Title: MOR-004: A Phase 3, Randomized, Double-Blind, Placebo-Controlled, Multinational Clinical Study to Evaluate the Efficacy and Safety of BMN 110 in Patients with Mucopolysaccharidosis IVA (Morquio A Syndrome)

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Principal Investigator: Paul Harmatz, MD

Synopsis:
This is a Phase 3, randomized, double-blind, placebo-controlled, multinational study in patients with Mucopolysaccharidosis IVA (Morquio A syndrome, MPS IVA). Approximately 162 patients (54 in each of the BMN 110 and placebo groups) will be enrolled at approximately 40 sites worldwide. CHRCO will enroll up to 20 patients.

MPS IVA is an inherited autosomal recessive disorder characterized by deficient activity of N-acetylgalactosamine-6-sulfatase (GALNS), resulting in macroscopic accumulation of the glycosaminoglycan (GAG) keratan sulfate (KS) in tissue macrophages, hyaline cartilage and other connective tissues, heart valve, and cornea as well as excretion in the urine. This accumulation causes multiple clinical manifestations including impaired functional capacity, endurance, and quality of life. There is currently no standard accepted treatment for MPS IVA other than supportive care. Enzyme replacement therapy (ERT) with BMN 110 (rhGALNS) may be a potential new treatment option for MPS IVA patients. BMN 110 is expected to reduce the progressive accumulation of KS and improve signs and symptoms of the disease.

This study will compare the effects and evaluate the efficacy and safety of 24 weeks of infusions of BMN 110 at doses of 2.0 mg/kg/week and 2.0 mg/kg/every other week (qow) with placebo in patients with mucopolysaccharidosis IVA (Morquio A Syndrome).

Primary objective:
• To evaluate the ability of 2.0 mg/kg/week BMN 110 and 2.0 mg/kg/qow BMN 110 compared with placebo to enhance endurance in patients with MPS IVA, as measured by an increase in the number of meters walked in the 6-minute walk (6MW) test from baseline to Week 24.

Secondary objectives:
• To evaluate the ability of 2.0 mg/kg/week BMN 110 and 2.0 mg/kg/qow BMN 110 compared with placebo to enhance endurance in patients with MPS IVA, as measured by an increase in the number of stairs climbed per minute in the 3-minute stair climb (3MSC) test from baseline to Week 24.
• To evaluate the ability of 2.0 mg/kg/week BMN 110 and 2.0 mg/kg/qow BMN 110 compared with placebo to reduce urine KS levels in patients with MPS IVA, as measured by a decrease in urine KS levels from baseline to Week 24.