

# **Ascending Dose Study of Genome Editing by the Zinc Finger Nuclease (ZFN) Therapeutic SB-318 in Subjects With MPS I**

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The purpose of the study is to evaluate the safety, tolerability of ascending doses of

SB-318. SB-318 is an intravenously delivered Zinc Finger Nuclease (ZFN) Therapeutic for genome editing. It inserts a correct copy of the  $\alpha$ -L-iduronidase (IDUA) gene into the Albumin locus in hepatocytes with the goal of lifelong therapeutic production of the IDUA enzyme.