Extension Study of Intrathecal Enzyme Replacement Therapy for MPS I

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This is a one-year extension study of the use of laronidase into the spinal fluid to treat spinal cord compression in mucopolysaccharidosis I. Mucopolysaccharidosis I is a rare genetic condition due to deficiency of the enzyme alpha-l-iduronidase. Spinal cord compression occurs in this condition due to accumulation of material called glycosaminoglycans (GAG). Laronidase is the manufactured form of the enzyme alpha-l-iduronidase that is deficient in mucopolysaccharidosis I patients. The aim of this study is to determine whether laronidase is safe and effective when given into the spinal fluid as a potential non-surgical treatment for spinal cord compression due to mucopolysaccharidosis I disease. Funding Source -- FDA OOPD