 200,000 persons in the US (about 1 in every 1,500 people). In 1983 Congress passed the **Orphan Drug Act**, which provides incentives and accommodations (such as requiring a smaller number of participants) to encourage clinical trials in rare diseases.

Outcome measures--A planned measurement described in the protocol that is used to determine the effect of interventions on participants in a clinical trial. For observational studies, a measurement or observation that is used to describe patterns of diseases or traits, or associations with exposures, risk factors, or treatment.

Placebo—A substance that does not contain active ingredients and is made to be physically indistinguishable (that is, it looks and tastes identical) from the actual drug being studied.

Protocol—The written description of a clinical study. It includes the study's objectives, design, and methods. It may also include relevant scientific background and statistical information.

Symptomatic treatment—A therapeutic intervention with a goal of addressing the symptoms of the disease (e.g. decreasing agitation or promoting a good night's sleep).

For More Information

ClinicalTrials.gov provides both general information and includes a registry of clinical studies around the world.

MedlinePlus Clinical Trials Information is a web-based health information service of the National Library of Medicine (http://www.nlm.nih.gov/medlineplus/)

The Association for Frontotemporal Degeneration *(www.theaftd.org/) supports funding for targeted FTD studies and disseminates information about current and prospective projects.*