HUNTINGTON'S DISEASE YOUTH ORGANIZATION

Clinical Trials in HD

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HDYO has more information about HD available for young people, parents and professionals on our site:

www.hdyo.org

There is a continued investment into research on Huntington's Disease (HD), whether looking at the treatment of symptoms or disease modification. It's important for the HD community to have access to this information in a clear and conversational manner to better understand what is being studied and how they may become involved if interested. This page summarizes some of the current major clinical trials underway that pertain to the young people that HDYO serves.

We have divided the different clinical trials into two sections:

1. Disease Modifying Trials
2. Symptom Improvement Trials

What is a Clinical Trial?

Also referred to as an interventional study, clinical trials are research studies that involve human participants to evaluate the safety and effectiveness of new treatments, such as drugs, medical devices, or behavioral interventions. Other studies like Enroll-HD, surveys and registries are considered Observational Studies. Check out the Observational Studies page for more on current studies in this domain.

To review other helpful research definitions, explore the HDYO Research Terminology.

Where to Find Trials Use the HD Trial Finder sites below which can provide clinical trial matching services directly. They provide a useful way for individuals with Huntington's Disease, caregivers, healthy volunteers, and physicians to connect with current research studies. It includes an easy-to-use website and free call center staffed by trained HD clinical trial navigators. People with HD, care partners, and healthy volunteers are all needed currently to participate in different aspects of HD research.

Visit HDSA TrialFinder

Visit EHA TrialFinder

ClinicalTrials.gov is a website that lists research studies from around the world and can also be used to find trials. However, this site can be challenging for the community to navigate if they don't have a scientific background. We suggest starting with the different HD Trial Finder sites above first or reviewing the resources below.

Disease Modifying Trials

These studies are trying to modify how Huntington's Disease progresses in individuals carrying the HD gene. To learn more about these approaches, visit the Current Research Focused on HD section of the HDYO Research Terminology.

Falcon-HD

- **Who is overseeing this study?** Skyhawk Therapeutics
- **What's its current status?** Recruitment Has Close
- **Where can people participate?** Australia & New Zealand
- **Who's potentially eligible?** People aged 25 to 65 with early-stage HD (Stage 1, 2, or mild Stage 3)
- **What are they investigating?** This study evaluated the safety, tolerability, and pharmacokinetics of SKY-0515 (a once-a-day pill) in healthy volunteers and individuals with early-stage HD. The trial is separated into three parts. Parts A and B evaluated SKY-0515 in healthy volunteers, and Part C will evaluate SKY-0515 in individuals with early-stage HD. The study has successfully demonstrated a reduction of mutant huntingtin in healthy volunteers using participants from Parts A and B. In addition, SKY-0515 was generally well tolerated at all doses tested in healthy volunteers. Given these findings, the company will continue studying dosing with Part C participants with early-stage HD and anticipates initiation of a Phase 2 study early next year.

Find Out More

Generation HD2

- **Who is overseeing this study?** Roche
- **What's its current status?** Active
- **Where can people participate?** Opened in North America, Europe, South America and Oceania.
- **Who's potentially eligible?** People aged 25 to 50 with prodromal (very early subtle signs of HD) or early manifest HD.
- **What are they investigating?** This study evaluates the safety, biomarkers and efficacy trends of different doses of the investigational drug tominersen (given as an injection by lumbar puncture) in people aged 25 to 50 with prodromal (very early subtle signs of HD) or early manifest HD.

Find Out More

HD-GENETRX2

- **Who is overseeing this study?** uniQure
- **What's its current status?** Active and recruiting.
- **Where can people participate?** United States, United Kingdom and Poland.
- **Who's potentially eligible?** Adults between the ages of 25 and 65 with early manifest HD.
- **What are they investigating?** In this trial, uniQure is investigating low and high doses of their drug AMT-130 – a one-time gene therapy candidate administered by neurosurgical procedure, designed to silence the huntingtin gene. This 5-year trial is evaluating the safety and potential impact of AMT-130 on disease progression. Interim results from the first two years showed a statistically significant slowing in disease

progression among patients receiving the high dose of AMT-130 when compared to an external control group. AMT-130 continues to remain generally well-tolerated, with a manageable safety profile at both doses.

Find Out More

INVEST-HD

- **Who is overseeing this study?** Novartis
- **What's its current status?** Active and recruiting.
- **Where can people participate?** Canada & US
- **Who's potentially eligible?** Adults between the ages of 21 to 70 with early-symptomatic HD.
- **What are they investigating?** This study will assess the safety, tolerability and efficacy of votoplam (taken orally) to slow disease progression in patients with early symptomatic HD compared to a control arm. The safety of votoplam (formerly known as PTC518) was confirmed by PIVOT-HD and is now ready for a full-scale Phase 3 study. It will aim to enroll 770 individuals.

