

**Why should I participate in research?  
Should I encourage members of my family to participate in research?**

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There are three compelling reasons why individuals with HD and those from HD families should participate in research:

- New treatments *must* be tested in HD.
- The more people that participate in HD research, the *faster* new treatments will be discovered.
- Participation in research *improves health and quality of life*.

**New treatments must be tested in humans.**

The two questions people should ask when a doctor recommends any new medication are: “Does it work?” and “Is it safe?” Suppose your doctor tells you about a new medication, “Well, this drug worked in mice, sheep and guinea pigs, and none of them had any side effects!” You might be a little skeptical about its safety and effectiveness. However, if your doctor said, “It was tried in 300 patients, and the patients who took the medication got much better, compared to a group of equally sick patients who did not take the medication.” Then if he added a description of the side effects that occurred in both groups of sick people, you would be a lot more willing to spend your hard earned money on the medication, and run the risk of having some side effects. New drug treatments must be rigorously tested in people to make sure they are safe and that they work. *There is no substitute for testing new medications in patients.*

**The more people participate in research, the faster new treatments will be discovered.**

Right now there are 5 major multi-center trials testing new medications in Huntington's disease. These trials are enrolling patients with Huntington's disease in the United States, Canada, Australia, New Zealand, and most of Europe. However, all of these studies are “behind schedule” because enough patients are not coming forward to participate in them. It is taking longer than we hoped to enroll people, which means it takes longer to find out if these drugs work. This slows down the process of finding the treatments for HD that we all hope will help patients now.

Drug discovery is a hit or miss process. A lot of drugs have to be tested to find the ones that really work. The longer it takes to find something that works, the more expensive it is for a drug company to sponsor the research. Drugs are very expensive to develop, which unfortunately is a growing concern in our current economy. A study that is “behind schedule” costs more to complete, and therefore, there will be less money to

study the next possible drug candidate. If HD patients can enroll in studies and get them done twice as fast, then the discovery of meaningful treatment will get here twice as fast. It is that simple.

**Those who participate in research tend to live longer and feel better.**

Studies show that patients who participate in clinical trials typically do slightly better than the average patient. Why is this? We are not sure, but people who feel like they are doing something to fight their disorder generally have a healthier attitude and less stress about their illness, and in the long run they tend to do better. People in clinical trials see a physician more often and have more screening laboratory tests than the typical patient, so if there are other health problems, they can be treated before they cause major problems. Participating in studies, regardless of their outcome, gives each participant additional satisfaction because they know that they have done something to help themselves, their families, and their community.

***Research participation is a way  
to take control over Huntington's Disease.***

**What kinds of research studies are available?**

The nice thing about HD research today is that everyone who has HD in his or her family is now able to participate in clinical research. There are two major types of studies: observational studies and treatment studies.

**Treatment studies:**

Patients with HD, who have symptoms, are sometimes eligible to participate in treatment trials. This type of research study involves having a patient sign up, take a medication or an identical placebo pill (with neither the research physician or the patient knowing which one it is), and at the end comparing the outcomes of patients on the study medication with the outcome of the patients on the placebo. It was this type of study that showed that Tetrabenazine was effective for controlling the chorea (involuntary movements) in HD. Patients who have a lot of chorea are likely to find it worthwhile to use this medication. Comparison with the placebo group was important, however, because it showed that Tetrabenazine, in addition to having good effects on chorea, also had some unattractive side effects: depression, stiffness, restlessness, and some slowed thinking. Those patients who have a little chorea and already have problems with depression and slowed thinking, therefore, may not be the best candidates for taking this new medication. Now patients and physicians can make some sensible choices about who should try this new medication, and because the side effects are well known, and the medicine can be stopped if these prove to be a problem for a given patient. This is the thought process behind every decision a patient and physician must make when choosing a medication for symptoms management in Huntington's Disease.

