Huntington's Disease Society of America

Clinical Trials Demystified: What it's Like to Participate and Why Do You Need ME?

Huntington's Disease Society of America

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HDSA encourages all attendees to consult with their primary care provider, neurologist or other healthcare provider about any advice, exercise, medication, treatment, nutritional supplement or regimen that may have been mentioned as part of any presentation.

Presenter Disclosures

Jeanette Garcia, Jimmy Pollard and George Yohrling

The following personal financial relationships with commercial interests relevant to this presentation existed during the past 12 months:

No relationships to disclose

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Presenter Disclosures

Stacey Barton

The following personal financial relationships with commercial interests relevant to this presentation existed during the past 12 months:

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Agenda

- **Welcome**
  Jimmy Pollard
- What to expect during a research visit
  Stacey Barton
- Interview: What it is like to be a participant in research
  Jimmy Pollard and Jeanette Garcia
- Call to action
  George Yohrling

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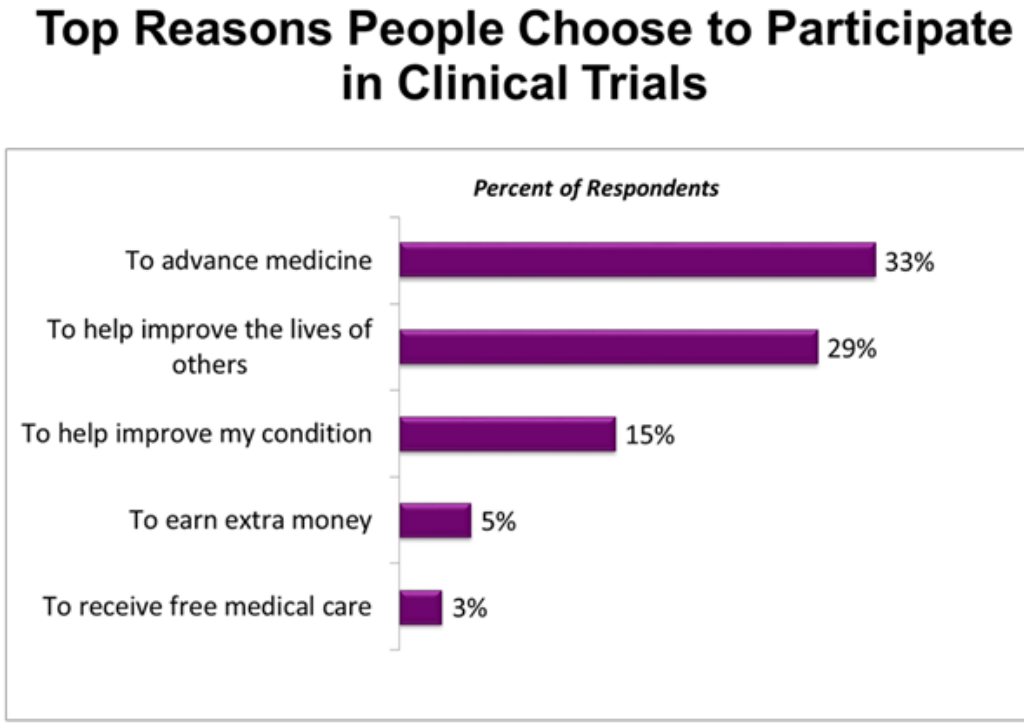
What does the scientific community want you to know

- **“HD is the most curable incurable disorder”** – Dr. Ed Wild
- **“If no patients or gene-positive people show up for trial participation there will be no novel treatments, ever!”** – Daniel P. van Kammen, M.D., Ph.D (via Gene Veritas’ blog)

What's in it for me? Why Participate in a Clinical Trial

- Gain access to new treatments not yet available to the public
- Obtain expert HD medical care
- Play an active role in your health
- Helping future generations by contributing to medical research
- Fight back against HD!
- You give HOPE to yourself and others
- Be an agent of change!



Source: CISCRP, 2013; N=5,701 people worldwide


I AM NOT THE CURE FOR
HUNTINGTON'S DISEASE
YOU ARE!!!!
HDTRIALFINDER.ORG
memegenerator.net

OK, maybe I'm sold.
But what is a study visit REALLY like?

- Make the contact
- Informed consent
- Types of studies: Clinical trials vs. observational studies
- Types of visits: Screening, Baseline, Follow up, Phone "visits"
- Study termination

Make the contact

- You can call the research site (we love to hear from you!) or you may be contacted directly
  - Limitations on research site reaching out to you

- Finding studies
  - **HD Trial Finder: www.hdtrialfinder.org – the easiest place to start!**

  - NIH: www.clinicaltrials.gov
  - HSG: www.Huntington-Study-Group.org
  - ENROLL-HD: www.enroll-hd.org

Informed Consent

- Informed consent is a *process* utilizing a *document*
- What you should know:
  - When you consent to participate in a study, you acknowledge you understand and accept all aspects of the research study—including any risks or benefits involved
  - Staff is obligated to discuss all pertinent information about the study
  - It is your responsibility to ask questions if you do not understand
    - If you do not understand, ask that the information be explained in another way, using everyday words
    - Documents /interpreters can be made available in your preferred language
  - Research staff should help you understand the info and give you enough time to ask questions and consider your options

Source: Centerwatch

Types of studies:
Clinical trials vs. observational studies

- Different studies require different things
  - HD clinical trials evaluate drugs or treatment
  - HD observational studies learn more about HD without changing anything about you
- Generally speaking, clinical trials will be more involved than observational studies, require more frequent visits and have more tests (e.g. for safety)

Types of visits: Clinical trials

- Screening visit – makes sure you are eligible for the study
  - Informed consent document signed at this visit
  - May include analysis of your health history, current health, blood work/vitals/scans, other medications
- Baseline visit – randomization (if applicable)
- Follow up visits – drug count, distributing more drug, questions about side effects, ongoing monitoring
- Phone “visits”
- Length of visits can vary quite a lot

	Visit 1	Visit 2	Visit 3	Visit 4	Visit 5- Phone	Visit 6	Visit 7	Visit 8 or Term Visit	Visit 9- Follow-up	Unscheduled Visit
Standard blood tests	X	X	X	X		X	X	X	X	
Blood for soluble biomarkers		X				X		X		
Blood for genomic analysis (including CAG)	X									
Blood sample for gene expression analysis		X				X		X		
Blood Sample for drug concentration levels			X	X		X		X		
Urinalysis	X									
Physical examination, including weight	X	X	X	X		X	X	X	X	
ECG (Visit 2 you will have three ECG's)	X	X	X	X		X		X	X	
Vital signs measurements	X	X	X	X		X	X	X	X	X
Blood Sample for Pregnancy Test	X	X	X	X		X	X	X	X	
Urine Sample for Pregnancy Test		X	X	X		X	X	X	X	
Discussion- use of effective contraception		X	X	X	X	X	X	X	X	X
MRI scan		X						X		
Scales & Questionnaires	X	X	X	X		X	X	X	X	
Dispense Study Drug		X	X	X		X	X			
Collect Study Drug			X	X		X	X	X		X
Home urine pregnancy test			After Month1 (Visit 3), at-home urine test for women of child-bearing potential every 28 (±2) days							
Site call to patient for result inquiry			Phone call within 72 hours of urine test date to inquire about results							
Medication Inquiry	You are asked about any medications or changes in the medications that you are currently taking									
Adverse Event Inquiry	You are asked about changes in your health since last visit, including any serious health events									

Adapted from www.enroll-hd.org

Types of visits: Observational studies

- May be more like a “typical” office visit except you are providing information, not getting feedback or counseling about your health. It’s a one-way street.
- First visit - informed consent process and sign form, medical history, medications you’re taking, demographic info, family history
- Confidentiality – all studies will assign you a study ID, some studies use an HDID
  - Variability of confidentiality
- All visits – whatever study procedures you have been told about - functional assessment, motor tests, cognitive tests, questions about mood, blood samples
- Visit length caries by study – for example, ENROLL takes ~1-2 hours.

Core Assessments	Written informed consent/parental permission/assent Creation of the unique HD identification (HDID) Review of Inclusion/Exclusion Criteria Local diagnostic laboratory CAG report (if available) Investigator and research genotyping determined classification of participants Socio-demographic information HD Clinical Characteristics (HDCC) Medical history Co-morbid conditions Current therapies (Pharmacotherapy, Nutritional supplements, non-pharmacologic therapies) Reportable Event monitoring **Motor Assessments** Unified Huntington's Disease Rating Scale (UHDRS) 99 Motor UHDRS Diagnostic Confidence Index **Functional Assessments** UHDRS '99 Total Functional Capacity UHDRS '99 Functional Assessment Scale UHDRS '99 Independence Scale **Behavioral** Problem Behaviors Assessment-Short (PBA-s) **Cognitive Assessments** Symbol Digit Modality Test Stroop Color Naming Stroop Word Reading Categorical Verbal Fluency **Research Genotyping** (conducted at the first visit for all new participants to the study or for participants from previous studies for whom a research genotype is not available)

Extended Assessments	**Global** Global Clinical Impression **Behavioral** Hospital Anxiety/ Depression Rating Scale (HADS) & Snaith Irritability Scale (SIS), combined Columbia Suicide Severity Rating Scale (CSSRS) **Cognitive** Stroop Interference Trail Making A & B Letter Verbal Fluency Mini Mental State Examination (MMSE) **Physiotherapy Outcome Measures** Timed Up and Go (TUG) 30-second Chair Stand Test **Quality of Life** Short Form Health Survey-12 (SF-12) Caregivers Quality of Life Questionnaire **Health Economics** Client Services Receipt Inventory (CSRI) Work Productivity and Activity Impairment-Specific Health Problem Questionnaire (WPAI-SHP)
Optional Assessments	Family History Biospecimens for biobanking

The Study Day

- Length varies
- Breaks, meals and snacks
- May have to move around the medical center – e.g. labs, seeing the doctor, scans
- Travel
- Overnights

Study Termination

- Studies end in several possible ways
  - You have completed the entire study (thank you!!)
  - You decide you want to quit
    - May or may not impact the research data
  - The researcher removes you from the study
  - The study ends early

- Will you find out which treatment group you were in?