Find Out More

PIVOT-HD

- **Who is overseeing this study?** PTC Therapeutics/Novartis
- **What's its current status?** Completed.
- **Where can people participate?** Germany, France, Netherlands, & UK.
- **Who's eligible?** Adults ages 25 and over with a CAG repeat between 42 and 50, with no functional or movement symptoms of HD.
- **What are they investigating?** PTC continues to investigate their drug PTC518, taken orally, which has been designed to reduce the production of the mutated huntingtin protein. PIVOT-HD was a 12-month placebo-controlled trial to assess safety of PTC518 at two dose levels (5mg and 10mg), relative to placebo, as well gain insight to biomarker data that could provide meaningful evidence of treatment effect. The results confirmed that PTC518 lowers Huntingtin protein and shows early signals of clinical benefit with a favorable safety profile. It also showed a dose-dependent lowering of mutant huntingtin (mHTT) protein in the blood and cerebrospinal fluid (CSF) as well as favorable effects on several HD-related functioning outcomes at 12 and 24-months. Based on these findings, PTC are exploring the potential for accelerated approval by regulatory agencies.

Find Out More

Phase I study of ER2001 in Early HD

- **Who is overseeing this study?** ExoRNA Bioscience

- **What's its current status?** Active and recruiting.
- **Where can people participate?** China
- **Who's potentially eligible?** Adults between the ages of 25 and 55 with early manifest HD
- **What are they investigating?** This clinical trial will assess the safety, tolerability, and pharmacokinetics of increasing doses of intravenously (injections) administered ER2001 in patients with early manifest HD. The study is expected to enroll 15 participants and run in Guangdong.

Find Out More

Phase I Study of SPK-1001

- **Who is overseeing this study?** Roche and Huntington Study Group (HSG)
- **What's its current status?** Active and recruiting.
- **Where can people participate?** United States
- **Who's potentially eligible?** Adults ages 25-65 who have genetically confirmed HD and mild clinical symptoms.
- **What are they investigating?** This trial is assessing the safety, tolerability and preliminary efficacy of SPK-10001 in patients suffering from Huntington's Disease. SPK-10001 is a gene therapy that delivers genetic instructions into the brain cells to reduce mutant huntingtin that causes brain atrophy and clinical symptoms affecting motor function, cognition, and behavior for individuals with HD. In this study, the researchers will slowly increase the dose for each small group of participants to find the best dose of a study treatment for participants to receive.

Find Out More

Phase 2/3 Study of SKY-0515

- **Who is overseeing this study?** Skyhawk Therapeutics
- **What's its current status?** Active and recruiting.
- **Where can people participate?** Australia, New Zealand and Georgia
- **Who's potentially eligible?** People aged 25 or older who have HD confirmed by genetic testing and meet certain requirements for physical ability and independence
- **What are they investigating?** The goal of these clinical trials is to test if the drug SKY-0515 (a once-a-day pill) can lower harmful proteins linked to HD and improve the symptoms of participants with HD. The studies are both randomized placebo-controlled trials that will evaluate the safety, pharmacodynamics, and efficacy of SKY-0515. SKY-0515 is designed to lower the amount of harmful huntingtin (HTT) protein that causes HD. It works by reducing the messenger RNA (mRNA) that tells cells to make the HTT protein, which means less of both the normal and mutant forms of the protein are produced. The drug also lowers levels of another protein called PMS1, which is involved in the process that makes the disease get worse over time. In early

studies, SKY-0515 has reduced HTT and PMS1 mRNA levels in a dose-dependent manner, with favorable safety and tolerability profiles in healthy volunteers.

Find Out More

Find Out More

POINT HD

- **Who is overseeing this study?** Roche
- **What's its current status?** Active and recruiting
- **Where can people participate?** New Zealand, Australia and Argentina
- **Who's potentially eligible?** Adults ages 25-65 who have genetically confirmed HD and mild clinical symptoms as well as who carry a specific genetic variation in the HD gene targeted by the drug being investigated
- **What are they investigating?** This Phase I study is testing the safety and tolerability of a drug called RG6496 at different doses in patients suffering from HD. RG6496 is being developed with the goal of lowering levels of the faulty huntingtin protein, while keeping healthy huntingtin protein. It works by targeting a specific genetic variation that some people carry in their HD gene.

Find Out More

SELECT-HD

- **Who is overseeing this study?** Wave Life Sciences
- **What's its current status?** Completed
- **Where can people participate?** Australia, Canada, Denmark, France, Germany, Italy, Poland, Spain, United Kingdom
- **Who's potentially eligible?** Adults aged 25 to 60 years with HD.
- **What were they investigating?** This Phase Ib/IIa trial investigated the safety and tolerability of WVE-003 in people with HD. WVE-003 is a drug designed to lower the toxic form of the huntingtin protein, delivered intrathecally (through the spine). The study demonstrated that WVE-003 selectively lowered the toxic, mutant huntingtin protein while preserving healthy, wild-type huntingtin protein for individuals with Huntington's disease. It also showed that WVE-003 was generally safe and well-tolerated. Based on these positive findings, Wave is planning activities for a global Phase 2/3 study of WVE-003.

