A Study in MPS VI to Assess Safety and Efficacy of Odiparcil

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Mucopolysaccharidoses (MPS) are a group of rare inherited disorders characterized by a deficiency of lysosomal enzymes responsible for the normal degradation of glycosaminoglycans (GAGs). Medical need for treatment of MPS is still very high due to the poor penetration of the recombinant enzymes into the blood brain barrier as well as the ocular barriers and into tissues that are poorly vascularized, such as cartilages and bones. Odiparcil is an orally active compound that allows the synthesis of soluble glycosaminoglycans (GAGs), mainly chondroitin sulfate (CS) and dermatane sulfate (DS). The neosynthesized solubles GAGs are then excreted in urine. By diverting endogenous GAG synthesis to the synthesis of soluble odiparcil linked GAGs, odiparcil should decrease the intracellular pool of GAGs and consequently decrease the lysosomal GAG accumulation.

The primary objective of the study is to assess the safety and efficacy of two doses of odiparcil in MPS VI patients and to provide evidence to enable the selection of the relevant dose of odiparcil for phase III study. The secondary objective of this study is to characterize the dose response, PK and PD of odiparcil.