Retrotope Expands its Drug Pipeline with the First Dosing of RT001 in patients with Friedrech’s ataxia (FA)

Three clinical trials centers now open for enrollment; two additional ones initiating

LOS ALTOS, Calif., January 9, 2020 -- Retrotope announced today that it has dosed its first patient in a Phase 2/3 clinical trial of RT001 in Friedrech’s ataxia, the most common of the inherited ataxias. RT001, a stabilized fatty acid drug, has been shown to reduce lipid peroxidation leading to cell death in patients across a wide swath of degenerative diseases, including FA. More information and a list of participating clinical sites for the current trial in FA can be obtained at ClinicalTrials.gov http://bit.ly/2T2FWA5. With this trial, the company expands its pipeline of indications for RT001 which is currently being studied in a pivotal trial in another fatal, neurodegenerative disease, Infantile Neuroaxonal Dystrophy (INAD), which is fully enrolled.

Retrotope previously demonstrated safety and an early signal of efficacy in a small, randomized, placebo-controlled trial in FA http://bit.ly/2QQk8Fi. In that study, subjects on drug outperformed those on placebo in generating peak workload in a controlled cardiopulmonary exercise test (CPET). After only a month of dosing, other measures of disease progression showed trends towards improvement in the Friedrech’s Ataxia Rating Scale, Neurological exam (FARS-Neuro), in an electronically-timed walk, and in maximum oxygen consumption during CPET. RT001 has been granted U.S. orphan drug designation for the treatment of Friedrech’s ataxia by the FDA.

“This Phase 2/3 clinical trial is an important milestone in the development pathway of RT001” commented Peter G Milner, MD of Retrotope. “We believe the CPET is a very sensitive probe of the mitochondrial function and stamina of patients with this disease who suffer from profound fatigue due to mitochondrial neuropathy, myopathy and cardiomyopathy. FDA has agreed with us that CPET may be a primary endpoint in a marketing approval study, and will be evaluated for approval with other secondary, supportive efficacy measures and validated scales important to patient function.”

Jennifer Farmer, Chief Executive Officer, Friedrech’s Ataxia Research Alliance (FARA) commented, “Friedrech’s ataxia is a progressive and debilitating disease for which there are no approved treatments, thus there is high unmet need for individuals and families diagnosed with FA. We are excited to see RT001 advance to a phase 2/3 efficacy study, and we are actively working to assist in patient recruitment efforts to ensure the study enrolls efficiently and meets its enrollment goals. FARA has been committed to creating and sharing the research community resources such as patient registries, natural history data, and validated clinical outcome
measures along with fostering patient engagement in research in order to assist Retrotope and other drug development partners in advancing clinical development programs.”

About Friedreich’s ataxia
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.

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. 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 a fatal, childhood neurodegenerative disease called Infantile Neuroaxonal Dystrophy. Expanded Access trials calibrating endpoint effects of RT001 in ALS, PSP Parkinsonism, and Huntington’s disease, and others are also underway. For more information about Retrotope, please visit www.retrotope.com.

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