Ataluren long-term

Study of Ataluren for previously treated patients with nmDBMD in Europe, Israel, Australia and Canada

Hub Summary

DMD is caused by a mutation in the gene which produces dystrophin. Dystrophin functions to maintain muscle structure and function. The loss of dystrophin in DMD leads to muscle weakness and loss of ambulation. A nonsense mutation is a specific type of mutation which is the cause of DMD in 10-15% of patients.

Ataluren is a drug designed to make the body's machinery less sensitive to nonsense mutations. This phase 3 trial is designed to assess the long-term safety and tolerability of Ataluren.

Study Number: NCT01557400

Description by PTC Therapeutics

Duchenne/Becker muscular dystrophy (DBMD) is a genetic disorder that develops in boys. It is caused by a mutation in the gene for dystrophin, a protein that is important for maintaining normal muscle structure and function. Loss of dystrophin causes muscle fragility that leads to weakness and loss of walking ability during childhood and teenage years. A specific type of mutation, called a nonsense (premature stop codon) mutation, is the cause of DBMD in approximately 10-15% of boys with the disease. Ataluren is an orally delivered, investigational drug that has the potential to overcome the effects of the nonsense mutation.

This study comprises a Phase 3, open-label study of ataluren in patients with nmDBMD who previously received ataluren at an investigator site in a prior PTC-sponsored clinical study. A separate open-label study (PTC124-GD-016-DMD) is being conducted for nmDBMD patients who previously received ataluren at an investigator site in the United States (US).

All participating sites must have had at least 1 patient that received ataluren treatment in a prior PTC-sponsored clinical study in DBMD. It is planned that up to ~96 patients will be enrolled.

Subjects will receive ataluren 3 times per day (TID) at respective morning, midday, and evening doses of 10 mg/kg, 10 mg/kg, and 20 mg/kg, for approximately 336 weeks. Study assessments will be performed at clinic visits during screening, on the first day of ataluren dosing, and then every48 weeks during the ataluren treatment period, except for weight, which will be measured every 24 weeks at a primary care physician (PCP). No measures of efficacy will be captured.

Primary Outcome Measures

- Long term Safety and Tolerability of Ataluren. [Time Frame: 336 weeks]

The primary objective of this study is to assess the long-term safety and tolerability of 10, 10, 20 mg /kg ataluren in patients with nmDBMD who had prior exposure to ataluren in a PTC-sponsored clinical trial. Safety profile characterized by type, frequency, severity, timing, and relationship to Ataluren of any adverse events or laboratory abnormalities.

Can I take part?

Inclusion Criteria

- Evidence of signed and dated informed consent/assent document(s) indicating that the subject (and/or his parent/legal guardian) has been informed of all pertinent aspects of the trial.
- Note: If the study candidate is considered a child under local regulation, a parent or legal guardian must provide written consent prior to initiation of study screening procedures and the study candidate may be required to provide written assent. The rules of the responsible Institutional Review Board/Independent Ethics Committee (IRB/IEC) regarding whether one or both parents must provide consent and the appropriate ages for obtaining consent and assent from the subject should be followed.
- History of exposure to ataluren in a prior PTC study in nmDBMD . Note: Patients are considered eligible only if they received ataluren during their participation in one or more prior PTC-sponsored studies of ataluren in nmDBMD. Note: Subjects who have participated in a prior or ongoing PTC study with ataluren in nmDBMD at a trial site in the US or Canada, but reside outside of the US and Canada, may be eligible for this study (with the approval of the PTC Therapeutics Medical Monitor).
- Male sex.
- In patients who are sexually active, willingness to abstain from sexual intercourse or employ a barrier or medical method of contraception during ataluren administration and the 6-week follow-up period.
- Willingness and ability to comply with scheduled visits, drug administration plan, study procedures, laboratory tests, and study restrictions. Note: Psychological, social, familial, or geographical factors that might preclude adequate study participation should be considered.

Trial Status
Trial complete

UK Locations
London - GOSH, Trial complete/terminated, Newcastle, Trial complete /terminated

Trial Sponsor
PTC Therapeutics

Phase
3

Recruitment Target
95

Ambulatory
Non-ambulant

Therapeutic Category
Stop-codon read through

Age
All

Mutation Specific
Mutation specific therapies, Nonsense mutations only

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

dmdhub.org

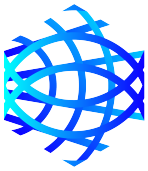
Exclusion Criteria

- Exposure to another investigational drug within 1 month prior to start of study treatment.
- Eligibility for another ataluren clinical trial that is actively enrolling study participants.
- Known hypersensitivity to any of the ingredients or excipients of ataluren (Litesse® UltraTM [refined polydextrose], polyethylene glycol 3350, Lutrol® micro F127 [poloxamer 407], mannitol 25C, crospovidone XL10, hydroxyethyl cellulose, vanilla, Cab-O-Sil® M5P [colloidal silica], magnesium stearate).
- Ongoing use of the following medications:

1. Coumarin-based anticoagulants (eg, warfarin), phenytoin, tolbutamide, or paclitaxel.
2. Systemic aminoglycoside therapy

- Ongoing uncontrolled medical/surgical condition, ECG findings, or laboratory abnormality that, in the investigator's opinion, could adversely affect the safety of the patient or make it unlikely that follow-up would be completed.

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



Duchenne UK

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