2. SYNOPSIS

Name of Sponsor/Company: PTC Therapeutics Inc. **Name of Finished Product:** Translarna **Name of Active Ingredient:** Ataluren (PTC124)	Individual Study Table Referring to Part of the Dossier Volume: Page:	*(For National Authority Use Only)*

Title of Study:
Long-Term Observational Study of Translarna Safety and Effectiveness in Usual Care

Study Center(s):
Multicenter (Austria, Brazil, Czech Republic, France, Germany, Greece, Hungary, Israel, Italy, Latvia, Portugal, Romania, Slovenia, Sweden, and the United Kingdom)

Publications (Reference):
Mercuri E, Muntoni F, Osorio AN, Tulinius M, Buccella F, Morgenroth LP, Gordish-Dressman H, Jiang J, Trifillis P, Zhu J, Kristensen A, Santos CL, Henricson EK, McDonald CM, Desguerre I; STRIDE; CINRG Duchenne Natural History Investigators. Safety and effectiveness of ataluren: comparison of results from the STRIDE Registry and CINRG DMD Natural History Study. J Comp Eff Res. 2020 Apr;9(5):341-360.

Muntoni F, Desguerre I, Guglieri M, Osorio AN, Kirschner J, Tulinius M, Buccella F, Elfring G, Werner C, Schilling T, Trifillis P, Zhang O, Delage A, Santos CL, Mercuri E. Ataluren use in patients with nonsense mutation Duchenne muscular dystrophy: patient demographics and characteristics from the STRIDE Registry. J Comp Eff Res. 2019 Oct;8(14):1187-1200.

Mercuri E, Osorio AN, Muntoni F, Buccella F, Desguerre I, Kirschner J, Tulinius M, de Resende MBD, Morgenroth LP, Gordish-Dressman H, Johnson S, Kristensen A, Werner C, Trifillis P, Henricson EK, McDonald CM; STRIDE and CINRG DNHS investigators. Safety and effectiveness of ataluren in patients with nonsense mutation DMD in the STRIDE Registry compared with the CINRG Duchenne Natural History Study (2015-2022): 2022 interim analysis. J Neurol. 2023 Aug;270(8):3896-3913. Erratum in: J Neurol. 2023 Sep;270(9):4583.

Studied Period: First patient enrolled: 30 March 2015 Last patient last visit: 22 July 2025	**Phase of Development**: 4

Objectives:
Study 025o (the STRIDE Registry) was designed to evaluate the long-term safety and effectiveness and utilization pattern of Translarna in routine clinical practice (also referred to as "usual care").

Methodology: This was a multicenter long-term study of patients receiving Translarna commercially or through other country-specific access programs outside of clinical trials.
Enrolled patients were followed for at least 5 years from their date of enrollment, or until patient withdrawal of consent or death, whichever occurred first. Patients who discontinued treatment with Translarna continued to be followed for the duration of the study unless they withdrew consent to participate in the study. Data were collected during this time period in conjunction with all routine care visits, which occurred approximately every 6 months.

Number of Subjects (Planned and Analyzed): The target sample size was approximately 360 patients, with at least 20 patients between ≥2 and <5 years of age. A total of 315 patients, 19 of whom were between ≥2 and <5 years of age, were included in the analyses.

Diagnosis and Main Criteria for Inclusion:
Patients who met the following inclusion criteria were included:

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

Test Product, Dose and Mode of Administration: No study medication was provided as part of this observational study. The treating physician made all treatment decisions according to their usual practice and provided prescriptions as appropriate.

Duration of Treatment:
The overall median duration of exposure to Translarna in the As-Treated Population was 6.46 years (range: 0.0 to 10.9 years), and total exposure was 1918.41 patient-years. End of data collection occurred on 23 September 2025.

Reference Therapy, Dose and Mode of Administration, Batch Number:
None.

Criteria for Evaluation:
Safety:
The safety assessment examined adverse events (AEs), clinical laboratory test results, electrocardiogram (ECG), and vital signs.

Statistical Methods:
Safety:
The safety assessment examined AEs, clinical laboratory test results, ECG, and vital signs. The data were summarized with descriptive statistics.
The treatment start date incorporated both prospective and retrospective data collection by subject consent.
All safety summaries were tabulated by corticosteroid use (yes or no) and total. Patients are considered in the corticosteroid use group if they had responded 'Yes' at any time during the study to the case report form (CRF) question, "Is the participant currently taking or has ever taken any corticosteroid medication during the study protocol?" The categorization of corticosteroid use (yes/no) refers to the use of prednisolone, prednisone, and/or deflazacort for the treatment of Duchenne muscular dystrophy (DMD).

SUMMARY – CONCLUSIONS
Safety 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.

CONCLUSION:
The STRIDE registry includes over 300 patients with nmDMD enrolled at expert DMD centers where they are receiving Translarna under the conditions of routine clinical practice. The median overall exposure to Translarna was 6.46 years, and the total exposure was approximately 1918 patient years. The large population in this study included a broad age range of patients, most of whom received >5 years of treatment with Translarna, making it a rich data source for informing long-term safety.

The results of this long-term registry study are consistent with the known safety profile of Translarna in clinical studies and postmarketing experience. No signals have emerged that would affect the favorable benefit-risk for the use of Translarna in patients with nmDMD.

Date of the report: 13 August 2026

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Regulatory Approval	Raphel Mattis Regulatory Approval I approve the document(s) 29-Sep-2026 13:52:31 GMT+0000

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