Clinical Trials: What Patients Need To Know

An overview of clinical trials, the pluses and minuses of participating in a clinical study and information about how to find clinical trials. This article originally appeared in the HCV Advocate, mid-month October 2015

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1. Phases of Clinical Trials – Clinical trials begin with pre-clinical studies conducted in test tubes or animals.If the results are positive the drugs can move through different phases—1 through 3—and a possibly 4th phase. A brief recap of the 4 phases is listed below.

- **Phase 1** studies usually include healthy people, but can include persons with the particular disorder that the study drug is being tested to treat. The primary reason for phase 1 studies is to establish the safety of the study drug. Another important part of a phase 1 study is to find the dose that combines the highest effectiveness with the lowest rate of side effects. (generally 20 to 80people are recruited for phase 1 trials).

- **Phase 2** only includes people who have the disorder or disease that the investigational drug is being tested into find the efficacy—also called effectiveness. In some phase 2 studies the study drug may be compared to a current drug approved by the Food and Drug Administration (FDA) or a placebo drug (sugar pill). Additional safety and side effect information is also obtained. (generally 100 to 300 people are recruited for phase 2 studies)

- **Phase 3** is similar to phase 2 clinical trials but have a larger patient population. They also compare the study drug to other medicines to treat the same disorder or disease—usually the current standard of care drugs—and in different patient populations. Since the patient population is much larger the effectiveness, side effect information and other information obtained is more realistic data compared to the information obtained in phase 1 and 2 studies.

(up to 1,000 or more people)

The pharmaceutical company will compile and review the phase 3 data and apply to the FDA for New Drug Application (NDA). After a period of review and if appropriate the FDA will approve the medication for a particular patient population. The FDA will also issue a package label.

- **Phase 4** is post-marketing studies. These are studies that the FDA may require as part of the FDA approval process. Phase 4 studies gather more information about the safety, effectiveness, and/or the use of the approved medication in certain patient populations.

2. Types of Studies – In clinical studies there are various types of studies including:

- Randomization means that some patients will receive the study drug and some will receive the drug that it is being compared against (an FDA approved drug)or a placebo (sugar pill),
- Open-label means that everyone will receive the study drug
- Prospective these are studies that look ahead look for certain outcomes. These are studies that recruit patients to find out if investigational drugs work.

3. Informed Consent – To participate in a clinical study,a document called 'informed consent' is filled out. The subject/patient is required to read, understand and sign the document. A copy is given to the subject/patient. A study nurse is assigned to each study. It is the nurse's responsibilities that every patient in the study understands the benefits and risks of the study. The informed consent form should be written in language (6th grade and lower) that is simple, and easy to understand. The sentences should be short, and non-technical. The person who is entering into a clinical trial should be encouraged to ask questions and understand every aspect of the clinical trial. Listed below is some general information that should be included in an informed consent form.

- The nature of the study,

- Why the candidates are being recruited for this study,
- What risks, benefits and alternatives are associated with the research, and
- What rights the subjects/patients have as research subjects.

4. Questions Patients Should Ask – If you are thinking of enrolling in a clinical trial, there are many questions you should ask yourself in order to make an informed choice:

- Do you think the study drug may be better than the current standard-of-care medications that have been approved by the FDA? If so, why?
- What is the current phase of the trial (phase 1, 2, 3 or 4)?
- Have you considered the possible side effects and the safety profile of the trial drugs?
- Is it too early in the clinical trial development that it may pose too many risks?
- Are your willing to take the possible risks, and side effects?
- What are the potential benefits?
- Is the cost of the study tests covered?
- Is it an open-label study—that is, does everyone receive the study drug?
- Is it a randomized study? If so, if you do not get the study drug are you offered the study drug at the end of the study?
- Who will be in charge of the patient care?
- Can you wait until the study drugs are FDA approved?

Note: Remember you can drop out of a clinical trial any time you want.

5. Next Steps There are many questions to think about before entering into a clinical trial. Some people would like to further the knowledge about a particular disease and treatment that will help their community. Others may want to receive medical care and treatment. Medical care and treatment may be particularly important for many people who do not have insurance or for those who have been denied coverage. Still others who have been treated but have not been cured may seek treatment and care through clinical trials. All of these reasons are valid and clinical trials are a good way to explore treatment and care. However, as with any treatment you do not want to rush into any decision. It is always good to do your research and work with your medical provider to make the best possible medical decision that is the best decision for you.

Ask your medical provider about clinical trials in your area. Many medical hospitals also conduct clinical trials. The best website that I have found is www.clinicaltrials.gov.

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