Hereditary Neurological Disease Centre

Specializing in Huntington Disease

www.huntingtonsdiseaseclinic.com

SUMMER

Talk To Us... We Make It Simple...

Available 24 Hours a day!

TF	888.232.4632
Wichita	316.609.3020
Fax	316.609.3070
Email	hndcentre@aol.com

Clinic Schedule 2017

Clinic dates are on Saturday - persons interested in attending our out-patient clinic need to call and schedule an appointment in advance. There are no "standing appointments."

To schedule a clinic assessment or if you have questions about scheduling a clinic appointment please contact HNDC.

NEXT 2017 CLINIC DATES ARE AS FOLLOWS

October..... 21
November..... 18
December..... TBD

Inside This Issue:

Gerald "Jerry" L. Howell, DDS

MEMORIALS

Jerry loved to look at smiling faces and never let someone's smile pass him by, so his lifelong career as a dentist fit him perfectly. Jerry passed away May 1, 2017 surrounded by his wife and family. He received his DDS from Washington University in St. Louis, MO and was still actively practicing dentistry in Wichita. Jerry dealt firsthand with multiple patients with Huntington's disease, so when he joined the Board of Directors in 2011 he knew the complexities of HD and wanted to help make a difference. He served as Treasurer for the Hereditary Neurological Disease Centre since 2012.

Jerry loved to give back to his community. In addition to serving on the HNDC Board of Directors, he was an instructor at Wichita State University Department of Dental Hygiene. Jerry had many passions including piloting his own plane, golfing, hunting, woodworking, traveling with his wife and volunteering/donating to a myriad of charitable organizations. Jerry was also the consummate WSU Shocker fan. He lived life with a smile on his face, knew no stranger and his love incorporated many friends. His contributions to HNDC will be long lasting and he will be greatly missed by us all.

John T. Suter

One might say the Hereditary Neurological Disease Centre (HNDC) exists, in part, due to the extensive pro bono (Free) work of attorney John Suter. In March 1997, the Hereditary Neurological Disease Centre was incorporated and the journey to becoming a 501 (c)(3) organization began. Under the guiding eyes of John, the IRS designation forms were completed, bylaws written, and a myriad of other 'legal stuff' was pushed our way... which in turn was sent his way to decipher. With a calm, guiding hand he walked us through the process. "I've filled out the forms and marked where you need to sign, send it back to me and I'll take care of it", was John's regular response. He was 'behind the scenes' player, not wanting recognition for anything.

He was generous, as was his nature, to never say 'No' to this organization. Not only was he generous with his time and professional services – which many HD families utilized as a resource for help – he was a regular financial donor. If we had any sort of legal questions – be it for the organization or on behalf of an HD family – he would have time to discuss the issue at length, providing direction and assurance. John was a family man, with a wonderful wife, nine children and twenty grandchildren; he always made time to spend with his family. Not unlike his own family, John fostered a connection to those with HD, giving up a Saturday morning in September to join us at our 2016 HD Walk of Hope. Such a relationship is developed over time and certainly one that will be difficult to replace.

IONIS-HTT Rx Study in HD Patients Completes Enrollment

Mathew Shanley

Ionis Pharmaceuticals announced the enrollment completion in its Phase 1/2a randomized, placebo-controlled, dose escalation study of IONIS-HTTRs in Huntington's Disease (HD) patients.

IONIS-HTTRs is an antisense drug, and the first therapy designed to directly target the cause of the disease by reducing production of the responsible huntingtin protein to enter clinical development. It was granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) in January 2016.

HD is an often fatal, rare genetic neurological disease in which patients experience depreciation of both physical control and mental abilities. Symptoms typically start to show between the ages of 30 and 50, and worsen over a 10-to-25-year period until eventual death.

Dosing in the final patient cohort continues, and Ionis intends to report top-line results from the study in the final quarter of 2017.

"We are encouraged by the safety profile of IONIS-HTTRx we have observed to date in the completed dosing cohorts in the Phase 1/2a study," said C. Frank Bennett, Ph.D., senior vice president of research at Ionis Pharmaceuticals.

