

# 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

### EU PAS number

EUPAS11275

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### Study ID

48265

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### DARWIN EU® study

No

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### Study countries

 Austria

 Brazil

 Czechia

 France

-  Germany
  -  Greece
  -  Hungary
  -  Israel
  -  Italy
  -  Latvia
  -  Portugal
  -  Romania
  -  Slovenia
  -  Sweden
  -  United Kingdom
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### **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.

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### **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 [sjohnson@ptcbio.com](mailto:sjohnson@ptcbio.com)

**Study contact**

[sjohnson@ptcbio.com](mailto:sjohnson@ptcbio.com)

### Primary lead investigator

Christine Keller

## Study timelines

### **Date when funding contract was signed**

Planned: 11/11/2014

Actual: 11/11/2014

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### **Study start date**

Planned: 30/03/2015

Actual: 30/03/2015

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### **Data analysis start date**

Planned: 23/04/2016

Actual: 23/04/2016

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### **Date of interim report, if expected**

Planned: 30/04/2023

Actual: 25/04/2023

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### **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

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## 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

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#### **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

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## **Non-interventional study design, other**

Long-term observational registry

# Study drug and medical condition

## **Medicinal product name**

TRANSLARNA

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## **Medical condition to be studied**

Duchenne muscular dystrophy gene carrier

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## **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)
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## **Estimated number of subjects**

## 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.

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### 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...](#)

## Data management

### ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

## Data sources

### Data sources (types)

[Disease registry](#)

[Other](#)

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### 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

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**Check completeness**

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

No