News from the Hereditary Disease Foundation

Trailblazing Research…Magical Moments
$800,000 Raised Toward HD Treatments and Cures
Dr. Anne Young, chair of our Scientific Advisory Board, said it best, "This is the golden age of Huntington's research." She was speaking at our 2018 Symposium on October 22, at the Harvard Club. The Symposium was the start of an evening celebrating 50 years of rewriting the future through trailblazing research. There were many magical moments as we heard how research is transforming our thinking about Huntington's disease and recognized one of the brilliant scientists who is truly rewriting the future for all of us. We raised $800,000 to help keep the momentum going!

HDF-funded scientists Andrew Yoo, Alina Li, Ai Yamamoto, Christopher Ng, and Ben Hoffstrom with Nancy Wexler

Science Changes Everything
A rapt audience listened intently as Dr. Shirley Tilghman, president emerita and professor of molecular biology and public affairs at Princeton University, spoke

Dr. Shirley Tilghman

about how genes are organized in the genome and regulated during early development. A member of the National Research Council's committee that set the blueprint for the United States effort in the Human Genome Project, she was also one of the founding members of the National Advisory Council of the Human Genome Project for the National Institutes of Health. Dr. Tilghman is a world-renowned leader in the field of molecular biology whose brilliant scientific career as a mammalian development geneticist has advanced our understanding of Huntington's disease and other disorders.

The mood within the room was electric with excitement and hope as Dr. C. Frank Bennett, senior vice president for research and franchise leader for neurological programs at Ionis Pharmaceuticals, took the podium. He gave a mesmerizing talk about his work developing antisense therapeutics for neuromuscular and neurological diseases. His pioneering work brought us the world's first approved treatment for spinal muscular atrophy (SMA). In 2005, Frank started a program to develop antisense therapies for Huntington's disease. IONIS-HTTrx is designed to treat patients with HD and is the first and only drug to demonstrate reduction of the neurotoxic abnormal huntingtin protein, the underlying cause of HD, in patients. Ionis is partnering with Roche for phase 3 of the trial, which is set to begin in the U.S. and abroad soon!! This is a groundbreaking advance for the field of HD!

Celebrating Scientific Innovation

Following a lively question and answer session, the beautiful and inspiring evening continued with dinner, an awards presentation and entertainment. The Leslie Gehry Brenner Prize for Innovation in Science was awarded to Dr. C. Frank Bennett from Ionis Pharmaceuticals for his leadership and continued commitment to developing antisense therapies for Huntington's disease. The prize was created in honor of renowned architect Frank Gehry's daughter, the late Leslie Gehry Brenner, who died of uterine cancer in 2008. It was the highlight of the evening and a poignant moment when Frank Gehry presented the award to Dr. Bennett.

Dr. Nancy Wexler said, "We are thrilled to recognize Frank Bennett for his innovative work in bringing us a totally novel class of drugs. We recognize the truly pathbreaking and monumental promise of Frank's creativity to mold DNA in a way that the toxic HD protein is silenced from mucking up the works in our brain cells." She added, "Frank's novel insight to use the body's own molecular capacity to create new medicine makes him an HD pioneer. His accomplishments honor the spirit and memory of Leslie Gehry by embodying originality, spontaneity, precision and rigor – all critical attributes in a scientist."

Nancy Wexler, C. Frank Bennett, Frank Gehry

"I am honored and grateful to be recognized by the Hereditary Disease Foundation and the Gehry family," said Dr. Bennett. "For nearly 30 years, our technology and learnings from developing drugs for diseases like Huntington's have paved the way for new potential therapies for diseases where no therapeutic approaches exist. I am proud of these achievements, but they would not have been possible without the vision and leadership of Dr. Stanley T. Crooke, founder of Ionis Pharmaceuticals, and the commitment of my colleagues to bringing new antisense therapies to patients living with unmet medical needs."

Celebrating HD Families and Supporters

The evening was capped off with a moving and spirited musical performance by Kate and Justin Miner of the band Miner. Kate's mother has HD and her sisters have both tested positive. Kate is actively engaged with the medical community and policymakers to help find a cure for HD and raise awareness, traveling to Washington D.C. to speak with legislators and working on behalf of charitable organizations.

Kate Miner, Justin Miner, 2018 Gala Chair Karen Newman

We thank all who attended and supported the evening, and all those whose continued support is opening up new pathways for research that is leading to treatments and cures. We celebrate you!

Innovating Research…Discovering Cures

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