Long-Term Observational study of Translarna Safety and Effectiveness in Usual Care (STRIDE)

First published: 13/10/2015

Last updated: 17/04/2025

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/48265

EU PAS number

EUPAS11275

Study ID

48265

DARWIN EU® study

No

Study countries

☐ Austria

- [ ] Brazil
- [ ] Czechia
- [ ] France
- [ ] Germany
- [ ] Greece
- [ ] Hungary
- [ ] Israel
- [ ] Italy
- [ ] Latvia
- [ ] Portugal
- [ ] Romania
- [ ] Slovenia
- [ ] Sweden
- [ ] United Kingdom

Study description

This is a multicenter, observational study of patients receiving Translarna. No study medication will be provided as part of this observational study: the treating physician will make all treatment decisions according to his or her usual practice and will provide prescriptions as appropriate. Patients will present to the study sites for scheduled routine clinic visits with no additional visits required for data collection in the study. Enrolled patients will be followed for at least 5 years from their date of enrollment, or until patient withdrawal of consent or death, whichever occurs first. Patients who discontinue treatment with Translarna will continue to be followed for the duration of the study unless they withdraw consent to participate in the study. Data will be collected during this time period in conjunction with all routine care visits, estimated to occur at 3-6 months intervals.

Study status

Ongoing

Research institutions and networks

Institutions

Fortrea - Real world Intelligence & Late Phase Solution

☐ Germany

☐ United Kingdom (Northern Ireland)

First published: 15/12/2015

Last updated: 31/10/2023

Institution | **Non-Pharmaceutical company** | **ENCePP partner**

Networks

TRiNDS

Contact details

Study institution contact

Shelley Johnson

Study contact

sjohnson@ptcbio.com

Primary lead investigator

Christine Keller

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 11/11/2014

Actual: 11/11/2014

Study start date
Planned: 30/03/2015

Actual: 30/03/2015

Data analysis start date
Planned: 23/04/2016

Actual: 23/04/2016

Date of interim report, if expected
Planned: 30/04/2023

Actual: 25/04/2023

Date of final study report
Planned: 30/10/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

PTC Therapeutics Int.

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Other study registration identification numbers and links

ClinicalTrials.gov Identifier: NCT02369731

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Effectiveness study (incl. comparative)

Main study objective:

The study is designed to evaluate the long-term safety and effectiveness, and utilization pattern of Translarna in real-world routine clinical practice.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Long-term observational registry

Study drug and medical condition

Name of medicine

TRANSLARNA

Medical condition to be studied

Duchenne muscular dystrophy gene carrier

Additional medical condition(s)

Nonsense mutation in the dystrophin gene

Population studied

Age groups

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Estimated number of subjects

360

Study design details

Outcomes

The objectives of the study are: • Obtain additional information on all safety concerns being tracked in the Risk • Management Plan and the long-term safety profile of Translarna • Obtain additional information on the long-term effectiveness of Translarna. • Monitor the utilization pattern of Translarna in usual care.

Data analysis plan

For any given safety event, the unadjusted incidence rate and exposure-adjusted event rate will be calculated, along with exact binomial confidence intervals. Further details of the analyses will be included in the SAP. Specific analyses on safety concerns with ataluren treatment will be performed when data available. The effectiveness of treatment with Translarna will be evaluated in the context of data were available: 6MWT, timed function tests, NSAA, PUL, cardiac and pulmonary functions. Data from these assessments will be summarized by visit. Changes in the relevant variable from baseline to each post-baseline visit will be summarized descriptively. Changes from baseline to each post-baseline visit will be analysed using paired t-tests. Results will be presented combined and stratified as appropriate. Subgroup analyses will be performed.

Documents

Study publications

Muntoni F, Desguerre I, Guglieri M, Osorio AN, Kirschner J, Tulinius M, Buccell…

Mercuri E, Muntoni F, Osorio AN, Tulinius M, Buccella F, Morgenroth LP, Gordish...

Data management

Data sources

Data sources (types)

Disease registry

Other

Data sources (types), other

Spontaneous reporting system, Prospective patient-based data collection, Prescription event monitoring

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No