EMA/245211/2021

European Medicines Agency decision

P/0195/2021

of 10 May 2021

on the acceptance of a modification of an agreed paediatric investigation plan for ataluren (Translarna), (EMEA-000115-PIP01-07-M11) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/83/2009 issued on 15 May 2009, decision P/123/2010 issued on 28 July 2010, decision P/0069/2012 issued on 4 April 2012, decision P/0202/2012 issued on 30 August 2012, decision P/0132/2015 issued on 12 June 2015, decision P/0002/2016 issued on 14 January 2016, decision P/0122/2016 issued on 29 April 2016, decision P/0283/2016 issued on 4 November 2016, decision P/0393/2017 issued on 19 December 2017, and decision P/0335/2019 issued on 11 September 2019,

Having regard to the application submitted by PTC Therapeutics International Limited on 17 December 2020 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 March 2021, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for ataluren (Translarna), granules for oral suspension, oral use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to PTC Therapeutics International Limited, 5th Floor, 3 Grand Canal Plaza, Grand Canal Street Upper, Dublin 4 - D04 EE70, Ireland.

EMA/PDCO/14788/2021
Amsterdam, 26 March 2021

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-000115-PIP01-07-M11

Scope of the application

Active substance(s):

Ataluren

Invented name:

Translarna

Condition(s):

Treatment of dystrophinopathy

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Granules for oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

PTC Therapeutics International Limited

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, PTC Therapeutics International Limited submitted to the European Medicines Agency on 17 December 2020 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/83/2009 issued on 15 May 2009, decision P/123/2010 issued on 28 July 2010, decision P/0069/2012 issued on 4 April 2012, decision P/0202/2012 issued on

30 August 2012, decision P/0132/2015 issued on 12 June 2015, decision P/0002/2016 issued on 14 January 2016, decision P/0122/2016 issued on 29 April 2016, decision P/0283/2016 issued on 4 November 2016, decision P/0393/2017 issued on 19 December 2017, and decision P/0335/2019 issued on 11 September 2019.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 4 January 2021.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of dystrophinopathy

The waiver applies to:

- preterm and term newborn infants (from birth to less than 28 days of age) and infants (from 28 days to less than 6 months of age);
- granules for oral suspension, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan

2.1. Condition:

Treatment of dystrophinopathy

2.1.1. Indication(s) targeted by the PIP

Treatment of nonsense-mutation dystrophinopathy

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 6 months to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Granules for oral suspension

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	1	**Study 1** Development of an age appropriate formulation (granules for oral suspension) for children less than 5 years of age
Non-clinical studies	4	**Study 8** 7-day tolerability and pharmacokinetic study of ataluren in neonatal Beagle dogs **Study 2** 28-day dose range finding juvenile toxicology and toxicokinetic study of ataluren in Beagle dogs

		Study 3 3-month juvenile toxicology and toxicokinetic study of ataluren in Beagle dogs with a 3-month recovery period **Study 9** 28-Day Investigational Juvenile Toxicology and Toxicokinetic Study of Ataluren in Beagle Dogs with an 8-Week Recovery Period
Clinical studies	4	**Study 4** Randomized, double-blind, placebo-controlled, multicentre, dose-ranging, efficacy and safety trial in patients 5 years of age and older (PTC124-GD-007-DMD) **Study 5** Open-label, long-term extension study (Europe) (PTC124-GD-019-DMD) **Study 6** Open-label trial to evaluate safety and pharmacokinetics of ataluren in children from 2 to less than 5 years with Duchenne Muscular Dystrophy (PTC124-GD-030-DMD) **Study 7** Open-label trial to evaluate safety and pharmacokinetics of ataluren in children from 6 months to less than 2 years with Duchenne Muscular Dystrophy

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By June 2023
Deferral for one or more studies contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of Duchenne muscular dystrophy

Authorised indication(s):

- Treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 2 years and older.

Authorised pharmaceutical form(s):

Granules for oral suspension

Authorised route(s) of administration:

Oral use