Donidalorsen Treatment in Children With Hereditary Angioedema

NCT07298447

Status	RECRUITING
Phase	Phase 3
Sponsor	Ionis Pharmaceuticals, Inc.
Enrollment	20 participants

Key Eligibility Criteria

Inclusion (5)

- Must be between the ages of 2 and less than 12 years, inclusive, at the time of informed consent and, as applicable, assent.
- Must weigh at least 9 kg at the time of informed consent and, as applicable, assent.
- Documented diagnosis of HAE-1/HAE-2 based upon both of the following:
- Documented clinical history consistent with HAE (SC or mucosal, non-pruritic swelling episodes without accompanying urticaria).
- Diagnostic testing results that confirm HAE-1/HAE-2: C1-inhibitor (C1-INH) functional level \<50% normal level AND complement factor C4 level below the lower limit of normal (LLN); OR a known pathogenic mutation in the SERPING1 gene.

Exclusion (3)

- Must not have any screening laboratory abnormalities or any other clinically significant abnormalities during screening that would render a participant unsuitable for inclusion.
- Must not have been treated with another investigational drug, biological agent, or device within 1 month of Screening, or 5 half-lives of investigational agent, whichever is longer.
- Concurrent diagnosis of any other type of recurrent angioedema, including idiopathic angioedema or HAE with normal C1-INH (HAE-nC1-INH or Type III).

Locations (3 total)

Ionis Investigative Site, Santa Monica, California, United States
Ionis Investigative Site, St Louis, Missouri, United States
Ionis Investigative Site, Cincinnati, Ohio, United States