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Do-or-die time for Huntington's clinical trials

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At Risk for Huntington's Disease

HD is a genetically caused brain disorder that causes uncontrollable bodily movements and robs people's ability to walk, talk, eat, and think. The final result is a slow, ugly death. Children of parents with HD have a 50-50 chance of inheriting the disease. There is no cure or treatment.

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Huntington's Disease Society of America
International Huntington Association

WEDNESDAY, AUGUST 24, 2011

Do-or-die time for Huntington's clinical trials

It's do-or-die time for the Huntington's disease community.

We must meet the challenge of participating in clinical trials – or run the risk of those trials failing. That means thousands of patients will need to sign up with the help of their physicians, caregivers, research centers, universities, and outreach programs such as the Centers of Excellence of the Huntington's Disease Society of America (HDSA).

Never before has the research towards treatments and a cure shown so much promise. As I have pointed out in previous articles, very soon scientists and drug companies will start testing those potential treatments for safety and efficacy (click here to read more).

The stakes are enormous.

Successful trials would produce the long-awaited treatments for the cruel condition that afflicts tens of thousands of families around the world and threatens the millions who are at risk or, like me, carry the abnormal, disease-causing gene. Other disease groups could benefit, too, such as Alzheimer's and Parkinson's patients.

Failure could relegate yet another generation to life in a wheelchair and the destruction of their brains.

Renewed urgency

My mother died of HD in 2006. Her symptoms began in her late 40s. At 51, I could experience onset very soon.

On July 30, I renewed my fight against HD with a speech in Seattle urging at-risk, gene-positive, and HD-affected individuals to participate in observational studies and/or clinical trials. I spoke at the 2011 Inaugural Clinical Research Symposium, sponsored by the Northwest Chapter of HDSA and Lundbeck.

Organized by Dr. LaVonne Goodman, the founder of Huntington's Disease Drug Works, and other members of the local chapter, this at times technical but mainly uplifting event featured me and four other main speakers presenting various angles of the potential clinical trials and providing some of the latest news about HD research.

Huntington's Disease Drug Works
Huntington's Disease Lighthouse
Hereditary Disease Foundation
Huntington's Disease Advocacy Center
Thomas Cellini Huntington's Foundation
HDSA Orange County (CA) Affiliate
HD Free with PGD!
Stanford HOPES
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HD Blogs and Individuals

Chris Furbee: Huntingtons Dance
Angela F.: Surviving Huntington's?
Heather's Huntington's Disease Page

Dr. Goodman (left), Gene Veritas, and HDSA NW Chapter President Linda Ingle

A matter of numbers

People need to confront HD now, I emphasized in my presentation, titled "What HD Families Should Know about Clinical Trials: Initial Thoughts from a Gene-Positive Activist."

"We need to encourage all of our families to become involved in this process so that when it's time to sign up for the trials, we have enough people," I told the approximately 80 people in the auditorium of the Evergreen Hospital Medical Center.

"Why is this so important? If you look at Alzheimer's disease, where there are millions of people with the disease, or you look at Parkinson's, where there are a million people, or heart disease, where there are millions of people, it's not all that hard to get people to sign up for trials.

"But how many HD people are there in the United States? Well, there's only about, tops, 30,000 symptomatic HD people in the United States. That's not a lot of people when you take into account the fact that sometimes a trial may need hundreds or maybe more than a thousand people. And you can't be in more than one trial at the same time.... Very quickly you're going to run out of the number of people you need to participate."

Old fears, new hope

I encouraged people to exit the "HD closet" and to combat the stigma and denial surrounding the disease.

"Many of us in the Huntington's disease community are hiding," I stated. "We're afraid of getting tested. We're afraid of telling our insurance plans that we have or are at risk for Huntington's disease. We don't want to tell our employers....

"Now, with the new hope of clinical trials, it's time, I believe, for us to exit this closet."

