The Department of Psychology & Psi Chi Club Presents

Examining Enzyme Replacement Therapy for Lysosomal Storage Diseases Using Human Neural Progenitor Cells

Tuesday, November 24th 3:00pm

Invited Speaker James Munoz, Ph.D.
Nova Southeastern University
Dr. Munoz's Website

Neuronal ceroid lipofuscinosis type 2 (CLN2) is an autosomal recessive, neurodegenerative lysosomal storage disorder due to a deficit of the metabolic enzyme tripeptidyl peptidase (TPP-1). Due to the lack of TPP-1, an aggregation of lysosomal waste leads to neurological complications and early death. Currently, a clinical trial of the experimental treatment Brineura® (cerilponase alfa) is the only approved treatment for CLN2. Cerilponase alfa is recombinant TPP-1, which is delivered directly to the brain via intracerebroventricular infusion. Diffusion models suggest TPP-1 will spread along a concentration gradient through the brain. We are examining proliferation, cell cycle kinetics, differentiation, and cell death in human neural stem/progenitor cells (hNPCs) following overexpression or exposure to human recombinant TPP-1. These results may have indications in clinical trials using intracerebroventricular infusion for enzyme replacement therapies and dosing.

Join Zoom Meeting
https://fau-edu.zoom.us/j/83754619902?pwd=d0JIam9RUFJncHZYUWV4T0xHSElFUT09

Meeting ID: 837 5461 9902
Passcode: 2f4X7e