



News from the Hereditary Disease Foundation

Celebrating Women's History Month



Nancy Wexler

Nancy Wexler, PhD, President, Hereditary Disease Foundation shares her thoughts. All my life I was inspired by my mother's intelligence, curiosity, warmth and love. When she was diagnosed with Huntington's disease when I was 23, her bravery inspired me. My father Milton Wexler began the Hereditary Disease Foundation in 1968 to find treatments and cures for my mom, his two daughters – my sister Alice and me, and families around the world stricken by this devastatingly catastrophic disease.

Since the creation of the HDF, we have been fortunate to have so many brilliant, innovative, collaborative and exceptional women who did and are doing paradigm-shattering research!

The Gene Hunters, a collaborative effort of over 100 international scientists, identified the DNA marker for HD in 1983 and the HD gene in 1993, which helped launch the Human Genome Project. Gillian Bates, Leslie Thompson and Anne Young were Gene Hunters. These women continue to play critical roles as researchers and on our Scientific Advisory Board; Anne leads as chair. They inspire a new generation of women in science as mentors.

Essential animal models were created by Gillian Bates, Jenny Morton and Marie-Francoise Chesselet, among others, and studied by everyone to create breakthroughs in our understanding of HD. Novel gene therapies are being tested in stem cells created by Ai Yamamoto. Ai, with Anne Simonsen, helped discover the gene Alfy, which extends life in someone with HD by up to 23 years. Anne's daughter Ellen Penney, following in her parents' footsteps as a movement disorder neurologist, recently received an HDF Milton Wexler Postdoctoral Fellowship.



Ai Yamamoto

We pay a special tribute to the amazing women of HDF. A few share their thoughts here.

What Motivated Your Career in Science?



Sarah Tabrizi

“I met patients with HD in 1996 when I was doing my PhD and was incredibly moved. My passion to find an effective treatment for Huntington’s disease drives everything I do. I still see patients and families every week and that reminds me of the devastating impact this disease has on families and children.” –Sarah Tabrizi, BSc, MB ChB, FRCP, PhD, FMedSci, University College of London.

Sarah is a member of the HDF Scientific Advisory Board and the clinical global principal investigator for the world’s first gene silencing trial in HD, sponsored by Ionis Pharmaceuticals. She received HDF’s Leslie Gehry Brenner Prize for Innovation in Science in 2017.

“An undergraduate lecture on Huntington’s disease, in which Nancy Wexler was mentioned for her obvious role in the discovery of the HD gene, is what motivated me. Having never heard of the disease before then, I was shocked and saddened seeing videos of patients in that lecture. I have always wanted to pursue research which will lead to viable treatments for this devastating disease, and I thank the HDF for allowing me to pursue this.” – Charlene Smith-Geater, PhD, University of California, Irvine.

Charlene is scientist in the lab of Leslie Thompson at the University of California, Irvine. She is using a technology that allows skin cells from HD patients and healthy individuals to be “reprogrammed.” These reprogrammed neurons are being used to expand our knowledge of the many different processes in HD.



Charlene Smith-Geater



Anne Simonsen

“I was always interested in medical research and was inspired by female faculty during my undergraduate studies to follow a career in science. During my postdoc, I had two kids and when I started to doubt that I would manage to combine kids and a career in science, I got a lot of support from my postdoc mentor. The most important, however, was my own realization that I had to do science.” –Anne Simonsen, PhD, University of Oslo, Norway.

Anne’s work was the first to identify Alfy, a very large protein with implications for HD. Her research could lead to therapies that will delay the age of onset of HD and push it out of the normal lifespan.

What Is Your Experience as a Woman in Science?

“I received excellent training at the University of California, Irvine laboratories of two female professors: Dr. Lee McAlister-Henn and Dr. Leslie M. Thompson. The mentorship and scientific training I received from them both was truly the most important of my scientific career. I have tried to pay that forward by mentoring up-and-coming young women scientists over the past 20 years.” –Joan Steffan, PhD, University of California, Irvine.

Joan is a member of the HDF Scientific Advisory Board. She discovered how the normal Huntington protein functions in a cellular process called autophagy, which enables cells to takeout and recycle their “garbage.” As the understanding of this process advances, new therapies can be developed.



Joan Steffan

“I have always been mentored particularly by women, initially by Professor Anita Harding who had a great influence on my work. I have clearly been influenced by great scientists, such as Nancy Wexler, and world-leading neurologists, such as Anne Young. My experience as a woman in science is that you have to be the best you can be, have a passion for what you do, and not try and use your gender to win arguments; it has to be a meritocracy.” –Sarah Tabrizi

How Has the Hereditary Disease Foundation Impacted Your Experience?



Gillian Bates

“Through the collaboration to clone the gene and at HDF Workshops, the HDF gave me the opportunity to form working relationships with some of the most talented scientists in the field. When I started by own lab, the grant support that I received from HDF was invaluable and was very important in allowing me to establish my independent research program.” –Gillian Bates, FMedSci, PhD, FRS, University College of London.

Gill was part of the team that cloned the gene for HD in 1993. She went on to develop the first mouse model of HD in 1996. Gill is a member of the HDF Scientific Advisory Board. She received the Leslie Gehry Prize in 2012.

“I think having the HDF be a mostly female organization is great, and Nancy Wexler definitely fosters a compassionate atmosphere. This helps promote a more collaborative network, which supports the younger researchers and also promotes sharing of ideas more freely to progress the work forward.” – Charlene Smith-Geater

[Hear](#) from more HD researchers about their experiences and the impact of the Hereditary Disease Foundation.

Huntington's disease, neuroscience, and research as a whole have benefited from the drive and intelligence of these outstanding women in science. We are indebted to their creativity, collaborative spirit, generosity and empathy.

On Television Soon...Don't Miss It!

PBS will air the Woody Guthrie All Star Tribute Concert on Thursday, March 21, 2019, on WNET/Thirteen in New York. This concert at the Hollywood Bowl was the very first fundraiser for what is now HDF. You'll see never before seen footage of this rare and historic event from 1970. [Check](#) your local listings for airtimes in your area.



Arlo Guthrie, Joan Baez, Country Joe McDonald

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