I stressed the importance of reevaluating our community's fear of genetic testing for HD. I explained that, from my own test in 1999, I know the experience can be frightening. Many people – such as my at-risk sister, the mother of three grown boys conceived long before we knew about HD – decline to test because of the lack of treatments.

"Why should I find out this horrible news about myself and then know that there's no treatment, that there's no cure?" I said, putting myself in untested people's shoes. "But the fact of the matter is that there is hope today, and I believe, from my own personal standpoint, that we now have a very big reason for getting tested and finding out our status and getting involved in the community. And that reason is the hope of the clinical trials and these potential drugs that are coming out of the pipeline."

Stressing the positive

I told the audience I knew I was "preaching to the choir," which, unfortunately, is but a small minority of the HD community. How, I asked, do we educate people about the new hope of HD research and the upcoming clinical trials?

"It's time to start to deemphasize the phrases, 'HD is incurable. HD is untreatable,'" I proposed. "As a scientific and medical fact, yes, those are accurate statements. But I strongly believe ... that in the next few years we're going to see some treatments that are going to attack the causes of Huntington's disease. I believe we're going to see in our lifetime Huntington's disease managed as a disease just as diabetes is managed and cholesterol is managed and other problems in the human body are managed with medication. I think we're going to find all kinds of revolutionary ways for doing that."

You can watch my entire presentation in the video below.

Gene Veritas: What HD Familes Should Know about Clinical Trials

from Gene Veritas

49:51

Gene Veritas: What HD Familes Should Know about Clinical Trials from Gene Veritas on Vimeo.

The basics of clinical trials

Later in the day Dr. Goodman, physician to several dozen HD patients, provided essential details about the workings of clinical trials in a talk titled "The A, B, C's of Clinical Research: HDSA's Initiative."

"I belong to an HD family, having lost my husband to Huntington's disease, and I have two biological children with my first husband who are at risk," Dr. Goodman began. "So I am in the same boat as many of you."

The chair of family services and education for the Northwest Chapter, Dr. Goodman noted that HDSA has placed an increased emphasis on involving people in clinical trials.

She stressed that all members of the HD community can and should join the effort.

Participating in a trial is "empowering" and "fulfilling" and allows people to take "control over this disease, as much as you can," she said. "If you help someone be in a clinical trial, but couldn't have been in one, then you have been very integral to the success of that clinical trial," she said.

Success will come only with hard work and perseverance, she added. "Most trials don't have positive outcomes. We have to be prepared for that too."

Critical studies

Dr. Goodman gave an overview of current and recent research studies, which lay the basis for clinical trials:

PREDICT-HD, examining asymptomatic, gene-positive individuals to understand the development of initial symptoms;

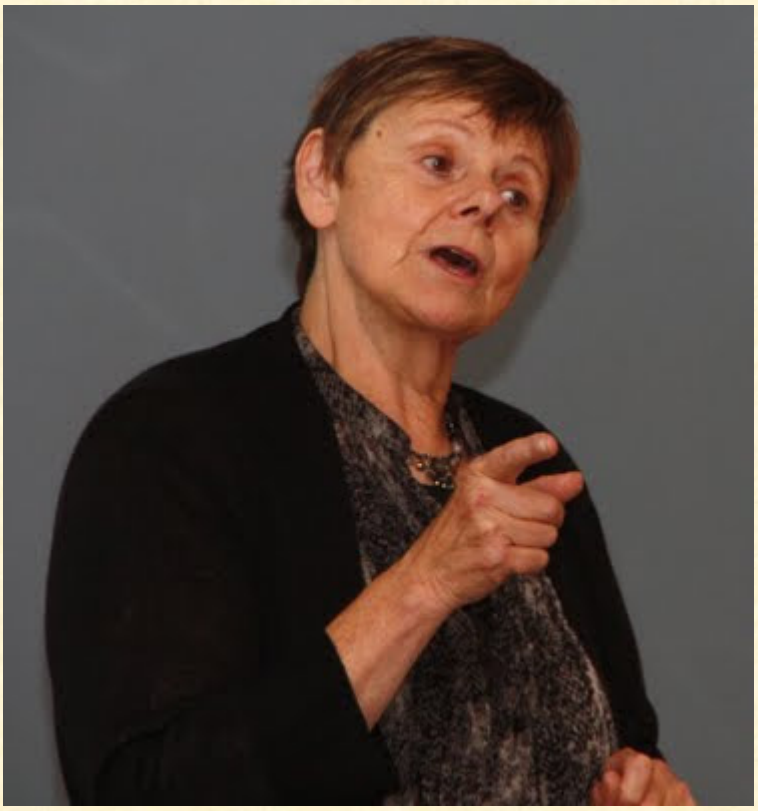
TRACK-HD, following the early to middle stages of the disease;

