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‘Navigating’ the Huntington’s disease community towards crucial 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.

Blog Archive

- ► 2021 (12)
- ► 2020 (16)
- ▼ 2019 (19)
  - ▼ November (2)
    - An 'electric,' inspiring Thanksgiving for the Hunt...
    - 'Navigating' the Huntington's disease community to...
  - ► October (2)
  - ► August (2)
  - ► July (1)
  - ► June (2)
  - ► May (2)
  - ► April (1)
  - ► March (3)
  - ► February (2)
  - ► January (2)
- ► 2018 (16)
- ► 2017 (14)
- ► 2016 (13)
- ► 2015 (24)
- ► 2014 (24)
- ► 2013 (30)
- ► 2012 (26)
- ► 2011 (33)
- ► 2010 (26)
- ► 2009 (21)
- ► 2008 (7)
- ► 2007 (7)
- ► 2006 (4)
- ► 2005 (17)

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SUNDAY, NOVEMBER 17, 2019

'Navigating' the Huntington's disease community towards crucial clinical trials

As scientists and drug companies expand the array of potential treatments for Huntington's disease, the Huntington Study Group (HSG), the world's largest HD clinical research network, is redoubling its efforts to educate the HD community for current and upcoming clinical trials and train the necessary medical personnel.

A record 700-plus participants focused on these themes at the 26th annual HSG Meeting, titled "HSG 2019: Navigating HD," November 7-9 at the Hyatt Regency hotel in Sacramento, CA. (Attendance at the HSG 2017 and 2018 meetings was over 600.)

Clinical trials are crucial for demonstrating drug safety and efficacy. The number of HD trials has increased in recent years, bringing hope for better treatment of the devastating symptoms and perhaps even an attack on the root causes. Key trials in progress include GENERATION HD1, run by Roche, and SIGNAL, administered by the HSG and Vaccinex.

"Figuring out how these trials are going to work, what they're aiming to do, and what an individual patient or family should do to get involved or not get involved has become complicated, to some extent," Andrew Feigin, M.D., the HSG chair and a professor of neurology at New York University Langone Health, told me in a November 6 interview. "That's my interpretation of the 'navigating HD.' We're trying to get at some of these novel therapies and clarify where they're headed, where they stand, how the HSG can get more involved, and figuring out where people can go for the cutting-edge therapies for Huntington's disease."

In the conference-opening "HSG State of the Union" presentation by HSG leaders and staff, executive director Shari Kinel, J.D., reported that the event involved 15 countries, 23 companies, 9 advocacy groups, 17 sponsors, and 15 exhibitors. The sponsors included Roche's American subsidiary Genentech and Vaccinex.

"This incredible showing […] is a sign that the HSG has more partners, more colleagues, more friends than ever who are engaged, dedicated, and committed to seeking treatments that make a difference for those impacted by Huntington's disease," Kinel told the audience.

Dr. Feigin affirmed that in the past year, the HSG has doubled its paid staff from four to eight, plus one part-timer, although he declined to reveal the organization's annual budget. Headquartered in Rochester, NY, the HSG is mainly funded by firms like Vaccinex that it partners with on clinical trials, he explained. Sponsors cover the cost of the annual meeting.

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The audience watches a presentation by Dr. Arthur Combs at the "HD Innovators Forum" at the 26th annual HSG Meeting (photo by Gene Veritas, aka Kenneth P. Serbin)

A full-service organization

The HSG was founded in 1993, the year of the discovery of the huntingtin gene. Dr. Feigin described the nonprofit organization as a "full-service" contract research organization that can carry out all aspects of an HD clinical trial.

In her speech, Kinel stated that the HSG member network includes 801 investigators (researchers), trial coordinators, scientists, and HD experts. Around the globe, the organization has credentialed 127 sites for HD trials, and HSG members have worked with more than 21,000 HD-affected individuals, she said.

The HSG also developed the Unified Huntington's Disease Rating Scale (UHDRS), the primary assessment tool in HD clinical trials. It consists of tests of a person's movements, cognition, behavior, independence, and functional capacity.

The "HSG State of the Union" presentation outlined the HSG's mission, accomplishments, clinical trials, educational activities, efforts to improve patient care, and plans for the future.

You can watch the presentation in the video below. Click here for my video album of the event, which included a variety of presentations on patient care, clinical trial techniques and measurements, new scientific findings, and innovations in drug and clinical trial development.

2019 Huntington Study Group 'State of the Union'

from **Gene Veritas**

1:09:29

Seeking a better drug to treat chorea

Prior to the main conference, the HSG held organizational meetings for KINECT-HD, a Phase 3 clinical trial by the HSG and San Diego-based drug developer Neurocrine Biosciences to test the efficacy of valbenazine to treat chorea, the involuntary movements typical in HD.

