# US FDA Allows Trial to Proceed for Retrotope's RT001 in the Treatment of Progressive SupraNuclear Palsy (PSP).

**LOS ALTOS, CALIF, April 25, 2020**– Retrotope announced today that it received a "Study May Proceed" letter from US Food and Drug Administration (FDA) related to its recently filed Investigational New Drug (IND) application for the use of RT001 in the treatment of PSP. In addition, the FDA provided useful guidance and suggestions for detailed design elements of the trial. Retrotope filed this IND for a PSP Phase 2/3 trial after 3 patients treated by physicians collaborating with Retrotope in PSP Expanded Access protocols showed encouraging results after more than a year on drug. RT001 is being tested in two additional Phase 2/3 trials in Infantile Neuroaxonal Dystrophy (INAD) and Friedreich's ataxia (FA), and has accumulated an impressive safety record based on over 500 patient months of dosing in other controlled and open label trials.

PSP is a serious neurodegenerative disease that profoundly affects the quality and length of life in adults[1]. Patients are typically severely disabled within 3-5 years of disease onset. It affects an estimated 17,500 adults in the US. In addition to the motor deficits noted above, affected individuals frequently experience personality changes and cognitive impairment. Symptoms typically begin after age 60 but can begin earlier. The exact cause of PSP is unknown and is often misdiagnosed as Parkinson's disease due to the similarity of symptoms in the early stage of disease. The cause of PSP is not known, but it is a form of tauopathy, in which abnormal phosphorylation and accumulation of the protein tau in the mid brain leads to destruction of vital protein filaments in nerve cells, causing their death. A regionally specific increase in lipid peroxidation damage, the target of RT001, has been observed in PSP.

RT001 is a chemically stabilized fatty acid that confers resistance to lipid peroxidation in mitochondrial and cellular membranes via a novel mechanism. RT001 has been shown to decrease levels of lipid peroxidation in PSP patient mesenchymal stem cells, and restore mitochondrial structure and function to damaged cells. As RT001 is distributed as an essential fat throughout tissues in human, it is expected to lower the amount of lipid peroxidation, restore normal mitochondrial function and prevent mitochondrial cell death.

Robert Molinari, Ph.D. CEO of Retrotope commented: "We hope to get this trial started rapidly after the current pandemic fears wane sufficiently that healthcare institutions go back to treating patients for longer term conditions. We are grateful to the researchers, patients and clinicians whose work contributed to the results supporting our filing of an IND application to FDA in this terrible disease."

Peter Milner, MD, Chief Medical Officer of Retrotope, added, "PSP is a disease involving modification and dysfunction of tau protein. RT001's mechanism of action both lowers lipid peroxidation and prevents mitochondrial cell death of neurons, both of which are associated with disease onset and progression. Also RT001 may have a synergistic downstream benefit in the pathophysiology of PSP by normalizing tau homeostasis preventing accumulation and cross linking of phospho-tau in the mid brain."

**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, and now in PSP which is also fatal. Expanded Access trials calibrating endpoint effects of RT001 in ALS, PSP, Huntington's disease, and others are also underway. If you have already contacted Retrotope about this trial and have given us your permission, you will be contacted with updates as the planning for the trial proceeds. For more information about Retrotope, please visit www.retrotope.com.

# # #

[1] https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Fact-Sheets/Progressive-Supranuclear-Palsy-Fact-Sheet

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

Contacts:
For Media: Robert J Molinari, Ph.D. CEO, bob@retrotope.com 650 575 7551
For Patients: Sarah Endemann, Clinical Coordinator, sarah@retrotope.com 619 206 5944
For Expanded Access Policy: Go to https://www.retrotope.com/eap