New Drug for Chorea
Tetrabenazine

Receives “Fast Track” Designation
From the FDA for Chorea Associated with Huntington’s Disease

WASHINGTON--(BUSINESS WIRE)--Sept. 16, 2004--Prestwick Pharmaceuticals, Inc., a CNS (Central Nervous System) specialty pharmaceutical company, announced today that tetrabenazine has been designated as a “fast track” product by the U.S. Food and Drug Administration (FDA). Tetrabenazine has been designated as an orphan product by the FDA. Tetrabenazine is being evaluated for the treatment of chorea associated with Huntington’s disease. Currently in pivotal clinical testing in the United States, tetrabenazine is a novel dopamine depletor that works by selectively blocking the VMAT2 (vesicular monamine transporter) in the CNS.

The Fast Track Program is designed to expedite the review of drug compounds for the treatment of patients with serious or life-threatening diseases where there is an unmet medical need for new therapeutic approaches. The benefits of fast track designation provide for multiple meetings, timely comments, and a priority review at the FDA.

“This treatment addresses an unmet medical need, as there are currently no approved drugs for the treatment of chorea in Huntington’s disease. The recent FDA action underscores the need for therapeutics for people with Huntington’s Chorea and is most encouraging,” said Kathleen Clarence-Smith, MD, PhD, Acting Chief Executive Officer, Prestwick Pharmaceuticals. “Prestwick is engaged in multiple programs to study the efficacy and safety of tetrabenazine in this patient population. We look forward to compiling the data generated from these trials and submitting it to the FDA.”

Tetrabenazine is approved for use in several European countries and Australia as Xenazine(TM) and in Canada as Nitoman(TM).

Dr. Hank Paulson at August support group

Dr. Henry Paulson spoke at the August 2004 HD Center of Excellence support group meeting. He provided the group with an update of the exciting developments in HD research. Dr. Paulson also spoke about a recent report by Dr. Beverly Davidson, one of his colleagues at the University of Iowa. Dr. Davidson and her research team were studying the usefulness of a new technique called RNA interference in the treatment of HD. Briefly, the research group used this technique in an HD mouse model population. Accordingly, they were able to turn off the HD gene and repair some HD related damage. Further research will be conducted in HD animal models before this new technique is tested on humans.

Furthermore, Dr. Paulson provided a brief update on a number of clinical trials related to HD: MINO-HD, TETRA-HD, and PHEND-HD. The MINO-HD and TETRA-HD studies have recently been completed. Look to see more information about these clinical trials in upcoming newsletters.

Dr. Paulson was asked to comment on the role of stem cell research in HD research. He noted two ways in which stem cells are typically used in research. First, researchers may one day be able to use stem cells to replace cells that have been damaged or destroyed in the body. For example, this type of research may lead to treatments for individuals with spinal cord injuries. Dr. Paulson noted that this type of research is less likely to benefit individuals with HD, due to the progressive nature of the disease and the genetic involvement in HD. For example, if cells destroyed by HD were successfully replaced, these new cells may also die, as the gene responsible for HD will still be present in these individuals. However, a second role of stem cell research is the ability to create a line of cells. This type of research may prove useful in HD research. Researchers may use stem cells to create a line of cells affected with HD and, in turn, use these cells to better understand the mechanisms responsible for HD and to test potential interventions for HD.

Dr. Paulson will address sleep problems in HD in a future issue.

Check our website at http://www.uihealthcare.com/depts/huntingtondisease/services/ask the team to read more about RNA interference.
Where in the US is the Huntington’s Quilt?

If you attended the June 2004 National convention in St Louis, you witnessed two beautiful quilts with squares made by individual families affected by HD. The first quilt was also displayed in the lobby of the University of Iowa Hospitals and Clinics the week prior to the Iowa HDSA meeting on July 17, 2004. Both quilts were showcased at the Iowa meeting.

Members of the Hunt-Dis on-line Huntington’s Disease support group initiated a national Huntington’s Disease Memorial Quilt project to honor all loved ones with Huntington’s Disease in late 1999. This was a major undertaking and requests for patches were sent to HDSA Chapters, HD Support Groups, HD Centers of Excellence, and from families and friends living with HD.

The Huntington’s Disease Memorial Quilt is available for loan from Hunt-Dis to any HDSA Chapter, Support Group, Center of Excellence or HD family supporting an HD awareness, educational or fund raiser event.

Each quilt comes with a book of bios and pictures of each square. The Quilts are a powerful and beautiful reminder of the terrible impact Huntington’s Disease has in all of our lives. They make a very visible statement to those attending an event.

The Quilt has been to International Huntington’s Disease (IHA) conventions, to the Canadian (HSC) convention, and every HDSA National Convention since it was unveiled in 2000. Additionally, the Quilts have traveled throughout the United States honoring people with Huntington’s Disease.