Upon completion and full analysis of this study, the next step for this program will be to conduct a study to investigate whether decreasing mutant huntingtin protein with IONIS-HTTRx can slow the progression of this terrible disease."

Per a press release, the safety and tolerability profile of IONIS-HTTRx in the concluded cohorts of the Phase 1/2a study backs its continued progression. Patients who finish the Phase 1/2a study will be eligible to participate in an open-label extension study that Ionis intends to start within the next year.

> "Together with our partners at Roche, we are committed to developing IONIS-HTTRX, which has the potential to transform the treatment of HD."
>
> *C. Frank Bennett, PhD.*

Roche, Ionis' partner for this drug, continues to advance and support the program, and has the option to license IONIS-HTT through the completion of the Phase 1/2a study.

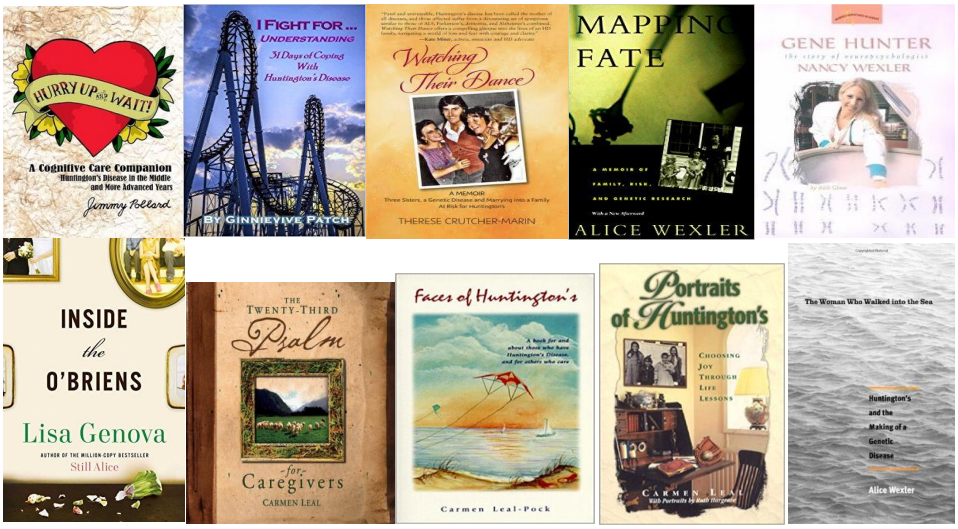
"We believe that IONIS-HTTRx, which is designed to reduce the production of all forms of the huntingtin (HTT) protein – the known cause of HD, represents the most promising opportunity to address this significant unmet medical need," said Bennett.

"Together with our partners at Roche, we are committed to developing IONIS-HTTRx, which has the potential to transform the treatment of HD."

For more updates on Huntington's Disease and this potential treatment option, be sure to follow Rare Disease Report on Facebook and Twitter.

Published Online: Thursday June 22, 2017

HD Reading Resources



RESEARCH 2017 HNDC

HDClarity -HDClarity is a planned multi-site cerebrospinal fluid (CSF) collection initiative to facilitate therapeutic development for Huntington's disease. It will consist of approximately 600 participants, across six clinical cohorts, with 30 site locations anticipated throughout Europe, North America and Australasia. The primary object of HDClarity is to generate high quality CSF sample collection for evaluation of biomarkers and pathways that will enable the development of novel treatments for HD. This is an observational study. Participant cohorts, 100 in number for each group who meet eligibility requirements, include: healthy controls, early pre-manifest, late pre-manifest, early manifest HD, moderate manifest HD and advanced manifest HD. Participants between the ages of 21-75 that meet inclusion criteria will be invited to participate in HDClarity.

