Moving Forward and Staying Connected Through Challenging Times
A Year to Forget Cannot Stop Our Progress

Dear Friends,

The COVID-19 pandemic has affected all aspects of our society. Over a million lives have been lost, millions more jobs are gone and countless businesses have been shuttered. The research community was not immune to the consequences of this virus. HD laboratories around the world locked their doors and recruitment to clinical trials in many cases was halted to curb the spread of the virus.

The pandemic forced HDSA to cancel its in-person Annual Convention for the first time in our history. However, cancelling Convention altogether was never an option, so, the HD community did what it always does... adapt and persevere. We came together quickly to convert our New Orleans Convention into a virtual event that safely reached more than 1500 people in over 90 countries. It was here that families got first-hand updates from scientists and clinicians from around the globe on our collective progress since we last met in Boston.

Despite the setbacks 2020 presented, this year marked several notable events in the history of HD research.

uniQure dosed the very first HD patient in the US with an experimental gene therapy that could permanently lower levels of the huntingtin protein with just one dose. Novartis announced plans to launch a Phase 2 trial to investigate an orally administered drug to lower huntingtin. Triplet Therapeutics began (and fully recruited) Shield-HD to better understand how the CAG repeats in the huntingtin gene expand to prepare them to test a drug that could interfere with this process. Azevan reported clinical trial data suggesting their drug, SRX-246 may improve irritability and aggression in HD patients.

While 2020 will undoubtedly go down in history as one of the most challenging years ever, I will remember it as a year of unprecedented expansion of our HD drug pipeline. An expansion based on solid science from observations in humans. So, while we anxiously await results from the current huntingtin lowering trials like Generation-HD1 and Precision-HD1/2, I am excited and more hopeful than ever by the depth and diversity of our clinical trial bench.

I hope that you enjoy reading about our expanding drug pipeline in this year’s The Marker and here is to a safe and happier 2021 with continued progress towards a world free of HD!

George Yohnling, PhD
Chief Scientific Officer & Chief Mission Officer, HDSA
In October of 2020, HDSA awarded four grants under the Society’s largest research initiative, the HDSA Huntington’s Disease Human Biology Project. These grants, totaling $523,000, represent HDSA’s patient-centric research focus which brings basic and clinical researchers together to facilitate Huntington’s disease science in the human condition. Applicants worldwide proposed projects addressing HD through small clinical studies or donated human samples. This year’s winners hail from Australia, Canada, Germany, and the United States.

2020 Human Biology Project Fellowships

Simon Laganiere, MD
Beth Israel Deaconess Hospital
Dr. Laganiere will use innovative imaging techniques to study how brain networks interact in presymptomatic individuals during challenging cognitive tasks. This type of mental stress test can show HD related brain changes earlier than they would otherwise appear. If successful, results from this study will help determine the optimal timing for eventual disease modifying treatments.

Bjoern von Einem, PhD
University of Ulm
Dr. von Einem’s project is focused on improving upon the technology to monitor the effectiveness of huntingtin-lowering ASO drugs that are currently in clinical trials. The goal is to noninvasively measure huntingtin in the spinal fluid, by sampling tiny bubbles called exosomes. This work will help guide the development of potential disease-modifying treatments for HD.

Yifat Glikmann-Johnston, PhD
Monash University
Dr. Glikmann-Johnston will carry out a large study to understand the key differences in gut bacteria between people with and without the HD gene. This foundational work will serve to determine whether there is a relationship between the microbiota in our intestines, and mood and cognition in people with HD. The gut may be an excellent target for drug development and dietary interventions.

Melanie Alpaugh, PhD
Université Laval
Dr. Alpaugh aims to identify factors in the blood, including a protein called tau, that could be used to track cognitive symptoms of HD. A reliable and noninvasive tool to predict which individuals are likely to develop cognitive deficits would have important implications for clinical trials as well as symptom management.
2020 Donald A. King Summer Research Fellowships

In May 2020, five exceptional undergraduate students were awarded HDSA’s Donald A. King Summer Research Fellowships. These awards are named in honor of Donald King, who served as HDSA Board Chairman from 1999 to 2003 and worked tirelessly to advocate for HD families until his death in 2004.

