

# HSG studies.....



**2CARE:** A Multi-center, Randomized, Double-blind, Placebo-controlled Study of Coenzyme Q10 (CoQ) in Individuals with HD.

The purpose of 2CARE is to find out if **Coenzyme Q10 (CoQ)**, a naturally occurring substance in the body, is effective in slowing the worsening symptoms of HD. CoQ is available for purchase over the counter as a nutritional supplement, but is being studied as an investigational drug at a higher dosage than is currently available for purchase. In this study, researchers also will learn about the safety and acceptability of long-term CoQ use by determining its effects on people with HD. This study is supported by a grant from the National Institute of Neurological Disorders and Stroke (NINDS).



**CO**operative **H**untington **O**bservation **R**esearch **T**rial

COHORT is a multi-center **observational** study which began in 2005 to collect ongoing information from individuals who are affected by HD and those who are part of an HD family. The HSG is collecting this information in order to learn more about HD, potential treatments, and to plan future research studies of experimental drugs aimed at postponing the onset or slowing the progression of HD. This study is sponsored by CHDI Foundation, Inc.



**CRE**atine **S**afety, **T**olerability, & **E**fficacy in HD.

The purpose of CREST-E is to study the effects of **Creatine Monohydrate** in persons who have clinical features of HD. CREST-E is designed to assess whether high doses of pharmaceutical grade creatine monohydrate can slow the functional decline that occurs in HD patients, in comparison to placebo. CREST-E is also designed to assess the general safety and tolerability of creatine and to examine its effects on the body. This study is sponsored by the National Center for Complementary and Alternative Medicine (NCCAM) and the U.S. Food and Drug Administration (FDA) Orphan Products division. Enrollment begins 3<sup>rd</sup> quarter of 2009.



**HART** A Multi-Center, North American, Randomized, Double-Blind, Parallel Group Study Comparing Three Doses of Pridopidine (ACR16) vs Placebo for the Symptomatic Treatment of HD.

The purpose of HART is to confirm whether the experimental drug, **Pridopidine (ACR16)** is efficacious in improving voluntary motor function in HD, symptoms that seem to be most important for the functional disability associated with the

disorder and to determine the most effective dose. Pridopidine 'modulates' the action of dopamine, an important chemical which contributes to brain damage in HD patients. Three different doses of ACR-16 will be compared to placebo over a 3 month period. This study is sponsored by NeuroSearch Sweden AB.



A Phase III, Randomized, Double-Blind, Placebo-Controlled, Safety and Efficacy Study of Dimebon in Patients with Mild-To-Moderate HD.

The purpose of HORIZON is to assess the safety and efficacy of the experimental drug **Dimebon** when taken over a period of 6 months. Efficacy is determined by exploring whether or not there is an effect on cognitive (thinking) abilities and overall functioning in people with HD. The study will also evaluate other HD symptoms including behavioral and motor (movement) symptoms. Research has shown that Dimebon might be effective in preventing the death of brain cells in animals and improving thinking and memory in Alzheimer disease. Results of a HD study in the United States and United Kingdom showed that Dimebon 60mg per day was safe and well tolerated and suggested that Dimebon may improve cognition (thinking abilities) in individuals with HD. This study is sponsored by Medivation, Inc in collaboration with Pfizer, Inc. Enrollment begins 3<sup>rd</sup> quarter of 2009.



**PREDICT**ors of **H**untington **D**isease

The PREDICT-HD study is an **observational** study that uses a variety of tests to examine the nature and pattern of neurobiological changes and neurobehavioral changes that occur in the period preceding an HD diagnosis. The intent of the study is to learn more about the beginning changes in cognition, emotion regulation, brain structure and brain function as a person transitions from health to HD. This study is supported by a grant from the National Institute of Health (NIH).



Study in **PRE**-manifest HD of Coenzyme **Q10 (U**biquinon**E)** **L**eading to Preventive Trials.

The purpose of PREQUEL is to assess the safety of **Coenzyme Q10 (CoQ)** in healthy people who have tested positive for the HD gene expansion and are "pre-manifest", that is, they have not been clinically diagnosed with HD. The study is not designed to see whether or not CoQ is an effective treatment for HD, but that it is safe and well tolerated over the course of 20 weeks in pre-manifest individuals. This study is supported by a grant from the National Institute of Neurological Disorders and Stroke (NINDS). Enrollment begins 3<sup>rd</sup> quarter of 2009.








.....a summary

# H • S • G

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Seeking Treatments That Make a Difference For Huntington Disease

# HSG studies.....

HSG STUDY	Location of participating sites	Length of study	No. of in-person visits	Investigational Drug	Main Inclusion Criteria		Enrollment Start Date	Currently Enrolling?
					Age	HD Status		
 COHORT	USA, Canada, Australia	Ongoing	1 per year	None. Observational Study	15+	All members of an HD family. Can also participate in other HSG interventional trials while participating in COHORT.	February 2006	Yes
 2CARE	USA, Canada, Australia	5 years	14	Coenzyme Q10 (CoQ)	16+	Clinical features of HD, and a confirmatory family history of HD or CAG repeat expansion $\geq 36$	April 2008	Yes
 CREST-E	USA, Canada, Australia, New Zealand, Europe	36 months	16	Creatine Monohydrate	18+	Clinical features of HD, and a confirmatory family history of HD or CAG repeat expansion $\geq 36$	Projected 3 <sup>rd</sup> Quarter 2009	No
 HART	USA, Canada	14-16 weeks	7	Pridopidine (ACR-16)	30+	Clinical features of HD, and a confirmatory family history of HD or CAG repeat expansion $\geq 36$	September 2008	Yes
 HORIZON	USA, Canada, Australia, Europe	6 months	8	Dimebon	30+	Clinical features of HD, and a CAG repeat expansion $\geq 36$	Projected 3 <sup>rd</sup> Quarter 2009	No
 PREDICT	USA, Canada, Australia, Europe	Ongoing	1 per year	None. Observational Study	18+	Gene positive with no diagnosis of HD or Gene Negative	September 2001	Yes
 PREQUEL	USA	20 weeks	7	Coenzyme Q10 (CoQ)	18+	CAG repeat expansion $\geq 36$ and pre-manifest based on a physical exam	Projected 3 <sup>rd</sup> Quarter 2009	No

.....at a glance