We Are HDSA!

Huntington's Disease Society of America

Support Group Newsletter • www.hdsa.org • (800) 345-HDSA • Issue 1, April/May 2014

RESEARCH in 2014

The first issue of *We Are HDSA!* in 2014 is dedicated to research. Inside, you will read about two exciting new studies happening around the country, Enroll-HD and First/ARC. These studies offer families an opportunity to help push forward the science of HD so we can eventually find an effective treatment and a cure.

There are many ways one can stay updated on HD research. The HDSA website, **www.hdsa.org/research**, provides information about current and upcoming trials and how drugs go through the trial process. The research webinar series provides information by the experts on what people with HD, family members, and professionals should expect to see in the upcoming years. Webinars are archived on **hdsa.org**.

HDTrials.org is a free, confidential online database where a person can sign up to receive e-mail updates when new clinical and observational trials are recruiting in their area. All one has to provide is an e-mail address and a zip code.

HDBuzz (**www.hdbuzz.net**) is another resource. This website, started by Drs. Ed Wild and Jeff Carroll, aims at helping people understand the research that is currently being undertaken around the world. You can sign up on **hdbuzz.net** to receive e-mail updates or follow HDBuzz on twitter at **@HDBuzzFeed**.

Most importantly, join the HDSA e-mail list, found at **www.hdsa.org** under the "Sign Up" button and stay connected to all the HDSA events, activities, and late breaking news. You can join the HDSA National Mailing list by reaching out to Anita Markpaul at **amarkpaul@hdsa.org** or via phone at **(800) 345-HDSA, ext. 219**.

Upcoming Research Webinars:

Topic: Reach2HD Clinical Trial Results

Presenter: Ray Dorsey, MD (University of Rochester)

Date: April 21, 2014

Time: 12-1pm EST

Topic: Progress in HD Animal Model Development

Presenter: David Howland, PhD (CHDI Foundation)

Date: May 22, 2014

Time: 12-1pm EST

To register for these and other research webinars, visit **www.hdsa.org/research** and click on **"Research Webinar Series."**

HD Human Biology Project- HDSA Research Fellowship

Huntington's Disease Society of America (HDSA) has adopted a patient-centric research strategy to facilitate the critical HD research that must be done to push the field closer to meeting our goal of identifying effective therapies to slow the progression or onset of HD. To do so, HDSA has built upon the cornerstone of its care network – the HDSA Centers of Excellence. As the models of exemplary and comprehensive HD clinical care in the United States, these Centers are unique to the HD community. They are valuable resources to HD families where patients are provided with a multidisciplinary approach to HD care and treatment.

The HD Human Biology Project was launched in 2013 with the goal of fostering innovative research at the HDSA Centers of Excellence with the goal of better understanding the biology of Huntington's disease as it occurs in humans. This year we reaffirm this goal. This grant is a one or two year grant mechanism to provide support for young scientists to work collaboratively with HDSA Centers of Excellence and their mentors. For more information, please visit **www.hdsa.org/research** or contact George Yohrling, PhD, at **gyohrling@hdsa.org**.

HDSA Information

Learn about applying for support group guest speakers:

The HDSA Educational Grant can be used to fund speaker travel and accommodations. For groups inviting a local speaker, funds for modest refreshments are also available. Please contact Jane Kogan at **jkogan@hdsa.org** for more information about educational grants.

Upcoming HDSA Educational Events

April 6	Long Island, NY
April 12	Providence, RI Sioux Falls, SD
April 26	Waukesha, WI
May 3	Grapevine, TX Des Moines, IA Sacramento, CA Salt Lake City, UT
May 24	Prescott, AZ

Enroll-HD: A Global HD Research Platform to Accelerate HD Drug Development

By: George Yohrling, PhD, Director of Medical and Scientific Affairs, HDSA and Simon Noble, PhD, Director, Scientific Communications, CHDI Management/CHDI Foundation

The Huntington's Disease Society of America (HDSA) has announced its endorsement of Enroll-HD, a worldwide research platform for Huntington's disease families. HDSA believes that Enroll-HD is a new breed of patient registry, with the highest levels of management, patient protection and commitment to sharing its data with scientists in order to understand HD better, test potential drugs more quickly and ensure that we are providing the highest quality of care for HD families.

