

A Study to Evaluate the Safety of AB-1003 (Previously LION-101) in Subjects With Genetic Confirmation of LGMD2I/R9 (Part1)

NCT05230459

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	AskBio Inc
Enrollment	10 participants

Key Eligibility Criteria

Inclusion (4)

- Male and female subjects aged 18 and 65 years with clinical diagnosis of LGMD2I/R9 and confirmation of FKRP gene mutation.
- Ability to walk/run 10 meters in ≤ 30 seconds.
- Able to understand and comply with all study procedures.
- Sexually active females of childbearing potential and female and male partners of male subjects receiving study intervention must use a barrier method of contraception for the first 6 months after dosing.

Exclusion (13)

- Significant cardiomyopathy as defined by echocardiogram (left ventricular ejection fraction $\leq 40\%$), evidence of conduction defect (increased PR and RR intervals, left bundle branch block and QTcF ≥ 480 ms), NYHA Class 3 or 4 heart failure, or MRI gadolinium enhancement evidence of clinically important myocardial fibrosis.
- Contraindication to MRI or hypersensitivity to contrast dyes, shellfish or iodine.
- Implanted spinal rods, cardiac pacemaker or other implantation that would distort cardiac MRI images.
- History of active, ongoing chronic liver disease (e.g. hepatitis, HIV-related liver disease, hemochromatosis, steatosis, etc.) or abnormal liver function tests (abnormal GGT and/or abnormal total/direct bilirubin \geq upper limit of normal [ULN] and/or elevated AST and ALT ≥ 2 ULN).
- Abnormal renal function (GFR ≤ 60 ml/min, using the Modification of Diet in Renal Disease equation).

... and 8 more (see full listing online)

Locations (6 total)

University of California - Irvine, Irvine, California, United States
University of Iowa, Iowa City, Iowa, United States
University of Kansas Medical Center, Kansas City, Kansas, United States
... and 3 more locations