

# ATTUNE: A Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Intrathecally-Administered ION440 in Participants With Methyl CpG Binding Protein 2 (MECP2) Duplication Syndrome (MDS)

NCT06430385

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Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Ionis Pharmaceuticals, Inc.
Enrollment	48 participants

## Key Eligibility Criteria

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### Inclusion (8)

- Males aged e 2 to d 65 years, depending on specific cohort and group, at the time of informed consent.
- Group A: e 8 to d 65 years old
- Group B: 2 to 7 years old, inclusive
- Participant has at least one parent or caregiver e 18 years old capable of providing informed consent and able to comply with all study requirements and activities.
- Participant has a documented diagnosis of MDS with genetic confirmation of MECP2 duplication.

... and 3 more (see full listing online)

### Exclusion (11)

- Documented diagnosis of severe MECP2 duplications including terminal duplication and/or translocation or MECP2 triplication OR clinical features associated with severe variant structure including (a) onset of seizures prior to age 5 (for those aged 5 and above at signing of ICF), (b) oxygen dependence, (c) microcephaly, IF MECP2 genetic structure information is unavailable.
- Clinically significant vital sign or ECG abnormality at Screening
- Known brain or spinal disease that would interfere with the LP procedure, or CSF circulation or presence of other factors would affect the safety of the LP procedure.
- Has any concomitant disease or condition or circumstance, or any finding at Screening that, in the opinion of the Investigator, makes the participant unsuitable for enrollment or that could interfere with the conduct of the study or that would pose an unacceptable risk to the participant in this study.
- Treatment with an investigational drug, biological agent, or device within 30 days of Screening, or 5 half-lives of investigational agent, whichever is longer.

... and 6 more (see full listing online)

## Locations (11 total)

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Rady Children's Hospital, San Diego, California, United States  
University of Colorado Hopsital - Anschutz Medical Campus, Aurora, Colorado, United States  
Kennedy Krieger, Baltimore, Maryland, United States

... and 8 more locations

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<https://clinicaltrials.gov/study/NCT06430385>

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