Retrotope initiates second patient for Compassionate Use of RT001 for an ultra-rare genetic neurological disease, Infantile Neuroaxonal Dystrophy.

LOS ALTOS, CA, November 16, 2017—Retrotope announced today that it has enrolled a second subject in a compassionate use trial of the ultra-rare, neurological disease, Infantile Neuroaxonal Dystrophy (INAD).

Retrotope enrolled a first compassionate use patient in March of 2017. RT001 appears to have arrested progression in that patient, and has led to a re-acquisition of lost development milestones in this otherwise strictly progressive disease. The company hopes to confirm a halting of progression and re-acquisition of lost milestones in a second patient. Retrotope has received Orphan Drug designation for RT001 in Friedreich’s ataxia and recently in PLA2G6 associated neurodegeneration (PLAN), of which INAD and young onset Parkinson’s Disease (YOPD) are subsets. If the second patient trial shows RT001 is safe and successful in re-acquiring lost development milestones as an endpoint, the company will seek a meeting with the FDA to discuss a rapid development pathway of RT001 for INAD. INAD is currently 100% fatal in children by roughly age 10, and has no approved therapy.

Dr. Peter Milner, acting Chief Medical Officer of Retrotope, commented: “A drug that could slow progression, never mind restore lost milestones, in this relentlessly progressive disease could be an important breakthrough, especially if we see it repeated in this second patient.”

Dr. Robert Molinari, CEO of Retrotope adds: “In INAD patients, lipid peroxidation products cannot be readily eliminated due to mutations in the Phospholipase 2G6 (PLA2G6) enzyme activity. The presence of free iron in the brain, which accumulates in NBIA patients, is a potent catalyst of excessive lipid peroxidation. With RT001, a “fireproofed” form of a key membrane fat that is susceptible to lipid peroxidation, the toxic insult is rendered non-toxic to the cell.”

About INAD
INAD is an ultra-rare, devastating life-shortening neuro-degenerative disorder that affects only a few hundred patients in the US. 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 14 to 18 months of age, when they begin to experience progressive mental and psychomotor development declines. 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 D-PUFAs
RT001 is the first-in-class of a new category of drugs called D-PUFAs. They are patented, chemically stabilized fatty acids that confer resistance to lipid peroxidation in mitochondrial and cellular membranes, a causative element of INAD. Orally available D-PUFAs stabilize
(“fireproof”) mitochondrial and cellular membranes against attack and restore cellular health. Retrotope and others have discovered that lipid peroxidation, the free-radical degradation of fatty acids in mitochondrial and cellular membranes, may be the primary source of cell death in several degenerative diseases.

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 called Infantile Neuroaxonal Dystrophy. For more information about Retrotope, please visit www.retrotope.com.

# # #

Retrotope Media Contact
Rick Roose
415-202-4445
roi.roos@gmail.com

SOURCE: Retrotope, Inc.
4300 El Camino Real, Suite 201
Los Altos, CA 94022
650-575-7551