

April 1, 2014

Research Progress Report: NNPDF Peter Pentchev Fellowship

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Date: October 2013 ~ March 2014

Lay Summary:

This project has focused on the evaluation of varied protein levels in cerebrospinal fluid from NPC1 patients compared to non-affected controls. We have carried out an exhaustive analytical study to validate the technique we are using to be able to incorporate it into our patient sample studies. To date, we have focused on increasing the number of patient samples analyzed to obtain the most comprehensive representation of differences within the disease and with and without pharmacological intervention. We have identified several proteins that are different in NPC1 patient samples compared to controls and these data represent novel findings and confirm the findings from previous studies.

Continued efforts will be focused on increasing the number of samples analyzed, validating results and investigating the potential utilizing these protein biomarkers in future studies