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HDSA's renewed commitment to critical research

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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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WEDNESDAY, NOVEMBER 28, 2012

HDSA's renewed commitment to critical research

After a budgetary crisis that practically eliminated support to HD scientists in recent years, the Huntington's Disease Society of America (HDSA) has committed itself to reestablishing a program of research projects critical for developing effective treatments.

In a September 28 e-mail message to "HDSA friends," HDSA CEO Louise Vetter announced the hiring of George Yohrling, Ph.D., to fill the new position of Director of Medical & Scientific Affairs.

"In the past we have relied upon the volunteer support and consultation of physicians and scientists," Vetter explained in an interview in San Diego on November 16. "We will continue to do so. But based on what the pace of HD research is right now and our vision to have a new research program, we felt it was the right time to bring that expertise in-house."

Dr. Yohrling, a molecular neuroscientist, has worked in the HD field since 2000.

"His first job was with an HDSA Coalition for the Cure lab," Vetter said. "He was funded with an HDSA grant, and he has continued to stay in the field.... He has real expertise in HD, which is incredibly important. His primary task is to launch a new research program for the organization."

The hire marks the first step in fulfilling a series of goals outlined in HDSA's first-ever [strategic plan](#), formulated after broad consultation with the HD community in 2011 and 2012, and launched by Vetter at the annual HDSA convention last June ([click here](#) to read a report on the drafting of the plan).

In response to recent criticisms of HDSA because of the drastic decline in research support ([click here](#) to read more), Vetter stated that HDSA is working "to have the most impact for the most people and help set the course for the best care and the best treatments as soon as possible."

(Watch the entirety of my interview with Vetter in the video below.)

HD Links

[Huntington's Disease Society of America](#)
[International Huntington Association](#)
[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](#)
[Earth Source CoQ10, Inc.](#)

HD Blogs and Individuals

[Chris Furbee: Huntingtons Dance](#)
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CEO Louise Vetter on HDSA's new directions

from [Gene Veritas](#)

12:24 |

Avoiding repetition

In crafting the new research program, HDSA planners took into account the organization's limited budget (about \$8.5 million annually) and strived to avoid duplicating efforts by other HD initiatives focused strictly on research, Vetter added.

Those initiatives include the [CHDI Foundation, Inc.](#), which spends tens of millions of dollars annually, and the [Hereditary Disease Foundation](#) (HDF). Unlike HDSA, they do not offer support groups, care centers, or other forms of patient outreach.

Both Vetter and Dr. Yohrling emphasized that the new research program will focus on HD research in humans and human cells. This contrasts sharply with – but also necessarily complements – the research conducted on animals.

Focusing on humans

“We’re calling it ‘Human HD Biology Project,’” Dr. Yohrling said in a November 26 interview. “All of the observations will be from human HD patients, and not from a worm or mouse or fly. There’ll be no arguing about the physiological relevance of the data we will acquire.”

Dr. Yohrling explained that the project will function like a post-doctoral or clinical fellowship program. Young scientists will partner with the directors of HDSA's 21 Centers of Excellence for Family Services and Research, which see patients and other HD family members on a regular basis. It replaces the Coalition for the Cure grant program, which ceased to exist with the onset of the budget crisis.



Dr. George Yohrling (CHDI photo)

The new researchers will focus on tasks such as the search for human biomarkers – specific signs of the disease within bodily fluids or tissues or the brain, for example – that will become crucial for measuring the effects of potential remedies.

“These are studies, not clinical trials, but their outcome could and should help support, drive, and steer clinical trials,” Dr. Yohrling explained, adding that he is currently recruiting HD specialists to serve on the program’s advisory committee, which will review the applications from researchers.

Grants will be relatively modest, Dr. Yohrling indicated. “These won’t be \$250,000 jobs,” he said. However, because of the clear emphasis on non-redundancy, “hopefully we’ll get a big bang for our buck,” he added.

HDSA has already raised funds to kick off the project, Dr. Yohrling said, although both he and CEO Vetter recognized the need to increase fundraising to expand support for research.

HDSA hopes to issue its first call for proposals in early 2013, Dr. Yohrling said.

HDSA also will continue to support an important consortium of HD stem cell researchers, he noted.

A pivotal player

Dr. Yohrling’s diverse experience in HD research makes him a pivotal player in the search for treatments.

After receiving his Ph.D. in pharmacology from Wake Forest University in 2000, he became the very first post-doctoral researcher in the lab of Jang-Ho Cha, M.D., Ph.D., at Harvard Medical School/Massachusetts General Hospital. (Dr. Cha is a member of the HDSA Board of Trustees and will chair the HD Human Biology Project Advisory committee.) During his two years under Dr. Cha, he “got to see and interact with HD firsthand.”

“Any human being with a soul in their body” would be motivated to help, Dr. Yohrling recalls of his initial, serendipitous contact with Dr. Cha and the HD team while in Boston searching for a post-doctoral position. “I was hooked. I felt it was my calling.”

Under Dr. Cha, Dr. Yohrling also received an HDF grant.

Dr. Yohrling spent the next five years conducting Alzheimer’s disease research at the pharmaceutical giant Johnson & Johnson, followed by two years at Galleon Pharmaceuticals, Inc., researching respiratory conditions.

In 2009 Dr. Yohrling joined CHDI, which he described as a “dream” of an opportunity to focus fully on HD research in a large, resource-rich organization. There he served as director of target assessment and then director of systems biology-pathway assessment. Working with firms and leading HD scientists, he managed millions of dollars in research contracts. He also led the development of [HD Research Crossroads](#), an online repository of HD-relevant drug target validation data.

Impacting patients with ‘all hands on deck’

CHDI was “more a behind-the-scenes operation,” Dr. Yohrling observed. “HDSA is more of a grassroots, family-oriented foundation. The opportunity to get back to that, while also getting involved in human biology research, was an opportunity too good to ignore.”







Responding to the argument that HDSA should let others concentrate on research while putting funds strictly into social services, Dr. Yohrling observed that the HDSA board and HD families want the organization “back involved in research and not to leave it up to the other entities like CHDI, HDF, and the government,” although his key responsibilities will include maintaining “an open line of communications” with those and other HD research organizations.

“We’re really glad to have someone like George working at HDSA,” said Robi Blumenstein, the president of CHDI Management, Inc., which carries out the day-to-day tasks of CHDI’s mission. “It just furthers our ability to collaborate.”

“I think that HDSA is well-positioned with their centers of excellence around the country,” Dr. Yohrling continued. “This is

an incredible resource. It's a huge benefit that HDSA has over other foundations or organizations, this access and close connection to the patients. A research program, although it might be limited initially, if the money is use properly and thoughtfully, can have a huge impact on the lives of patients."

"We need all hands on deck," said Vetter, adding that everybody in the HD community can take small but important steps to help the cause by keeping in touch with attending physicians, learning about HD research, and participating in clinical trials and research studies. "As we look at the dawn of a new time of HD therapeutic development, the only way those drugs are going to come to market is if people get involved. Getting involved is *the* most important thing that the HD community can do right now."

Posted by [Gene Veritas](#) at [12:54 PM](#)      

Labels: [Alzheimer's](#) , [biology](#) , [biomarker](#) , [care](#) , [Center of Excellence](#) , [CHDI](#) , [clinical trial](#) , [fundraising](#) , [Hereditary Disease Foundation](#) , [Huntington's](#) , [Louise Vetter](#) , [neuroscientist](#) , [stem cell](#) , [systems biology](#) , [treatments](#)

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