News from the Hereditary Disease Foundation

So Much to Celebrate!
Thanks to our wonderful donors, friends, family and extraordinary HDF-funded scientists for joining us at the Celebrating Discoveries in Neurosciences Symposium and Gala and making it a night to remember! It was an exciting evening filled with hope and enthusiasm as we celebrated scientific progress and looked toward a future without Huntington’s disease. To top it all, we raised over $1 million to keep the momentum going!

Anna Canoni, Deborah Fine, Nancy Wexler, Anne Young, Alice Wexler, and Nora Guthrie
Lesley Stahl, 60 Minutes, CBS News correspondent, who was our host in 2016 and again this year, spoke of the progress and the partnership between donors and scientists that makes it all happen.

She said, “When I was here three years ago, some of the discoveries we are celebrating tonight were concepts in the laboratory. Now they are realities that are likely to change the lives of patients and families. Thanks to your generous support and the work of the brilliant scientists in this room, there are clinical trials underway that are working to silence, modify and edit the gene that causes Huntington’s disease.”
There was a standing ovation as Lesley led a toast to HDF President Nancy Wexler, congratulating her on receiving the prestigious Double Helix Medal from Cold Spring Harbor Laboratory and the Academy Medal for Distinguished Contributions in Biomedical Science from the New York Academy of Medicine.

During the Symposium that preceded dinner, a standing-room crowd listened intently as the noted geneticist and neuroscientist Dr. Cori Bargmann, Head of Science at the Chan Zuckerberg Initiative and former member of the HDF’s Scientific Advisory Board, spoke about the power of science to move medicine forward. She highlighted the importance of scientists involving patients, not just as research subjects but as participants and drivers of research, noting that HDF was one of the pioneers of this approach.

Scott Zeitlin, the distinguished HD researcher and recipient of the 2019 Leslie Gehry Brenner Prize for Innovation in Science, discussed his work to understand the structure and function of the Huntington’s disease protein and develop new
therapeutic strategies. Scott’s laboratory at the University of Virginia School of Medicine has developed mouse models with normal and abnormal HD genes that can be regulated and are being used to understand the mechanism of HD pathogenesis. Scott’s mice are now the most widely used model by scientists worldwide to discover drugs that could be used to treat HD.

In accepting his award, he thanked his colleagues in the research community and families with HD who are the motivation for finding treatments and cures. Scott is eager to use his $100,000 prize to further accelerate the research in his lab.

Capping the evening, Charles Sabine, Emmy award-winning journalist and HD advocate, poignantly recounted his experiences as a member of an HD family and his work to eliminate HD’s stigma. Charles shared his recent conversation at an HD youth summer camp where a teenage girl asked him: “Tell me something you know for certain?”
“No generation, yours included, will ever need to fear this disease as much as mine did,” he optimistically replied.

Those words from Charles say it all!

**Exchanging Ideas…Advancing Research**
Collaboration and exchange of ideas is key to developing effective treatments and cures for HD. The Milton Wexler Workshops, a centerpiece of HDF, are a unique opportunity for scientists from different disciplines to brainstorm.

In conjunction with our Celebrating Discoveries in Neuroscience Symposium and Dinner, we held a Workshop to discuss mechanisms and therapies for HD. Participants were a diverse mix of 40 scientists - members of the HDF’s Scientific Advisory Board, currently funded researchers, some early in their careers and others as senior researchers, from a variety of scientific and geographical backgrounds.

Several hot topics were discussed including: informal updates on the most recent clinical trials, genetic modifiers of the age of symptom onset in HD, how DNA repairs itself in HD, how to get drugs into the brain past the blood brain barrier, biomarkers to track disease progression, and finally the use of systems biology and functional genomics to better understand critical pathways in HD.

**Thank You!**
Speaking about the exciting HDF Fall events, Nancy Wexler said, “We are exhilarated and inspired by the science that is moving us toward a future without HD. During this special season to give thanks, we say a big thank you to our visionary supporters, our brilliant researchers, and the amazing HD families. The cure is within our grasp!”

**Innovating Research…Discovering Cures**

[Donate Today](#)

**Contact Us:**
[Web](#)
[Twitter](#)
[Facebook](#)

*Published November 2019*