In order to further meaningful discoveries about Huntington’s disease and ensure that bright young scientists are steered towards the field, this program supports undergraduates who have committed their summers to training in an HD research lab. Applications are accepted between December and March each year and are reviewed by HDSA’s Scientific Advisory Board.

This year’s applicants were evaluated by the quality of their personal academic achievements, mentoring plan, experimental design, and the feasibility of achieving their scientific goals. After rigorous review by the HDSA’s Scientific Advisory Board, five young scientists, the most in the history of this program, were awarded 2020 Donald A. King Summer Research Fellowships. Due to this year’s unusual circumstances, these projects were extended throughout 2020.

Funding for this year’s Dan King Fellows was made possible, in part, by a generous gift from Hal and Isabelle Pilskaln. Funding HD research has been a life-long passion for the Pilskalns and HDSA is indebted to them for their support.

Sophia Friedman
Wellesley College
Sophia is completing her project at the Massachusetts Institute of Technology with David Houman, PhD, exploring genes known to alter the onset of HD symptoms.

Amber Keith
University of California at Irvine
Amber worked with Sarah Hernandez, PhD, a 2017 HDSA Berman-Topper Career Development Fellowship recipient, in the laboratory of Dr. Leslie Thompson, to develop a model of the blood-brain barrier derived from human cells.

Lav Patel
Ohio State University
Lav is doing HD research in the laboratory of Richard Fischl, PhD, where he aims to visualize DNA repair proteins that affect the expansion of CAG repeats.

Tasneem Sadok
University of California at Los Angeles
Tasneem is working under the guidance of Lindsay DeBiase, PhD, to study how changes in energy production affect the brain’s support cells in areas vulnerable to HD.

Kadambari Vyas
University of Central Florida
Kadambari was mentored by former HDSA Human Biology Fellow Dr. Amber Southwell and studied aggression in mouse models of HD.
As part of HDSA’s commitment to developing the next generation of passionate and innovative Huntington’s disease scientists, the Berman-Topper Family HD Career Development Fellowships provide up to $80,000 of funding per year for three years to young scientists and clinicians who desire to make HD part of their long-term career plan. These prestigious awards are made possible due to the generosity of the Berman and Topper families.

HDSA received applications from researchers all around the world for this competitive grant. In May, HDSA was pleased to announce that Dr. Yasaman Gholamalipour, of the University of Massachusetts Medical School had been granted this distinguished award for 2020.

Dr. Gholamalipour’s project will attempt to reverse the CAG repeat expansion sequence in the mutant huntingtin gene back to the normal range (less than 36 CAG repeats) in cellular and animal HD models using the CRISPR-Cas9 nickase genome-editing tool.

Upon hearing the announcement of Yasaman’s selection, Michael Berman said, “Dr. Gholamalipour joins a terrific group of young scientists who have added both knowledge and enthusiasm to the field of HD research, and we are sure that her contribution will be significant.”

In 2015, to facilitate participation in clinical trials for both families and researchers, HDSA created HD Trialfinder, a clinical trial matching service. It is a way for individuals with Huntington’s disease, caregivers, healthy volunteers, and physicians to connect with current research studies.

HD Trialfinder includes an easy-to-use website and free call center staffed by trained HD clinical trial navigators. Through involvement in the worldwide HD research community, HDSA keeps track of national, international, and local clinical trials so that the HD Trialfinder listing stays up-to-date with studies that need participants. Anyone can visit the website to read about ongoing studies, and by creating a profile for yourself, a loved one, or a patient, users can find out which nearby studies they are eligible for, and locate contact information to get involved directly. This year we saw the number of HD Trialfinder users grow to over 7,500. They have undoubtedly contributed to the speedy US recruitment of three huntingtin-lowering trials.