The HSG ran the successful clinical trials of two other drugs for chorea, Xenazine and Austedo, the only HD-specific medicines to receive approval from the U.S. Food and Drug Administration (FDA). On November 14, it issued a press release announcing the start of the 18-week trial, which seeks to enroll HD-affected individuals with chorea at 55 sites in the U.S. and Canada.

In 2017, valbenazine was approved by the FDA with the name Ingrezza for the treatment of tardive dyskinesia, an irreversible involuntary movement disorder. This status allowed Neurocrine and the HSG to take it directly into a Phase 3 trial for HD.

Like Xenazine and Austedo, valbenazine is a VMAT2 inhibitor. Xenazine requires three daily doses, and Austedo two.

"The upside thing of valbenazine is that it's a drug that can be dosed once daily," said Dietrich Haubenberger, M.D., the Neurocrine medical director, in a presentation forming part of the "HD Research Round-Up" at the close of the scientific sessions on November 8.

Wearable sensors and the search for biomarkers

In the quest for HD treatments, researchers hunt for new biomarkers, that is, signs of the disease and the effect of remedies. Biomarkers are especially critical in brain-related diseases, because doctors cannot do biopsies on the organ.

With a key innovation, KINECT-HD will also look for biomarkers. It will be the HSG's first trial in which participants use wearable sensors – for continuous monitoring of their movements and other biological functions, even at home. Researchers hope this more detailed monitoring will provide both a better understanding of chorea and valbenazine's impact on it.

Called BioStamp nPoint, the sensors were designed by MC10, Inc., and cleared for use by the FDA. MC10 is based in Lexington, MA.

MC10 chief medical officer Arthur Combs, M.D., described the system at the conference's "HD Innovators Forum."

"It weighs less than eight grams [0.28 oz.]," Dr. Combs said, explaining that the sensor can be placed anywhere on the body and worn even during showers and swimming. "It's like putting on a Band-Aid."

MC10 developed 44 algorithms for the system to help measure trial participants' data. In addition to chorea, BioStamp nPoint will help investigators observe individuals' gait, heart rate, sleep, posture, and other bodily functions, Dr. Combs added.

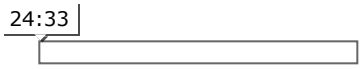
In one previous study, "patients with symptomatic Huntington's disease spent 50 percent of their day" lying down, he explained. That may be a response to exhaustion or the risk of falling, he said. Thus, the BioStamp nPoint system could help determine whether lying down is a "marker" for the disease, and whether less time at rest is a sign of drug efficacy, he said. It also accounts for the uniqueness of each patients, he added.

To obtain continuous data in GENERATION HD1, Roche developed an HD Digital Monitoring Platform, with participants wearing a smartwatch and using a smartphone.

You can watch Dr. Combs' presentation in the video below.

BioStamp nPoint: End-to-End System for Remote Physiological Monitoring

from **Gene Veritas**



The latest clinical trial news

In addition to Neurocrine, other firms reported on their clinical trials during the "HD Research Round-Up": Voyager Therapeutics, uniQure, Wave Life Sciences, Vaccinex, and Roche.

The Roche GENERATION HD1 update of the company's historic Phase 3 clinical trial of the drug RG6042 was one of the most anticipated. A gene-silencing drug, RG6042 is aimed at the roots of HD and caused a stunning improvement in the health of HD-affected mice. On October 14, Roche announced that it was expanding the number of trial participants from 660 to 801 and adding China to the nearly 20 countries in the study.

The announcement noted that recruitment in the U.S. had "exceeded expectations" and was now complete. Expanding the number of volunteers and adding China will allow for more abundant data and the study of a more diverse population, Roche said.

Enrollment for the Roche HD program has been "absolutely electric," with over 800 individuals already in 2019 in GENERATION HD1 and related HD studies, said Scott Schobel, M.D., M.S., Roche's associate group medical director and clinical science leader for RG6042 (click here to watch Dr. Schobel's presentation). If the trial is successful, Roche will apply for drug approval from the FDA and regulatory agencies in other countries.

On November 9, HSG held a "Family Day" for the HD community, with presentations by advocates like me, presentations by scientists, and an update on GENERATION HD1.

In upcoming articles, I will report on Family Day and more of the scientific and clinical developments discussed at the meeting.

Disclosure: my travel expenses were covered by the HSG and the Department of History of the University of San Diego.

Posted by Gene Veritas at 10:08 PM

Labels: Andrew Feigin , chorea , clinical trials , Genentech , HSG , huntingtin , Huntington Study Group , Huntington's disease , KINECT-HD , Neurocrine Biosciences , researchers , Roche , Shari Kinel , symptoms , treatments , Vaccinex

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