EMA/633001/2020

European Medicines Agency decision

P/0508/2020

of 22 December 2020

on the acceptance of a modification of an agreed paediatric investigation plan for testosterone (EMEA-001529-PIP02-14-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 testosterone (EMEA-001529-PIP02-14-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/0045/2017 issued on 17 February 2017, the decision P/0132/2018 issued on 16 April 2018, and the decision P/0236/2019 issued on 16 July 2019,

Having regard to the application submitted by Acerus Biopharma Inc. on 31 July 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 13 November 2020, 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 and to the deferral.

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

[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 testosterone, nasal gel, nasal use, including changes to the deferral, 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 Acerus Biopharma Inc., 2486, Dunwin Drive, L5L 1J9 - Mississauga, Ontario, Canada.

EMA/PDCO/440544/2020
Amsterdam, 13 November 2020

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

EMEA-001529-PIP02-14-M03

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 Biopharma Inc.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Acerus Biopharma Inc. submitted to the European Medicines Agency on 31 July 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/0045/2017 issued on 17 February 2017, the decision P/0132/2018 issued on 16 April 2018, and the decision P/0236/2019 issued on 16 July 2019.

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

The procedure started on 15 September 2020.

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 and to the deferral 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 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 12 years of 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 12 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.

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 12 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 12 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 May 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes