

HD Clinical Research: Getting Involved

HDSA Center of Excellence at UC Davis

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Clinical research studies, especially clinical trials, are how new knowledge is generated, and they help to find new treatments. Being a research participant is not for everyone, but without participants there cannot be new treatments in the future.

Source: The Huntington Study Group Web Site

Questions

- What is clinical research?
- What is an observational study?
- Who can participate in research studies?
- How much time does it require?
- What about confidentiality?
- How do people decide whether to volunteer ?
- Which HD clinical trials are enrolling right now?
- How can I stay up to date about research?

What is Clinical Research?

- The **goal** of clinical research is to study information collected about the people enrolled in order to learn about the manifestations of a disease, or to test the safety, benefit, side effects and risks of an intervention designed to help people affected by a disease.

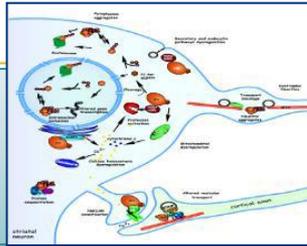
- Types of clinical research:
 - Observational studies
 - Clinical trials
 - Double blind, randomized control trials
 - Phase I, II, III

What Are Observational Studies?

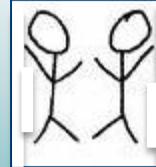
- These are studies that follow research participants over time without making a treatment intervention.
- Observational studies allow researchers to study the progression of HD over time in a number of individuals, and help us to understand the symptoms people experience, the signs noticed by investigators, the rate of progression, and sometimes markers of HD such as brain scan or blood test changes.

The Pathway to Finding New Drugs

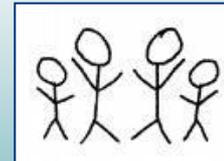
Pre-clinical: Models



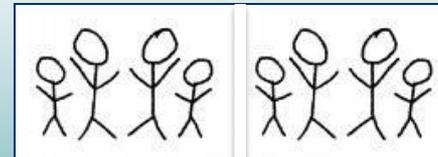
Phase I: Test in healthy humans for safety



Phase II: Test in small population with disease for safety and dosing



Phase III: Test in larger population for efficacy



FDA Approval



What Are Double Blind, Randomized, Controlled Studies?



- Designed to help prevent judgment or bias by the investigators or research subjects from affecting the study results.
- Double blind: neither the investigator nor the participant can tell if the participant is taking the active drug or a placebo.

What Are Double Blind, Randomized, Controlled Studies?



Randomized: The study participant is assigned randomly to either active drug or placebo.

Controlled: The study drug is compared to a known treatment (active control) or to a placebo (inactive compound that appears identical to the active drug).



Who Can Participate In Clinical Trials?

- The study protocol is a research plan or roadmap designed to best answer the question posed by the study.
- The protocol includes criteria for who is eligible. This could include:
 - People with the HD gene expansion who don't yet have symptoms
 - People with HD at early, mid- or late-stage
 - People at 50% risk for the HD CAG expansion
 - Family members who are not at risk for HD, or people who have undergone predictive testing and don't have the HD CAG expansion



Who Can Participate In Clinical Trials?

Persons with:

- Ability to give informed consent
 - Or research proxy (person you designate to make research decisions for you)
- Who meet age criteria
- Who have characteristics of HD: early, mid-stage, etc
- Other medical conditions
 - Safety labs
- Psychiatric conditions
 - Must be stable
- Other medications
 - Some may not be allowed



How Much Time Is Required?

Studies vary in duration, frequency of study visits and in the number of activities carried out.

- Observational studies can last many years
 - Visits are usually just once a year
- Clinical trials could last a few weeks to several years
 - Visits are frequent for Phase II trials (every 1-4 weeks)
 - Visits every 1 – 6 months in longer Phase III studies
- Initial study visits usually last 2 - 3 hours
- Follow-up visits may take as little as 15 minutes, or as long as several hours
- Sometimes there may be telephone contacts made between visits

What Kinds Of Activities Take Place During Study Visits?

➤ Initial visit: informed consent

- Principles of protection of human subjects require approval of Institutional Review Boards or Ethics Committees.
- Informed consent includes a description of the aims of the study, the procedures that will take place, the potential benefits, side effects and risks to participants.
- Participants are invited to ask questions for clarification
- Often 18 pages long
- Takes about 45 minutes