Visit www.hdtrialfinder.org or call 1-866-890-6612 to learn more.
Clinical Trial Updates

ENROLL-HD

Enroll-HD Evolving to Meet Future Clinical Trial and Researcher Demands

Enroll-HD is an observational study that is just one component of the entire Enroll-HD research platform that serves to enable all future HD clinical studies. Enroll-HD is sponsored and managed by CHDI Foundation, a not-for-profit biomedical research organization dedicated to rapidly developing therapies that slow the progression of Huntington’s disease.

The COVID-19 pandemic has expectably had a negative impact on the number of in-person visits conducted at the study sites, with the lowest number of visits conducted in April 2020. While we saw a slight rebound in the late summer/early fall, the second wave of COVID-19 is slowing down progress on this important study once again.

The ultimate goal of HD researchers is to prevent HD gene expansion carriers from ever getting sick. To do so, pre-symptomatic clinical trials will be required. To make this goal a reality, the new Enroll-HD recruitment strategy is focusing on the recruitment of at risk, pre-manifest, and early-stage participants. As of November 1, 2020, there are nearly 20,000 active participants enrolled at 158 active sites in 20 countries around the world. The highest recruiting HDSA Centers of Excellence for the Enroll-HD study are the University of California at San Diego (UCSD) and Vanderbilt University. Both have recruited over 300 participants.

WAVE LIFE SCIENCES

Wave Life Sciences Announced Top-Line Results from PRECISION-HD2 Trial

Wave Life Sciences is investigating huntingtin-lowering ASOs in two Phase 1b/2a clinical trials called PRECISION-HD1 and PRECISION-HD2. Whereas the Roche drug lowers all huntingtin, Wave’s approach focuses on lowering only the mutant form of huntingtin protein and leaving the healthy form intact. Participants in these early safety trials (and any future recipient of these ASOs) must have not only the HD mutation, but an additional variation in their HD gene that acts like a “genetic GPS” directing the drug to the right spot. This is true for 61% of people with HD.

On December 30, 2019, after we had published The Marker for 2019, Wave announced encouraging topline results from PRECISION-HD2, their Phase 1b/2a placebo-controlled trial evaluating the investigational therapy WVE-120102 targeting SNP2. When they compared all patients treated with WVE-120102 to patients who received placebo and they saw a statistically significant reduction of 12.4% in mutant huntingtin protein in the cerebrospinal fluid (CSF).

In addition to demonstrating a reduction in mutant huntingtin protein, WVE-120102 was safe and well-tolerated across all patients. In hopes of lowering huntingtin even more, Wave added a higher dose (32mg) group to both the PRECISION-HD1 and HD2 trials. We expect results from these studies in early 2021.
**Clinical Trial Updates**

**uniQure**

**uniQure Administers the World’s First Gene Therapy for HD in Clinical Trial of AMT-130**

This year marked an exciting milestone in the history of HD research. This June, the very first HD patient in the world was treated with a gene therapy aimed at decreasing the expression of the gene that causes HD. The drug, called AMT-130, is a piece of man-made genetic code called a microRNA. It is packaged inside a type of harmless virus and delivered deep into the brain via a neurosurgery. Once the virus containing the drug enters a brain cell, the virus will make the huntingtin-lowering micro-RNA that will instruct the cell to make less toxic huntingtin protein. In theory, with only one surgical procedure, the drug will keep attacking toxic huntingtin indefinitely. This experimental procedure has so far been successful at lowering huntingtin in animals like mice and mini-pigs.

The first two patients were observed for an initial period of 90 days to ensure this approach was safe. An independent Data Safety Monitoring Board (DSMB) reviewed the data on the first two participants and determined that it was safe to proceed with the dosing of additional people. When the study is complete, 26 people will have undergone neurosurgery and will have received AMT-130 or an imitation surgery where no drug is injected into the brain. For 18 months after the surgery, participants will have frequent follow-up visits, with imaging, neurological exams, blood draws, and other tests to determine the safety of the drug. Then they will have yearly visits until 5 years after the surgery.

