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**Research Progress Report: NNPDF Peter Pentchev Fellowship**

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**Sponsor: Forbes D. Porter**

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**Lay Summary:**

We have developed a method to analyze which proteins are altered in the cerebrospinal fluid from NPC1 patients compared to controls. This method is robust and is currently being used to evaluate a large number of patient samples. Our initial studies were to compare data from patients with NPC1 but not on miglustat. Now, we are currently focusing our efforts on evaluating patient samples in which the patient takes miglustat. Taken together, this data will provide us with the means to establish what proteins are altered in the disease and any differences that may be found with miglustat treatment.