A Study of Intrathecal Enzyme Therapy for Cognitive Decline in MPS I

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This is a 24-month study of the use of laronidase administered into the spinal fluid to treat cognitive decline in mucopolysaccharidosis I (MPS I). MPS I is a rare genetic condition due to deficiency of the enzyme alpha-l-iduronidase. Laronidase is the manufactured form of the enzyme alpha-l-iduronidase.

MPS I is a heterogeneous disease with several clinical phenotypes ranging from the most severe, Hurler syndrome, to the attenuated forms, Hurler-Scheie and Scheie. Although patients with milder forms of MPS I may not have grossly observable problems with cognition, these patients do have learning difficulties that are apparent in school and with neuropsychological testing. The goal of this study is to evaluate whether intrathecal recombinant human alpha-l-iduronidase (rhIDU) injections can stabilize or improve cognitive decline in individuals with MPS I.