A Double-Blind Study to Evaluate the Efficacy and Safety of BMN 110 in Patients With Mucopolysaccharidosis IVA (Morquio A Syndrome)

Oakland, California, United States
Wilmington, Delaware, United States
Washington, District of Columbia, United States
Chicago, Illinois, United States
New York, New York, United States
Seattle, Washington, United States
Cordoba, , Argentina
Campina Grande, , Brazil
Porto Alegre, , Brazil
Montreal, , Canada
Sherbrooke, , Canada
Toronto, , Canada
Bogota, , Colombia
Copenhagen, , Denmark
Lyon, , France
Paris, , France
Mainz, , Germany
Monza, , Italy
Tokyo, , Japan
Seoul, , Korea, Republic of
Amsterdam, , Netherlands
Coimbra, , Portugal
Doha, , Qatar
Riyadh, , Saudi Arabia
Taipei, , Taiwan
Birmingham, , United Kingdom
London, , United Kingdom
Manchester, , United Kingdom

This Phase 3 study will evaluate the efficacy and safety of 2.0 mg/kg/week BMN 110 and 2.0 mg/kg/every other week BMN 110 in patients with mucopolysaccharidosis IVA (Morquio A Syndrome).

There is currently no standard accepted treatment for MPS IVA other than supportive care. Enzyme replacement therapy (ERT) may be a potential new treatment option for MPS IVA patients. BMN 110 is administered to MPS IVA patients by IV infusion, allowing cellular uptake by the mannose-6-phosphate receptor and transportation to the lysosomes.

This enzyme uptake into the lysosomes is hypothesized to promote increased catabolism of keratan sulfate (KS) in tissue macrophages, hyaline cartilage, other connective tissues, and heart valve, and reduce the progressive accumulation of KS which is responsible for the clinical manifestations of the disorders.