

PROTOCOL SYNOPSIS

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	Nataional 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	<p>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.</p> <p>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.</p>
STUDY OBJECTIVE(S)	<p>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.</p> <p>The secondary objectives of this study are to assess:</p> <ul style="list-style-type: none">▪ 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