



News from the Hereditary Disease Foundation

Brilliant Science... Extraordinary Results

Gene Therapy Pioneer Beverly Davidson Leads the Way!

“It’s an exciting time be in science and see the marriage of different disciplines working together toward a common goal,” says Beverly L. Davidson, PhD, longtime member of the Hereditary Disease Foundation Scientific Advisory Board, 2015 recipient of the HDF’s Leslie Gehry Brenner Prize for Innovation in Science, and Arthur V. Meigs Chair in Pediatrics and Chief Scientific Strategy Officer at the Children’s Hospital of Philadelphia (CHOP) and Professor of Pathology and Laboratory Medicine at the University of Pennsylvania.



Dr. Beverly L. Davidson

From the laboratory bench to the patient's bedside, physicians, microbiologists, computer scientists and researchers from varied fields are focused on finding improved treatments and ultimately cures for Huntington's disease and other devastating brain disorders.

"Bev is a rock star. She's leading the way," says Nancy Wexler, President of HDF. "Her trailblazing research is making gene silencing for the treatment of HD a reality. She has shown that the havoc-wreaking activity of disease-causing genes in the brain can be silenced."



Nancy and Bev

"Cells have garbage in them. Just like your house, if that garbage is not removed, it will build up and cause a toxic, polluted and uninhabitable environment. Gene silencing addresses how to remove the garbage from cells – how to throw out toxic proteins that cause HD and other diseases," explains Nancy.

The Davidson lab at CHOP has been working for the past decade to develop safe and effective gene silencing strategies for the treatment of HD. Bev's large team of Research Investigators, postdoctoral fellows,

graduate students and technicians conducts imaginative research focused on HD and other disorders. The team was the first to show that gene silencing could improve disease symptoms in mouse models. In collaboration with colleagues experienced in delivering gene-based medicine to humans, the team is now advancing these exciting preclinical studies to clinical trials in HD patients. Bev's research is focused on experiments to better understand the biochemistry and cell biology for HD and other inherited genetic diseases that cause central nervous system dysfunction. Her scientific quest is to develop strategies for getting cures for HD into cells.

"Gene therapy approaches have already been successful in treating a form of genetic blindness, and it could be harnessed as a treatment for HD," Bev says. She is using genetic vectors for delivering gene editing machinery to the brain, where her team has shown that they can remove the mutant allele while leaving the normal allele intact. This holds enormous opportunity and the next challenge is figuring the best delivery systems by which to accomplish this.

Always digging deeper and looking at the most novel approaches, Bev is also currently exploring the link between HD and heart disease, trying to understand why the heart is impacted by HD.

From the Plains of Nebraska to the Labs of Philadelphia

Bev's scientific journey started in the plains of Nebraska where she grew up. Her father was a doctor and her mother a nurse.

"I was always interested in how things worked, how things are put together, and how to solve problems. And I loved going to the hospital with my dad to help with anything (e.g., holding a hand, or retrieving a jar to hold an extracted fish hook), or just to watch him care for people with all kinds of maladies. I read Scientific American and National Geographic from an early age and my parents always encouraged my interest in science. I worked in labs throughout my undergraduate career, even published my first paper studying the changes in various urea cycle enzymes as the tadpole transitioned to a frog, combining field work with anatomy and basic biochemistry and enzyme assays." Once she entered graduate school, she married her love of biochemistry with molecular biology, and identified mutations that caused the neurological

disorder Lesch-Nyhan syndrome. "It was during this training that my fate as a neuroscientist was sealed," says Davidson.

In 2002, early in her career, Bev attended an HDF workshop on gene silencing, one of the first workshops held on this topic. It was organized by Nobel Laureate Philip Sharp and Nobel Laureate and long-standing HDF Scientific Board member H. Robert Horvitz. Just as the 1979 HDF conference that made the audacious proposal that we could use DNA markers to find the gene for HD was a landmark event heralding the beginning of a new era in genetic science, so too was the 2002 conference. It led Bev to expand her research portfolio to include Huntington's disease.



