Retrotope Initiates Project to Test RT001 in an Animal Model of Lung Damage to protect against COVID-19 impact

LOS ALTOS, CALIF, April 16, 2020 – Retrotope announced today that it has initiated experiments to determine if RT001, a drug that is able to downregulate pro-inflammatory fatty acid oxidation in a number of diseases, may provide benefits against COVID-19 initiated lung damage.

The mechanisms by which COVID-19 causes severe illness and death in the elderly and in patients with pre-existing comorbid conditions is well documented. Hence, even when treated with the current standard of care, the recovery rate for patients progressing onto ventilator support is low. The rationale for the high mortality rate remains unclear. Several theories abound, one of which points to an overly-stimulated immune system leading to long-term lung damage or death. Inflammation induced by lipid peroxidation may play a role in lung damage.

RT001 is a chemically stabilized fatty acid that resists oxidation in mitochondrial and cellular membranes as well as in atherosclerosis models via a novel, proven mechanism. Lipid peroxidation and its by-products are molecules that are believed to serve as signaling moieties for the immune system. Hence it is appropriate to test whether RT001, being developed as an oral drug for several intractable diseases of degeneration such as Infantile Neuroaxonal Dystrophy (INAD), ALS, Progressive SupraNuclear Palsy (PSP), and Friedreich’s ataxia (FA), has a beneficial effect by protecting against immune mediated tissue damage.

In the first test of the effect of RT001 on lung damage, rodents will be orally dosed with either RT001 or a non-active control. At the end of the drug loading period, the treated animals and controls will be challenged with a well-known pro-inflammatory toxin that induces inflammation, edema, pulmonary tissue damage, and cell death. A post treatment tissue evaluation will determine if RT001 is capable of ameliorating inflammatory lung injury. Because, as an essential fat, RT001 is distributed throughout tissues in humans, it is expected to fortify the lung and other tissues against inflammatory cytokine induced cell death.

Robert Molinari, Ph.D. CEO of Retrotope commented: “While we cannot predict to what extent this proposed mechanism will offset COVID-19 related death, it is a reasonable approach to mitigate disease progression or long-term disability in survivors with a safe drug candidate. RT001 uses a novel pathway to mitigate cell death, which differs mechanistically from current antiviral, vaccine, and other approaches, thus providing a potentially complementary (with other drugs) therapy for the treatment of COVID-19 affected patients. It is incumbent upon every company with compounds under testing, which can create useful hypotheses to assist during this world-wide public health crisis.”

Peter Milner, MD, Chief Medical Officer of Retrotope, added, “As we know from prior work that RT001 appears to be a very safe drug in over 50 patient-years of dosing in other indications, we believe the risk of trying it in concert with other more directed approaches is low. Should the animal experiments indicate positive results, we will seek immediate regulatory approvals to begin human trials.”
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 trigger inflammation and other pathways leading to cell death in several degenerative diseases. The presence of D-PUFAs (RT001) can help protect (“fireproof”) against this attack at the cellular level.

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. For more information about Retrotope, please visit www.retrotope.com.

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