

Now Enrolling: 2CARE Trial of Coenzyme Q10 in Huntington Disease

By Lisa J. Bain – March 2008

In March, 2008, a clinical trial began that will test the ability of a naturally occurring compound called Coenzyme Q10 (CoQ10), to slow progression of Huntington disease (HD) in its early stages. The trial, called 2-CARE, follows an earlier study that tested CoQ10 at a lower dosage with slight, but not statistically significant benefits. This new study expands on the earlier study using the compound at a much higher dosage and longer treatment period.

Why Coenzyme Q10?

CoQ10 is a vitamin-like substance produced in the human body that is involved in energy production in cells. Because it has anti-oxidant properties and scavenges free radicals, it is thought to protect cells and tissues from damage. Several studies have suggested that levels of CoQ10 are reduced in many disease states, including HD. CoQ10 is widely available as a supplement in health food stores and has been promoted for a wide variety of conditions, including high blood pressure, Alzheimer disease, heart disease, HIV/AIDS, and migraine. However, there is limited evidence supporting these uses, and since it is sold as a supplement rather than a drug, it is not regulated by the Food and Drug Administration (FDA). This means that the purity and strength of the product you buy in the store may be inconsistent.

The first trial of CoQ10 in people with HD followed animal studies that showed some benefits in HD mouse models. In this first clinical trial, subjects received a dosage of 600 mg/day. While this dose did not seem to slow progression of the disease at what statisticians would call a “statistically significant” level, a trend towards improvement motivated the researchers to try again at a higher dosage and longer treatment duration.

Who will be eligible to take part in the trial?

People can participate in this trial if they have symptoms suggesting that they are in the early stages of HD and have either a family history of the disease or had genetic testing showing that they carry the HD mutation. People who have taken CoQ10 in the past will be allowed to participate in the trial, but only if they stop taking the supplement before starting the trial. A total of 608 participants will be enrolled in the trial.

Who is conducting the trial, and where?

The trial is being run by the Huntington Study Group (HSG) at approximately 40 different HSG sites in the United States and Canada. Dr. Merit Cudkowicz, a neurologist at the Massachusetts General Hospital, leads the study along with biostatistician Michael McDermott and neurologist Karl Kieburtz from the University of Rochester Medical Center.

What will participants in the trial do?

Participants in the trial will be randomly assigned to receive either CoQ10 or [placebo](#), but neither they, nor their treating physicians, will not know which they are getting. Each participant will be followed for five years, undergoing a series of tests every six months to determine if the disease has progressed and, if so, by how much.

Is this higher dosage safe?

In preparing to study whether CoQ10 would be effective if given at a higher dosage, the clinicians running the study first needed to show that a higher dosage was safe. In a preliminary study called Pre2CARE, they tested doses ranging from 1200 mg/day to 3600 mg/day for 20 weeks. This study showed that other than some mild upset stomachs, the drug did not cause any significant adverse effects. A dosage of 2400 mg/day was therefore chosen for subsequent studies, since higher dosages did not raise the level of the CoQ10 in the blood.

What if a significant difference in progression of the disease or adverse effects becomes apparent before the end of the trial?

A separate group of investigators, called the “Data Safety Monitoring Board,” will monitor the trial and can stop it if the study drug raises safety concerns. In addition, the trial could be stopped if an interim analysis showed either that the treatment appeared to offer no benefits or if the benefits were so significant that it would be unethical to continue a clinical trial.

Why should people take part in this trial knowing that they might get placebo rather than CoQ10?

According to Dr. Cudkowicz, patients are eager to take part in this trial even though they may be assigned to the placebo arm of the study. “HD patients and their families understand that the fastest way for the community to get an answer on whether a treatment works or not is to do these placebo controlled trials,” said Dr. Cudkowicz, adding that “it’s still altruistic for them to participate in trials.”

Dr. Cudkowicz also noted that the dosage being used in this trial is much higher than that typically used by people who buy CoQ10 over the counter, so it might not be wise for people to try this higher dosage outside of a clinical study where there is close safety monitoring.

Although Dr. Cudkowicz will not be a treating physician for this trial, she will refer her patients to another physician if they are interested. “I tell them that the science behind this drug is exciting and that data from different laboratories shows that CoQ10 improves how your cells make energy.” Clinical trials are needed to determine if a new treatment works in HD, she said.