



UPDATE ON THE 2CARE STUDY “COENZYME Q₁₀ IN HUNTINGTON’S DISEASE

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There is a strong rationale for testing coenzyme Q₁₀ (CoQ₁₀) in people with HD based on the following observations: (1) CoQ₁₀ targets a relevant disease pathway, mitochondrial bioenergetics; (2) CoQ₁₀ has been shown to be neuroprotective in *in vitro* and *in vivo* models of HD and other neurodegenerative disorders; and (3) CoQ₁₀ at 600 mg/day appeared to slow disease course in a previous randomized, double-blind, placebo-controlled trial in people with HD followed for 30 months. Initial studies in both Parkinson's disease and Progressive Supranuclear Palsy showed beneficial clinical effects.

Taken together, this information strongly supports the testing of high-dosage CoQ₁₀ in people with HD.

There are currently no data available to determine whether results (positive or negative) of an intervention study in a mouse model of HD can predict corresponding effects in humans. An answer to this question will require the establishment of treatments that actually slow disease progression in people with HD. There is also known variation in results between different laboratories studying the same interventions in mouse models of HD. Therefore, it is important to consider all the data available on a possible therapeutic agent to decide whether to pursue its development as a therapy for HD. For a disease without a known treatment, it would not be appropriate to discard a therapy because a particular laboratory did not see an effect in a particular mouse model.

The Huntington Study Group is currently conducting a study called 2CARE (Coenzyme Q₁₀ in HD) to learn if CoQ₁₀ can help to slow the worsening of everyday activities of daily living in people with HD. Forty -three centers in the US, Canada and Australia who specialize in the care of people with HD are seeking participants for this 5-year study. Individuals with mild to moderate HD will be screened for eligibility and if enrolled, will have study visits at Months 1, 3, 6 and every 6 months following enrollment. Some travel assistance may be available.

The study doctor at each site is a specialist in Huntington's disease. Participants will receive either active study medication (CoQ10) or a matching inactive substance in this randomized, double-masked study and provide blood and urine samples in the beginning of the study and periodically over the 5 years to check general health and for research analysis.

The study is being led by Drs. Merit E. Cudkowicz (Massachusetts General Hospital) and Michael P. McDermott (University of Rochester) in collaboration with the National Institute of Neurological Disorders and Stroke (NINDS). For more information about the study, we encourage you to visit the HSG website (<http://www.huntington-study-group.org/>), where contact information for the participating sites is located, and get in touch. The individuals listed can answer your questions and provide further details about the study.