JOHNS HOPKINS MEDICINE

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IONIS Website Post

A Phase 1-2, Double-Blind Sham-Controlled Multiple Ascending Dose Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Intrathecally-Administered ION440 in Patients with MECP2 Duplication Syndrome (ATTUNE)

Description: The study will assess the safety and tolerability of intrathecally-administered ION440 (an antisense oligonucleotide) in children and adults with MECP2 Duplication Syndrome. Participation in Part 1 is expected to last 42 weeks and involves six multi-day study visits (with in-person and remote components) at Kennedy Krieger Institute and Johns Hopkins, as well as two drug or sham administrations and one overnight hospital admission. The study drug is given as an injection into the spinal canal also called lumbar puncture or spinal tap. The sham will involve a lumbar puncture, but no drug will be injected. Participants who complete the first part of the study will have the opportunity to participate in Part 2, during which they will receive 13 additional cycles of study drug over 156 weeks.

Eligibility & Criteria

IRB #: IRB00459764

Principal Investigator: Constance Smith-Hicks, MD, PhD

Eligible Age Range: 2-65 years old

Gender: Males only

Diagnosis: Interstitial MECP2 duplication