www.huntingtonsdiseaseclinic.com

Hereditary Neurological Disease Centre

Specializing in Huntington Disease

SPRING 2018

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

huntingtonsdiseaseclinic.com

Clinic Schedule 2018

HNDC clinic is composed of a volunteer medical team including Neurology, Speech Therapy, Dietary, Physical Therapy and Social Work. There are no "standing appointments."

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

NEXT 2018 CLINIC DATES ARE AS FOLLOWS

June	9
July	No Clinic
August	18
September	15
October	20
November	17
December	No Clinic

Inside This Issue:

MAY WAS HD AWARENESS MONTH

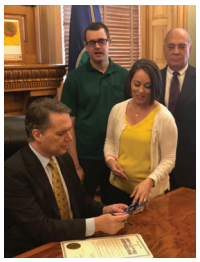
Dear Friends,

May is Huntington disease (HD) awareness month. We mark this time of year with special events to promote awareness of HD, be it through activities in support groups, special events, focused television programming, or as was the case last month, meeting with Governor Jeff Colyer, MD, to sign a Proclamation "May is HD Awareness Month" in Kansas. Here are some of our highlights to promote awareness:

- On April 23rd, a group of us met with Kansas Governor, Jeff Colyer, MD, for Proclamation signing at the Capitol.
- On April 28th, the Fourth Annual Lubbock, TX, Walk of Hope was held and on May 5th, the Third Annual Walk of Hope in Lawrence, KS. Both generate funds in support of local HD family members and research for HD – Walk Local... Support Local"
- On June 2nd, the Fourth Annual Walk of Hope – Cairo, NE, will be held. If you are in or near the Cairo/Grand Island, NE vicinity, please plan on joining us for this special event to benefit local HD family members and generate HD research dollars.
- Quietly, but certainly worthy of mention, the 25th Anniversary of the finding of the gene responsible for Huntington disease (1993) has come and gone...our first appointment for direct gene testing was March 14, 1994. The genetic test for HD has forever changed our understanding of genetics and is an important resource for young people at risk for HD...we are now testing individuals who only have known a time that a test for the HD gene exists...and are seeking that information at an early age in growing numbers. We have assisted over 2000 people ascertain their HD genetic information.
- Each year we send out a mailing to promote May as HD Awareness Month and to ask for your continued support of our clinical and research programs. All of us should continue to learn as much about HD as possible, to share it with others, and to step up to the challenge of taking part in a CURE. Enroll-HD, the global clinical trial database, as of April 1, 2018, has a total of 15,201 active participants enrolled at 153 active sites in 16 countries around the world (we are the fourth largest enrolling site in the world; largest site in North and South America!)... however, we need up to 10,000 more participants. Your support of $25, $50, $100, $250 or more IS important. Your participation IS important. Much work remains to be done before we can declare the defeat of HD.

With Sincere Appreciation,

Gregory Suter
Executive Director



Press Release: IONIS Pharmaceuticals

New Data from IONIS-HTT Rx Phase 1/2 Study Demonstrates Correlation Between Reduction of Disease-causing Protein and Improvement in Clinical Measures of Huntington's Disease

First drug to demonstrate lowering of mutant huntingtin, the disease-causing protein, in people with Huntington's disease

CARLSBAD, Calif., April 24, 2018/ PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), the leader in antisense therapeutics, today presented top-line data from the Phase 1/2 study of IONIS-HTTRx (RG6042) in people with early stage Huntington's disease (HD) at the 70thAmerican Academy of Neurology (AAN) meeting in Los Angeles, California. Results from exploratory analyses of data from the study demonstrated correlations between reductions in mutant huntingtin (mHTT), the disease-causing protein, and improvements in clinical measures of Huntington's disease.

HD is a rare, progressive, neurodegenerative disease caused by genetic mutation in the huntingtin gene, resulting in the production of mHTT protein, which gradually destroys neurons in the brain and results in deterioration in mental ability and physical control. Ionis designed IONIS-HTTRx (RG6042), a Generation 2+ antisense drug, to specifically reduce the production of the huntingtin protein, including mHTT.

"Since the discovery of the gene that causes Huntington's disease 25 years ago, we've been working to discover a drug that targets the cause of the disease—the mutant huntingtin protein. With the results from the Phase 1/2 study with IONIS-HTTRx, we have cleared the first major hurdle in developing such a drug. The substantial lowering of the mutant huntingtin protein, combined with additional data from exploratory clinical measures presented today and the good safety profile we observed in the study, give us hope that this new drug may have the potential to slow, or perhaps halt, the progression of this devastating disease," said Dr. Sarah Tabrizi, professor of clinical neurology, director of the University College London's Huntington's Disease Centre and the global lead investigator on the study. "The next step is to advance the drug into a larger study designed to demonstrate the potential clinical benefit of reducing the toxic mutant huntingtin protein in people with Huntington's disease."