ENROLL-HD - Enroll-HD is a worldwide clinical research platform and observational study for Huntington's disease that has three main goals:

- To better understand HD as it happens in people to give insight into developing new drugs
- To improve the design of clinical trials to rapidly provide clear outcomes - better, smarter, faster clinical trials will identify effective treatments as quickly as possible
- To improve clinical care for HD patients by identifying the best clinical practices across all Enroll-HD sites around the world and ensure that all families receive that standard of care

In support of these goals, Enroll-HD is helping coordinate and expedite experimental medicine studies and the development of biomarkers and clinical assessment tools for HD. Sites for this include North America, Latin America, Europe, Asia and Australia. Ancillary studies will be added on to the Enroll-HD study for additional research opportunities.

As of **August 1st** there are a total of **13,047 active participants** enrolled at **145 active sites** in **14 countries** around the world. HNDC is the top enrolling site in North America and in the top 5 in the WORLD.

Our Organization – Be an INFORMED supporter of the H.N.D.C

The Hereditary Neurological Disease Centre is a free-standing, non-profit organization. We often use short version- HNDC- and this has apparently brought some confusion with the national HD organization, Huntington's Disease Society of America (HDSA). We are not, and never have been, an affiliate of the national organization. We share a common goal to assist those with Huntington's Disease. Our direction, purpose, mission, and funding sources are dramatically different. ALL HNDC funds provide direct research and patient care programs for our regional area. ALL MONEY is used for these important program services; none is used for overhead or salaries. If you want to know where your dollars are used, please contact us and arrange an opportunity to learn more about WHERE your donations go, to WHOM they help directly in your area. PLEASE... Be an informed supporter and KNOW where your time, talent and donations are going.

Wave Life Sciences Initiates Two Phase 1b/2a Clinical Trials: PRECISION-HD1 and PRECISION-HD2 in Patients with Huntington's Disease

Wave Life Sciences Ltd. (WVE), a biotechnology company focused on delivering transformational therapies for patients with serious, genetically-defined diseases, today announced the initiation of the Company's PRECISION-HD program, which includes PRECISION-HD1 and PRECISION-HD2, the Company's two Phase 1b/2a clinical trials evaluating WVE-120101 and WVE-120102, respectively, for patients with Huntington's disease (HD).

"Wave's PRECISION-HD program is the first to target the underlying cause of Huntington's disease with an allele-specific approach," said Michael Panzara, MD, MPH, Neurology Franchise Lead of Wave Life Sciences. "Obtaining approvals to initiate these global studies as part of our first clinical program marks an important milestone for Wave. More importantly, these investigational compounds have the potential to address a critical unmet need for the HD patient community where no disease-modifying treatments are currently approved."

PRECISION-HD1 and PRECISION-HD2 are Phase 1b/2a multicenter, randomized, double-blind, placebo-controlled studies that will primarily evaluate the safety and tolerability of single and multiple doses of WVE-120101 and WVE-120102, respectively, administered intrathecally in HD patients. Additional exploratory objectives include assessing the impact that each compound has on the toxic mutant protein known to cause loss of brain cells in HD, as well as evaluating potential clinical effects and impact on brain atrophy as measured by magnetic resonance imaging (MRI). Both PRECISION-HD trials will follow the same protocol, and each will target a single nucleotide polymorphism, or "SNP," that marks a separate and distinct location on the mutant huntingtin (HTT) gene transcript. Wave intends to enroll approximately 50 patients globally in each of the two studies through multiple sites, in Canada initially, with Europe and the United States to follow.

The PRECISION-HD trials for WVE-120101 and WVE-120102 will include adult patients with early manifest HD who carry a SNP at the rs362307 ("SNP1") or the rs362331 ("SNP2") location, respectively. Potential HD patients for the PRECISION-HD program will be pre-screened for the presence of SNP1 or SNP2, and directed to the appropriate study upon qualifying for entry. Approximately two-thirds of all HD patients are expected to carry either SNP1, SNP2, or both, in association with the HD gene.

SNPs are a common type of genetic variation that normally occur in all humans, but may also act as biological markers to aid in locating genes associated with a particular disease. Previous HD research has identified multiple SNPs that are associated with the disease-causing expanded cytosine-adenine-guanine (CAG) repeat, which is an abnormality present in all HD patients that results in the production of mutant huntingtin protein, and causes HD. Therefore, Wave is utilizing common SNPs to precisely target the underlying cause of the disease.

