The Phases of HD Research: A Focus on Translational Research

Research aimed at finding treatments and a cure for HD can be divided into three phases: basic research, applied or translational research, and clinical trials. HD research is currently progressing in all three phases, but translational research is often seen as the element that bridges the gap between basic research and clinical trials.

The primary goal of basic research is the identification of targets for possible intervention. Scientists work to understand HD at the level of individual brain cells. They study the process of brain cell malfunction and death in HD. Once these processes are better understood, interventions can be identified that prevent the processes from occurring.

Clinical trials are aimed at determining whether potential interventions are successful in treating HD. Clinical trials occur under the watch of the Food and Drug Administration (FDA). Three phases of clinical trials determine whether the drug is safe for humans and effective at treating HD before the drug can be made available to the general public.

Applied or translational research, therefore, is designed to discover interventions that “hit” the targets identified by the basic researchers. Researchers screen potential compounds for their ability to stop or prevent the disease process from occurring. These compounds are first tested on HD cells in a test tube and later in animal models. Once a compound succeeds at this level, it moves to the clinical phase of research.

The National Institutes of Health (NIH) has made translational research a priority. It has launched and funded centers of translational research through its Clinical and Translational Science Award (CTSA) program started in 2006. The University of Iowa is one of 24 CTSA funded academic centers already established.

Translational research is often referred to as “bench-to-bedside” or translating research into practical applications or new treatments. The goal of basic science to produce new drugs and treatments for patients (clinical trials), and this requires a flow of information from hospitals, clinics and study participants to facilities that collect specimens, research-based facilities and labs.

The need to generate and analyze data accelerates the knowledge database. Collaboration and data sharing is of fundamental importance. If you are participating in an HD clinical trial, expect a statement in the consent document asking to retain your blood samples at a repository supported by National Institute of Health (NIH). It is the compilation of data that continues to inform and spur new projects.

The second part of translational research is aimed at enhancing the adoption of best practices in the community, which includes increasing public knowledge. This means bringing results of studies into everyday clinical practice and into common health decisions. The process starts with health care professionals but includes patients, public health administrators and consumers.

Researchers continue to need your participation to move the process along of finding a treatment for HD. Thank you for your participation!
A Day in the HD Clinic
By Sean Thompson
Editorial Associate

In a cramped conference room on the third floor of the University of Iowa Hospitals and Clinics, about 10 doctors and research assistants have gathered as they do every month to provide an important service to a handful of patients with HD.

This is the UI Huntington’s Disease Center’s monthly clinic for patients with HD. It’s my first time visiting clinic and I listen as Dr. Peg Nopoulos, a psychiatrist, discusses the first patient with social worker Anne Leserman. The clinic staffers, who have multiple duties with their own research and other clinical responsibilities, display sharp focus when they talk about the patients.

On this day, I’m privileged to observe two types of examinations with a female patient in her sixties who had been previously diagnosed with HD. The first examination, conducted by Dr. Nopoulos, involved questions designed to get a sense of his psychiatric condition. Nopoulos asked her about everything from what she eats for breakfast to whether she was feeling depressed or suicidal. The patient took the personal questions in stride and answered earnestly. I don’t think I could have handled such questioning so well.

Next, Dr. Teri Thomsen, a neurologist, performed a motor examination on the same patient. She tested her ability to complete a variety of tasks meant to assess her movement, balance and neurological symptoms.

Since joining the psychiatry staff at the University of Iowa a few weeks earlier, this was my first time witnessing the devastating effect HD can have on one’s ability to do simple physical tasks. The patient was able to complete some of the tasks, like walking back and forth and turning her hand over and under her other hand. But every task seemed to require multiple attempts and a lot of concentration.

A topic that wasn’t easy for anyone in the examination room to discuss was the patient’s driving ability. Because of the deterioration of her motor skills, both Thomsen and Nopoulos recommended that she should no longer drive, even for short distances.

“I never say these things lightly because I know what it means to be able to drive,” Nopoulos told the patient.

I could see the disappointment in the patient’s face when Thomsen advised her husband to take her keys from her.

During the clinic visit, patients are also evaluated by a neuropsychologist. Patients go through written and verbal testing to assess their memory, speed at which they process information and ability to multitask. The tests paint a clearer picture of the effect HD is having on their cognitive ability.

I left the clinic that day with a better understanding for the difficulties people with HD live with every day. It was a sobering couple of hours. But I was lifted by the resolve of the patients and their families. Though weary at times, they displayed a steadfastness and pragmatic approach to their condition that was impressive and inspiring.

