

Michigan Chapter Social Worker

Hello everyone, I hope all is well. I have some exciting news about a certain drug that has finally been approved, Tetrabenazine!

The U.S. Food and Drug Administration has approved Tetrabenazine for the treatment of chorea in people with Huntington's disease. Tetrabenazine is a dopamine depletor. It works by inhibiting the vesicular monoamine transporter.

Since Tetrabenazine also reduces serotonin and norepinephrine. Depression is a possible side effect. However, if depression occurs it can be treated by antidepressants.

"Tetrabenazine or (Xenazine) represents hope for patients and families with this difficult disease," said Timothy Cote, M.D., M.P.H., director of FDA's Office of Orphan Products Development. "For the first time, there is a treatment that can help patients with this disease gain some quality of life."

The most common side effects reported by patients using Tetrabenazine in clinical trials include, depression, drowsiness, restlessness, and nausea.

For more information

FDA: Questions and Answers on Risk Evaluation and Mitigation Strategies

http://www.fda.gov/cder/regulatory/FDAA/FR_QA.htm

FDA: Office of Orphan Products Development

www.fda.gov/orphan

National Institute of Neurological Disorders and Stroke: Huntington's disease Information Page

www.ninds.nih.gov/disorders/huntington/huntington.htm

I also attended the national meeting in Pittsburgh, Pennsylvania and it was very educational. The most significant piece of the meeting that resonated throughout me was the clinical trials. Here is a brief guide to what the clinical trials consist of:

WHAT ARE THE HUNTINGTON CLINICAL TRIALS?

- They are research studies utilizing human volunteers to answer specific disease questions or to test treatments.
- Each is conducted according to a specific protocol. The protocol describes the type of patient that will be included, the schedules of tests and procedures, the drug and dosages that will be tested, the length of the study, and the type of outcome measures. Outcomes are usually measures of motor, cognitive, or behavioral function.
- You must agree to follow the study procedures.
- Your participation in any study is voluntary and you can stop at any time.

WHY DO CLINICAL TRIALS?

- To find ways to speed up clinical trials. Understanding the natural course of the disease will allow development of biomarkers that can be used in therapeutic trials to test drugs. PREDICT AND COHORT are examples in the U.S. TRACK HD is a similar trial offered in Europe and Canada.
- To find out if supplements or available drugs are safe and effective treatments.
- To find out if new experimental drugs are safe and effective.

WHAT ARE THE BENEFITS OF PARTICIPATING IN CLINICAL TRIALS?

- To gain access to new research treatments.
- To receive expert medical care.
- To help others by contributing to medical research and treatment advances, though there may be no direct benefit to you.



WHAT ARE THE POSSIBLE RISKS?

- There may be unpleasant, serious, or even life threatening side effects. Risks may vary on the type of study and treatment being used.
- The treatment might not be effective. Participation in a trial may be stressful and time consuming.

HOW ARE VOLUTEERS PROTECTED?

- The FDA must determine that a new treatment has passed (Tetrabenazine) safety studies in animals, and has scientific basis for potential benefit before it gives permission for a clinical trial.
- The Institution Review Board (IRB) determines if protocols are ethical before allowing the start of any trial at institutions or clinical sites.
- Safety committees review results during trials to check for unexpected safety problems

QUESTIONS TO ASK BEFORE ENROLLING IN A CLINICAL TRIAL?

- What is the main purpose of the study?
- Does the study involve a placebo or a treatment already on the market?
- How will the treatment be given?
- How long will the study last and what will I be asked to do?
- What has already be learned about the study treatment?
- Is there any reimbursement for travel or child care?
- Will I be able to see my own doctor?
- Will I be able to continue drugs or supplements that I am already taking?
- If the treatment works for me, can I keep using it after the study?
- Can anybody find out that I'm participating in a clinical trial?
- How will my information be kept confidential?
- Will I receive follow up care after the study is completed?
- How long will the study last?
- What has already been learned about the study treatment?

Here are the sources for clinical trial information:

Huntington Study Group (HSG) www.huntingtonstudygroup.org

National Institute of Health (NIH): 1-800-411-1222 – www.Clinicaltrials.gov

Centerwatch Listing Service: 617-856-5900 – www.CenterWatch.com

PhRMA Database: www.clinicalstudyresults.org

To Sign up for Clinical Trials go to: www.hdtrials.org

Please feel free to call me if you have any questions @ 1-800-909-0073

LET'S FIGHT THE GOOD FIGHT!!!!!!!!!!!!

Danny Berry – Social Worker/Family Service Coordinator