

HDSA Minnesota Chapter Highlights

SPRING 2018

Groundbreaking Drug Provides Hope for HD

This is an excerpt from HDSA's 2017 Research Report. For the full article, visit hdsa.org

Since late 2015, the very first therapy aimed to combat the underlying genetics of Huntington's disease has been undergoing a safety and tolerability trial in around 40 adults with early-stage HD. The trial is testing a huntingtin-lowering therapy known as IONIS-HTTRx. On December 11th, 2017, Ionis announced that the completed Phase I/2a study met its safety and tolerability endpoints and successfully lowered levels of mutant huntingtin protein. While the entire HD community is excited about this study, we must remember that its sole purpose was to evaluate the safety of Ionis-HTTRx in HD patients. A Phase III study is planned next to evaluate the effectiveness of the drug to improve HD symptoms.

FAQs about the Ionis Huntingtin-lowering trial

What is Ionis-HTTRx? IONIS-HTTRx is an investigational drug being developed for the potential treatment of HD. IONIS-HTTRx offers a unique mechanism to moderate the underlying genetic cause of HD by decreasing the production of the toxic huntingtin protein. IONIS-HTTRx is an antisense drug designed to reduce the amount of huntingtin RNA in the brain, and with less RNA "message" available, less huntingtin protein is made. IONIS-HTTRx is designed to reduce the production of all forms of the huntingtin (HTT) protein, which in its mutated variant (mHTT) is responsible for HD. As such, IONIS-HTTRx offers a unique approach to treat people with Huntington's disease, irrespective of their individual HTT mutation.

What was the Phase I/2a trial designed to do? The Phase I/2a study was a randomized placebo-controlled Phase I/2a clinical study to evaluate the safety and tolerability of increasing doses of IONIS-HTTRx in people with early stage Huntington's disease. Phase I/2a study participants who are eligible for the open-label extension (OLE) study will have the opportunity to continue on drug in this trial.

How was the study performed? Each patient received multiple injections of a specific dosage of the drug, with different groups of patients receiving placebo and a range of doses. The treatment was administered as an intrathecal injection, commonly called a lumbar puncture or

"spinal tap." The drug is injected into the lower back, enters the cerebrospinal fluid that bathes the nervous system, and reaches the brain. None of the patients or physicians participating in the study knew who was receiving active drug or placebo, known as a double-blind design.

Who participated in this phase of the study? The study enrolled approximately 40 patients with early manifest, Stage I HD (defined as Total Functional Capacity of 11-13), aged 25 to 65 years. Participants were enrolled at six centers in Canada, the United Kingdom and Germany only. All patients had genetically confirmed HD by direct DNA testing, passed additional eligibility screening, and had informed consent. Participation in the Ionis-HTTRx study lasted for about 8 months.

What are the plans for further clinical development? The next step for this program will be to conduct a safety and efficacy study to investigate if decreasing mutant huntingtin protein with IONIS-HTTRx can benefit people with Huntington's disease. Future studies for the program will be conducted globally, including the U.S. Roche will announce details about studies, including eligibility criteria and planned start dates, as this information becomes available. All relevant information on upcoming studies will also be posted on HDTrialFinder.org and ClinicalTrials.gov.

*Help for Today.
Hope for Tomorrow.*



HDSA is a national, voluntary health organization dedicated to improving the lives of people with Huntington's disease and their families.

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**Interested in becoming a
volunteer?**

Contact Brianna Wallen Lindell at
(952) 334-2211 or
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Long-Term Care Options for Huntington's Families



Jessica Marsolek, LGSW

Did you know that in Minnesota we have the most care options of any location in the world for those who are living with Huntington's Disease?

As a social worker, this makes my job much easier. I hear from social workers around the country, struggling to find places for their clients to receive quality care. Providing care for someone affected by HD at the mid to end of life

is a very important job. It doesn't come without its challenges, but is very crucial that they are in a safe, understanding and compassionate care environment.

Those living with HD tend to be rougher on equipment and furniture, and negative behaviors can be very challenging if misunderstood. We are very thankful that we have a variety of group homes and nursing home units that have dedicated their care to those affected by HD. These may or may not be an appropriate setting for you or your loved one, but it is always nice to know what options may be out there before you need them.

Dedicated care partners do their very best to keep their loved one at home as long as they can, but there may be a point in your own health, or your loved one's functioning that causes "Your Plan" to change. Let me know how I can be of service to you and your family in making these tough decisions. It is always better to discuss options and "what-ifs." It is recommended to not promise that someone with HD will never have to go to a care setting. Keeping your loved one at home may be the goal, but cannot always be guaranteed. With changing psychological symptoms and safety issues,

this promise may not be able to be honored, so keeping the possibility open is recommended when talking about long term planning with your loved one living with HD.

How do I know if I or my loved one is ready to move into a care setting?

This is not an easy question to answer, nor is it one size fits all. Questions I recommend asking yourself are:

- Can I physically provide the care that is required?
- Is my loved one safe alone?
- Is my home safe for further accessibility needs (stairs, bathtub, and toilet)?
- Is there a back-up person if I am not able to care for my loved one—in case of emergency or for whatever reason (you are in hospital, or something outside of your control happens)?

If you know of a care provider that would like to learn more about HD and how to improve care for those with HD, please let me know, so I can coordinate an in-service or consultation.

I am always looking for great medical, dental, or therapist care you may be receiving. HD-informed care is key to greater understanding and I want to hear about who is doing a great job serving those in our community!

Jessica Marsolek, LGSW 612-371-0904, jmarsolek@hdsa.org

Support Groups

Support group meetings are for people who are at risk for HD, persons with HD, and families of people with HD. For more information, including how to start a support group in your area, contact Jessica Marsolek, LGSW, at (612) 371-0904 or jmarsolek@hdsa.org.

Richfield, MN

Oak Grove Lutheran Church
7045 Lyndale Ave. S.

Every third Saturday, 10:30 a.m.-12 p.m.

Facilitators: Jessica Marsolek, Nina Ross

Rochester, MN

Bethany United Methodist
1835 19th Ave. NW

Every third Saturday, 3 p.m.

Facilitator: Jessica Marsolek

RSVP required to Jessica at

(612) 371-0904 or jmarsolek@hdsa.org

Upcoming Events

For event updates, visit www.minnesota.hdsa.org or
www.facebook.com/hdsamnchapter

Fun Outta the Sun Bowl-a-Thon

Saturday, April 7, 12-2pm | Park Tavern Bowling, 3401 Louisiana Ave., St. Louis Park

HDSA National Convention

June 7-9 | Los Angeles, CA

For more information or scholarship opportunities, visit hdsa.org/annual-convention

Twin Cities Team Hope Walk & 5K

Saturday, August 4, 8:30am | Purgatory Creek Park, 13001 Technology Dr., Eden Prairie

Duluth Team Hope Walk

Saturday, August 25, 8:30am | Endion Station Public House, 200 Lake Pl. Dr., Duluth

HDSA Motorcycle Ride for Hope

Saturday, September 22, 9:30am | Twin Cities Harley Davidson, 1355 98th Ave. NE, Blaine | After party to follow at Route 65 in East Bethel

Education Day 2018

Saturday, October 6, 12-4pm | Calvary Lutheran Church, 7520 Golden Valley Road, Golden Valley | HD social hour to follow (Location TBD)

In Memoriam



Mary Barlow
Art Dunlap
John Wilkey