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Resources for Staying up to Date on HD Research

- HDSA Research Webinar Series
  - Monthly research seminars given by HD scientists
  - Archived for future reference @ www.hdsa.org (click on YouTube)
- HD News App for iOS and Android – Free!
  - Search iTunes and Google Play App Store for “HDSA” or “Apptomics”
- New HD Trial Finder - www.hdtrialfinder.org – available now!
  - Replaces the outdated HDtrials.org
  - Easy to use:
    - Create Account
    - Create Profile (zip code and who for)
    - Answer short questionnaire
    - Review personalized results

Huntington's Disease Society of America HDTrialfinder

Huntington's Disease Society of America HDTrialfinder

What's there?

- Only currently recruiting trials in North America.
- Observational and Interventional HD trials
- Important experimental medicine studies **not** listed in ClinicalTrials.gov
- Patient-friendly summaries
- Local contact info
- Mobile-friendly

Questions? Comments?

Additional Resources

- HDSA Research Webinars: www.hdsa.org/research
- HD Trial Finder: www.hdtrialfinder.org
- HD Buzz: www.hdbuzz.net
- HSG: www.Huntington-Study-Group.org
- ENROLL-HD: www.enroll-hd.org

Remember, NO drug to treat HD will ever be approved without clinical trials!

And clinical trials can only happen with your help!

Our Finish Line:
Treatments for HD

Huntington's Disease Society of America

Extra slides if needed

Informed Consent

- Makes sure you know and understand what will happen during the study so you can decide whether or not participating is right for you
- Your rights are to:
  - Know purpose, risks, side effects, expected discomforts, benefits
  - Know what will happen including whether procedures/drugs differ from standard treatment
  - Know and compare options available vs. being in the clinical trial
  - Ask questions prior to consenting and during the study
  - Be allowed ample unpressured time to decide whether to participate
  - Terminate participation for any reason at any time
  - Receive a signed and dated copy of the informed consent form
  - Be told of any treatments available if complications occur

Source: Centerwatch

Informed Consent: Sample Questions to Ask

- What is the main purpose of the study and why is it important to me?
- What are the chances that this drug will work?
- What are the risks?
- How much time will this take?
- Is there a placebo? What are my changes of getting placebo vs. drug?
- What kinds of tests will be done? Will they hurt? If so, for how long?
- How will the tests compare to the tests I would otherwise have anyway?
- Can I continue to see my own doctor during the study?
- Can I continue to take my regular medications during the study?
- If I have side effects, can they be treated and who pays for the treatments?
- Who has access to my research information?
- What if I decide to quit the study?
- Can you take me out of the study even if I want to continue?
- Does it cost me anything? If so, will insurance cover these costs?
- Am I paid or is my travel reimbursed?

Source: Centerwatch

Study Period	Screening		Long Term Treatment										Follow up		UNS	
	First HD	BL	Titration													
Activity **Week**		0	1	2	3	4	5	8	15	28	Q 13 weeks	End of Drug Visit	1 wk	4 wk	UV[3]	UTC
In-Person Clinic Visit		X		X		X		X	X	X	X	X	X		X	
Telephone Contact			X		X		X							X		X
Evaluate Capacity for informed consent		X														
Written Informed Consent		X														
Research Advance Directive		X														
Review Eligibility Criteria		X														
Update Medical History		X														
Vital Signs/Weight/ Height	X[2]	X[2]		X		X		X	X	X	X	X	X		X	
Physical Examination	X[2]											X				
Brief Physical Examination										X						
Complete Neurological Examination	X[2]											X				
Heart Rhythm (ECG)	X[2]			X [1]		X [1]		X				X				
General Health Blood Samples	X[2]							X		X	X (Q 26)	X				
Blood Sample for CAG Length	X[2]															
Blood sample for CYP2D6 protein	X[2]															
Study Drug Blood Samples															X[1]	
Assess movement, mood, speech, swallowing		X[2]		X		X		X	X	X	X	X	X		X	
Assess your chorea		X[2]	X	X	X	X	X	X	X	X	X	X	X		X\	X
Assess your memory and thinking		X				X		X	X	X	X	X	X			
Assess your behavior		X		X		X		X	X	X	X	X	X			
Assess your everyday functioning	X[2]									X		X				
Assess daily activities, functioning, well-being	X[2]									X	X (Q 52)	X				
Assess your sleepiness and restlessness		X[2]		X		X		X	X	X	X	X	X			
Evaluate/Adjust Dose of Study Drug			X	X	X	X	X	X							X[1]	X[1]
Discuss problems since last visit , review current meds	X[2]	X[2]	X	X	X	X	X	X	X	X	X	X	X	X	X	X
Return study drug												X				
Dispense, instruct abt side effects, review compliance	X	X	X	X	X	X	X	X	X	X	X	RC			X[1]	X[1]