

Safety and Dose Ranging Study of Human Insulin Receptor MAb-IDUA Fusion Protein in Adults and Children With MPS I

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AGT-181 is a fusion protein containing alpha-L-iduronidase that is intended to deliver the enzyme peripherally and to the brain, when administered intravenously. This is a safety and tolerability study to obtain safety and exposure data as well as information on the biological activity of the investigational drug.

This is a two-stage, sequential, single and multi-dose study of AGT-181 in patients with MPS I. The first stage will be an open-label, single-dose, dose-escalation cohort study and the second stage will be an open-label, multi dose, adaptive dose escalation cohort study.