



RETROTOPE Retrotope Receives FDA Clearance to Commence RT001 Phase 2/3 Clinical Trial in Patients with Infantile Neuroaxonal Dystrophy (INAD)

Patient enrollment anticipated to start in July 2018

LOS ALTOS, Calif., June 28, 2018 (GLOBE NEWSWIRE) -- Retrotope announced today that the U.S. Food and Drug Administration (FDA) has granted the company approval to conduct an open-label Phase 2/3 clinical trial of its investigational drug RT001 to evaluate efficacy and safety in patients with infantile neuroaxonal dystrophy (INAD). 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 numerous neurodegenerative diseases including INAD. To date, Retrotope has enrolled two INAD patients in two separate Expanded Access trials, the first patient having begun treatment in March 2017 and the second patient having enrolled in November 2017. Promising results from the first study were presented at the recent annual meeting of the American Academy of Neurology, and treatment of both children remains ongoing. RT001 has been granted U.S. FDA orphan drug designation for the treatment of PLA2G6 associated neurodegeneration (PLAN), which includes INAD.

“The Phase 2/3 clinical trial is an important milestone in the development pathway of RT001 for the treatment of INAD,” commented Robert Molinari, Ph.D., CEO of Retrotope. “Preclinical models suggested that RT001 could reduce high levels of lipid peroxidation induced cell death in PLA2G6 deficient *Drosophila* and human fibroblast models. These findings have been further supported by the encouraging results we have seen in two children currently receiving Expanded Access treatment. We look forward to the start of the trial and expect to begin patient enrollment in the next month.”

The RT001 Phase 2/3 clinical trial will enroll patients between the ages of 18 months and 10 years old with INAD who are not dependent on a ventilator for mechanical respiration (CPAP use acceptable). The primary endpoint of the study is to define the effectiveness of treatment with RT001 in subjects with INAD as measured via a quantitative scale of both Activities of Daily Living (ADLs) and deficits in vital functions affecting INAD children. Secondary objectives include how RT001 treatment affects standardized scores in a variety of childhood developmental milestone scales, as well as evaluations of the safety, PK, and tolerability of RT001 in INAD subjects. The study will be conducted at two major medical centers in the United States, but families living abroad are welcome to travel to the U.S. for participation. It is estimated that the study centers will be able to start accepting patients as early as July 2018.

Leena Panwala, President and Co-founder of INADcure (www.inadcure.org), noted, “INAD is a devastating disease for which there is currently no treatment. We are excited that RT001 will move forward in formal clinical testing so that its effects can continue to be more thoroughly investigated.” The INADcure Foundation is a 501(c)(3) nonprofit charity whose mission is to support the development of treatments, including a cure, for Infantile Neuroaxonal Dystrophy (INAD) and other forms of PLA2G6-related neurodegeneration (PLAN).

Peter G. Milner, MD, Retrotope’s Chief Medical Officer, commented, “We are grateful for the FDA’s clearance to start our INAD study. To the best of our knowledge this trial will be the first and only interventional clinical trial available to children with classic INAD. We are excited to be able to offer hope to families for a therapy that could potentially be available in their children’s lifetime.”

For more information please visit <https://bit.ly/2N5r1JF> at clinicaltrials.gov.

About INAD

INAD is an ultra-rare, devastating life-shortening neurodegenerative disorder that affects only a few hundred patients worldwide. It is caused by a genetic defect in the PLA2G6 housekeeping gene that removes damaged lipids from cells. Infants with INAD appear to develop normally until approximately 6 to 18 months of age, when they begin to experience progressive mental and psychomotor development declines as they lose developmental milestones. Later-stage disease typically involves a feeding tube and breathing via ventilator. Life-threatening complications typically develop by the end of the first decade.

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 Friedreich’s Ataxia (FA) and 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, INAD, ALS, Friedreich’s ataxia (FA), Late Onset Tay Sachs (LOTS), Familial Encephalopathy with Neuroserpin Inclusion Bodies (FEIN or neuroserpinosis), mitochondrial myopathies, and retinopathies. RT001, Retrotope’s first lead candidate, is being tested in clinical trials for the treatment of FA, a fatal orphan disease, and in compassionate use studies for the fatal, neurodegenerative diseases such as INAD, LOTS,

FEIN, and a genetic form of Alzheimer's disease. For more information about Retrotope, please visit www.retrotope.com.

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