PLEASE NOTE: *This study has been imported from ClinicalTrials.gov without additional data checks.*

Trial Description

Title

A Phase 3 Extension Study of Ataluren (PTC124) in Patients With Nonsense Mutation Dystrophinopathy

Trial Acronym

[---]*

URL of the trial

[---]*

Brief Summary in Lay Language

Dystrophinopathy is a disease continuum that includes Duchenne muscular dystrophy, which 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. A specific type of mutation, called a nonsense (premature stop codon) mutation is the cause of dystrophinopathy 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. 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.

Brief Summary in Scientific Language

This Phase 3, open label safety and efficacy study will be performed at participating sites worldwide. The study will enroll ~ 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.

Organizational Data

- DRKS-ID: **DRKS00007237**
- Date of Registration in DRKS: **2015/05/07**
- Date of Registration in Partner Registry or other Primary Registry: **2014/03/17**
- Investigator Sponsored/Initiated Trial (IST/IIT): **no**
- Ethics Approval/Approval of the Ethics Committee: **[---]***
- (leading) Ethics Committee Nr.: **[---]***

Secondary IDs

- Primary Registry-ID: **NCT02090959 (ClinicalTrials.gov)**
- Sponsor-ID: **PTC124-GD-020e-DMD (PTC Therapeutics)**

Health condition or Problem studied

- Free text: **Muscular Dystrophy, Duchenne**
- Free text: **Muscular Dystrophies**
- Free text: **Muscular Disorders, Atrophic**
- Free text: **Muscular Diseases**
- Free text: **Musculoskeletal Diseases**
- Free text: **Neuromuscular Diseases**
- Free text: **Nervous System Diseases**
- Free text: **Genetic Diseases, X-Linked**
- Free text: **Genetic Diseases, Inborn**

Interventions/Observational Groups

- Arm 1: **Drug: Ataluren**

Characteristics

- Study Type: **Interventional**
- Study Type Non-Interventional: **[---]***
- Allocation: **Single arm study**
- Blinding: **[---]***
- Who is blinded: **[---]***
- Control: **Uncontrolled/Single arm**
- Purpose: **Treatment**
- Assignment: **Single (group)**
- Phase: **III**
- Off-label use (Zulassungsüberschreitende Anwendung eines Arzneimittels): **[---]***

Primary Outcome

- Long term safety of ataluren in boys with nonsense mutation dystrophinopathy, as determined by adverse events and laboratory abnormalities; time frame: Baseline and 96 weeks

Secondary Outcome

- Physical Function; time frame: Baseline and 96 weeks; 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; time frame: Baseline and 96 weeks
- Quality of Life; time frame: Baseline and 96 weeks
- Pulmonary function; time frame: Baseline and 96 weeks
- Ataluren blood levels; time frame: Baseline and 96 weeks

Countries of recruitment

- US **United States**
- AU **Australia**
- BE **Belgium**
- BR **Brazil**
- CA **Canada**
- CL **Chile**
- CZ **Czech Republic**
- FR **France**
- DE **Germany**
- IL **Israel**
- IT **Italy**

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- KR **Korea, Republic of**
- PL **Poland**
- ES **Spain**
- SE **Sweden**
- CH **Switzerland**
- TR **Turkey**
- UK **United Kingdom**

Locations of Recruitment

- **University Hospital Medical Center Freiburg, Freiburg**
- **University of Essen-Duisburg, Essen**

Recruitment

- Planned/Actual: **[---]***
- (Anticipated or Actual) Date of First Enrollment: **2014/03/31**
- Target Sample Size: **220**
- Monocenter/Multicenter trial: **Multicenter trial**
- National/International: **International**

Inclusion Criteria

- Gender: **Male**
- Minimum Age: **7 Years**
- Maximum Age: **18 Years**

Additional 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.

Addresses

■ **Primary Sponsor**

PTC Therapeutics

Telephone: **[---]***
Fax: **[---]***
E-mail: **[---]***
URL: **[---]***

■ **Contact for Scientific Queries**

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- **Contact for Public Queries**

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Sources of Monetary or Material Support

- **[---]***

Bitte wenden Sie sich an den Sponsor / Please refer to primary sponsor

Telephone: **[---]***
Fax: **[---]***
E-mail: **[---]***
URL: **[---]***

Status

- Recruitment Status: **Recruiting ongoing**
- Study Closing (LPLV): **[---]***

Trial Publications, Results and other documents

- Further trial documents **[---]***

The parameters in ClinicalTrials.gov and DRKS are not identical. Therefore the data import from ClinicalTrials.gov required adjustments. For full details please see the DRKS FAQs.

- Translation on version: 1

- Last processed date by ClinicalTrials.gov: 2014/11/05

* *This entry means the parameter is not applicable or has not been set.*

*** *This entry means that data is not displayed due to insufficient data privacy clearing.*