**George Yohrling,** Chief Scientific Officer and Chief Mission Officer at HDSA said, “There is an urgent need for disease-modifying options to treat Huntington’s disease, and we’re excited to have an investigational gene therapy now available for HD patients. Based on the promising preclinical data presented on AMT-130 over the years, we are optimistic about its potential to alter the course of this devastating disease.”

This first HD gene therapy trial is an extraordinary milestone in HD clinical research, and HDSA will continue to work closely with uniQure to provide families with up-to-date information about the progress of the study.

**AMT-130 will be injected into the striatum, a deep part of the brain affected in the early stages of Huntington’s disease.**

**ROCHE**

**GENERATION-HD1 Trial of Roche Drug Tominersen Continues — Results Expected in 2022**

GENERATION-HD1 is an ongoing trial to test the effectiveness of a huntingtin-lowering drug in development by the pharmaceutical company Roche. In February of 2020, Roche announced that the drug had been given an official name: tominersen. This Phase 3 trial is the first of its kind, and is fully recruited, with nearly 800 participants at about 100 sites across the globe. Tominersen is an antisense oligonucleotide (ASO) designed to attack the RNA message that produces huntingtin protein, and it is delivered via spinal injection. The trial participants are visiting study sites to receive the drug or a placebo every 2 months over the course of two years.

Roche announced this year that the results of the study are expected in 2022. If it proves effective at slowing down the progression of HD symptoms, the next step would be the process of getting approval from regulatory agencies like the FDA.

**NOVARTIS**

**Novartis SMA Drug Branaplam Receives Orphan Drug Designation to be Tested in HD Patients**

In October of 2020, Novartis announced that they had received special status from the FDA, known as Orphan Drug Designation, to study an experimental drug called branaplam in HD patients. Orphan Drug Designation is a set of financial incentives that makes it easier, more attractive, and more efficient for companies to develop treatments for rare diseases. Branaplam was developed for the treatment of a genetic disease in children called spinal muscular atrophy (SMA). While testing it in animals and humans, Novartis discovered that it can lower levels of the huntingtin protein, which is the culprit in HD. The exciting part: this drug is already known to be safe in patients with SMA, and is taken by mouth. The next step will be testing branaplam in larger numbers of people, to make sure it is safe for HD patients and see whether it could help with HD symptoms. Novartis plans to start a trial of branaplam in HD patients in 2021, and receiving Orphan Drug status will help them do so.
Neurocrine Biosciences is conducting the KINECT-HD study, a Phase 3 clinical trial of a drug to treat HD movement symptoms (chorea). The medication, valbenazine, is already approved for people with tardive dyskinesia, a condition that also causes involuntary movements of the face and limbs. Neurocrine plans to enroll 120 participants with HD, ages 18 to 75, for this 18-week study. The KINECT-HD trial began in early 2020 and is ongoing at more than 25 sites in North America.

Vaccinex Pharmaceuticals
Unfortunately, we learned in September of 2020 that the SIGNAL trial being conducted by Vaccinex Pharmaceuticals did not meet its primary endpoints. This Phase 2 trial was designed to test whether a drug called pepinemab could help with HD symptoms, measuring participants’ performance on cognitive tests and other measurements of their well-being. At the end of the SIGNAL trial, the drug was deemed safe and tolerable, but participants who received pepinemab did not perform better than those who received the placebo. Nevertheless, the data collected during study visits is invaluable to our understanding of HD progression, and the results of the SIGNAL trial will inform trials of pepinemab in Alzheimer’s and head and neck cancer patients.

Prilenia Therapeutics
Prilenia was founded with the goal of developing pridopidine, a drug that was tested several years ago in HD patients in a Phase 2 trial called PRIDE-HD. Although that study did not meet its key clinical goals, some patients showed improvements in function over the course of the trial, in their ability to manage at work and at home. In November, Prilenia launched PROOF-HD, a larger and longer trial of pridopidine. This Phase 3 study will involve 480 participants at an early stage of disease, who will take the drug for about 18 months to determine if it could preserve brain function in HD.

