FDA approves first gene therapy for an inherited disease.

Could Huntington disease be next?
The clock is ticking for people like Tacie and her already diagnosed family members, who struggle to be patient, even as their lives unravel around them. But HD researchers and clinicians, fully aware of the imperativeness and urgency of their tasks, continue to plow ahead. In fact, sitting in the conference sessions and listening to these men and women speak I was impressed by just how seriously they take their jobs. In the words of Tacie Fox, “For them, it’s not just a job and a career; it’s a life mission.”

Since the conference, two important breakthroughs have occurred in the fight against HD. First, IONIS Pharmaceuticals, Inc., announced that its drug IONIS-HTTRRx successfully and safely lowers the toxic mutant huntingtin protein (mHTT) in people with HD. The company now is planning a larger trial to test whether IONIS-HTTRRx slows disease progression. Experts all agree that this is an unprecedented step toward finding an effective treatment for HD.

Second, Spark Therapeutics received approval by the Food and Drug Administration in December for its gene therapy Luxturna, which treats retinal dystrophy, a rare inherited form of blindness. The treatment actually restores vision to people who were born blind. With the approval of Luxturna by the FDA, there’s little doubt that a gene therapy will soon be available for HD.

Both of these recent developments are described in more detail in this issue of HD Insights. In addition, we’ve included an interview with Tacie Fox, with the goal of helping you—our readers—feel even more connected to your friends and colleagues in the HD community.

As the new deputy editor of HD Insights, I find myself becoming entrenched in this community, along with the scientists, clinicians, communicators, patient advocates, and most importantly patients and families, who comprise it. It’s an incredible feeling, to join ranks with these people, each of whom contributes his or her own positive, hopeful, and determined energy toward finding a cure.

As always, if you have suggestions for future content, we would love to hear them. We also would like to post any jobs you may have available. Contact me directly at hdinsights@hsglimited.org or submit your jobs and stories ideas online at http://huntingtonstudygroup.org/ hd-insights/.
MEET THE FOUNDATION
Tacie Fox discusses her family’s foundation to support HD research.
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BY THE NUMBERS

50-120
The typical weight of a minipig in kilograms. Minipigs are an emerging large animal model for studying HD.

19
The day in December 2017 when Luxturna, the first gene therapy to treat an inherited disease, was approved by the FDA.

2018
The year in which IONIS Pharmaceuticals plans to publish its results from the IONIS-HTTRx trial. IONIS-HTTRx successfully lowers the toxic mutant huntingtin protein (mHTT) in people with HD.

30,000
Approximate number of Americans who have HD.

25
The Huntington Study Group will hold its 25th Anniversary Celebration November 8-10 in Houston, Texas.

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Image, including cover, courtesy of Spark Therapeutics Inc.

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IONS announces that IONS-HTTRx successfully lowers the toxic mutant huntington protein (mHTT) in people with HD.
Page 7
**RESEARCH ROUND-UP**

### IN BIOMARKERS

By Lise Munsie, PhD

Finding biomarkers that track with Huntington disease (HD) progression continues to be an important challenge in the assessment of clinical trials. Tracking levels of mutant huntingtin (mHTT) is an attractive option since HTT knockdown is a promising therapeutic.

An assay published in the *Journal of Huntington’s Disease* detects mHTT in human cerebrospinal fluid (CSF) using a novel ultrasensitive immunoassay. The technique is called single-molecule counting (SMC) and is a bead-based fluorescent immunoassay.

Guided by U.S. Food and Drug Administration (FDA) standards, the group clinically validates its assay’s ability to detect mHTT in the CSF of HD patients. This is a promising biomarker for quantifying mHTT knockdown therapies in the brain.

Previous work shows alterations of expression patterns of microRNAs (miRNA) in the brains of neurodegenerative disease patients, including those with HD. Dr. C.M. Chen of the Chang Sung Memorial Hospital Linkou Medical Center and College of Medicine and Chang Sung University, and colleagues looked at expression levels of different miRNAs in peripheral leukocytes of HD patients to see if they tracked with disease. They found that miR-9*, an miRNA thought to be involved in neuronal differentiation, may be a marker of neurodegeneration in HD that is detectable in the blood; however, it currently does not seem to track with disease progression. A larger sample size would be required to ascertain if miR-9* tracks with HD progression in blood samples.

