PTC Ataluren Phase 3

Phase 3 Extension study of Ataluren (PTC124) in Patients with Nonsense Mutation Dystrophinopathy

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 of Ataluren in boys with nonsense dystrophinopathies. The study will also assess changes in clinical measures such as muscle function and pulmonary function.

Study Number: NCT02090959

Description by PTC Therapeutics

The main goal of this Phase 3 extension study is to obtain long-term safety of ataluren in boys with nonsense mutation dystrophinopathy as determined by adverse events and laboratory abnormalities. The study will also assess changes in physical function, pulmonary function and other important clinical and laboratory measures.

This Phase 3, open-label safety and efficacy study will be performed at participating sites worldwide. The study will enrol ~ 220 boys with nonsense mutation dystrophinopathy who participated in a previous Phase 3 study of ataluren (Protocol # PTC124-GD-020-DMD). Patients will receive 10, 10, 20 mg/kg of ataluren TID at morning, midday, and evening for approximately 96 weeks. Study assessments will be performed at clinic visits every 12 weeks.

Primary Outcome Measures

Long-term safety of ataluren in boys with nonsense mutation dystrophinopathy, as determined by adverse events and laboratory abnormalities

Secondary Outcome Measures

- Physical Function: North Star Ambulatory Assessment, Timed Function Testing, Upper Limb Function, 6 Minute Walk Test
- Patient and/or parent-reported activities of daily living and disease symptoms
- Quality of Life
- Pulmonary function
- Ataluren blood levels

Can I take part?

Inclusion Criteria

- ✔ Completion of study treatment in the previous Phase 3, double-blind study protocol (Protocol PTC124-GD-020-DMD).
- ✔ Evidence of signed and dated informed consent/assent document(s) indicating that the patient (and/or his parent/legal guardian) has been informed of all pertinent aspects of the trial.
- ✔ Willingness to abstain from sexual intercourse or employ an approved method of contraception during the period of study drug administration and 6-week follow-up period.
- ✔ Willingness and ability to comply with scheduled visits, drug administration plan, study procedures, laboratory tests, and study restrictions.

Exclusion Criteria

- ✘ Known hypersensitivity to any of the ingredients or excipients of the study drug
- ✘ Ongoing participation in any clinical trial (except for studies specifically approved by PTC Therapeutics).
- ✘ Prior or ongoing medical condition (eg, concomitant illness, psychiatric condition, behavioral disorder, alcoholism, drug abuse), medical history, physical findings, ECG findings, or laboratory abnormality that, in the investigator's opinion, could adversely affect the safety of the subject, makes it unlikely that the course of treatment or follow-up would be completed, or could impair the assessment of study results.

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

Trial Status
Trial complete

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

Trial Sponsor
PTC Therapeutics

Phase
3

Length Of Participation
96 weeks

Recruitment Target
219

Ambulatory
Ambulant

Therapeutic Category
Stop-codon read through

Age
7-18

Mutation Specific
Mutation specific therapies, Nonsense mutations only

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

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