"Reducing the disease-causing mutant huntingtin while preserving the healthy protein would be an important breakthrough for the HD community," said Dr. Edward Wild, Principal Researcher at University College London Huntington's Disease Centre, Consultant Neurologist at the National Hospital for Neurology and Neurosurgery, London, and member of the PRECISION-HD Clinical Advisory Committee. "The pre-clinical data for Wave's targeted compounds are encouraging and I am thrilled that we are beginning to explore the potential of these compounds in HD patients in this exciting programme

Teva and the Huntington Study Group Announce Publication of Data for AUSTEDO™ (deutetrabenazine) Tablets in Huntington Disease from ARC-HD Study in JAMA Neurology. Data support overnight conversion from tetrabenazine to AUSTEDO™ for the treatment of chorea associated with Huntington disease.

JERUSALEM--(BUSINESS WIRE)--Jul. 11, 2017—

Teva Pharmaceutical Industries Ltd. (NYSE and TASE:TEVA) and the Huntington Study Group today announced results from the Phase III open-label, single-arm switch cohort of the ARC-HD (Alternatives for Reducing Chorea in HD) study were published in JAMA Neurology. Results from the study showed patients with chorea associated with Huntington disease (HD) were able to safely switch overnight from three times daily tetrabenazine to twice daily AUSTEDO™ (deutetrabenazine) tablets. The ARC-HD study was conducted by Teva in partnership with the Huntington Study Group. A rare and fatal neurodegenerative disorder, HD affects approximately 35,000 people in the United States. Chorea – involuntary, random and sudden, twisting and/or writhing movements – is one of the most striking physical manifestations of this disease and occurs in approximately 90% of patients. HD may also manifest as cognitive deterioration and behavioral and/or psychological problems.

"Chorea is a debilitating symptom of Huntington disease that can impact the safety, function and quality of life of many patients," said Michael Hayden, M.D., Ph.D., President of Global R&D and Chief Scientific Officer at Teva. "We are pleased to share the ARC-HD study results to allow those physicians treating HD patients to increase their knowledge of AUSTEDO™."

"Human-based research is critical to evolving our understanding of HD and to providing more options and meaningful treatments," said Samuel Frank, M.D., principal investigator of the ARC-HD study and Director of the Huntington's Disease Society of America Center of Excellence at Beth Israel Deaconess Medical Center. "It could not be done without the dedication of patients and their families who participate in clinical trials, for which we are extremely grateful."

The ARC-HD study enrolled 37 patients who had been on a stable tetrabenazine regimen (≥8 weeks). Patients were converted from tetrabenazine to an initial AUSTEDO™ daily dose of approximately half the prior tetrabenazine daily dosage. One week after overnight conversion to AUSTEDO™, investigators could begin weekly dose adjustments, if needed, to achieve optimal chorea control. Changes from baseline in United Huntington's Disease Rating Scale (UHDRS), Total Maximal Chorea Score (TMC) and Total Motor Score (TMS) were evaluated as efficacy endpoints.

For more information regarding this JAMA Neurology publication, visit http://jamanetwork.com/journals/jamaneurology/fullarticle/2643174.

HD Community Events in Review

3rd Annual Cairo Walk of Hope
The Nebraska Community celebrated another successful walk on June 3, 2017

Cairo Walk

Claudia Hohnbaum, MA, RDN, LD

On May 9th Claudia Hohnbaum, MA, RDN, LD spoke to the Wichita HD support group. Claudia has been a volunteer member of the HNDC medical team since 1987. Claudia's professional roles have included that of a hospital dietitian, nursing home consultant and assistant director of a nonprofit for the nutrition education of kids.

Dr. Mallonee

William Mallonee, MD, spoke to the Wichita HD support group on August 8th. Dr. Mallonee is a Board Certified Neurologist in private practice at the Hutchinson Clinic. Dr. Mallonee has over 30 years of experience with Huntington Disease and other neurological diseases. He is also the volunteer medical director for Hereditary Neurological Disease Centre and volunteers at our monthly HD clinic.