Dr. Sarah Tabrizi, professor of clinical neurology, director of the University College London’s Huntington Centre, and her group published on tracking levels of mHTT in patient peripheral blood mononuclear cells (PBMCs) using an ELISA-based mesoscale discovery electrochemiluminescence immunoassay. In agreement with previous studies, this simple and scalable assay shows increased levels of mHTT during disease progression in blood samples. The group was able to detect different conformations of HTT and relate the levels back to disease stage.

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IN ANIMAL MODELS
By Lise Munsie, PhD

Mouse models and other small organisms classically have been used for Huntington disease (HD) research. Yet, larger and more complex models may be better to model the human disease.

Two recent manuscripts describe the results of using the Q73-humanized HD-sheep model (OVT73) for studying the mechanism of disease, as well as testing therapies. Dr. Russell Snell, professor at the University of Auckland, and colleagues performed RNA-seq studies on brain tissue from 5-year-old OVT73 animals. These animals were still in the prodromal phases of HD, so the group was looking at early stage pathophysiology. Their study revealed alterations in urea, indicating metabolic defects and elevated concentrations of urea in the brain in early stage-HD.

Dr. Neil Aronin, professor at the University of Massachusetts Medical School, and his group used the same model to examine the safety and efficacy of using a virus to deliver miRNA-targeting human huntingtin (HTT). The benefits of this treatment over the current clinical trials involving intrathecal delivery of antisense oligonucleotides is that viral delivery of miRNA will be more sustained and more targeted. The treatment was able to specifically target the human mutant HTT (mHTT), and knockdown was noted at one month and six months post injection in the caudate and putamen. This study demonstrates the safety and efficacy of this approach in a large model.

Another new large animal model for studying HD is the minipig. The name of these pigs is a misnomer, as these pigs typically weigh from 50-120 kg, which is the size of an adult human; in addition, the brain is similar to the size of a human brain at 90-100 g. HD transgenic pigs harbor a 124Q mHTT transgene. A study in PLOS ONE characterizes behavioral tests of these pigs so they can be used in future preclinical studies. This study describes prodromal pig behavior up to age 4 and ascertains baseline values for many tests, including functional, cognitive, and behavioral tests, that can be used in the future when these pigs become symptomatic.
RESEARCH ROUND-UP

IN STEM CELLS
By Lise Munsie, PhD

Induced pluripotent stem cells (iPSCs) are both an important tool for research as well as a potential therapeutic avenue for Huntington disease (HD).

A recent article in *Human Molecular Genetics* outlines the use of iPSC from control and HD patients in discerning if the ubiquitin-proteasome system is impaired in HD. The focus of this study is on the FOXO protein family. The researchers looked at this system both in undifferentiated iPSC, as well as iPSC differentiated to neural precursor cells (NPC), and found that the protein FOXO4 had a role in proteasome activity in both cell types. Additionally, they showed that alterations in FOXO4 protein in HD cells are modulated by the kinase AKT. The results of this paper implicate a role of the proteasome in HD pathogenesis.

Two recent papers examine the use of neural stem cells (NSC) derived from iPSC in treating HD mouse models. Julien Rossignol, assistant professor at Central Michigan University College of Medicine, and her team looked at intra-striatal transplants of NSCs derived from mouse iPSCs into the YAC128 model. The team found improved locomotor deficits and increased BDNF levels in these mice. Leslie Thompson, professor at the University of California, Irvine, and her group examined the use of GMP-grade, human-derived NSCs in treating both R6/2 and Q140 full-length mouse models. The transplant of these cells into the striatum led to improved motor deficits in both models as well as grafts showing that the cells were electrophysiologically active. The transplant of these cells led to increased BDNF expression and a reduced accumulation of mutant protein. Although the cells were not transplanted long enough to mature to full post-mitotic neurons, imaging by electron microscopy showed that these cells may be able to make contacts with endogenous neurons.

Together, these studies show promise for the use of regenerative medicine for treating HD. Of note, both of these studies use allogeneic cell sources, and it is possible that autologous cells would have even greater benefit; this is an area of future study.

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DRUG SUCCESSFULLY LOWERS MUTANT HUNTINGTIN PROTEIN

By Sara LaJeunesse

IONIS-HTT$_{RX}$ successfully lowers the toxic mutant huntingtin protein (mHTT) in people with Huntington disease (HD), and it does so safely, according to a press release issued by Ionis Pharmaceuticals, Inc. The drug will be licensed by Roche following the completion of its Phase 1/2a randomized, placebo-controlled, dose escalation study. IONIS-HTT$_{RX}$ is the first therapy in clinical development designed to target the underlying cause of HD by reducing the production of mHTT.