To support its endorsement, HDSA will be launching an educational campaign on Enroll-HD across its network of 54 Chapters and Affiliates nationwide. Clinical trial education is already a critical component of HDSA's educational, research and advocacy work. The Society's over 170 support groups, 40 social workers and specially trained Clinical Trial Diplomats and Research Ambassadors provide ongoing education to HD families about the role of observational and clinical trials in finding treatments for HD.

We appreciate that HD families are under enormous pressures just to make it through their daily activities and that for some, participation in research studies is often too much to consider. However, for families who are looking for one effective way to fight back against HD, joining Enroll-HD might be right for them.

Enroll-HD began in 2012 and now includes families in North America, Europe, Latin America, Australia and parts of Asia. Enroll-HD will likely become the largest observational study of any neurodegenerative disease in the world and is only possible because of the unique collaborative spirit that exists among HD researchers and HD families around the globe.

Enroll-HD builds upon the knowledge gained from the COHORT study in the U.S., Canada and Australia and the similar REGISTRY study in Europe. Like those studies, Enroll-HD is sponsored and managed by the CHDI Foundation, a private not-for-profit biomedical research organization dedicated to developing therapies that slow the progression of HD as soon as possible. The idea is that by combining the previous studies into one study around the world, with as many HD family members as possible participating, all taking the same tests at their annual study visits, the data and samples collected will mean a much more powerful study that can identify factors important in the course of HD progression. That gives researchers vital clues on what might make an effective therapy.

The main objective of Enroll-HD is to speed up the development of new effective drugs to treat HD. The study will do this in a number of ways:

- First, participants in Enroll-HD will provide clinical data and blood samples to help better understand how HD happens in patients (because animal models can only tell us so much). All patient data and samples (after very careful de-identification so that they are anonymous) will be available to HD researchers around the world to answer critical questions about HD. The idea is to get as many researchers as possible working on HD.
- Second, Enroll-HD will help recruitment for clinical trials of new drugs by building

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HDSA CEU Course for Social Workers:

A five-part HDSA CEU Course for Social Workers is available online. Each one-hour segment can earn you one free Continuing Education Unit from the National Association of Social Workers. To take the course and post-test, please visit **www.hdsa.org/ceu**. For questions, please contact Seth J. Meyer at **smeyer@hdsa.org**.

HDSA Annual Convention:

29th Annual HDSA Convention

June 20-22, 2014

Galt House Hotel

Louisville, KY

www.hdsa.org/convention

For more information email Robert Coffey **rcoffey@hdsa.org**

Enroll-HD: A Global HD Research Platform *(Continued from Page 2)*

an engaged group of families who have shown they are active in research. Because of the thousands of participants within Enroll-HD (in a de-identified manner) researchers will be able to conduct better, smarter, and faster clinical trials of promising new drugs.

- Third, Enroll-HD will also serve as a platform to help improve the clinical care of people with HD. By comparing clinical practice (things like physiotherapy, speech/occupational therapy, current drugs used, exercise and sleep patterns, etc.) in different sites and countries around the world in thousands of patients, clinical researchers will be able to see which sites or countries have patients who do better. They can then try to identify what it is at those sites that helps patients do better and establish new guidelines for the best clinical care for all HD patients.

Enroll-HD is truly a family study; any member of a family affected by HD can participate, including:

- People who have tested positive for the expanded huntingtin gene, whether or not they show any symptoms of the disease.
- At-risk family members who have not undergone genetic testing.
- Individuals who have a family history of HD but know they do NOT carry the expanded huntingtin gene.
- Spouses/partners of family members with HD.