Waiting on Raffle

Fun Walkers

Lawrence Ks. 2nd Annual Walk of Hope was held at Holcom Park on April 29th, 2017
It was a cold and rainy morning, but that didn't stop the Lawrence community from being "Champions for HD!" It was warm and cozy in the Holcom Park Recreation Center where our dedicated walkers completed the mile walk inside the gym then gathered around for the raffle and silent auction.

Up Coming Events

HUTCHINSON KS

4th annual Walk of Hope
When: October 14th @ 9:00am
Where: Carey Park Homebuilders Shelter
6 Emerson Loop

CUNNINGHAM KS

Inaugural Walk of Hope:
When: October 28th @ 8:30am
Where: Hilltop Manor
403 S. Valley St.

LUBBOCK TX

3rd annual Walk of Hope
When: November 4th @ 8:30am
Where: Hillside Christian Church
6202 Milwaukee Ave.

FORT SMITH AR

17th annual Walk of Hope
When: November 11th @ 8:30am
Where: Creekmore Park
3301 S. M Street

I AM A CHAMPION FOR HD

CHAM▪PI▪ON - A Person who supports or stands up for a cause or on behalf of someone else.

Hereditary Neurological Disease Centre Support Groups

Wichita Area

The Wichita Area Support Group meets the 2nd Tuesday of every month at the Abay Medical Plaza Auditorium.

The 2017 Wichita Area Support Group is listed below:

September 12, 2017 - Regular Support Group - 7:00pm

***October 10, 2017 -** *(Speaker – Estate Planning/Legal)* - 7:00pm

November 14, 2017 - Regular Support Group - 7:00pm

December 2017 - Happy Holidays No Support Group

*Dinner is provided at all Support Groups with a Guest Speaker. Dinner is provided starting at 6pm and the featured speaker starts at 6:30pm. There is NO CHARGE and the support group is OPEN to ANYONE who has interest in attending. **Please call 316-609-3020 or email norberta@hndcentre.com at least 72 hours prior if you have interest in attending for food planning purposes.**

Support Group meets at the Abay Medical Plaza Auditorium, located at 3223 N. Webb Rd. Wichita Ks.

Lawrence Area

Huntington's Disease Support Group
Meets the 3rd Tuesday of every month at
Lawrence Memorial Hospital
330 Arkansas entrance
Conference Rm D South
For more information please contact HNDC at 888-232-4632

The Fort Smith Area

Huntington's Disease Support Group
Meets the third Thursday of each month at 6 p.m. at the **Fort Smith Public Library**

3201 Rogers Avenue
2nd Floor in the Davis Room
Fort Smith, Arkansas
For more information,

Sandee Farley 479-462-2734
Pat Valley 479-785-5209

Call 1-888-232-4632

For updated meetings or Additional meeting information.

Find us on: facebook

To learn more about our site & our services

Hereditary Neurological Disease Centre Specializing in Huntington Disease

3223 N. Webb Road, Suite 4
Wichita, Kansas 67226

Like us on Facebook @ Hereditary Neurological Disease Centre

www.huntingtonsdiseaseclinic.com

Memorials

Memorial contributions in support of the mission of the Hereditary Neurological Disease Centre are important and can be a lasting tribute year after year. Our condolences go out to the Family, Friends, and Loved Ones.

We want to thank those families that have established Memorials, as well as all those that contributed to the Memorial Fund. Unless otherwise restricted, Memorial gifts are directed to the Huntington's Disease Resource Fund.

*** Nan Morris * Gerald Howell * Nicola Beard ***
*** John Suter * Mary Reimers * Rodney Wilson ***

Genetic Testing Services

We continue to offer genetic testing at our cost for laboratory fee only.
Call 1-888-232-4632 for further details or to schedule an appointment.

This Newsletter is printed by the HNDC, 3223 N. Webb Rd, Suite 4, Wichita, KS 67226, in support of its mission to provide diagnosis, disease management, and support services. Should you have items of interest to contribute, questions, comments, or would like to volunteer to help make a difference, contact the HNDC office.