

RETROTOPE

Poster Presentation, American Academy of Neurology

“RT001 to Treat Neurodegeneration – Case Study: Improvement in a Single Patient with Late Onset Tay-Sachs disease (LOTS)”

At the May 2019 American Academy of Neurology Scientific Sessions, Dr. Robert Molinari presented Retrotope’s poster presentation entitled: “RT001 to Treat Neurodegeneration--Case Study: Improvement in a Single Patient with Late Onset Tay-Sachs Disease (LOTS).

- Lipid peroxidation (LPO) is a self-propagating, free-radical chain reaction that is thought to play a role in the cause of LOTS.
- RT001 is a di-deuterated isotope of linoleic acid (D-LA) that is readily incorporated into membranes and prevents oxidative damage.
- RT001 was readily absorbed and incorporated into RBC membranes within 30 days
- Improvement trends were seen in activities of daily living, walk speed, cough, fine motor skills and falling were noted during the 45 weeks of drug administration.
- RT001 was well-tolerated without any toxicities reported during the exposure period.
- Studies of RT001 is being developed in multiple neurodegenerative conditions in which LPO is seen including Friedreich’s ataxia, infantile neuroaxonal dystrophy and amyotrophic lateral sclerosis. A larger trial in LOTS patients may be warranted.