Bev receiving the Gehry Brenner Prize at the HDF Symposium and Dinner in 2015

"I was invited to speak about my research into gene silencing for the inherited neurological disorder Spinocerebellar ataxia. Nancy asked why I wasn't also working on Huntington's disease, and right then she recruited me into the fold."

The Hereditary Disease Foundation was the first to fund Bev's laboratory at the University of Iowa. As a result of this seed funding, she went on to receive many other prestigious and highly competitive awards from the NIH.

Bev became a member of the HDF Scientific Advisory Board in 2005.

“We invite people to join our Scientific Advisory Board early in their careers because we want to learn from them and have them play a vital role in shaping the science,” says Nancy. “It helps us fund the most catalytic and imaginative research.”

Bev says, “The Board actively shapes the critical thinking about what the future holds. I’m passionate about getting the work to the next level and training the next group of scientists.”

“Bev is a phenomenal mentor,” says Nancy. “She’s a perfectionist who expects the best of people and gets it. Everyone who has been touched by her is still working on HD.”

Among Bev’s mentees is Alex Mas Monteyts at CHOP who is receiving HDF funding for his working on a switch to turn off or control the bad actors that impact the brain in HD. Paul Ranum, also at CHOP, has just received HDF funding this year for work on critical genes that impact Huntington’s. Other mentees who have received HDF funding and are dedicated to finding treatments and cures for Huntington’s disease and other neurological disorders include Jodi McBride of Oregon Health & Science University (OHSU), Scott Harper of Ohio State University (OSU), Sarah Fineberg at Yale and John Lee, who is finishing his MD fellowship at the University of California at Los Angeles (UCLA).

Along her path-breaking scientific journey, Bev has received many accolades, contributed to hundreds of publications in the scientific literature and served on scientific advisory boards for Sarepta Therapeutics, Intellia Therapeutics, and Homology Medicines, among others. She is also on the editorial board of Molecular Therapy, EMBO Reports and Human Molecular Genetics. She received her PhD in biological chemistry from the University of Michigan and served on the faculty there. Prior to joining the Children’s Hospital of Philadelphia in 2014 as the Chair of the Center of Cellular and Molecular Therapeutics and Professor of Pathology and Laboratory Medicine at Penn, she was at the University of Iowa as Associate Director of the Center for Gene Therapy, the Roy J. Carver Biomedical Research Chair in Internal Medicine, and Vice Chair of the Department of Internal Medicine and a

Professor in Internal Medicine, Neurology, Physiology & Biophysics. In 2007 she was named a fellow of the American Association for the Advancement of Science, and in 2014 was named to the NINDS advisory council. In 2015, Bev received the Leslie Gehry Brenner Prize for Innovation in Science from the HDF for her work on gene silencing. In 2017, Bev was elected to the American Academy of Arts and Sciences, joining with world leaders in the arts and sciences, business, philanthropy, and public affairs. Bev is one of the few female pioneers in the biomedical industry focused on gene-based medicines. She is a co-founder of Spark Therapeutics, Inc. and Spirovant Therapeutics.

A brilliant scientist and a visionary leader, Bev is also the mother of two children who are pursuing careers in the medical sciences. Her son is currently a 4th year medical student at the University of Iowa and her daughter is a Master's graduate of Sarah Lawrence's genetic counselor training program, and now works for GeneDx. Talk about mentorship!

Mark Your Calendars!

The HDF Symposium and Dinner will be at the Harvard Club on Monday, October 28. It will be a special evening to share with friends and family and hear from outstanding scientists who are at the forefront of finding treatments and cures for HD. It will also be a joyous celebration of Nancy Wexler, recipient of the 2019 Double Helix Medal from Cold Spring Harbor Laboratory. Stay tuned for more details!

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Published August 2019