

Ascending Dose Study of Genome Editing by the Zinc Finger Nuclease (ZFN) Therapeutic SB-913 in Subjects With MPS II

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The purpose of the study is to evaluate the safety, tolerability and effect on leukocyte and plasma Iduronate 2-Sulfatase (IDS) enzyme activity of ascending doses of SB-913. SB-913 is an intravenously delivered Zinc Finger Nuclease (ZFN) Therapeutic for genome editing. It inserts a correct copy of the IDS gene into the Albumin locus in hepatocytes with the goal of lifelong therapeutic production of the IDS enzyme.