Clinical Trial Overview

Therapy to Improve Cognitive Dysfunction in Huntington Disease

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Background Information

- Huntington disease (HD) is a hereditary neurological disorder
- Clinically characterized by motor disorder, cognitive dysfunction, and psychiatric disturbance
- No currently approved medications for HD-associated cognitive dysfunction

Intervention

- The proposed treatment is an experimental drug for the treatment of HD-associated cognitive dysfunction
- Placebo group will be included to provide blinding but will not contribute to the outcome

Primary Outcome

- Dose limiting toxicity to determine the maximum tolerated dose of an experimental drug in HD patients
- Challenge:
  - Choosing the dose range to study so that we identify a biologically relevant and tolerable dose

Challenges to Trial Design

- Recruitment
- Drug compliance
- Patients may experience a toxic event that is unrelated to the drug
- We are excluding those with severe cognitive impairment and it is unknown if there could be an effect in these patients

Pre-mortem

- Explore different dose range?
- Different length of follow-up?
- Have a real time measure that the drug is doing what we think it should in the brain (e.g. brain image)?