



## **Retrotope announces open enrollment for Friedreich's ataxia clinical trial**

**Los Altos, CA, August 17, 2015** – Retrotope announces the opening of enrollment for a 28-day, first-in-human, randomized, double-blind, controlled, ascending dose study of orally dosed RT001 to evaluate the safety, tolerability, pharmacokinetics (PK), disease state, and exploratory endpoints in patients with Friedreich's ataxia (FA).

Robert Molinari, Ph.D., CEO of Retrotope comments: *"Retrotope is excited to begin human trials working with FARA and the Friedreich's ataxia community to develop a treatment for this devastating disease. Retrotope and others have discovered that free radical lipid peroxidation cascades in the mitochondrial membrane represent a common mechanism by which many degenerative diseases, and especially FA, trigger and amplify damage that kills cells. In FA, free iron associated with the disease is a catalyst for lipid peroxidation of exactly the type we can mitigate with our drug, RT001, a chemically stabilized form of a natural membrane fatty acid that is resistant to lipid peroxidation."*

The RT001 protocol is a two center study planned for 18 patients with FA who are ambulatory (with or without an assistive device). The primary endpoints are to evaluate the safety and tolerability of two dose levels of RT001 when administered orally to patients with FA for 28 consecutive days; to determine the PK profile of RT001 at both dose levels following a single and multiple oral administration; and to determine the dose for future studies. The secondary endpoints are to evaluate the effects of RT001 on disease state endpoints using the Friedreich's Ataxia Rating Scale neurological sub-score and the Timed 25-Foot Walk (T25FW) performance measurement relevant to ataxia.

*“FARA has been working with Retrotope for the past few years as RT001 has gone through pre-clinical development and we believe this provides an excellent example of the power of patient engagement early in the drug development process. FARA provided assistance in developing partnerships with academic collaborators who have tested RT001 in FA cellular models, grant support for manufacturing, attendance at regulatory meetings and engagement of the patient community in focus group discussions on diet modifications recommended for this early phase study. We are excited to continue our partnership and support as we approach this new and important milestone,”* said Jennifer Farmer, MS, CGC, Executive Director at FARA.

Two of FARA's Collaborative Clinical Research Network sites, the University of South Florida and the University of California Los Angeles, will be conducting the study and FARA will utilize the Patient Registry to assist the sites in recruitment. The University of South Florida is open for enrollment, the University of California Los Angeles will be opening in the early fall for enrollment.

For more information on this study, visit:

<https://clinicaltrials.gov/ct2/show/NCT02445794>

**About RT001:** Retrotope has discovered that a mechanism common to many degenerative diseases, namely, the free radical degradation of lipids in mitochondrial and cellular membranes, may actually cause disease. Free radicals attack and degrade the polyunsaturated fats (PUFAs) that are essential components of cellular membranes. We and others have shown that the degradation products of these fats are associated with many diseases of neurodegeneration and aging, and create further damage cascades that are toxic to cells. Retrotope’s lead compound (RT001) is a patented, orally available, stabilized fatty-acid that shuts down this degradation and stabilizes (“fireproofs”) cellular membranes against further attack.

**About Retrotope, Inc.:** Retrotope, Inc. is a privately held clinical-stage pharmaceutical company that is leading the advance of a revolutionary new unifying theory of aging and degeneration that can result in dramatically new approaches to therapy. It is creating a new category of drugs composed of proprietary compounds that are chemically stabilized forms of essential nutrients that can treat degenerative diseases. The first indication is for the treatment of Friedreich's ataxia, a fatal orphan disease with a disease mechanism common to many other diseases of aging and degeneration. For more information about Retrotope, please visit [www.retrotope.com](http://www.retrotope.com).

**About Friedreich's ataxia (FA):** FA is a debilitating, life-shortening degenerative neuro-muscular disorder that affects approximately 6,000 people in the United States. Onset of symptoms can vary from five years old to adulthood, with the childhood onset tending to be associated with a more rapid disease progression. A progressive loss of coordination and muscle strength leads to motor incapacitation, the full-time use of a wheelchair, and ultimately early death from cardiac complications. Most young people diagnosed with FA require mobility aids such as a cane, walker or wheelchair by their teens or early 20's. There are currently no approved treatments for FA.

**About FARA:** The Friedreich's Ataxia Research Alliance (FARA) is a national, public, 501(c)(3), non-profit, tax exempt organization dedicated to curing Friedreich's ataxia (FA), a rare neuromuscular disorder, through research. For more information about FA, visit the Friedreich's Ataxia Research Alliance (FARA) website at [www.curefa.org](http://www.curefa.org).

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