“The results of this trial are of ground-breaking importance for Huntington disease patients and families,” said Dr. Sarah Tabrizi, professor of clinical neurology, director of the University College London’s Huntington Centre, and the global lead investigator on the Phase 1/2a study, in the press release. “For the first time, a drug has lowered the level of the toxic disease-causing protein in the nervous system, and the drug was safe and well tolerated. The key now is to move quickly to a larger trial to test whether IONIS-HTT$_{RX}$ slows disease progression.”

Ionis and Roche plan to present results from this study at medical conferences in the first half of 2018 and to submit the study results for publication in a peer-reviewed medical journal. In addition, Ionis and Roche recently have initiated an open-label extension (OLE) study for patients who completed the Phase 1/2a study.

An article by HD Buzz, calls IONIS-HTT$_{RX}$ “one of the biggest breakthroughs in Huntington’s disease since the discovery of the HD gene in 1993.” The article notes that “The success of this first trial sets the stage for a larger study in hundreds of HD patients, as soon as possible...Until we conduct the next trial, we won’t know if this reduces the impact of HD.”


Dr. Sarah Tabrizi

Roche following the completion of its Phase 1/2a randomized, placebo-controlled, dose escalation study. IONIS-HTT$_{RX}$ is the
FIRST GENE THERAPY TO TREAT AN INHERITED DISEASE IS APPROVED

By Sara LaJeunesse

The first gene therapy to treat an inherited disease was approved by the Food and Drug Administration on December 19, 2017.

Developed by Spark Therapeutics, the therapy—called Luxturna—treats retinal dystrophy, a rare inherited form of blindness.

“This milestone reinforces the potential of this breakthrough approach in treating a wide range of challenging diseases,” said FDA Commissioner Scott Gottlieb in a press release; the treatment does, indeed, have implications for Huntington disease (HD).

A study conducted by Spark Therapeutics involving 31 participants found Luxturna to be effective, with more than 90 percent of the treated patients showing at least some improvement in their ability to complete the obstacle course at low light levels compared to the control group.

Spark Therapeutics has followed its patients for more than three years and has found that the treatment’s effects to be long-standing. The team found no significant adverse reactions to the gene therapy.

In a previous interview with HD Insights, Katherine High, president and chief scientific officer at Spark Therapeutics, said that the treatment’s approval by the FDA would signify “fulfillment of a career spent trying to establish a basis for gene therapy for genetic diseases.” Upon learning of the treatment’s approval by the FDA, she noted that it is time to pursue a gene therapy for HD. In the near future, she said, “I really believe that genes will become medicines.”

Image courtesy of Spark Therapeutics Inc.

For more information about HTTRx, read our interview with Katherine High: huntingtonstudygroup.org/HD-Insights/meet-the-company-spark-therapeutics/
A MEETING OF MINDS
By Sara LaJeunesse

Not only was the elevation a “mile high” at the HSG 2017 conference, which was held in Denver, Colorado, in November, but emotions also soared—with excitement about the rapid progress of Huntington disease (HD) research.

The 24th annual meeting of the Huntington Study Group brought together scientists, clinicians, health practitioners, company representatives, caregivers, and patients, among many others from around the world to learn about and discuss the latest advances in HD research and treatments.

“No other biomarker has shown such a strong association with HD.”

Of note was Dr. Lauren Byrne’s and Dr. Ed Wild’s discussion of their work on a neurofilament light protein (NfL) as a potential prognostic marker of neurodegeneration in HD patients. They found that mean concentrations of NfL in blood plasma at baseline were significantly higher in HTT mutation carriers than in controls, and the difference increased with disease stage. “No other biomarker has shown such a strong association with HD,” said Byrne, research assistant at UCL Institute of Neurology. Wild is a clinician scientist at the institute.

Dr. Jody Corey-Bloom, neurologist at UC San Diego Health, discussed a different type of biomarker, one that could simply and safely detect HD: saliva. Her group has found that the huntingtin (Htt) protein can be detected in salivary samples. “Overall, measurements of salivary Htt appear to offer significant promise as relevant, non-invasive biomarkers for HD,” she said.

