



*Reinforcing the molecules of life*

## US FDA Grants Orphan Drug Designation for Retrotope's RT001 in the treatment of Friedreich's ataxia

*First-in-human clinical trial for RT001 is now fully enrolled*

**LOS ALTOS, CA, June 1, 2016** – Retrotope announced today that the U.S. Food and Drug Administration (FDA) Office of Orphan Products Development granted orphan drug designation for its stabilized fatty acid drug (RT001) for the treatment of Friedreich's ataxia (FA). This follows the recent announcement that RT001 was well tolerated with no serious adverse events or dose limiting toxicities in the first cohort of its Phase 1/2 clinical trial in FA patients.

FA is a debilitating, life-shortening neuro-degenerative disorder that affects approximately 5,000 people in the United States, and over 20,000 people worldwide. A progressive loss of coordination and muscle strength leads to motor incapacitation, the full-time use of a wheelchair, and ultimately early death, typically from cardiomyopathy. RT001 is a chemically stabilized form of a natural fatty acid that confers resistance to lipid peroxidation in mitochondrial and cellular membranes via a novel mechanism. There is currently no approved treatment for FA.

Robert Molinari, Ph.D. CEO of Retrotope commented: "We are pleased that FDA has granted us orphan status with the benefits that such status accrues to our program. We have been fortunate to have had the support of the Friedreich's Ataxia Research Alliance (FARA) since the inception of our clinical program for RT001. We are also grateful to the patients and clinicians who are participating in our first-in-human clinical trial of RT001 for this devastating condition."

Jennifer Farmer, MS, CGC, Executive Director of the Friedreich's Ataxia Research Alliance (FARA), said "FARA is pleased to continue our support of Retrotope. We utilized our Patient Registry to help recruit patients for the Phase 1 that is now fully enrolled. We look forward to a continued partnership as Retrotope's clinical development advances to further evaluate their new approach to treating FA. We are excited by the potential of RT001 and hope this new drug benefits FA patients."

Retrotope is conducting a study at two sites: the University of South Florida Ataxia Research Center and the Collaborative NeuroSciences Network in Long Beach, CA. The trial has recently been fully enrolled. For more information on this study, please visit: <https://clinicaltrials.gov/ct2/show/NCT02445794>.

### **About RT001**

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 and others have shown that the degradation products of these fats create toxic cascades that have been associated with many illnesses of degeneration, and particularly ones with mitochondrial lipid damage. RT001 is a patented, orally available modified fatty-acid therapeutic that stabilizes ("fireproofs") mitochondrial and cellular membranes against attack and restores 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 for the treatment of Friedreich's ataxia, a fatal orphan disease. For more information about Retrotope, please visit [www.retrotope.com](http://www.retrotope.com).

**About FARA:** The Friedreich's Ataxia Research Alliance (FARA) is a national, public, 501(c)(3), non-profit, tax exempt organization dedicated to curing Friedreich's ataxia (FA), a rare neuromuscular disorder, through research. For more information about FA, visit the FARA website at [www.curefa.org](http://www.curefa.org).

**About the Orphan Drug Act**

The US FDA Orphan Drug Act (ODA) provides orphan designation for drugs and biological products to treat a rare disease or condition upon request of a sponsor. Orphan drugs are usually defined as those products intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S., or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug. Orphan designation qualifies the sponsor of the drug for various development incentives of the ODA, including tax credits for qualified clinical testing. A marketing application for a prescription drug product that has received orphan designation is not subject to a prescription drug user fee unless the application includes an indication for other than the rare disease or condition for which the drug was designated.

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**Retrotope Media Contact**

Rick Roose, CoActive PR  
415-202-4445  
[rick@coactivepr.com](mailto:rick@coactivepr.com)

SOURCE: Retrotope, Inc.  
4300 El Camino Real, Suite 201  
Los Altos, CA 94022  
650-917-9256