Hunt-Diser Marie Nemec has, once again, graciously agreed to help with the Phase III of the Huntington’s Disease Memorial Quilt. The quilt is made from individual twelve-inch blocks. These blocks will be incorporated into the new section of the HD Memorial Quilt. Contact Marie Nemec by phone (970) 434-8323 or email rmnemec@earthlink.net

HDSA Support Groups:

Des Moines
Iowa Lutheran Hospital
University Ave at Pennsylvania
4th Friday at 6:30 pm

Lincoln, Nebraska
Perkins Family Restaurant
121 North 48th St
1st Tuesday at 7:30 pm

Omaha, Nebraska
Village Inn Restaurant
78th and Dodge
2nd Monday at 6:00 pm

SW Iowa
600 N. 21 st
Clarinda, IA
1st Monday at 7:00 pm

Iowa City
University of Iowa Hospitals and Clinics
Della Ruppert Conference Room
4th Sunday at 1:00 pm

What Huntington’s Cannot Do

Huntington’s is so limited
It cannot cripple love
It cannot shatter hope
It cannot erode faith
It cannot destroy peace
It cannot kill friendship
It cannot suppress memories
It cannot silence courage
It cannot evade the soul
It cannot steal eternal life
It cannot conquer the spirit.

Author unknown
from Faces of Huntington’s
by Carmen Leal-Pock

Many Families affected by HD joined the HD Center of Excellence staff at the 2nd annual In Harmony for Huntington’s Disease at the Mill in Iowa City on October 8th. They were entertained by 4 local bands. Raffle prizes and silent auction events were highlighted. Stay tuned next addition for pictures and information on funds raised.
NEW FACES in PAULSEN LAB

There are many new changes in the Paulsen lab this fall with five new Research Assistants hired on a variety of projects.

Please welcome Ellen Samuel, a recent University of Iowa B.A. graduate in Psychology and Spanish. Ellen worked as a student in the lab last year and was impressed with the many different research projects. She grew up in the famous community of Oak Park, Illinois—birthplace of Ernest Hemingway and home of Frank Lloyd Wright. Ellen is the coordinator of STARTLE and is also working on neuroimaging projects.

Sara Van Der Heiden has her B.A. in Psychology from Coe College in Cedar Rapids, Iowa. She is working on a variety of projects including neuroimaging (analyzing MRI and PET scans). She is also the coordinator of a bone marrow transplant study that investigates cognitive functioning. She is a rollerblader who uses her blades to complete her work activities; she only needs to learn to stop.

Kimberly Bastic graduated from the University of Iowa with a B.A degree in Psychology. She was working on a research project examining chronic pain at the University of Wisconsin before returning to Iowa. Kimberly is the current coordinator for the Predict-HD study. She claims that she is not very interesting but seems to be full of stories. Maybe she is making them up.

For more information about these projects and new studies that start soon, call or email Elizabeth Penziner at (319) 353-4292 or elizabeth-penziner@uiowa.edu

PREDICT-HD

This trial will help define the earliest changes in HD and this information helps researchers evaluate the effectiveness of future treatments more accurately. Predict comprises individuals who are gene tested and are pre-symptomatic. Participants are evaluated once a year. Evaluation includes neuropsychological testing and MRI scans.

PHAROS

Individuals who participate in this trial help researchers define the natural history of HD in its earliest stages. This study gives researchers a more complete picture of the disease’s initial signs and the factors that influence age of onset. PHAROS participants are evaluated at nine-month intervals. The study is not accepting new participants.

PET

Positron Emission Topography, a type of brain scan, is being used to examine brain activity in persons at risk for HD. Individuals who are gene tested and are pre-symptomatic are being recruited.

MRI

Participants receive a magnetic resonance imaging scan, which produces a picture of an HD brain structure. This provides useful information about the disease process in HD. Individuals who are gene tested are eligible to participate.

STARTLE in Pre-symptomatic HD

The purpose of this trial is to characterize the startle reflex (an unlearned response to a startling stimulus). Participants listen to varying decibel levels of sound through headphones, and their eye blink activity is measured.
How to Talk to your MD
About Your Disease or Disorder...

What is my diagnosis?
What caused my condition?
Can my condition be treated?
How will this condition affect me now and in the future?
Should I watch for any particular symptoms and notify you if they occur?
Should I make any lifestyle changes?

About My Treatment...
What is the treatment for my condition?
When will the treatment start, and how long will it last?
What are the benefits of this treatment, and how successful is it?
What are the risks and side effects associated with this treatment?
Are there foods, drugs, or activities I should avoid while I’m on this treatment?
If my treatment includes taking a medication, what should I do if I miss a dose?
Are other treatments available?

About My Tests...
What kinds of tests will I have?
What do you expect to find from these tests?
When will I know the results?
Do I have to do anything special to prepare for any of the tests?
Do these tests have any side effects or risks?
Will I need more tests later?

Understanding your doctor’s responses are essential for good communication. Here are a few more tips:
If you don’t understand your doctor’s responses, ask questions until you do understand.
Take notes, or get a friend or family member to take notes for you. Or, bring a tape-recorder to assist in your recollection of the discussion.
Ask your doctor to write down his or her instructions to you.
Ask your doctor for printed material about your condition.
If you still have trouble understanding your doctor’s answers, ask where you can go for more information.

Other members of your health care team, such as nurses, pharmacists and social workers are good sources of information. Talk to them, too.

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