Study of ARO-DUX4 in Adult and Adolescent Patients With Facioscapulohumeral Muscular Dystrophy Type 1

NCT06131983

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Arrowhead Pharmaceuticals
Enrollment	60 participants

Key Eligibility Criteria

Inclusion (5)

- Genetically confirmed FSHD1 based on Screening evaluation or source verifiable medical record
- Clinical severity score between 3 and 8 (scale, 0 to 10)
- Must have eligible lower extremity muscle for biopsy as determined from MRI by a central reader
- A 12-lead electrocardiogram (ECG) at Screening with no abnormalities that may compromise participant's safety in the study
- Participants of childbearing potential and their partners must use highly effective contraception during the study and for at least 12 weeks following the end of study or last dose of study medication, whichever is later. Males must not donate sperm during the study from Day 1 until at least 12 weeks following the end of study or last dose of study medication, whichever is later.

Exclusion (8)

- Human Immunodeficiency Virus (HIV) infection as shown by presence of anti-HIV antibody (seropositive) at Screening
- Seropositive for hepatitis B (HBV) or hepatitis C (HCV) at Screening
- Uncontrolled hypertension
- Severe cardiovascular disease
- History of thrombolic events

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

Locations (17 total)

Research Site 2, Liverpool, New South Wales, Australia
Research Site 3, Auchenflower, Queensland, Australia
Research Site 1, Birtinya, Queensland, Australia
... and 14 more locations