EMA/501091/2012

European Medicines Agency decision

P/0202/2012

of 30 August 2012

on the acceptance of a modification of an agreed paediatric investigation plan for ataluren (EMEA-000115-PIP01-07-M03) 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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on the acceptance of a modification of an agreed paediatric investigation plan for ataluren (EMEA-000115-PIP01-07-M03) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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, the decision P/123/2010 issued on 28 July 2010, and the decision P/0069/2012 issued on 4 April 2012,

Having regard to the application submitted by PTC Therapeutics Limited on 25 May 2012 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 17 August 2012, 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, granules for oral suspension, age appropriate formulation, other, 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 Limited, 12 York Gate, Regent's Park, NW1 4QS – London, United Kingdom.

Done at London, 30 August 2012

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/476743/2012

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

EMEA-000115-PIP01-07-M03

Scope of the application

Active substance(s):

Ataluren

Condition(s):

Treatment of dystrophinopathy

Pharmaceutical form(s):

Granules for oral suspension

Age appropriate formulation, other

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

PTC Therapeutics Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, PTC Therapeutics Limited submitted to the European Medicines Agency on 25 May 2012 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, the decision P/123/2010 issued on 28 July 2010, and the decision P/0069/2012 issued on 4 April 2012.

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

The procedure started on 19 June 2012.

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Scope of the modification

Some timelines 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 annex(es) and appendix.

London, 17 August 2012

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman
(Signature on file)

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

1. Waiver

1.1. Condition: treatment of dystrophinopathy

The waiver applies to:

- preterm and term newborn infants (from birth to less than 28 days) and infants (from 28 days to less than 6 months),
- for granules for oral suspension and age-appropriate formulation, other, 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.
- Age-appropriate formulation for children, other.

2.1.4. Studies

Area	Number of studies	Description
Quality	1	Development of an age appropriate formulation, other
Non-clinical	2	Juvenile toxicology dose range-finding study Juvenile toxicity study
Clinical	3	Randomized, double-blind, placebo-controlled, multicentre, dose-ranging, efficacy and safety trial in patients 5 years of age and older Open-label, long-term extension study (Europe) Open-label safety and PK study in patients aged 6 months to less than 5 years

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes.
Date of completion of the paediatric investigation plan:	By December 2016.
Deferral for one or more studies contained in the paediatric investigation plan:	Yes.