RETROTOPE

Reinforcing the molecules of life

US FDA Grants Orphan Drug Designation for Retrotope's RT001 in the Treatment of Phospholipase 2G6 (PLA2G6)-Associated Neurodegeneration

LOS ALTOS, CALIF, November 2, 2017 – Retrotope announced today that the U.S. Food and Drug Administration's (FDA's) Office of Orphan Products Development (OOPD) granted orphan drug designation for its chemically-modified polyunsaturated fatty acid drug (RT001) for the treatment of PLA2G6 associated neurodegeneration (PLAN). Physicians collaborating with Retrotope have previously received approval from the FDA's Division of Neurology Products to test RT001 in Expanded Access trials of two patients having Infantile Neuroaxonal Dystrophy (INAD), a severe childhood PLA2G6-induced neurodegeneration. These trials are underway.

Robert Molinari, Ph.D. CEO of Retrotope commented: "We want to sincerely thank the FDA's OOPD for granting us orphan status in an indication class (PLA2G6-associated neurodegeneration) broader than our original request (INAD) with a rapid 6 week turnaround in a background of a large backlog for such designations. We are also grateful to the researchers, patients and clinicians whose work contributed to the results supporting our filing to FDA for orphan status."

Peter Milner, MD, Acting Chief Medical Officer of Retrotope, added, "We applaud FDA's conclusion to grant orphan designation using an inherited enzyme defect to define the molecular basis of disease. We are energized and excited by the potential of RT001 in this whole class of indications encompassed by FDA's designation of PLA2G6-Associated Neurodegeneration, including INAD, non-infantile onset PLAN, and even PLA2G6 induced young-onset Parkinson's disease (YOPD).

About INAD

INAD is an ultra-rare, devastating life-shortening neuro-degenerative disorder that affects only a few hundred patients in the US. Infants with INAD appear to develop normally until approximately 14 to 18 months of age, when they begin to experience progressive mental and psychomotor development declines. RT001 is a chemically stabilized fatty acid drug that confers resistance to lipid peroxidation in mitochondrial and cellular membranes, a causative element of the disease. Life threatening complications typically develop by the end of the first decade, and there is no approved treatment.

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 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 has shown that the degradation products of these fats create toxic cascades in many illnesses of degeneration.

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, neuromuscular diseases, and retinopathies.

RT001, Retrotope's first lead candidate, is for the treatment of rare orphan neuromuscular diseases, and is being tested in clinical trials for Friedreich's ataxia and INAD. For more information about Retrotope, please visit www.retrotope.com.

INADcure Foundation: The INAD cure foundation seeks to provide support for families of affected children, and funds research for the characterization, treatment and cure for diseases caused by impaired PLA2G6 gene function. www.inadcure.org It is assisting Retrotope in its trials.

The Orphan Drug Act
The US FDA Orphan Drug Act (ODA) provides a drug with orphan designation for rare indications, that qualifies sponsoring companies for expedited treatment and other incentives during FDA approval.

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