PROTOCOL SYNOPTIC

STUDY TITLE  A Pilot, Randomized, Double-blind, Placebo-controlled Phase I Study to Determine the Safety and Tolerability of Varenicline (Chantix®) in Treating Spinocerebellar Ataxia Types 1, 2, 3, and 6

SPONSOR  National Ataxia Foundation; Bobby Allison Ataxia Research Center (Sites: U of South Florida, U of Chicago, UCLA, Emory U of Florida, U of Minnesota)

CLINICAL PHASE  2

STUDY RATIONALE  Spinocerebellar ataxia (SCA) is a group of inherited disorders characterized by cerebellar degeneration leading to imbalance, incoordination, speech difficulties, and problems with walking. Recently, individual case reports have suggested that varenicline, a drug used in smoking cessation, produces substantial improvement in patients with several inherited ataxias. A modest response was noted in 5 patients with SCA, suggesting that it is potentially efficacious in this disorder as well. Although this agent is available for off-label use, the severe side effects noted with its use and the lack of long-term toxicity data demand that it be systematically assessed. The present study will test whether varenicline is safe and potentially efficacious in a heterogeneous cohort of adults with SCA.

STUDY OBJECTIVE(S)  The primary outcomes will be the changes in the patient’s SARA Rating Scale total score and frequency and severity of dose-limiting adverse events.

The secondary objectives of this study are to assess:

- the effect of varenicline on quality of life in patients with spinocerebellar ataxia
- the effect of varenicline on depression and anxiety ratings
- the effect of varenicline on the activity of daily living (ADL) in patients with spinocerebellar ataxia

TEST ARTICLE  Varenicline

STUDY DESIGN  This is a double-blind, parallel group, randomized, placebo-controlled, crossover pilot study

NUMBER OF SUBJECTS  40 subjects overall
6 sites

STUDY DURATION  175 days (± 3 days) per subject