



Division of Medical Genetics
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To: National Niemann Pick Disease Foundation
Re: Edward Schuchman Research Fellowship Progress Report
Period: October 1st – April 1st 2015

Lay Summary

We applied to the National Niemann-Pick Disease Foundation's Edward Schuchman Research Fellowship to fund a natural history clinical research study. Our goal was to not use the grant funds for salary support but to apply the funding to support patient care including MRI's, cardiology evaluations and laboratory testing. During the first few months of this funding period we have been able to apply to our clinical research unit so that we have resources necessary to examine patients. Now, our goals are to focus on seeing more patients to collect data from the medical evaluations. So far, we have examined 6 patients and we expect to reach our yearly recruitment goals. Future goals include the analysis of the relationship between genetic information and clinical course. We would also like to explore biomarkers that could be used to predict disease burden.

A handwritten signature in black ink, appearing to read "Melissa Wasserstein", written over a horizontal line.

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