CLINICAL RESEARCH PROCESS

How Research Turns into Treatments for HD

You can become involved in finding treatments for Huntington's disease (HD) that may lead to improvements and potentially a cure by volunteering to help doctors and researchers learn more about HD

Types of Studies

Clinical Trials (Interventional studies)	studies the effectiveness and safety of potential treatments on people
Observational Studies	Observes data or collects information from participants

Stages of Research

Pre-clinical	researchers investigate a new idea in their lab usually using animals or cell models. If promising, they apply to the FDA for an Investigational New Drug Application.
Clinical	3 Phases of trials to understand the effectiveness and safety of treatments on volunteers.
FDA New Drug Application	reviewing and approval leads to the next stage.
After FDA Approval	researchers continue to monitor research volunteers for side effects and they submit their safety reports to the FDA.

Phases of Clinical Trials

	involves healthy volunteers finding out if a drug or therapy is safe and

Phase 1	exploring different ways to administer treatments such as pills or injections.
Phase 2	involves volunteers with HD to test if the experimental treatment is safe and effective. Some volunteers receive a placebo.
Phase 3	given to a larger group of volunteers to learn about safety, effectiveness, and test for different dosage amounts.

Key Terms

Informed Consent	explains the purpose of the study, how long it's expected to last, tests or procedures being done, risks and benefits, and whom to contact for further information.
Inclusion/Exclusion Criteria	are the reasons a person can/cannot participate in a trial. Each trial uses different criteria.
Open-Label Extension (OLE)	allows eligible participants to take the active drug instead of a placebo if a drug is found to have the potential for benefit.
Compassionate Use (Expanded Access)	allows the use of the treatment without FDA approval and outside of the clinical trial if an individual has a life-threatening condition.

Key Questions to Ask

- [ ] What expenses do you cover?
- [ ] Do you provide a stipend for travel/food/lodging?
- [ ] Do you provide a stipend for my partner?
- [ ] Can I bring my children? What accommodations do you have for them? Do you provide a stipend for childcare?
- [ ] Do you have a stipend for pet care expenses?
- [ ] Do you offer a concierge service?
- [ ] What is your policy on open-label extension (OLE)?
- [ ] What is your policy on compassionate use?
- [ ] If I participate, will I be able to participate in other studies or future studies?
- [ ] Are you flexible on dates and times of visits?
- [ ] How many visits should I anticipate each month (or year)?
- [ ] When will the results from the study be available?
- [ ] How long will it take to administer the drug/therapy?
- [ ] Can I work/do other activities while receiving the drug/therapy?
- [ ] Who can I call between visits if I have questions or concerns?

- [ ] Do you provide any professional support such as a social worker or therapist?
- [ ] What other questions have other participants asked?