

HDSA Minnesota Chapter Highlights

FALL 2017

Update on Gene-based Therapies



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There is nothing more exciting in HD research than the possibility of therapies directed against the gene itself. The gene-silencing treatment in an HD patient was administered in September 2015, and Ionis, the sponsor of this groundbreaking study, announced earlier this summer that the project has completed enrollment, and initial results of the trial should be available to the public within a year!

That is exciting news.

Two other companies are also developing gene silencing therapies. Wave Life Sciences has developed a different kind of gene silencing treatment, which should more specifically target just the abnormal HD gene, without also (potentially) silencing the normal gene, than the Ionis product. But the downside is that the Wave product(s) will not work for everyone. They have designed two molecules, which target the two genetic “markers” that are most commonly seen close to abnormal HD genes, but not commonly seen close to normal HD genes. The Wave molecule “recognizes” that marker next to the abnormal and binds to it, blocking the cell’s protein-making machinery from having access to the abnormal genetic recipe. Although many people with HD test positive for one of the two genetic markers that the Wave product targets, not all of them do.

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UniQure, a European-based company, is developing a modified virus to be injected directly into the brain cells, which will “infect” the cells with a genetic molecule that blocks the abnormal HD gene. Two other companies, Spark Therapeutics, and Voyager Therapeutics, are working on similar kinds of treatments.

Another gene-based therapy that is still in its infancy, is gene editing, using an enzyme called Crispr Cas9. You may have read about Crispr in the popular media, because there is a lot of debate about the possibility that doctors and patients will (want to) use it to create “designer babies.” But a more immediate purpose for Crispr is to “cut out” or “edit” gene mutations that cause diseases—like HD.

The work on Crispr is still in its early stages, not ready for human trials yet. But it is clear that a new era in HD research is upon us, one that should give the HD community great hope. We need to start thinking now about some of the ethical and logistical challenges that may come with these new treatments. What if the treatment requires brain surgery (the Ionis product is delivered by monthly spinal tap infusions) or other invasive procedures? What if there are risky side effects? What if the treatment is very expensive? What if you decide to participate in a research study and it doesn’t work? What if the treatment works for some people but not others? I encourage everyone to learn everything you can about current and future treatments for HD, so that we are prepared to use them in a safe and ethical manner.

*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
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Stay Independent Longer While Living With HD



Jessica Marsolek, LGSW

When meeting with families, I often get asked questions regarding how one can stay independent as long as possible while living with HD and about care options for later stages of HD. Below, please see suggestions that I have seen work wonders for others, and are worth the share...

1) Keep your circle of family and friends close and maintain those relationships. They may be your biggest support now and/or later. There are so many ways that friends and family can support someone living with HD and their care partner: Offering to help with meals, spend time with the person who has HD while their care partner takes a few minutes to themselves, help give rides to appointments, and just being available to listen, when venting is necessary. When they offer, say "Yes" or be open to accepting help. Those who offer want to help. They may not know exactly what you need—let them!

2) Every support system is comprised of different dynamics, and the more support someone living with HD has, the more likely they will be able to live in a more independent setting longer. Consider allowing help in the home earlier than later; it will be easier to add supports as needed down the road. Some individuals I work with will begin with a friend or

hired PCA (Personal Care Attendant) or Companion services to help with light housekeeping, meal preparation, laundry and then add to these as needs increase.

3) Request Long-Term Care Consultation (LTCC) Services from your local county human services agency. The county's goal is to keep those who need care out of Long-Term care facilities and in community/home settings as long as possible. Because that is the ultimate goal, they will offer supports and resources available to you through a meeting such as a Long-Term Care Consultation. When you call your local county, request this consultation and they will send a public health nurse or social worker out to your home within a week or so, ask about what areas of daily living the individual living with HD needs assistance with (even if it is once, or once in awhile, it is important to be honest) and then provide information about resources that may be available to you in your area that will assist you in your goal of remaining as independent in your home as possible. This can be done more than once, so don't hesitate to call and request this helpful and resourceful service.

4) Accept help when it comes your way. To connect with other families that are living with HD, consider attending a support group or online group. HD is too hard to go through alone, so don't make life harder than it needs to be. Be kind to yourself and allow others to give, to contribute to you.

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. More details coming soon.

Spring 2018

Fun Outta the Sun Bowl-a-Thon
Be The Hope Reception: A Celebration of Hope

Summer 2018

Wade Wallen Memorial Golf Tournament
Twin Cities Team Hope Walk & 5k
Duluth Team Hope Walk—Saturday, Aug. 25,
8:30am | Endion Station Public House

Fall 2018

Education Day

In Memoriam

Richard "Rick" Barlow

Tom Carillo

Patti Goodchild

Charlie Jennings

Marilee Lockwood

Wade Wallen

Richard (Rich) Wiechman

