EMA/94941/2017

European Medicines Agency decision

P/0045/2017

of 17 February 2017

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for testosterone (EMEA-001529-PIP02-14) 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 application submitted by Acerus Pharmaceuticals SRL on 11 April 2014 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 27 January 2017, in accordance with Article 18 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

(4) It is therefore appropriate to adopt a decision granting a waiver.

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

Has adopted this decision:

Article 1

A paediatric investigation plan for testosterone, nasal gel, nasal use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for testosterone, nasal gel, nasal use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver testosterone, nasal gel, nasal use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to Acerus Pharmaceuticals SRL, Durant Business Centre, Suite B, BB17097 - Christ Church, Barbados.

EMA/PDCO/763389/2016
London, 27 January 2017

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMEA-001529-PIP02-14

Scope of the application

Active substance(s):

Testosterone

Condition(s):

Treatment of male hypogonadism

Pharmaceutical form(s):

Nasal gel

Route(s) of administration:

Nasal use

Name/corporate name of the PIP applicant:

Acerus Pharmaceuticals SRL

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Acerus Pharmaceuticals SRL submitted for agreement to the European Medicines Agency on 11 April 2014 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 21 May 2014.

Supplementary information was provided by the applicant on 7 November 2016. The applicant proposed modifications to the paediatric investigation plan.

A meeting with the Paediatric Committee took place on 26 January 2017.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 18 of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset of the paediatric population, and Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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

2. The measures and timelines of the agreed 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 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 male hypogonadism

The waiver applies to:

- girls from birth to less than 18 years of age;
- nasal gel, nasal use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset;

  and

- boys from birth to less than 10.5 years bone age;
- nasal gel, nasal use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of male hypogonadism.

2.1.1. Indication(s) targeted by the PIP

Treatment of male hypogonadism.

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

Boys from 10.5 years bone age to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Nasal gel.

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of an age-appropriate nasal gel
Non-clinical studies	0	Not applicable.

Area	Number of measures	Description
Clinical studies	2	**Study 2** Open-label, variable dose, two arm uncontrolled trial to evaluate pharmacokinetics of testosterone administered as nasal gel in boys from 10.5 years bone age to less than 18 years of age with hypogonadism. **Study 3** Open-label uncontrolled trial to evaluate efficacy and safety of testosterone nasal gel in testosterone-naïve boys from 10.5 years bone age to less than 18 years of age with hypogonadism.
Extrapolation, modelling and simulation studies	1	**Study 4** Modelling & simulation study to support dose selection of testosterone nasal gel
Other studies	0	Not applicable.
Other measures	0	Not applicable.

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 November 2021
Deferral for one or more measures contained in the paediatric investigation plan:	Yes