Triplet Therapeutics
Boston-based company Triplet Therapeutics is taking a novel approach to HD drug development. One recent focus of HD research has been the phenomenon of somatic CAG repeat expansion. This is the observation that the number of CAG repeats in some cells of the body and brain can increase as a person with HD ages. The continued expansion of CAGs is believed to accelerate the onset of symptoms. Triplet Therapeutics aims to target the DNA repair machinery to slow down or stop this process. In parallel to developing therapies, they have begun an observational study called SHIELD-HD, which recruited in record time. It will follow about 60 adults (ages 18–63) with HD for seven study visits over the course of 2 years. It will measure a range of symptoms and abilities along with testing of blood and spinal fluid to better understand how CAG repeats lengthen over time in different individuals. This information will inform future drug studies to attempt to shrink CAG repeats or slow their growth.

Annexon Biosciences
Annexon Biosciences is a company based in San Francisco that is developing therapies to preserve the connections between neurons, called synapses, in the HD brain. Their target is a part of the immune system called the classical complement cascade, which goes awry in neurodegenerative diseases and immune disorders. In 2020 Annexon began conducting a small trial of their drug ANX005 in people with HD. Twenty-five adults age 18 and up will be recruited at eight sites in the US. This clinical trial was born out of pre-clinical studies originally conducted by Dr. Daniel Wilton during his HDSA HD Human Biology Fellowship, which is a testament to the impactful, human-centric work we fund.
The Nobel Prize in Chemistry was awarded this year to Emmanuelle Charpentier and Jennifer A. Doudna, who discovered the gene editing technology, CRISPR/Cas9. This technology has allowed scientists to manipulate genetics in unprecedented ways, opening new doors to the study of gene function and disease. The technology has allowed HD researchers all over the world to create new models and explore new questions about HD biology. Although CRISPR techniques are unlikely to be used for treating HD in humans in the near future, there are scientists working to improve upon CRISPR and other DNA editing technology for potential use in brain diseases. It is worth noting that Doudna and Charpentier are the first women-only duo to be awarded a Nobel Prize in any category.

HDSA continues to provide families with the latest information about clinical trials, HD research news, and HD research activities.

RESEARCH WEBINARS
Research webinars give academic and industry scientists a direct forum to share their latest findings with families.

THIS WEEK IN HD RESEARCH
Dr. Leora Fox provides a roundup of HD research activities in HDSA’s blog, This Week in HD Research.

HDBUZZ
HDSA supports HDBuzz, a website devoted to clear and effective communication about HD research and clinical studies.

SOCIAL MEDIA
HDSA shares relevant scientific news on our social media channels.

CONVENTION
The 2020 Virtual HDSA Convention featured a variety of research sessions, including researcher Q&As, science trivia with HD scientists from HD families, trial updates, a clinical showcase featuring studies that are soon to be recruiting, and novel approaches to HD therapy.

HD-COPE
Now in its third year, the Huntington’s Disease Coalition for Patient Engagement (HD-COPE) continued to provide patient and family input to help industry researchers better understand HD and to guide their clinical plans. Numerous companies met virtually in 2020 with members of the HD-COPE team to help shape their future clinical development plans.
The Huntington Study Group is a clinical research network focused exclusively on Huntington disease. Each year they host a conference that provides education and training for HD clinicians and researchers, with a final day reserved for families and the wider HD community. Their virtual event, HD in Focus, took place in late October. Many pharmaceutical companies presented brief updates on their novel approaches to HD treatments.

AZEVAN PHARMACEUTICALS
A highlight of the conference was a presentation of clinical trial data from Azevan Pharmaceuticals. Azevan recently analyzed results from a Phase 2 trial called STAIR of a drug called SRX246, which was designed to decrease aggression and irritability in people with HD. The STAIR study showed that SRX246 was safe for HD patients and lessened the frequency of aggressive outbursts in those who experience them. This is the first drug to focus specifically on a behavioral symptom in HD patients.

