uniQure to Conduct Phase I/II for the First One-Time Administered Gene Therapy to Enter Clinical Testing for the Treatment of HD.

In June 2019, Dan Leonard, the Senior Director of Patient Advocacy at uniQure released a letter to the HD community to introduce the company and their gene therapy clinical development program. uniQure has a long history in developing gene therapies, but is a relative newcomer to the HD community. uniQure was founded as Amsterdam Molecular Therapeutics (AMT) in the Netherlands in 1998. In 2012, the research team there developed the first gene therapy ever approved in Europe. The clinical trial uniQure is planning to conduct in HD uses a gene therapy known as AMT-130. This investigational treatment will be the first one-time administered gene therapy to enter clinical testing for the treatment of HD. AMT-130 is administered only once by neurosurgical procedure. There are two key components to AMT-130, a vector and a gene encoding a microRNA. The vector acts as a delivery system and is based on a non-disease causing adeno-associated virus (AAV) that has been changed to carry and deliver a gene encoding a microRNA that will recognize, bind and non-selectively lower the human huntingtin protein. microRNA (or miRNA) are small pieces of genetic material that can prevent production of a given protein. To “non-selectively lower” human huntingtin protein means that production of both the disease-causing mutant (mHTT) and normal huntingtin protein (HTT) will be decreased.

The objectives of the Phase I/II clinical trial are to assess the safety, tolerability and efficacy of AMT-130 in patients with HD using a dose-escalating, randomized, and double-blinded control design. uniQure expects to open several clinical sites in the United States and begin enrolling patients in the second half of 2019. The specific location of sites will be updated on an ongoing basis when the site is ready to screen patients for eligibility. To check the status please goes to clinicaltrials.gov and/or HDTrialfinder.org where the trial will be listed in the coming weeks.

To read the full statement from uniQure with more details on this study, visit www.hdsa.org.
Successful Spring Events!

We had a BLAST this Spring! First at our annual Strike Out HD bowling event at Scorz Sports Center and then at the wine tasting event hosted by Skyybox Grill & Bar with proceeds benefiting our HDSA Omaha Affiliate! Thanks so much to all who came out and made these events so much fun!!!

Strike Out HD Fun!

Smiles all around at the wine tasting event

We are looking for volunteers to help carry out our awareness and fundraising events. Volunteers can be anyone in our lives that want to give back to their community. They don’t need to have HD in their families – simply an interest in helping us execute our HDSA mission. If you are interested in volunteering on an event committee, or serving on our affiliate board, please contact Lindsay Rice at Omaha@hdsavolunteer.org
Two BIG HDSA Omaha Affiliate Events Coming up!!

The HDSA Team Hope Walk is our biggest event of the year! It’s a fantastic time for the community to come together to support our local HD families and to honor those we have lost. We hope to see you on August 25th at Zorinsky Lake (Shelter 5) for fun (including face painting, balloon animals, snow cones, ponies), food (lunch to be provided after the walk by Jimmy Johns and When Pigs Die BBQ), and comradery - we like to call this our big family picnic.

For registration and day of event details click the link below:
https://hdsa.donordrive.com/index.cfm?fuseaction=donorDrive.event&eventID=1656

Join us at Skybox Grill & Bar on Saturday, September 7th for the 3rd annual Tailgate for HD as we root on our Huskers as they take on Colorado! There’s nothing like a football party in Nebraska to bring our friends and families together to support our local HD community. For more information on this event visit the HDSA Omaha Affiliate Events page on Face Book or click the link below to pre-purchase tickets
https://hdsa.donordrive.com/index.cfm?fuseaction=donorDrive.event&eventID=1759