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

Status: RECRUITING

Eligibility Criteria

Age: 18 years to 65 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

1. Male and female subjects aged 18 and 65 years with clinical diagnosis of LGMD2I/R9 and confirmation of FKRP gene mutation. 2. Ability to ascend 4 stairs between 2.5 and 10 seconds. 3. Ability to walk/run 10 meters in \<30 seconds. 4. Able to understand and comply with all study procedures. 5. 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 Criteria:

1. Significant cardiomyopathy as defined by echocardiogram (left ventricular ejection fraction \<40%), evidence of conduction defect (increased PR and RR intervals, left bundle branch block and QTcF \>480m/sec), NYHA Class 3 or 4 heart failure, or MRI gadolinium enhancement evidence of clinically important myocardial fibrosis. 2. Contraindication to MRI or hypersensitivity to contrast dyes, shellfish or iodine. 3. Implanted spinal rods, cardiac pacemaker or other implantation that would distort cardiac MRI images. 4. 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 \>upper limit of normal \[ULN\] and/or elevated AST and ALT \>2 ULN). 5. Abnormal renal function (GFR \<60 ml/min, using the Modification of Diet in Renal Disease equation). 6. Any life-threatening disease, including malignant neoplasms and medical history or malignant neoplasms within the past 5 years prior to screening (except basal and squamous cell skin cancer). 7. In the opinion of the investigator, a pre-existing medical condition that predisposes the subject to risks that outweighs the potential benefits. 8. Requirement for daytime ventilatory support. 9. Change in glucocorticosteroid treatment within 3 months prior to screening visit. 10. Exposure to another investigational drug within 3 months prior to study treatment or any previous treatment with gene therapy. 11. Ongoing participation in any other therapeutic clinical trial. 12. Neutralizing antibody titer to AAV9 \>1:5. 13. Female subjects who are pregnant, plan to become pregnant in the next 12 months, or breastfeeding.

Conditions & Interventions

Interventions:
GENETIC: AB-1003 dose level 1, GENETIC: AB-1003 dose level 2, OTHER: Placebo
Conditions:
Limb Girdle Muscular Dystrophy, Limb-Girdle Muscular Dystrophy Type 2, LGMD2I, Muscular Dystrophy, LGMD2, LGMD, FKRP, FKRP Mutation, Fukutin Related Protein
Keywords:
gene therapy, LGMD2I, LGMD2I/R9, gene augmentation therapy, FKRP, fukutin related protein, FKRP mutation

More Information

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