Domagrozumab extension [TERMINATED]

Please note that Pfizer stopped the development of this drug after the Phase 2 failed to meet its primary endpoint.

Hub Summary

This study is designed to evaluate the safety and efficacy of Domagrozumaub, a myostatin inhibitor. Myostatin is a protein in the body which inhibits muscle growth and it is required to stop muscles from growing too large. It it thought that inhibiting myostatin may help preserve or improve muscle function in patients with DMD.

This is an open-label extension study of the Phase 2 study of Domagrozumaub - subjects are only eligible for this study if they were enrolled into and completed the phase 2 study.

Study Number: NCT02907619

Description by Pfizer

This study is an open-label extension to protocol B5161002 and will provide an assessment of the long-term safety, efficacy, pharmacodynamics and pharmacokinetics of intravenous dosing of PF 06252616 in boys with Duchenne muscular dystrophy. Approximately 105 eligible subjects will be assigned to receive a monthly individualised maximum tolerated dose based on their tolerability profile/data from B5161002. This study will not contain a placebo comparator. Subjects will undergo safety evaluations (Laboratory, cardiac monitoring, physical exams, x-ray, MRI), functional capacity evaluations (4 stair climb, range of motion, strength testing, Northstar Ambulatory Assessment, upper limb functional testing, six minute walk test and pulmonary function tests) and pharmacokinetic testing.

Primary Outcome Measures

- Incidence and/or rate of intolerability or dose limiting treatment related adverse events
- Incidence and/or rate, severity and causal relationship of treatment emergent adverse events (TEAEs) and withdrawals due to TEAEs.
- Incidence and magnitude of abnormal laboratory findings.
- Abnormal and clinically relevant changes in liver MRI and physical examinations.

Secondary Outcome Measures

- Mean change from baseline in functional capacity assessments
- Mean change from baseline in pulmonary function tests
- Mean change from baseline in muscle strength measured by myometry
- Pharmacokinetic: Trough serum concentrations for all subjects receiving active drug.
- Immunogenicity: Incidence of neutralising and anti-drug antibodies

Can I take part?

Inclusion Criteria

- Subjects with Duchenne muscular dystrophy who enrolled and completed study B5161002.
- Signed and dated informed consent document (ICD) indicating that the subject's parent or legal guardian/caregiver has been informed of all pertinent aspects of the study.
- Subjects and their legal guardians/caregivers who are willing and able to comply with scheduled visits, treatment plan, laboratory tests, and other study procedures.

Trial Status
Trial terminated

UK Locations

Trial Sponsor
Pfizer

Age
6 - 18

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
Yes

Phase
Phase 2 extension study

Length Of Participation
Up to 4 years

Recruitment Target
105

Ambulatory
Non-ambulant

Therapeutic Category
Myostatin inhibitor

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- Subject have;
    1. Adequate hepatic function on screening laboratory assessments
    2. GLDH less than 20 units/litre (2 x upper limit of normal [ULN])
    3. Iron content estimate on the liver MRI within the normal range.

Exclusion Criteria

- Unwilling or unable (eg, metal implants) to undergo examination with closed MRI.
- All male subjects who are able to father children and are sexually active and at risk for impregnating a female partner, who are unwilling or unable to use a highly effective method of contraception. In addition, all sexually active male subjects who are unwilling or unable to prevent potential transfer of and exposure to drug through semen to their partners by using a condom consistently and correctly. .
- Subjects who are investigational site staff members directly involved in the conduct of the study and their family members, site staff members otherwise supervised by the Investigator, or subjects who are related to Pfizer employees directly involved in the conduct of the study.
- Other severe acute or chronic medical or psychiatric condition or laboratory abnormality that may increase the risk associated with study participation.
- Participation in other studies involving investigational drug(s), with the exception of B5161002.
- History of allergic or anaphylactic reaction to a therapeutic or diagnostic protein or additives of this investigational product.

For contact details and to find out more, please refer to dmdhub.org.

Duchenne UK