Find Out More

VO659

- **Who is overseeing this study?** Vico Therapeutics
- **What's its current status?** Active and recruiting

- **Where can people participate?** Europe
- **Who's eligible?** Adults ages 25 to 60 early manifest HD
- **What are they investigating?** This Phase 1/2a clinical trial is designed to assess the safety and tolerability of four doses of VO659 administered intrathecally (through the spine) every four weeks in participants with early manifest HD. VO659 has a unique allele-preferential mechanism of action that targets the underlying CAG repeat expansion that causes HD. Interim data suggested that VO659 may be able to reduce levels of the toxic HD protein in the small group of participants tested so far. Still, interim data presented also showed that 4 (out of 6) people who received at least three doses of 40 mg experienced radiculitis, a painful condition related to nerve damage. Vico plans to mitigate this issue by lowering the amount of drug given to people in the trial.

Find Out More

Studies Targeting Symptom Improvement

These studies are targeted at suppressing symptoms such as those caused by chorea, cognitive decline and behavioral changes. They are not modifying the disease course, but aim to improve daily life instead in regards to symptom severity.

KINECT-HD2

- **Who is overseeing this study?** Neurocrine Biosciences and the Huntington Study Group
- **What's its current status?** Active; enrollment closed.
- **Where can people participate?** Canada and United States.
- **Who's potentially eligible?** Adults aged 18 to 75 with a diagnosis of motor manifest HD.
- **Which symptom(s) is being targeted?** Chorea
- **What are they investigating?** KINECT-HD2 is an open-label rollover study of the previous trial KINECT-HD. This trial is investigating valbenazine (whose trade name is INGREZZA®) for the treatment of chorea in HD. Initial results from combining KINECT-HD with current KINECT-HD2 participants recently supported the ability of the U.S. Food and Drug Administration (FDA) to approve INGREZZA® capsules for the treatment of adults with chorea associated with HD. More recently, Neurocrine has introduced an oral granules formulation of INGREZZA (called INGREZZA® SPRINKLE) that provides an alternative administration option for those who have difficulty swallowing or prefer not to swallow whole capsules.

Find Out More

PROOF-HD

- **Who is overseeing this study?** Prilenia

- **What's its current status?** Completed; Analyzing Data
- **Where could people participate** Austria, Canada, Czech Republic, France, Germany, Italy, Netherlands, Poland, Spain, United Kingdom, United States.
- **Who was potentially eligible?** Adults aged 25 and over with early manifest HD.
- **Which symptom(s) is being targeted?** Chorea, Cognition and Behavioral
- **What are they investigating?** PROOF-HD is a Phase III trial to evaluate the efficacy and safety of the drug pridopidine on functional capacity in adults with early manifest HD. Pridopidine is a small molecule drug thought to target a protein called the sigma-1 receptor. Taken orally, it is hoped pridopidine will have beneficial effects on nervous system cells. Preliminary analysis showed the study did not find a significant effect on change in functional capacity when looking across all study participants (the primary endpoint). However, there was evidence of a potential benefit in the subset of patients that were not taking neuroleptics (e.g., antipsychotics) or chorea medications. Additionally, there were other potential benefits shown on secondary endpoints capturing motor scores. Pridopidine was well-tolerated with no serious treatment-related adverse events.

Find Out More

Phase II Study of SOM3355 in Huntington's Disease Chorea

- **Who is overseeing this study?** SOM Innovation Biotech SA
- **What's its current status?** Completed
- **Where can people participate?** UK and Europe
- **Who's potentially eligible?** Individuals over the age of 21 diagnosed with Huntington's Disease and suffering with choreatic movements.
- **Which symptom(s) is being targeted?** Chorea
- **What are they investigating?** This trial assessed the efficacy and safety of SOM3355 in patients suffering from HD with choreatic movements. SOM3355 is an oral antihypertensive drug which has been used for years, and is now being tested to treat chorea in HD. he trial showed benefit of SOM3355 on chorea when given at the dose of 600mg daily in patients that were not taking neuroleptics (e.g., antipsychotics). Moreover, the safety profile of SOM3355 was also confirmed. The study results support continued development of SOM3355 for Chorea in individuals with HD who are not taking neuroleptics (e.g., antipsychotics).

Find Out More

Observational study evidence for Sertraline

- **Who is overseeing this study?** Investigators from the University of Barcelona
- **What's its current status?** Closed
- **Where can people participate?** At Enroll-HD sites across the globe
- **Who's potentially eligible?** Enroll-HD participants with HD from both pre-motor

manifest and motor manifest stages with a minimum of a 3-years of follow-up data

- **Which symptom(s) is being targeted?** Chorea (e.g., motor symptoms) and daily functioning
- **What are they investigating?** This study compared people with HD taking sertraline (either alone or with other antidepressants) to people taking other medications or no antidepressants at all. They found that people taking sertraline showed a slower decline in functional capacity, meaning they maintained their ability to work, manage finances, handle domestic chores, and care for themselves better over time. Specifically, people taking sertraline showed better scores on tests measuring total functional capacity, functional assessment, and independence. They found no evidence of an impact on motor problems or Chorea symptoms.

Find Out More