COHORT, examining patients and at-risk adolescents over the long run;

Enroll-HD, replacing COHORT, aiming for a worldwide database of 10,000 people, including patients, at-risk, and gene-positive individuals, and including a care component focusing on available drugs, stigma, qualify of life factors, and the design of a standard of care for patients to assist the many physicians unfamiliar with HD;

FuRST-pHD, developing a scale of measurement to assess clinical trials in patients with early symptoms;

and CAB-Beta, seeking to improve cognitive testing to be used in clinical trials.



Dr. Goodman (photo by Gene Veritas)

Boosting planning and recruitment

Dr. Goodman pointed out that the research studies aim to speed up the

planning and administration of the clinical trials, as well as the crucial recruitment of participants, and reduce the duration of the trials.

"Except for the first CoQ 10 trial and the Xenazine trial by Lundbeck, every single HD trial we've had has been delayed because of slow recruitment – every single one," she stated emphatically, referring to Coenzyme Q10, an anti-oxidant, and to the commercial name for tetrabenazine, the very first drug approved for HD in the U.S, to help reduce the shaking movements typical of the disease. "They've had some going on for years looking for 100 people.... We don't have that much time."

She added: "One of the big costs is when the clinical trial is dragged out by slow recruitment. So not only are little companies discouraged by this, but big companies, too. Now if we show drug companies that we can come to clinical trials quickly, they're going to be much, much more interested in us. We want all of those drug companies to come on. The more, the better."

Dr. Goodman cited the example of a clinical trial in the U.S. for ACR-16, another drug in development for treating the shaking movements, known as chorea: it took the trial administrators two years to recruit 250 people.

The sooner and faster trials can be implemented, the earlier the benefits of treatments can reach patients.

Heroes and roses

For an individual, taking the first step towards a research study or clinical trial can be hard, Dr. Goodman said. But that step is important.

"We learn from each clinical trial, even if it's negative," she said. "To win a war, we're going to have to take our time, energy, sweat, and tears."

And, she added, "there will be some risk," which researchers and the federal Food and Drug Administration (FDA) are striving to minimize with the required pre-testing in animals. "But the first person is still the first person," she said.

Yes, that first person will face risks, she allowed, "but there will also be heroes."

Dr. Goodman then asked all past trial participants in the audience to stand, the cue for volunteers to distribute roses to each one in recognition of their contribution to HD research.

Mike Hyer, a participant in the Predict-HD study (photo by Gene Veritas)

"These are the people who are going to help bring treatments," Dr. Goodman said.

She also recognized the people unable to participate in trials but who are also "heroes for living with HD."

You can watch Dr. Goodman's presentation in the video below.

The ABCs of HD Clinical Trials: A Talk by Dr. LaVonne Goodman

from **Gene Veritas**

The ABCs of HD Clinical Trials: A Talk by Dr. LaVonne Goodman from Gene Veritas on Vimeo.

