Retrotope Announces Peer-Reviewed Publication of Positive Phase 1b/2a Findings for RT001 in Friedreich’s Ataxia

Protocol for Pivotal Clinical Trial Has Been Submitted to the US FDA

LOS ALTOS, Calif., April 18, 2018 (GLOBE NEWSWIRE) -- Retrotope announced today peer-reviewed publication of positive Phase 1b/2a trial results for the company’s lead candidate, RT001, in patients with Friedreich’s ataxia (FA). RT001 is the first in class of a new category of drugs called D-PUFAs (deuterated polyunsaturated fatty acids), which are designed to protect against free radical damage resulting in cell death that is a hallmark of several degenerative diseases, including FA. In an article titled, “Randomized, Clinical Trial of RT001: Early Signals of Efficacy in Friedreich’s Ataxia” (DOI:10.1002/mds.27353), appearing online in Movement Disorders, results of the randomized, double-blind, comparator-controlled Phase 1b/2a trial demonstrated early signals of drug effect (including statistically significant improvements in peak exercise workload compared to placebo) and positive safety and tolerability.

Theresa Zesiewicz, M.D., FAAN, Director of the University of South Florida Ataxia Research Center and principal investigator of the Phase 1b/2a study, commented, “These are the first findings published in a peer-reviewed journal demonstrating that a D-PUFA can show both safety and early indications of possible efficacy over a short treatment window of 28 days in patients with a progressive neurodegenerative disease such as FA. While biological activity was not a primary goal of the study, we are encouraged by the study results and look forward to further progress of the program.”

The randomized, double-blind, placebo-controlled, two-dose Phase 1b/2a study met all primary safety, tolerability, and pharmacokinetic (PK) goals in 18 patients who completed the 28-day period of treatment. Patients were randomized 2:1 (RT001:placebo) in either a low dose or high dose cohort. RT001 was found to be safe and tolerable, with plasma levels approaching saturation by 28 days. An additional subject (#19) with a low body mass index experienced steatorrhea taking the highest dose and discontinued the study. This is a common complication of high polyunsaturated fatty acid dosing, for example, hypercholesterolemia, and self-resolved in several hours. Other adverse events during the study were either very mild or not drug related. Fatty acid metabolites of RT001 were also detected and demonstrate that the drug participated in normal fatty acid processing. For the 18 patients who completed the study, there was an improvement in peak workload during cardiopulmonary exercise testing (CPET) in the drug group compared to placebo (0.16 watts/kg; p = 0.008), as well as improvement trends in peak oxygen consumption and in stride speed.
Peter G. Milner, M.D., Retrotope’s Chief Medical Officer, stated, “The Phase 1b/2a trial provides an early signal that RT001 may be able to address one of the most important concerns of FA patients, namely, the ability to generate additional energy during exercise and avoid the profound fatigue in performing most tasks. Based on these findings and additional positive results from the trial, we intend to move RT001 forward in this disease and have submitted a pivotal study protocol to the US FDA for review.”

The company also announced publication of a letter titled, “Strong Correlations Among Four Measures of Disease Progression in Friedreich’s Ataxia” (DOI:10.1002/mds.27351), appearing online in Movement Disorders. The letter reported on the high correlation of CPET measures with other well-studied disease severity measures during the Phase 1b/2a trial, and supports using CPET as a primary endpoint in larger, longitudinal studies of FA.

**About Friedreich’s Ataxia (FA)**
FA is a debilitating, life-shortening neurodegenerative disorder that affects approximately 5,000 people in the United States, and over 30,000 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. In 2017, the FDA granted Retrotope Orphan Drug Designation for RT001 in FA. Retrotope is greatly indebted to the Friedreich’s Ataxia Research Alliance (FARA) for its support in all aspects of its clinical trial design and execution, including recruiting and financial support for patients’ travel costs to participate in the trial.

**About RT001**
RT001 is a patented, first-in-class, orally available D-PUFA, a deuterated polyunsaturated fatty acid, that incorporates into mitochondrial and cellular membranes and stabilizes them. Retrotope and others have discovered that lipid peroxidation, the free-radical damage of polyunsaturated fats (PUFAs) in mitochondrial and cellular membranes, may be the primary source of cell death in several degenerative diseases, including FA and Infantile Neuroaxonal Dystrophy (INAD). The presence of D-PUFAs (RT001) can help protect (“fireproof”) against this attack and potentially 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 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 being tested in clinical trials for the treatment of Friedreich’s ataxia, a fatal orphan disease, and in compassionate use studies for a fatal, childhood neurodegenerative disease, Infantile Neuroaxonal Dystrophy. For more information about Retrotope, please visit www.retrotope.com.

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