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Retrotope announces Phase I/II Clinical Trial Results of RT001 in Treatment of Friedreich's ataxia

First-in-human trial achieves safety, tolerability, and PK goals, with early signals of efficacy

LOS ALTOS, CA, September 15, 2016 – Dr. Theresa Zesiewicz, principal investigator in Retrotope's first-in-human clinical trial of RT001 in Friedreich's ataxia (FA), today presented early results from Retrotope's Phase I/II trial conducted at the University of South Florida Ataxia Research Center and the Collaborative NeuroSciences Network in Long Beach, CA. The trial, a randomized, double-blind, comparator controlled, two-dose study of RT001 in 18 FA patients for 28 days, met all of its primary safety, tolerability and pharmacodynamics (PK) goals. While biological activity was not a primary goal of the study, a number of clinically important activity measures were tested, found to be highly correlated to well-studied disease severity scales and showed multiple, unexpected, robust signals of drug effect at one or more doses.

Dr. Theresa Zesiewicz, principal investigator of the study, said, "It is impressive that multiple efficacy indications moved in the right direction in only 28 days in such a small study. This drug clearly deserves immediate further study in FA, which results in a slow, but steady decline in muscular and neurological function."

Curtis Scribner, MD, CMO of Retrotope commented: "This study met and exceeded all of its goals. The trial creates a profile of an extremely well-behaved drug: safe, well-tolerated at high doses, and rapidly adsorbed to target levels determined in preclinical studies."

Retrotope was also able to identify its maximum tolerated dose level (MTD) of RT001 due to one severe adverse event at the highest dose (9g/day), uncontrolled diarrhea, experienced by a subject with very low body mass index (BMI). This is a common complication of high fish oil dosing in hypercholesterolemia. Other adverse events were either very mild or not drug related. Fatty acid metabolites of RT001 were also detected in multiple blood compartments demonstrating the drug participated in normal fatty acid processing.

Retrotope is announcing an extension study in which the same patients which will re-randomize into treatment and comparator arms for 6 months. The goals of this study will be to refine dose determination under a relaxed (from the original study) low polyunsaturated fatty acid (PUFA) diet, establish longer term durability of clinical effects, and longer term safety. For more information on the current study, please visit: https://clinicaltrials.gov/ct2/show/NCT02445794

FA is a debilitating, life-shortening neuro-degenerative disorder that affects approximately 5,000 people in the United States, and over 20,000 people worldwide. A progressive loss of coordination and muscle strength leads to motor incapacitation, the full-time use of a wheelchair, and ultimately early death, typically from cardiomyopathy. There is currently no approved treatment for FA. Earlier this year, the FDA granted Retrotope Orphan Drug Designation for RT001 in FA.

Bob Molinari, Ph.D., CEO of Retrotope said: "This study would not have been possible without the support and guidance provided by the Friedreich's Ataxia Research Alliance (FARA) and the clinicians in its network, particularly Dr. Rob Wilson at the University of Pennsylvania Perlman School of Medicine, Dr. Susan Perlman at UCLA, and importantly, Jen Farmer and her team at FARA. They tirelessly assisted in organizing patient recruitment, paid large parts of patients' travel costs to trial sites, and consulted with us on all aspects of trial design. This organization makes FA trials possible and successful."

About RT001
RT001 is a patented, orally available modified fatty-acid therapeutic that stabilizes ("fireproofs") mitochondrial and cellular membranes against attack and restores cellular health. Retrotope and others have discovered that lipid peroxidation, the free-radical degradation of lipids in mitochondrial and cellular membranes, may be causative of a wide range of degenerative disease phenotypes. Free radicals attack and degrade polyunsaturated fatty acids (PUFAs) that are essential cellular membrane components. The degradation products of these fats then create toxic cascades that have been associated with many illnesses of degeneration, and particularly ones with mitochondrial lipid damage.

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 studied as disease modifying therapies for many intractable diseases such as Parkinson's, Alzheimer's, mitochondrial myopathies, and retinopathies. RT001, Retrotope's first lead candidate, is for 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 (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 FARA website at www.curefa.org.

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