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

**First published:** 13/10/2015

**Last updated:** 20/10/2026

Study Finalised

## Administrative details

**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**

Finalised

## 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**

Elena Heider eheider@ptcbio.com

**Study contact**

eheider@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

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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

Actual: 13/08/2026

## 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 topic:**

Human medicinal product

**Study type:**

Non-interventional study

**Scope of the study:**

Drug utilisation

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

**Data collection methods:**

Primary data collection

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**Study design:**

Multicenter long-term study

**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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**Study drug International non-proprietary name (INN) or common name**

ATALUREN

**Anatomical Therapeutic Chemical (ATC) code**

(M09AX03) ataluren

ataluren

**Medical condition to be studied**

Duchenne muscular dystrophy gene carrier

**Additional medical condition(s)**

Nonsense mutation in the dystrophin gene

## Population studied

**Short description of the study population**

Patients receiving Translarna

**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.

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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.

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**Summary results**

The results of the STRIDE registry are consistent with the known safety profile of Translarna in clinical studies
and postmarketing experience and confirm the favorable safety profile of Translarna in long-term routine clinical practice.

• The frequency and nature of treatment-emergent adverse events (TEAEs) were consistent with the established safety profile. No unexpected or new events signaling a safety concern were observed.

• Translarna was generally well tolerated with a favorable safety profile in 315

patients with nonsense mutation DMD (nmDMD) with a median exposure of 6.46 years and a total exposure of approximately 1918.41 patient-years.
• Few TEAEs were considered by the investigator to be related to Translarna.
• No deaths or serious adverse events (SAEs) were considered by the investigator to be related to Translarna.
• No clinically meaningful changes in laboratory test results, vital signs, or ECGs were observed.

## Documents

**Abstract of study report**

PTC124-GD-025-DMD Final CSR Synopsis - Redacted.pdf (145.9 KB)

**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

## 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 source(s), other**

STRIDE registry

**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