Phase 1/2 Study Results:

- Significant, dose-dependent reductions in mHTT were observed in CSF of treated participants with mHTT reductions of up to approximately 60% and mean reductions of approximately 40% in CSF observed at the two highest doses, 90 mg ($p<0.01$) and 120 mg ($p<0.01$).
- A 40% to 60% reduction in CSF corresponds to an estimated 55% to 85% reduction in mHTT in the cortex and 20% to 50% in the caudate regions of the brain in humans, based on a predictive model developed from data collected in rodents and non-human primates.
- mHTT levels were continuing to

decline at the last measurement time in the study with further decreases in mHTT anticipated; based on modelling and clinical results, maximum reduction predicted at approximately six months after first dose.

- No serious adverse events were reported in treated participants and most adverse events (AEs) were mild and considered unrelated to study drug. No participants discontinued from the study.

Exploratory Clinical Outcome Results:

- In an exploratory post-hoc analysis, the degree of mHTT lowering was correlated with improved scores at three months in several clinical measures commonly used in Huntington's disease clinical studies.
- Total Motor Score (TMS): rho=0.39 (p=0.007)
- Symbol Digit Modalities Test (SDMT): rho=-0.30 (p=0.044)
- Stroop Word Reading Test (SWRT): rho=0.08 (p=0.60)
- Total Functional Capacity (TFC) score: rho=-0.27 (p=0.066)
- In addition, a significant correlation was observed with the degree of mHTT lowering and the Composite Unified Huntington's Disease Rating Scale (cUHDRS) score at Day 85 (rho=-0.41, p=0.004).

"These important clinical results further demonstrate that targeting the reduction of the toxic mutant huntingtin protein with IONIS-HTTRx has the potential to be disease-modifying," added Dr. C. Frank Bennett, senior vice president of research and franchise leader for the neurological programs at Ionis Pharmaceuticals. "Following SPINRAZA for the treatment of patients with spinal muscular atrophy, this is our second antisense drug to show good target engagement in the CNS. These drugs, along with the two others we have in clinical studies and the five we have in preclinical development further validate the broad potential of our antisense technology to treat patients with neurological diseases."

About the Phase 1/2 Study

The study was a randomized, placebo-controlled dose escalation study in 46 people with early stage Huntington's disease. Study participants were treated for 13 weeks with four intrathecal injections of 10 mg, 30 mg, 60 mg, 90 mg or 120 mg of IONIS-HTTRx (RG6042) or placebo (3:1 active to placebo), administered monthly. The study's primary objective was to evaluate the safety and tolerability of IONIS-HTTRx (RG6042). The study was also designed to measure the effect of IONIS-HTTRx (RG6042) on levels of the mutant huntingtin protein in the cerebral spinal fluid (CSF). Exploratory analyses included several clinical measures commonly used in Huntington's disease studies.

An open-label extension (OLE) study for patients who participated in the Phase 1/2 study is ongoing.

Ionis' partner, Roche, exercised its option to license IONIS-HTTRx following the completion of a Phase 1/2 study and is responsible for all development and commercial activities. Planning is already underway for Roche to advance IONIS-HTTRx (RG6042) to a pivotal study to demonstrate the clinical efficacy and safety of IONIS-HTTRx.

About Huntington's Disease (HD)

Huntington's Disease (HD) is a rare, genetic, progressive, neurodegenerative disease resulting in deterioration in mental abilities and physical control. In the U.S., there are approximately 30,000 individuals (one in 10,000) with symptomatic HD and more than 200,000 people at risk of having inherited HD. HD is referred to as a triplet repeat disorder and is one of a large family of genetic diseases in which certain gene sequences are mistakenly repeated. In HD, the trinucleotide sequence in the gene that encodes for the HTT protein is repeated more than 36 times. The resulting mHTT protein is toxic and gradually damages neurons in the brain. Symptoms of HD usually appear between the ages of 30 to 50 years and continually worsen over a 15- to 20-year period. Ultimately, the weakened individual succumbs to pneumonia, heart failure or other complications. Presently, there is no effective disease-modifying treatment for HD, and current products focus only on managing disease symptoms.

About IONIS-HTTRx (RG6042)

IONIS-HTTRx (RG6042) is an antisense drug designed to reduce the production of all forms of the huntingtin protein (HTT), including its mutated variant, mHTT, which is the driver of HD. IONIS-HTTRx (RG6042) offers a unique approach to treat all patients with HD, irrespective of their individual HTT mutation. IONIS-HTTRx (RG6042) has been granted orphan drug designation by the U.S. Food and Drug Administration (FDA) and by the European Medicines Agency (EMA) for the treatment of patients with HD.

HNDC Kicked off the 2018 Walk of Hope with the
Inaugural Glendale AZ Walk of Hope – March 3,
4th Annual Lubbock TX Walk of Hope – April 28
3rd Annual Lawrence Walk of Hope – May 5

Walk Local ~ Support Local!

