



Put to the Test

Ten must-ask questions about joining a clinical trial.

BY KIMBERLEE ROTH

Pat Gianone jokes that she is a “human guinea pig.” The 62-year-old, who lives in the Chicago suburbs, was diagnosed with Parkinson’s disease almost four years ago. Since that time she has participated in three clinical trials at Rush University Medical Center in Chicago and is contemplating a fourth trial, of a medication.

Investigative studies of drugs, vaccines, or medical devices are important because they further our knowledge about diseases and help identify new treatments, says Nancy Barbas, M.D., M.S.W., clinical assistant professor in the University of Michigan Department of Neurology, where she directs the Cognitive Disorders Clinic. Dr. Barbas has been conducting clinical trials for 15 years.

What are the benefits for trial enrollees? Participants receive cutting-edge treatments, typically at no or reduced cost. “It gives them options beyond the regular standard of care,” says Joanne Lord, L.P.N., a certified clinical research coordinator at the University of Michigan.

In past trials, Gianone has tested a supplement, a drug, and an electronic home monitoring device. She might not directly benefit from the results, but she is committed to doing her part to hasten a cure for Parkinson’s. “I feel like I’m actively doing something to fight the disease. Without the studies, I would feel like I’m...waiting to fall apart. I’m not sitting around letting that happen,” she says.

Richard Bedlack, M.D., associate



University of Michigan neurologist R. Scott Turner, M.D., Ph.D., interviewing a clinical trial participant at the Michigan Alzheimer’s Disease Research Center.

PHASES OF THE TRIAL

PHASE I: Researchers test a new drug or treatment in a small group of people for the first time to evaluate its safety, determine a safe dosage range, and identify side effects.

PHASE II: The treatment is given to a larger group of people to see if it is effective and to further evaluate its safety.

PHASE III: The treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow it to be used safely.

PHASE IV: Studies are done after the treatment has been marketed to gather information on its effect in various populations and any side effects associated with long-term use.

professor and director of the Duke University Amyotrophic Lateral Sclerosis (ALS) Clinic, finds that during trials, patients also appreciate the more frequent contact with medical professionals. “I have family members [of patients] who say they love coming to clinic every month, versus every three or four months like before.”

But participating in clinical trials is not without risks, and there are a few

questions every patient should ask before signing on. All prospective participants for a trial should receive an informed consent document. Read it carefully, as it spells out the details of the study in simple language.

1 WHAT IS THE END POINT OF THE TRIAL? Trials can be designed to investigate different end points: disease prevention, screening methods, diag-

Clinical trial participants receive cutting-edge treatments, often for free.

nosis, quality of life, or the safety and efficacy of particular treatments. The end point of the study should be stated clearly in the informed consent document. If you're not clear about the objective of the research, ask the trial coordinator for an explanation.

2 WHO IS RUNNING THE TRIAL, AND WHAT ARE THEIR CREDENTIALS?

Generally, the principal investigator (PI) of the study spearheads the research, while the clinical trial coordinator handles the logistics and acts as the primary contact for patients. Each study will also have a sponsoring firm or institution. Ask about any potential financial conflicts of interest that the PI may have. Does he or she own stock in the company sponsoring the study and stand to benefit financially from positive results? If so, you might want to reconsider participating.

3 WHAT ARE THE RISKS AND POSSIBLE SIDE EFFECTS OF THE EXPERIMENTAL THERAPY?

Potential problems should be spelled out clearly in the informed consent document. You can also talk to the trial coordinator or your primary care doctor about possible adverse reactions. While certain studies may involve risk, the treatment likely will have been tested on animals and in healthy human volunteers first. Some trials test drugs that have been approved by the U.S. Food and Drug Administration for different uses, so the risks associated with these drugs should be well documented. You can find this information at fda.gov or pdr.health or by asking your doctor or pharmacist.

You can also ask the trial coordinator what phase the trial is; generally the

earlier the phase, the more risk involved. (See "Phases of the Trial.")

4 HOW LONG WILL I BE PARTICIPATING, AND CAN I STOP IF I CHANGE MY MIND?

Length of time varies with each particular trial protocol, but you are always free to drop out of a research study at any time. The informed consent document you sign at enrollment, which should inform you of this right, is not a contract that commits you to finish the trial.

5 WHAT ARE THE CHANCES I WILL RECEIVE THE ACTIVE TREATMENT RATHER THAN A PLACEBO OR ALTERNATE TREATMENT?

In open trials all subjects get the drug or treatment in question; in randomized trials participants are divided into groups, and some get the active treatment while others get a placebo. In double-blind studies, neither investigator nor patients know who received which. Ask what the odds are that you will receive the drug or placebo, as subjects usually don't learn whether they received the drug or the placebo until the study ends.

6 WHAT IS REQUIRED OF ME? Be sure you're clear about your obligations, such as how frequent the medical visits will be, and what procedures—such as a blood test or MRI—you will undergo.

7 IS THERE ANY COST TO ME? There usually isn't a cost to participants for the treatment being tested, but there could be for related medical services, such as monthly blood work. Some trials offer incentive pay to participants and may even reimburse travel expenses.

Clarify your financial responsibilities up front. If there is a high cost to you to partici-

pate, you should investigate the PIs and the study further.

8 WHAT HAPPENS IF I ENCOUNTER A PROBLEM DURING THE STUDY?

Find out who to contact in case you experience any adverse reactions and who pays for the associated medical care. The trial sponsor may cover some of these expenses; also check with your insurance carrier about what it will and won't cover.

9 HOW WILL MY PARTICIPATION AFFECT MY CURRENT TREATMENT?

Talk with your primary care doctor about your participation in the trial and keep him or her up to date as it progresses. If your doctor has questions or concerns at any point, he or she can speak to the PI or the trial coordinator.

10 HOW WILL MY MEDICAL DATA BE HANDLED?

The informed consent document should clearly spell out who has access to your study information. If you have particular concerns about how your information will be shared, talk to the trial coordinator.

"When you find a trial [everything] will be explained to you ahead of time," says Dr. Bedlack. "There shouldn't be any uncertainty by the time you're ready to start." If something is unclear, don't be shy about asking for further explanation.

"You never go into [a trial] not knowing what could happen," says Gianone. "You have to weigh the decision—whether you're helping find a cure—against the risk." NN

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