Regarding treatments for HD, Dr. Sarah Tabrizi, professor of clinical neurology at UCL Institute of Neurology, spoke about IONIS-HTTRx, an antisense oligonucleotide (ASO), which she and her team found successfully and safely lowers the mutant huntingtin protein (mHTT) in people with HD. In mice, it improves phenotype and survival. The researchers began their human trials in 2015 and expect to publish their results in 2018.
MEET THE FOUNDATION
FOX FAMILY FOUNDATION

IMAGE COURTESY OF THE HUNTINGTON STUDY GROUP
MEET THE FOUNDATION

NAME: FOX FAMILY FOUNDATION
U.S. HEADQUARTERS: MEDFORD, NJ

Tacie Fox, a co-trustee of The Fox Family Foundation, speaks with HD Insights about the foundation’s efforts to support Huntington disease research.

HD INSIGHTS: Tacie, why did your family create the Fox Family Foundation?

FOX: We started the foundation before my father, Bill Fox, died from Huntington disease in 2007. He owned the largest residential real-estate brokerage company in the tri-state Philadelphia area and was loved by his 3,000 employees and agents. My dad was my mentor and the sort of person who made birthday calls every year to everyone in his company to wish them a happy birthday. Near the end of his life, he was devastated that he had to stop calling them; they thought he was a prank caller because his speech was so slurred. Before my father died, we decided as a family we weren’t going to hide from this disease and dedicated ourselves to promoting and supporting HD research. We sold our interest in the family business and funded the foundation from the proceeds for the sole purpose of helping to find a near term treatment and cure for HD for our family members and other families like ours.

HD INSIGHTS: What types of projects does The Fox Family Foundation support?

FOX: When we started the foundation, our goal was to help support scientists, especially younger ones, to participate in working group meetings, so they could collaborate with one another. We still support that goal by being one of the sponsors for the HSG annual meeting, but now we focus more on funding research that has a potential near-term outcome, such as testing a drug that is already approved for another treatment. We give much more—up to $100,000 more—out of our foundation every year than we legally need to because we know that delays in funding critical research will delay finding treatments that will slow or stop the progression of HD. We are committed to this goal and if a treatment-related project comes forward that we believe can alter the progression of HD now, for those who are currently symptomatic, we would seriously consider using a lot of the foundation’s principal to help support it. We want our money to matter, and while we don’t have the resources to fund a drug trial, we hope that our financial support can help in meaningful ways to speed up the achievement of a critical milestone or provide for expanded research or analysis on an already funded project.

HD INSIGHTS: Why did the foundation shift its mission?

FOX: My sister and my cousin were diagnosed with HD, and they courageously battle the disease as it ravages their brain and impacts their lives on a daily basis. We are terrified for them, and know that the rest of us may have the HD gene as well. Our foundation’s key objective is to help support a treatment that will make a meaningful positive impact on their battle with HD in the next five years.

HD INSIGHTS: Do you have Huntington disease?

Continued, page 12
FOX: I don’t know. I’ve gone back and forth for years on whether or not to be tested. I know I don’t want to be tested until I’m symptomatic because I am greatly inspired by all of the research, and I feel that I can be more effective in contributing to the fight against HD when I am propelled by hope and knowledge, with a “half-full glass of water” perspective. At times, the fear of the unknown is terrifying, but the alternative (with no current treatment) is to know definitively that I have a death sentence, and that my two boys then have a 50 percent chance of getting HD. I choose to stay positive and continue to actively support HD researchers, while praying that the many brilliant scientists working on our behalf will come through soon—for my sister, my cousin, and everyone else suffering from HD.

HD INSIGHTS: You have two teenage boys, and your sister and cousin both have children. Are you worried about them getting HD?

FOX: Yes, but I really do believe that by the time they’re facing a potential HD diagnosis 15 to 20 years from now, there will be treatments in place. I have confidence that the HD scientists will save them from suffering my father’s fate.

HD INSIGHTS: What areas of research seem most promising to the foundation?

FOX: We are very excited about the use of mutant huntingtin in plasma and cerebral spinal fluid as a biomarker. The fact that these technologies were not feasible just two years ago and now they are being integrated into clinical trials is impressive. It is incredibly exciting and points to the increasing rate of progress in HD research.

HD INSIGHTS: What would you say to the research community?