All of the patients seen at Hereditary Neurological Disease Centre receive beneficial services at little or no cost to them or their families. ALL funds raised support research services and help with local HD community.

Many thanks to all of our sponsors, donors and families that attend our walks!!

Up Coming Events

FORT SMITH AR

Annual Walk of Hope:

When: November 10th @ 9am

Where: Creekmore Park

3301 S. M Street

CAIRO NE

Annual Walk of Hope:

When: June 2nd @ 9am

Where: Cairo Community Centre

305 Said Street

HUTCHINSON KS

Annual Walk of Hope:

When: September 22nd @ 9am

Where: Abay Medical Plaza

3223 N. Webb RD

WICHITA KS

NY Brain Bank Donation

In keeping with our pledge to Walk of Hope planners and participants, a portion of funds generated from each of our Walk of Hope events is directed to HD research. Each separate Walk of Hope community designates what proportion of Walk of Hope proceeds is sent for research dissemination through the Hereditary Neurological Disease Centre (HNDC).

On February 8th, 2018, HNDC Executive Director Gregory Suter presented a check to JeanPaul G. VonSattel, MD, of the New York Brain Bank. The check, representing 2017 Walk of Hope research fundraising, brings HNDC donated funds to assist in this critical research facility to over $110,000.00.

"Our long-standing collaboration, in addition to your precious financial support, are essential for the NYBB to continue being able to provide basic scientists with optimally prepared HD and control samples", reports Dr Vonsattel. Further, he states, "If the NYBB is able to fulfill such complex tasks so promptly, it is thanks to the collaboration the NYBB has with the Hereditary Neurological Disease Centre and the financial support the Centre keeps providing."

PHOTO: Gregory Suter, Etty Cortés, M.D., JohnPaul G VonSattel, MD

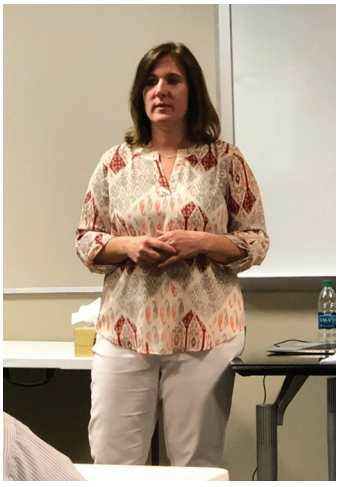
HD Community Events in Review

Serenity Hospice

On February 13, Serenity Hospice was our first guest speaker at the Wichita Area Support Group. Members learned about Advance Care Directives, What is Hospice and How to Access Hospice.

Juliet Gay

Wichita Area Support Group hosted speaker, Juliet Gay, a physical therapist from Norman, OK. Juliet educated the group on the importance of exercise with maintaining function and independence as well as good form when lifting or assisting with transfer of a person.



Up Coming Wichita Area Support Group Speakers

Jeremy Tan, MD

Dr. Tan is a graduate of the University of Oklahoma Medical School. He is married with 4 children. Dr. Tan is a psychiatrist at the Horizon Mental Health Center in Hutchinson, KS. Dr. Tan has working knowledge with Huntington Disease and has been on the HNDC medical team since 2008, assisting with HD clinical trials and general advice.

Julie Scherz, PhD, leads a very full professional life and is a volunteer Speech-Language Pathologist at the Hereditary Neurological Disease Centre. She currently serves as an Associate Professor and holds the position of Chair of the Communication Disorders and Sciences Department at Wichita State University (WSU). In addition, she is the NCAA Faculty Athletic Representatice, the liaison between academics and athletics, appointed by the WSU President. For the past 10 years, she has also served as an accreditation team site visitor for the American Speech-Language-Hearing Association.

Julie Scherz, PhD

Wichita Area

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

The 2018 Wichita Area Support Group is listed below:

January 9th - Regular Support Group - 7:00pm
***February 13th -** *(Speakers - Serenity Hospice Care)* - 6:00pm
March 13th - Regular Support Group - 7:00pm
***April 10th -** *(Juliet Gay - Physical Therapist)* - 6:00pm
May 8th - Regular Support Group - 7:00pm
***June 12th -** *(Speaker - Jeremy Tan, MD - Psychiatrist)* - 6:00pm
July - Summer Break No Group
***August 14th -** *(Julie Scherz, PhD - Speech Language Pathologist)* - 6:00pm
September 11th - Regular Support Group - 7:00pm
***October 9th -** *(Paul Good - Attorney)* - 6:00pm
November 13th - Regular Support Group - 7:00pm
December - Christmas Break No 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.

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 the 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.

Hereditary Neurological Disease Centre *Specializing in Huntington Disease*

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

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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.

∗ Barbara Faurot ∗ Tom Noonan ∗ Ronald Cox ∗ James Black ∗
∗ Philip Hammon ∗ Maeola McQueen ∗ Brian Schwarz ∗

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.