NOVARTIS
Novartis announced the exciting news of their plans to initiate a Phase 2 trial of an oral drug called branaplam, and revealed its mechanism of action for huntingtin-lowering in HD. (See Clinical Trial Updates on page 10.) This therapy is in clinical trials for the fatal childhood disorder spinal muscular atrophy, and was also found to lower huntingtin in patients’ cerebrospinal fluid.

NEUEXCELL THERAPEUTICS
NeuExcell Therapeutics hopes to rebuild damage in the HD brain by converting the brain’s support cells into new neurons. They plan to initiate clinical trials for HD patients in 2022.

MITOCHON PHARMACEUTICALS
Mitochon Pharmaceuticals is exploring the use of existing drugs that target mitochondria (the energy powerhouses in our cells). The goal is to stop the production of reactive oxygen species in HD patients which are thought to cause damage to the HD brain.

NEUBASE THERAPEUTICS
Neubase Therapeutics has developed a genetic technology administered intravenously which can spread throughout the body and brain. They hope to move forward with this platform to target mutant huntingtin in human studies.

CHDI FOUNDATION
2020 CHDI HD Therapeutics Conference
The Huntington’s Disease Therapeutics Conference, hosted by CHDI, is the largest annual gathering of HD researchers. In February of 2020 participants gathered in Palm Springs, California for a 3-day conference featuring presentations from academic scientists, industry leaders, and research nonprofits.

A highlight of this year’s conference was the huntingtin-lowering session, in which uniQure presented the science and design behind the world’s first trial of an HD gene therapy, delivered via brain surgery. (See Clinical Trial Updates on page 9.) This small safety trial began in June 2020 with two participants, and an additional two underwent the procedure in September 2020. Voyager Therapeutics presented a similar surgical gene therapy approach which is also slated for human trials in the near future. PTC Therapeutics shared their intention to launch a trial of a huntingtin-lowering small molecule, a particularly exciting development because this drug could be taken by mouth.

Other compelling sessions focused on the path to future trials that could prevent HD symptoms, novel imaging techniques to enable more precise visualization of the HD brain, insights into the huntingtin protein, and advanced computational modeling to help clinicians predict, monitor, and treat symptoms. The final session explored findings from large scale genetic studies, focusing on other genes that might shape the course of HD by influencing types of symptoms and age of onset. Such findings are beginning to drive HD drug development in the areas of DNA repair and personalized genetic medicine.
Huntington’s Disease Society of America Announces Fifty 2020 HDSA Centers of Excellence

In February of 2020, HDSA announced that fifty outstanding Huntington’s disease (HD) care facilities had been awarded the designation of HDSA Centers of Excellence for 2020. The HDSA Centers of Excellence are multi-disciplinary care teams with expertise in Huntington’s disease that share an exemplary commitment to providing comprehensive care.

The 2020 HDSA Centers of Excellence program expanded to 50 Centers from 47 in 2019, and from just 20 in 2015. The four new Centers of Excellence for 2020 are:
- Henry Ford Hospital (Michigan)
- Stony Brook University Hospital (New York)
- University of Kansas Medical Center
- University of Miami

The strategic expansion of the Center of Excellence program allows HDSA to increase access to expert HD clinical care and clinical trial opportunities to more families across the United States. With new Centers in Florida, Kansas, Michigan, and New York, HDSA now offers care locations in 33 States plus the District of Columbia. This year, HDSA will be awarding a total of $1,550,000 to the Centers of Excellence program.

The HDSA Centers of Excellence provide an elite team approach to Huntington’s disease care and research. Patients benefit from expert neurologists, psychiatrists, therapists, counselors, and other professionals who have extensive experience working with families affected by HD and who work collaboratively to help families plan the best HD care program throughout the course of the disease. Applications to become an HDSA Center of Excellence are open to all clinics in the United States who share HDSA’s commitment to high-quality, comprehensive care and access to clinical research.

“The growth of the HDSA Center of Excellence program has been nothing short of amazing. The Centers provide crucial multidisciplinary care, opportunities to participate in clinical research, and education for the medical and non-medical community,” said Dr. Donald Higgins, HDSA Board member, Chair of HDSA’s Centers Programs and Education Advisory Committee and National Director of Neurology for the Veteran Health Administration. “Kudos to HDSA for the unwavering support of this important program and to the Center Program and Education Advisory Committee tasked with reviewing applications for designation!”