Biology, leadership, and stem cells

The symposium's three other featured speakers were Dr. Keith Elliston, the Vice President and Senior Research Leader of Systems Biology at CHDI Management, Inc., the so-called "cure Huntington's disease initiative" backed by a wealthy, anonymous donor; Judy Roberson, the president of the Northern California Chapter of HDSA and the Joseph P. Roberson Foundation; and Dr. Jan Nolta, Professor in the Department of Cell Biology and Human Anatomy and Director of the Stem-Cell Program at the University of California, Davis.

Dr. Elliston's presentation focused on new approaches to seeking treatments for HD through the techniques of systems biology, "a way of studying biological systems not as individual sets of components, like a collection of parts lying on a highway, but as an integrated working system, like a working vehicle."

You can watch his fascinating presentation in the video below.

Systems Biology: A New Approach to Developing Therapies for Huntington's

from **Gene Veritas**

45:33

Systems Biology: A New Approach to Developing Therapies for Huntington's from Gene Veritas on Vimeo.

Roberson told the moving story of her late husband's fight against HD and her courageous campaign to raise funds and awareness for HD research and the care of patients at the HDSA Center of Excellence at UC Davis. Roberson was recently named as a representative of the HD community in the FDA's planning process for HD clinical trials.

You can watch her inspiring presentation on "making a difference for people with HD" in the video below.

Get Involved: Making a Difference for People with Huntington's Disease

from **Gene Veritas**

31:08

Get Involved: Making a Difference for People with Huntington's Disease from Gene Veritas on Vimeo.

Dr. Nolta spoke on her efforts to use a well-known type of stem cells, called "mesenchymal stem cells" (MSC), to develop two potential treatments for HD. Dr. Nolta referred to the MSC as "paramedics" because of the way they congregate around and repair damaged cells. Injected into the brain of an HD patient, the MSC might be able to repair damaged neurons (brain cells) and restore the vital connections between them.

You can watch Dr. Nolta's presentation, which includes the latest developments in her research, in the video below.

Towards Stem-Cell Treatments for Huntington's Disease: Talk by Dr. Jan Nolta

from **Gene Veritas**

50:24

Towards Stem-Cell Treatments for Huntington's Disease: Talk by Dr. Jan Nolta from Gene Veritas on Vimeo.

Posted by Gene Veritas at 12:25 PM

Labels: at risk , caregiver , chorea , clinical trial , cure , Enroll-HD , gene-positive , genetic testing , Huntington's , mother , research , scientist , stem cell , stigma , symptoms , treatment , wheelchair

3 comments:

Anonymous said...

My father-in-law was recently diagnosed, and we will be going to Baylor in Houston to start visits. We would like to participate in a trial, but there are so many.... What would be the appropriate approach to choosing one?

4:55 PM, August 25, 2011

Sean Thompson, PREDICT-HD Public Relations Coordinator said...

Thanks for making such an excellent case for participating in clinical and observational HD trials, and thanks in particular for mentioning our study.

Speaking for observational trials like PREDICT-HD, even

though we aren't introducing an experimental treatment as would happen in a clinical trial, we are seeking to contribute to finding effective treatments and hopefully a cure for HD. In PREDICT-HD, we're attempting to pinpoint the earliest signs of HD so we can look to treat those signs before they even begin to show up as clinical symptoms and start affecting peoples' lives in a negative way.

We're also seeking to find the best measures for the early signs of HD. The strongest measures we're able to identify in observational research will be used to see if a treatment in a future clinical trial is effective. The stronger the measure, the fewer people we'll need to complete a clinical trial and the faster we can reach a conclusion on whether or not a treatment is effective.

As always, thanks for your advocacy Gene, and a big thanks to every single person in the HD community who has taken part in a research study. You're making a real difference toward finding treatments and a cure.

3:36 PM, August 30, 2011

penny greene (gussler) said...

My grandmother,my moter passed away at 44 & my sister who is 37 is in mountian manor of paintsville ky, has the last stages of HD.I am 43 & have 4 children. I have not been tested.I have taken the CQ 10.Hope to see a cure.I live in Lawrence county kentucky

6:18 PM, September 29, 2011

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