FOX: That I am profoundly grateful for their passion and commitment. For them, it’s not just a job and a career; it’s a life mission. There are very few people in this world like our HD scientists, who dedicate so much of their heart, soul, and mind to finding a cure that will impact multiple generations across the globe.
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<th>STUDY AGENT</th>
<th>PHASE</th>
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<td>Beijing Pins Medical Co., Ltd</td>
<td>NCT02263430</td>
<td>PINS Stimulator System</td>
<td>I</td>
<td>Jia Fumin, PhD 010-59361265 <a href="mailto:pins_medical@163.com">pins_medical@163.com</a></td>
<td>Randomized, double-blind, parallel-group, sham-controlled trial of Globus Pallidus Deep Brain Stimulation in HD</td>
<td>1 year</td>
<td>Beijing, China</td>
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<td>Azevan Pharmaceuticals</td>
<td>STAIR</td>
<td>SRX246</td>
<td>I/II</td>
<td>Neal Simon, PhD 610-419-1057 <a href="mailto:ngsimon@azevan.com">ngsimon@azevan.com</a></td>
<td>Randomized, placebo-controlled, double-blind, 12 week, 3-arm dose escalation study of SRX246 in individuals with irritability and early symptomatic HD</td>
<td>12 weeks</td>
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<td>Ionis Pharmaceuticals</td>
<td>NCT0342053</td>
<td>IONIS-HTTRx</td>
<td>I/II</td>
<td>Ionis Pharmaceuticals 800-679-4747 <a href="mailto:patients@ionisph.com">patients@ionisph.com</a></td>
<td>An open-label extension study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of IONIS-HTTRx for patients who participated in prior IONIS-HTTRx studies.</td>
<td>74 weeks</td>
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<td>Wave Life Sciences Ltd</td>
<td>PRECISION-HD1</td>
<td>WVE-120101</td>
<td>I/II</td>
<td>Clinical Operations 855-215-4687 <a href="mailto:clinicaltrials@wavelifesci.com">clinicaltrials@wavelifesci.com</a></td>
<td>Randomized, double-blind, placebo-controlled study to evaluate safety, tolerability, pharmacokinetics, and pharmacodynamics of WVE-120101 in adults with early manifest HD</td>
<td>2 years</td>
<td>Toronto, Ontario, Canada</td>
<td>Currently enrolling</td>
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<td>Wave Life Sciences Ltd</td>
<td>PRECISION-HD2</td>
<td>WVE-120102</td>
<td>I/II</td>
<td>Clinical Operations 855-215-4687 <a href="mailto:clinicaltrials@wavelifesci.com">clinicaltrials@wavelifesci.com</a></td>
<td>Randomized, double-blind, placebo-controlled study to evaluate safety, tolerability, pharmacokinetics, and pharmacodynamics of WVE-120102 in adults with early manifest HD</td>
<td>2 years</td>
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<tr>
<td>Azidus Brasil</td>
<td>NCT03252535</td>
<td>Cellavita HD</td>
<td>II</td>
<td>Joyce Macedo, PI +55(19)3829-6160 <a href="mailto:joyce.macedo@azidusbrasil.com.br">joyce.macedo@azidusbrasil.com.br</a></td>
<td>First in human, dose-escalation study to evaluate the safety of the stem-cell based therapy Cellavita HD in HD</td>
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<td>None listed</td>
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<td>Heinrich-Heine University</td>
<td>NCT02535884</td>
<td>ACTIVA® PC neuro-stimulator</td>
<td>II</td>
<td>Susanne Harnisch +49 6421 2866553 <a href="mailto:susanne.harnisch@kks.uni-marburg.de">susanne.harnisch@kks.uni-marburg.de</a></td>
<td>Randomized, double-blind, parallel-group, sham-controlled, multi-centre trial of Globus Pallidus Deep Brain Stimulation in individuals with HD</td>
<td>3 months</td>
<td>10 total - Germany, Austria, and Switzerland</td>
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<td>TRIHEP3</td>
<td>Triheptanoin oil</td>
<td>II</td>
<td>Fanny Mochel, MD, PhD <a href="mailto:fanny.mochel@upmc.fr">fanny.mochel@upmc.fr</a></td>
<td>Randomized, double-blind, controlled study of Triheptanoin oil, an anaplerotic therapy, in early manifest HD</td>
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<td>2 total - France and Netherlands</td>
<td>Currently enrolling</td>
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<td>Teva Pharmaceutical Industries</td>
<td>NCT02215616</td>
<td>Laquinimod</td>
<td>II</td>
<td>Sarah Boe, Teva 610-727-3486</td>
<td>Randomized, double-blind, placebo-controlled, parallel-group study evaluating efficacy and safety of Laquinimod (0.5 or 1.0 mg/day) in HD</td>
<td>12 months</td>
<td>52 total - worldwide</td>
<td>Active, not enrolling</td>
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<td>University of Auckland</td>
<td>VCAS-HD</td>
<td>Varenicline</td>
<td>II</td>
<td>Ailsa McGregor, PhD +64 3 479 4235 <a href="mailto:ailsa.mcgregor@otago.ac.nz">ailsa.mcgregor@otago.ac.nz</a></td>
<td>Randomized, double-blind, placebo-controlled trial of varenicline using the standard dosing regimen for smoking cessation in patients with HD</td>
<td>16 weeks</td>
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<td>Vaccinex Inc.</td>
<td>SIGNAL</td>
<td>VX15/2503</td>
<td>II</td>
<td>Andrew Feigin, MD, Huntington Study Group: 800-487-7671</td>
<td>Randomized, double-blind, placebo-controlled study to assess the safety, tolerability, pharmacokinetics, and efficacy of VX15/2503 in individuals with late prodromal and early manifest HD</td>
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<td>Hôpitaux de Paris</td>
<td>REVHD</td>
<td>Resveratrol</td>
<td>III</td>
<td>Fanny Mochel, MD, PhD <a href="mailto:fanny.mochel@upmc.fr">fanny.mochel@upmc.fr</a></td>
<td>Randomized, placebo-controlled study to evaluate the therapeutic potential of Resveratrol on caudate volume in HD patients, using volumetric MRI</td>
<td>1 year</td>
<td>1 total - France</td>
<td>Currently enrolling</td>
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</table>