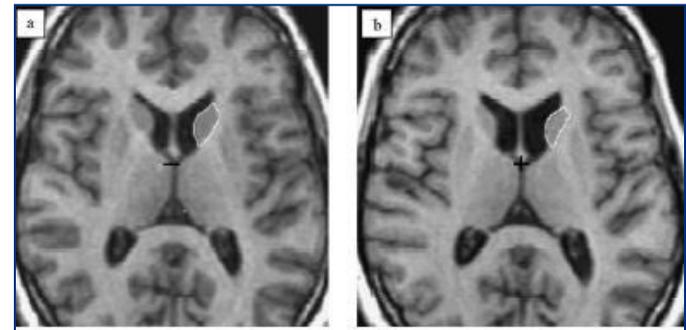
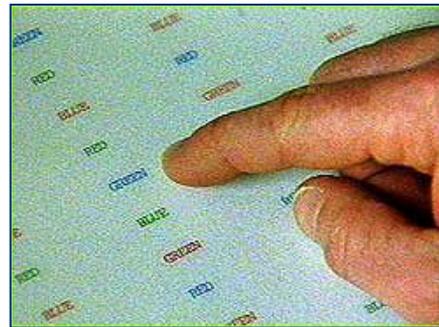
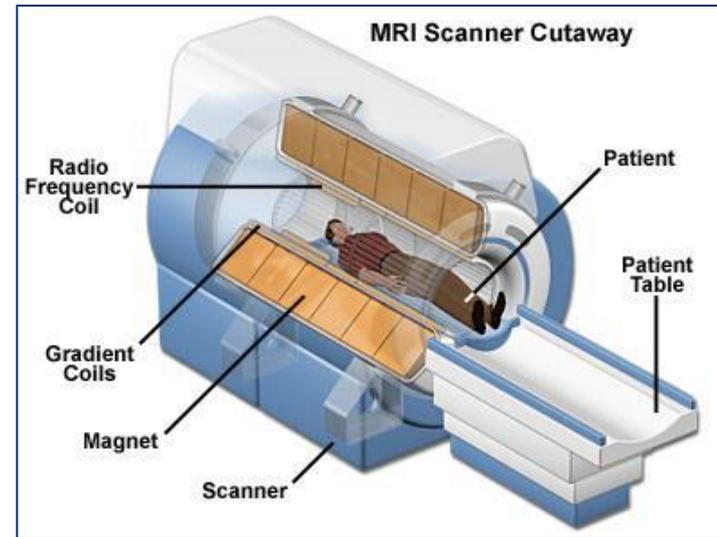
What Kinds Of Activities Take Place During Study Visits? (Cont.)

- Measuring “vital signs”: blood pressure, pulse, weight
- Drawing blood samples
- Meet with site investigator to ask how participant is doing, any health changes, any side effects, perform physical and neurological exam



What Kinds Of Activities Take Place During Study Visits? (Cont.)

- Meet with site coordinator to ask about mood or behavior changes, test thinking, turn in empty study medication bottles and dispense study drug
- MRI brain scan
- Cognitive tests (memory, thinking, etc)



What About Privacy?

- Confidentiality is a prime concern
- Participant identities are known to study site personnel, but NOT to anyone else involved in the study
- Participants are assigned an ID code at the sites; all communications with the central site use ID codes, not names
- Blood samples are assigned bar codes to further protect privacy
- To date, there have been no inadvertent disclosure of identity or gene status



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What Happens After A Clinical Trial Is Over?

- The Huntington Study Group is committed to communicating the results of completed clinical trials to participants
- Timing: as soon as the study data is “locked”
- Mechanism:
 - 1st: conference call with participants
 - HSG website, press releases
- Learning whether participant was assigned to active drug or control group may take weeks or months longer.



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Getting Involved In Research: Five Good Reasons For Participating

From Terry Tempkin

1. You've always wanted to help
2. You want to contribute to the efforts for a cure
3. You have the time
4. You care about HD research and future generations
5. Participating in research makes you feel good about helping to advance treatment and find a cure

Reasons For **NOT** Participating In Clinical Research

- I don't have enough time
 - Work, school, family activities
 - Caregiving for family members
- I'm concerned about my privacy.
- It's too stressful to think about HD.
- I don't want to have physical exams or thinking tests; I'm already worried enough.
- I hate having blood tests!
- It's too far to drive to the nearest study site.
- I don't want to be on a placebo.

Enroll-HD

Longitudinal Observational Trial



The Goal:



- To create opportunities for research participation for HD families on a **global scale**
- Through this effort, ENROLL- HD is creating a repository of unified clinical information and bio-samples critical for HD scientists world-wide.

Why Is This Important?

- This repository of data creates a wealth of information to better understand the disease and provide an important tool for future HD studies.
- Often studies require large numbers of individuals in order to be sufficiently powerful
- Enroll-HD investigators will be collaborating from across the globe to provide those large numbers of participants



STAIR



- An Exploratory Phase II Study to Test the Safety, Tolerability, & Activity of a Novel Vasopressin 1a Receptor Antagonist (SRX246) in **Irritable** Subjects with HD
- Schedule: Screening, Baseline, 5 clinic visits, + 2 telephone visits over ~ 18 weeks

For More Information....

Contact Your Local Study Coordinator!

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Online Resources

<http://clinicaltrials.gov>

<https://en.hdbuzz.net/>

<http://huntingtonstudygroup.org/>

<http://hdsa.org/hd-research/enroll-in-a-clinical-trial/>

HD Clinical Research

We need your help. Please consider volunteering for HD research.

Thank you!

