A Phase 3 Study of Zodasiran in Adolescent and Adult Subjects With Homozygous Familial Hypercholesterolemia (YOSEMITE)

NCT07037771

Status	RECRUITING
Phase	Phase 3
Sponsor	Arrowhead Pharmaceuticals
Enrollment	60 participants

Key Eligibility Criteria

Inclusion (8)

- Age e12 years, non pregnant, non lactating, do not plan to become pregnant during the study
- Body weight e35 kg at Screening as patients could theoretically be \<35 kg as the study continues.
- HoFH based on a supportive genetic test or a clinical diagnosis (total cholesterol \>500 mg/dL\[13 mmol/L\] OR treated LDL-C concentration of e300 mg/dL \[e8 mmol/L\] either accompanied by TGs \<300 mg/dL \[3.4 mmol/L\] AND both parents with documented total cholesterol \>250 mg/dL \[6.5 mmol/L\] OR cutaneous or tendinous xanthoma before 10 years of age)
- LDL-C e70 mg/dL (1.8 mmol/L). For adolescents 12 to \<18 years of age, screening LDL-C e116 mg/dL (3 mmol/L).
- Hemoglobin A1c (HbA1c) d9.5%

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

Exclusion (7)

- Use of a hepatocyte-targeted siRNA within 365 days before Day 1 (except inclisiran, which is permitted; administration of inclisiran and study drug must be separated by at least 4 weeks)
- Use of an antisense oligonucleotide molecule within 3 months before Day 1 (except inclisiran, which is permitted; administration of inclisiran and study drug must be separated by at least 4 weeks)
- Use of evinacumab within 3 months before Day 1. Evinacumab use is prohibited during the study.
- Non-response to evinacumab, defined as LDL-C reduction \<15% from baseline after 2 doses
- Use of any other investigational agent or device within 30 days or 5 half-lives (whichever is longer) before Day 1

... and 2 more (see full listing online)

Locations (22 total)

Research Site 7, Park Ridge, Illinois, United States
Research Site 2, New York, New York, United States
Research Site 1, Cincinnati, Ohio, United States
... and 19 more locations