Since the launch of Enroll-HD, we have seen tremendous participation by the HD community; to date, there are over 1,800 participants in the 52 actively recruiting Enroll-HD sites, around 1,300 of those in the U.S. alone. There are already more than 12,000 HD family members participating in the REGISTRY study in Europe, and the large majority of those will switch over to Enroll-HD over the next year or two.

When all is said and done, we expect there to be 15,000 to 20,000 HD patients and family members at over 200 Enroll-HD research sites in 27 countries. Getting as many HD patients and families to join in this new HD research platform is essential to ensure we maintain the positive momentum the HD community has created over the past few years, in particular to encourage more and more pharmaceutical and biotechnology companies to invest their resources and innovative technologies into finding effective treatments for HD. Building and maintaining a global research platform like Enroll-HD will undoubtedly encourage these companies to seriously consider jumping into HD drug development.

If you'd like to learn more about Enroll-HD or find a clinical site near you then please visit **www.enroll-hd.org** or **www.hdsa.org**, and look out for the Enroll! newsletter on the HDSA website.

First-HD and ARC-HD Trials

Expanded Eligibility & Financial Assistance for Participants

The Huntington Study Group (HSG) and Auspex Pharmaceuticals announced that they have modified the protocols for the First-HD and ARC-HD clinical trials so that patients previously excluded can now consider participating.

Originally, when the two trials were announced last year, patients who had previously taken Tetrabenzine (Xenazine®, Nitoman®) were excluded from participation in First-HD. The protocol for First-HD has since been changed to permit patients who have previously taken Tetrabenzine to enroll, as long as they have not taken Tetrabenzine in the past 6 months. In addition, reimbursement for travel and lodging costs incurred by trial participants is now available for both First-HD and ARC-HD.

Loosening the Tetrabenzine criteria and providing financial assistance to HD families increases the number of subjects who can participate and should decrease the amount of time required to complete the study.

First-HD is a Phase 3 clinical trial of an investigational drug called SD-809 Extended Release (ER) in persons who have a diagnosis of Huntington disease (HD). SD-809 has the same biological mechanism of action as Tetrabenzine. Both drugs are thought to positively impact chorea by inhibiting the packaging and release of an important neurotransmitter in the brain called dopamine. High dopamine levels in the brain are often associated with uncontrolled movements.

First-HD will investigate how safe, tolerable and effective SD-809 ER is compared to a placebo (inactive drug) in reducing chorea. First-HD is enrolling approximately 90 participants at approximately 30 sites across North America who have been diagnosed with HD and who have not taken Tetrabenzine in the past 6 months. Participants will be involved in First-HD for approximately 4 months. In addition, First-HD participants will receive SD-809ER and medical supervision at no cost for up to 12 months if they continue on to the ARC-HD trial.

ARC-HD is an open-label long-term safety and tolerability clinical trial of SD-809 ER in persons who have a diagnosis of HD. ARC-HD will look at how safe, tolerable and effective SD-809 ER is in HD participants and consists of two groups. One group (Rollover) is comprised of participants who complete the First-HD study and roll over into the open-label trial. The second group (Switch) consists of patients taking Tetrabenzine who are willing to switch from Tetrabenzine to SD-809ER. ARC-HD Switch is enrolling approximately 36 participants across 9 sites in the United States who have been diagnosed with HD and who are currently taking Tetrabenzine. Subjects will be involved in ARC-HD for up to 14 months and will remain on the study drug for a total of 54 weeks.

The First-HD and ARC-HD trials are being conducted by the HSG under the leadership of Samuel Frank, MD, Principal Investigator, (Boston University School of Medicine) and Claudia Testa, MD, PhD, Co-Principal Investigator (Virginia Commonwealth University). Drs. Frank and Testa recently presented an overview of the First-HD and ARC-HD trials to the HD community during a recent HDSA Research Webinar. An archived recording of this informative presentation can be found at **www.hdsa.org/research/research-webinar-series/first-hd.html**.

For more information on the First-HD and ARC-HD trials or to find a trial site near you, please visit: **www.HDSA.org** or **www.Huntington-Study-Group.org**

Thank you Auspex

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Please scan here to visit the HDSA website