Patient Registry Translarna (Ataluren)

Long-term observational study of Translarna safety and effectiveness in usual care

Hub Summary

This phase 4 clinical study is designed to assess the safety of Translarna, also known at Ataluren. This study will follow patients who are receiving Translarna as part of their usual care for 5 years. At the patients usual visits, data will be collected to determine the safety and effectiveness of Translarna.

Study Number: NCT02369731

Description by PTC Therapeutics

This is a multicentre, observational study of patients receiving Translarna based on inclusion of their data in a registry. This study is intended to enrol approximately 200 patients across ~60 care centres in Europe and other regions over a period of ~ 2 years. The study population will include patients who are receiving usual care treatment with commercial supply of Translarna (or receiving care within a named patient early access program) and who provide consent. Patients will be followed for at least 5 years from their date of enrolment. Safety and efficacy data will be collected in conjunction with routine visits conducted as per usual care. Although there are no protocol-mandated procedures, it is expected that physicians and other caregivers will follow published treatment guidelines and standards of care.

Primary Outcome Measures

- Incidence of adverse events.

Other Outcome Measures

- Changes in laboratory parameters.
- Changes in blood pressure.
- Prescriber and patient compliance with prescribing information according to the approved labelling.

Can I take part?

Inclusion Criteria

- Receiving or will be receiving usual care treatment with commercial supply of Translarna (or receiving care within a named patient early access program)
- Willing to provide written informed consent to allow the study data collection procedures (either by the patient or through authorisation by a legal guardian)

Exclusion Criteria

- Patients who are receiving ataluren or placebo in a blinded, randomised clinical trial, or ataluren in any other ataluren clinical trial or cohort early access program that prevents participation in this study

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

Duchenne UK

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Trial Status
Fully recruited

UK Locations
London - Evelina, Fully recruited, London - GOSH, Fully recruited, Alder Hey, Fully recruited, Birmingham, Fully recruited, Bristol, Fully recruited, Cambridge, Fully recruited, Leeds, Fully recruited, Manchester, Fully recruited, Newcastle, Fully recruited

Trial Sponsor
PTC Therapeutics

Age
Child, adolescent and adult

Mutation Specific
Mutation specific therapies, Nonsense mutation

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

Phase
4

Length Of Participation
5 years

Recruitment Target
Approximately 200

Ambulatory
Ambulant and non-ambulant

Therapeutic Category
Ribosome targeting

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