Even though HD has changed their lives forever, they are living each day the best they can.

HD Support Groups:
Des Moines
Valley View Village Conference
2571 Guthrie Avenue
Third Sunday at 1:30 p.m.
Mark Hillenbrand
(515) 208-3511

Omaha, Nebraska
Perkins Restaurant
108 L. Street
Second Monday at 6 p.m.
Cathy McNeil
(402) 537-0739

Iowa City
University of Iowa Hospitals and Clinics
Della Ruppert Conference Room
Fourth Sunday at 1 p.m.
Anne Leserman
(319) 353-4307

University of Iowa HD Clinic Fast Facts

What: Clinic where individuals with HD can get specialized care from providers with HD experience and expertise

Where: UI Hospitals and Clinics

When: Two Tuesdays of every month

Contact: Anne Leserman at (319) 353-4307
Huntington ACR16 Treatment (HART)
University of Iowa

HART is a multi-center, randomized, and double-blind study comparing three doses of ACR16 versus placebo for the symptomatic treatment of HD. The HART study at the University of Iowa is directed by the Principal Investigator, Jane Paulsen, Ph.D. This drug study will investigate the clinical effects of ACR16 on voluntary motor function in participants with HD, and its effects on brain function, behavior, and symptoms of depression and anxiety. The sister study, European MermaiHD (Multinational European Multi-centre ACR16 study In HD) is in Phase III trials.

What is HART trying to learn?
- The effects of ACR16 compared to placebo on voluntary motor function in individuals with HD.
- The outcomes of ACR16 versus placebo on brain function, behavior and symptoms of depression and anxiety.
- The levels of safety and tolerability and the most effective dose of ACR16.

Who can participate in HART?
- Participants must be age 30 or older.
- Participants must have early or mild HD symptoms.
- Participants must have a gene positive HD test (or if untested, an HD diagnosis).
- Participants must have an available caregiver or family member to accompany them to both the Baseline Visit and Visit 04.
- Participants cannot be currently taking any antipsychotic medication or tricyclic antidepressants.
- Participants must be healthy enough to engage in all protocol activities.

What study procedures will occur?
- A screening visit will determine whether this trial is right for you.
- The trial involves seven outpatient visits and one telephone contact over a period of about 16 weeks.
- Research visits include physical exams, neurological exams, thinking tests and surveys. There are blood draws and electrocardiograms to evaluate health and safety.

What else do I need to know about participating?
- Compensation for participation is not provided. You will be reasonably reimbursed for your travel expenses and other out of pocket expenses incurred as a result of participating in this study up to $75.00 per face-to-face visit.
- Study visits can be set up around your schedule (e.g. specific dates, weekends).
- Optional studies on other HD-related topics (e.g. functional brain imaging, eye tracking, and genetic discrimination) may be completed during a HART visit.

Who is paying for HART?
- The University of Iowa is receiving payment for conducting this research study from an arrangement between the University of Rochester, NY and NeuroSearch Sweden AB

How can I find out more information about HART?
Contact: Nancy Hale, B.S., R.N. Nancy-hale@uiowa.edu 319-353-4537
William H. Adams, B.A. William-H-Adams@uiowa.edu 319-353-4411
HSG (Huntington Study Group): http://www.huntington-study-group.org/
Funeral Preplanning

More people are planning their funeral in advance to relieve their families of the financial burden and much of the decision making. Last year, more than one million Americans planned their own (or a relative’s) funeral. The benefits of preplanning are:

> Saves your family from having to make decisions at an emotional time.
> Allows you to clarify your wishes and make them known.
> Gives you a chance to personalize your funeral to reflect your life and values. This may make it more meaningful for family and friends.
> Making arrangements in advance allows time to review your plan with family and take their thoughts and feelings into consideration.
> Eliminates concerns about emotional overspending. Preplanning gives you control of planning an affordable funeral.
> Prefunding the funeral relieves family of financial burden of a sudden expense. It preserves your life insurance and estate for your survivors.
> Saves money. Prepaying at today’s lower costs guarantees future costs are covered.
> Protects funds from potential depletion by nursing home expenses.
> Provides flexibility to make changes in the prearrangements as needed.
> Provides the security of knowing that everything is taken care of and your wishes will be carried out.

Printed with permission of Ken Holmes, Certified Preplanning Consultant, Lensing Funeral Services, Iowa City, IA