

# PHASE 3 TRIAL OF COENZYME Q<sub>10</sub> IN MITOCHONDRIAL DISEASES

This study is sponsored by the FDA Orphan Products Division, Tishcon Corp., & General Clinical Research Centers funded by the National Institutes of Health.

## Study Information

The study will help provide information to determine if Coenzyme Q<sub>10</sub> (CoQ<sub>10</sub>) is a safe and effective treatment for children with mitochondrial diseases. There are no cures for mitochondrial diseases at this time. Treatments have sought to help reduce the symptoms or delay the progression of the disease. CoQ<sub>10</sub> is a mitochondrial cofactor and antioxidant that is a part of the process by which food and oxygen are converted into energy by cells.

## Eligibility

Children age 12 months to 17 years with biochemical or genetic proof of a mitochondrial disease. There are factors that could exclude some children with a mitochondrial disorder from participating. Ask the study coordinator if your child is eligible to participate.

If your child is not eligible, that does not mean that he/she does not have a mitochondrial disorder, as there are many specific factors that are part of the selection process.

## Participation

Your participation is important because: It will contribute to medical knowledge about mitochondrial diseases, help determine the benefit of CoQ<sub>10</sub> in treating mitochondrial diseases, and help future clinical research trials look for specific treatments for people with mitochondrial deficiencies.

## Potential Benefits

- 1) Developmental evaluation of your child
- 2) Evaluation of his/her motor skills
- 3) Objective evaluation of effectiveness of CoQ<sub>10</sub>

## *S t u d y D e s i g n*



### Visits

Total of 5, one every 3 months (for about 1-2 days), at one of the participating centers.

### Meds

CoQ<sub>10</sub> everyday for 6 months and placebo everyday for 6 months. In addition, each child will be given a nutritional cocktail (vitamins) and vitamin daily. Your child will be allowed to continue taking all medications necessary for his/her health.

You will be asked to keep a daily medication diary of the study medications during the course of the trial.

### Tests

- Medical History
- Physical Exam
- Neuromuscular exam
- Six minute walk test (if able)
- Blood and urine tests
- Speaking with a dietician about your child's diet
- Completing developmental, sleep, behavior and quality of life questionnaires



### **PARTICIPATING CENTERS**

- ◆ General Clinical Research Center, *University of Florida*, Gainesville, Florida
- ◆ William T. Dahms Clinical Research Unit, *University Hospitals Case Medical Center*, Cleveland, Ohio
- ◆ *Cincinnati Children's Hospital Medical Center*, Cincinnati, Ohio
- ◆ *Hospital for Sick Children*, University of Toronto, Toronto, Ontario, Canada

### **FOR MORE INFORMATION CONTACT:**

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### **STUDY COORDINATOR AT:**

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