PTC - Ataluren (PTC124-041)

A Phase 3, Randomized, Double-blind, Placebo-controlled Efficacy and Safety Study of Ataluren in Patients With Nonsense Mutation Duchenne Muscular Dystrophy and Open-Label Extension

Summary

This study is a randomized, double-blind, placebo-controlled, 72-week study, followed by a 72-week open-label period. The purpose is to characterize the long-term effects of ataluren-mediated dystrophin restoration on disease progression.

Study Number: NCT03179631

Description by PTC Therapeutics, Inc.

This study is a randomized, double-blind, placebo-controlled, 72-week study, followed by a 72-week open-label period. The purpose is to characterize the long-term effects of ataluren-mediated dystrophin restoration on disease progression. Participants will be randomized in a 1:1 ratio to ataluren or placebo. Participants will receive blinded study drug three times daily (TID) at morning, midday, and evening for 72 weeks, after which all participants will receive open-label ataluren for an additional 72 weeks (144 weeks in total). Study assessments will be performed at clinic visits every 12 weeks during the double-blind period and every 24 weeks during the open-label period. The total sample size of ~250 subjects will include ~160 subjects who meet the criteria for inclusion in the primary analysis population (age 7 to 16 years old, baseline six minute walk distance (6MWD) greater than or equal to (>=) 300 meters, supine to stand >= 5 seconds). The study will be conducted in the United States and other countries around the world.

Primary Outcome Measures

- Slope of Change in 6-Minute Walk Distance (6MWD) Over 72 Weeks [Time Frame: 72 weeks]

Secondary Outcome Measures

- Change from Baseline to Week 72 in 6MWD [Time Frame: Baseline, Week 72]
- Change from Baseline to Week 72 in Time to Run/Walk 10 Meters [Time Frame: Baseline, Week 72]
- Change from Baseline to Week 72 in Time to Climb 4 Stairs [Time Frame: Baseline, Week 72]
- Change from Baseline to Week 72 in Time to Descend 4 Stairs [Time Frame: Baseline, Week 72]
- Change from Baseline to Week 72 in North Start Ambulatory Assessment (NSAA) Total Score [Time Frame: Baseline, Week 72]
- Time to Loss of Ambulation Over 72 Weeks [Time Frame: 72 weeks]
- Time to Loss of Stair-Climbing Over 72 Weeks [Time Frame: 72 Weeks]
- Time to Loss of Stair-Descending Over 72 Weeks [Time Frame: 72 weeks]
- Risk of Loss of NSAA Items Over 72 weeks [Time Frame: 72 weels]
- Number of Treatment-Emergent Adverse Events Considered Related to Study Drug [Time Frame: 72 weeks]

Can I take part?

Inclusion Criteria

- Males aged 5 years and older (≥5 years)
- Phenotypic evidence of Duchenne Muscular Dystrophy
- Nonsense point mutation in the dystrophin gene
- Use of systemic corticosteroids (prednisone/prednisolone or deflazacort)for a minimum of 12 months immediately prior to start of study treatment, with no significant change in dosage or dosing regimen for a minimum of 3 months immediately prior to start of study treatment
- 6-minute walking distance (6MWD) of greater than or equal to (≥) 150 meters
- Ability to perform timed function tests within 30 seconds
- Willingness and ability to comply with scheduled visits, drug administration plan, study procedures, laboratory tests, and study restrictions.

Exclusion Criteria

AUSNMD

Trial Status
Trial complete

Locations
Brisbane - Queensland Children's Hospital, Fully recruited, Melbourne - Melbourne Children's Campus, Trial complete/terminated, Perth - Children's Hospital, Fully recruited, Sydney - Westmead Children's Hospital, Fully recruited

Trial Sponsor
PTC Therapeutics, Inc.

Age
5 and above

Mutation Specific
Yes, Nonsense mutations only

Muscle Biopsy
No

MRI
Yes

Phase
3

Length Of Participation
144 weeks

Recruitment Target
250

Ambulatory
Yes

- Any change in prophylaxis treatment for cardiomyopathy within 1 month prior to start of study treatment.
- Ongoing intravenous (IV) aminoglycoside or IV vancomycin therapy.
- Prior or ongoing therapy with ataluren.
- Known hypersensitivity to any of the ingredients or excipients of the study drug
- Exposure to another investigational drug within 6 months prior to start of study treatment, or ongoing participation in any interventional clinical trial.
- History of major surgical procedure within 12 weeks prior to start of study treatment, or expectation of major surgical procedure during the 72-week placebo-controlled treatment period.
- Requirement for daytime ventilator assistance or any use of invasive mechanical ventilation via tracheostomy.
- Uncontrolled clinical symptoms and signs of congestive heart failure
- Elevated serum creatinine or cystatin C at screening.

Other inclusion/exclusion criteria apply.

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

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Therapeutic Category

Stop-codon read through