RETROTOPE

Reinforcing the molecules of life

Retrotope advances RT001 in clinical trials to treat Friedreich's ataxia

Announces open enrollment of highest dose cohort after safety review

LOS ALTOS, CA, February 19, 2016 – Retrotope announced today the successful completion of the first dose cohort and the opening of patient enrollment for the highest dose cohort in its ongoing 28-day study of orally dosed RT001 in Friedreich's ataxia (FA) patients. RT001 was well tolerated and no serious adverse events or dose limiting toxicities were observed.

The RT001 is a chemically stabilized form of a natural fatty acid that confers resistance to lipid peroxidation in mitochondrial and cellular membranes via a novel mechanism. In FA, free iron is a catalyst for lipid peroxidation of exactly the type that can be mitigated with our drug, RT001. FA is a debilitating, life-shortening neuro-degenerative disorder that affects approximately 6,000 people in the United States. 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. There are currently no approved treatments for FA.

Robert De Jager, M.D., Chief Medical Officer of Retrotope commented: *"We are very pleased that RT001 appears to be safe and well tolerated in this ongoing first-in-human study in FA patients. Primary endpoints are safety, tolerability, and the pharmacokinetic profile of orally dosed RT001. Secondary endpoints are the disease-related Friedreich's Ataxia Rating Scale neurological score, the timed 25-foot walk and various exploratory measures and biomarkers."*

Jennifer Farmer, MS, CGC, Executive Director at the Friedreich's Ataxia Research Alliance (FARA) said "*FARA is pleased to continue our support of Retrotope and this clinical study of a new approach to treat FA. We are excited by the potential of RT001 and we are grateful to the individuals who have volunteered for this study*."

Retrotope is conducting the study at two sites: the University of South Florida Ataxia Research Center, and Collaborative NeuroSciences Network in Long Beach, California. FARA will utilize its Patient Registry to assist in recruitment. For more information on this study, please visit: https://clinicaltrials.gov/ct2/show/NCT02445794.

About RT001: Retrotope has discovered that lipid peroxidation, the free-radical degradation of lipids in mitochondrial and cellular membranes, may be causative of a wide range of degenerative diseases. Free radicals attack and degrade polyunsaturated fats (PUFAs) that are essential membrane components. Retrotope and others have shown that the degradation products of these fats create toxic cascades that have been associated with many illnesses including Parkinson's, Alzheimer's, retinopathies and FA. RT001 is a patented, orally available modified fatty-acid therapeutic that is expected to stabilize ("fireproof") mitochondrial and cellular membranes against further attack and to restore cellular health.

About Retrotope: Retrotope, a privately held clinical-stage pharmaceutical company, is creating a new category of drugs to treat degenerative diseases. Composed of proprietary compounds that are chemically stabilized forms of essential nutrients, these compounds are being developed as disease modifying therapies for many intractable diseases, such as Parkinson's, Alzheimer's and retinopathies.

The first indication for RT001, Retrotope's lead candidate, is the treatment of Friedreich's ataxia, a fatal orphan disease. For more information about Retrotope, please visit www.retrotope.com.

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

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