**Observational studies:**

This type of research involves careful and systematic observation of people with HD or with HD in their family. Typically these studies try to determine how to best measure the severity of HD. Since HD changes slowly over time, it can take a long time to tell if a medication is working or not. Right now, the co-enzyme Q 10 study (2-CARE) is designed to take 5 years to see if this medication actually slows down HD. If there was a special scan or blood test that could tell if the medicine was working within a few months, it would be possible to test 20 medications in the 5 years planned to test just this one medication. Researchers are particularly interested in the earliest changes with HD. Observational studies have shown that some changes may appear as early as fifteen years before a person is actually diagnosed with HD. By measuring these changes, it may be possible to test medications in individuals with the HD gene, even before they have enough symptoms to warrant a diagnosis. It just makes sense that the sooner treatment can be started in HD, the more likely it will prevent the symptoms that are seen when patients are diagnosed.

**Why is there a placebo in research treatment studies?**

As indicated above, it is known that people who participate in clinical trials feel better and do better than those who don't. Depression, for example, which is a significant part of HD, tends to respond very well to placebos. When testing new medications, patients want something more than just a placebo effect. Furthermore, it is important to know what sorts of side effects a treatment can be expected. If someone followed 150 HD patients for 6 months even without treatment, it is likely that a small percentage would have headaches, backaches, depressed mood, irritability, weight loss, rashes and even things like colds and the flu. By doing a placebo controlled study, then the two groups of patients (the ones on medication and the ones on placebo) can be compared both for good effects and side effects. So, it is essential that new medications be tested in this rigorous fashion.

This is not to say researchers are not interested in the possibility of finding unexpected medications that might help HD. HD patients all over the world are treated with different medications for a variety of different problems, and many patients use supplements or alternative medications. Thus there are plenty of medication trials all the time in HD patients that are not placebo controlled. If there are medications or alternative approaches that are remarkably effective, the internet, support groups, and physician groups interested in HD will find out, and arrange to put promising medications or treatments to more rigorous tests. Experience, however, has shown that very few new treatments for diseases are found this way. There have been concerted efforts by millions of people to find alternative therapies and medications that cause weight loss, younger looks, or better memory. As of yet, these exhaustive searches by very large numbers of highly motivated people have come up empty handed. Twenty years ago, before the discovery of the HD gene, such a random approach was about all that could be done for HD. Now there is an evolving pipeline of new treatment possibilities designed to fix HD, and large coordinated study groups like the HSG and CHDI. It may make sense to continue to hope for some random discovery among people trying new medications and

alternative therapies. But it makes more sense to focus the majority of available resources on the promising prospect of drug discovery in HD by human engineering and science.

### **What happens during a research study?**

All clinical research studies, observational or treatment studies begin with informed consent. This is a written document that the study center goes over with you regarding the details of the study and the best available information regarding risks and benefits of participation. A committee of individuals from the host's institution (or from a central committee if the host is not part of a major academic medical center) carefully screens informed consent procedures for each study. This committee helps to ensure that the study is conducted appropriately and that anyone who signs up for the study is aware of what is involved. Risks are explained, as well as options and rights a study participant has in choosing to participate. Every study allows the participant the right to withdraw if the participant so chooses, at any point during the study.

Most studies involve blood tests, physical examinations, and other tests such as EKG, urine studies etc. Sometimes other tests are needed like brain scans or muscle tests or tests that require more time or effort. Individuals who participate in studies should know that their ability to make the regular visits in the study is very important. Participants also need to know that the accuracy of the research study depends upon taking the medication as directed. Reporting any and all possible side effects or other changes in their health is important as well. If for some reason, these three steps are not something that a person can comply with, research studies may not be something that will work out well. On the other hand, if a patient has successfully completed a research trial with a center that does studies, when possible they are often asked back to participate again.

For more information, check out these links:

Links:

General information about clinical trials from the FDA:

<http://www.fda.gov/oashi/clinicaltrials/default.htm>

"Your Rights and Informed Consent" by WebMD:

<http://www.webmd.com/content/pages/13/65821.htm>

HD Trials registry:

<http://hdtrials.org>

Where to learn more about Clinical Trials:

Huntington's Study Group: [Huntington Study Group](#)

CHDI Foundation, Inc: <http://www.highqfoundation.org/>

European Huntington's Disease Network: [Clinical Research Portal at the University College London](#)

Hereditary Disease Foundation: [Hereditary Disease Foundation](#)  
HD Lighthouse: <http://hdlighthouse.org/research/trials/>