HDSA CENTERS OF EXCELLENCE

Centers of Excellence

By the Numbers

$1,550,000

awarded in 2020

250%

program growth since 2015

92%

of Centers provide clinical trials

50 +

HDSA Centers of Excellence

6

partner sites

33 states and D.C.

Albany Medical College (NY)
Beth Israel Deaconess Medical Center (MA)
Cleveland Clinic (OH)
Columbia Health Sciences/ NYS Psychiatric Institute (NY)
Dartmouth-Hitchcock Medical Center (NH)
Duke University (NC)
Emory University (GA)
Georgetown University (DC)
Hennepin County Medical Center (MN)
Henry Ford Hospital (MI)
Indiana University
Johns Hopkins University (MD)
Massachusetts General Hospital
Northwestern University (IL)
Ochsner Health System (LA)
Ohio State University
OSF-Illinois Neurological Institute
Rocky Mountain Movement Disorders Clinic (CO)
Rush University Medical Center (IL)
Sanford Health (ND)
Stanford University (CA)
Stony Brook University Hospital (NY)
University of Alabama, Birmingham
University of Buffalo (NY)
University of California, Davis Medical Center
University of California, Irvine
University of California, Los Angeles
University of California, San Diego
University of California, San Francisco
University of Colorado
University of Florida
University of Iowa
University of Kansas Medical Center
University of Louisville (KY)
University of Miami (FL)
University of Nebraska Medical Center
University of Pennsylvania Medical Center
University of Pittsburgh Medical Center (PA)
University of Rochester (NY)
University of South Carolina School of Medicine
University of South Florida
University of Texas Health Science Center – Houston
University of Utah
University of Vermont, Frederick Binter Center for Parkinson’s Disease & Movement Disorders
University of Virginia
University of Washington (WA)
University of Wisconsin
Vanderbilt University Medical Center (TN)
Virginia Commonwealth University
Washington University School of Medicine (MO)

LEVEL 1 PARTNER HD CLINICS

Kaiser Permanente (CA)
Oregon Health Sciences University
Cole Neuroscience Center, University of Tennessee Medical Center
University of Tennessee, Erlanger Medical Center
University of Mississippi Medical Center
University of South Alabama
A Special Thanks to the HDSA Scientific Advisory Board

We are grateful to the HDSA Scientific Advisory Board (SAB) members who so generously donate their time and talent as volunteers! The HDSA SAB is comprised of leading experts in their fields. The role of the SAB is to provide scientific review of research proposals to ensure that the research programs at HDSA are scientifically sound, pertinent and provide a high impact to the HD research community. Additionally, the SAB advises the HDSA Board of Trustees and management on a range of issues influencing the scientific direction of the Society.
Huntington's Disease
Society of America

**OUR MISSION**
To improve the lives of people with Huntington's disease and their families.

**OUR VISION**
A world free of Huntington's disease.

**Huntington's disease (HD)** is a fatal genetic disorder that causes the progressive breakdown of nerve cells in the brain. It deteriorates a person's physical and mental abilities often starting in their prime working years. Currently, there is no cure for Huntington's disease.

HD is known as the quintessential family disease, because every child of a parent with HD has a 50/50 chance of carrying the faulty gene that causes Huntington’s disease. Today, there are approximately 41,000 symptomatic Americans and more than 200,000 individuals at-risk of inheriting the disease.

**The Huntington's Disease Society of America (HDSA)** is the premier nonprofit organization dedicated to improving the lives of everyone affected by HD. From community services and education to advocacy and research, HDSA is the world's leader in providing help for today and hope for tomorrow for people with HD and their families.

Across the United States HDSA supports 50 volunteer-led Chapters & Affiliates, 50 HDSA Centers of Excellence, more than 60 social workers and 80 support groups specifically for HD families.