To update or add a clinical trial, please e-mail HDInsights@hsglimited.org.
Sources: [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and [apps.who.int/trialsearch/](http://apps.who.int/trialsearch/)
**HD Therapeutic Pipeline**

**As of January 2018**

### Disease-modifying therapies

- **Cellavita HD** (Azidus Brasil)
- **SRX246** (Azevan)
- **Laquinimod** (Teva)
- **VX15/2503** (Vaccinex)

**Sources:** [www.clinicaltrials.gov](http://www.clinicaltrials.gov), HDSA's Therapies in Pipeline, and company/developer websites

To add or correct a therapy in development, please email [HDInsights@hsglimited.org](mailto:HDInsights@hsglimited.org).

### Neuroprotective compounds

- **Resveratrol**
- **Triheptanoin oil**

### Symptomatic treatments

- **Varenicline**
- **Pridopidine** (Teva)
- **Deutetabenazine** (Teva)
- **Tetraabenazine**

### Gene-targeting therapies

- **AAV-miRNA** (uniQure)
- **AAV-RNAi** (Voyager/Genzyme)
- **AAV-shRNA** (Spark Therapeutics)
- **WVE-120101** (WAVE Life Sciences)
- **WVE-120102** (WAVE Life Sciences)
- **IONIS-HTTRx** (Ionis)
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25TH ANNIVERSARY CELEBRATION

JOIN US

HSG 2018: UNLOCKING HD
NOV. 8-10, 2018
HOTEL ZAZA
HOUSTON MUSEUM DISTRICT
HOUSTON, TEXAS

- Celebrate HSG’s 25th anniversary
- Learn the latest in Huntington disease research and care
- Hear about new compounds and potential treatments
- Network, interact, and engage with the world leaders in HD
- Attend the 12th annual Peter Como HD Symposium

www.huntingtonstudygroup.org  |  +1 800-487-7671  |  info@hsglimited.org

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Dr. Amber Southwell, assistant professor at the University of Central Florida, discussed another potential ASO therapy. Her team’s ASO prevents the onset of HD in pre-symptomatic mice and enables post-symptomatic mice to recover their motor and psychiatric phenotypes.

Dr. Davina Hensman Moss, clinical research fellow at UCL Institute of Neurology, spoke about her research on genetic modifiers of HD, including genes whose locations on DNA may influence the age of onset of HD. She hopes this basic research will provide knowledge with which to develop drugs to treat the disease.

Toward the end of the meeting, journalist Charles Sabine brought the audience to tears when he showed his documentary video of his HDdennomore initiative in which he brought HD patients from Latin America to Rome to hear Pope Francis publicly acknowledge the disease.

It was a fitting way to end the conference—full of hope for a new future. Indeed, attendees left feeling inspired to continue their work and to implement the many new ideas that came to them throughout the course of the meeting.