EMA/12304/2017

European Medicines Agency decision

P/0030/2017

of 31 January 2017

on the agreement of a paediatric investigation plan and on the granting of a deferral for 2-hydroxypropyl-ß-cyclodextrin (EMEA-001866-PIP01-15) 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 Vtesse Europe Ltd on 23 November 2015 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 16 December 2016, in accordance with Article 18 of Regulation (EC) No 1901/2006 and Article 21 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.

(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.

[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 2-hydroxypropyl-ß-cyclodextrin, solution for injection, intrathecal 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 2-hydroxypropyl-ß-cyclodextrin, solution for injection, intrathecal 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

This decision is addressed to Vtesse Europe Ltd, Festival House, 39 Oxford Street, RG14 1JG – Newbury, United Kingdom.

EMA/PDCO/654919/2016
London, 16 December 2016

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

EMEA-001866-PIP01-15

Scope of the application

Active substance(s):

2-hydroxypropyl-ß-cyclodextrin

Condition(s):

Treatment of Niemann-Pick disease, type C

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intrathecal use

Name/corporate name of the PIP applicant:

Vtesse Europe Ltd

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Vtesse Europe Ltd submitted for agreement to the European Medicines Agency on 23 November 2015 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 4 January 2016.

Supplementary information was provided by the applicant on 26 September 2016. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

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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.

   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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of Niemann-Pick disease, type C

2.1.1. Indication(s) targeted by the PIP

Treatment of progressive neurological manifestations in children and adolescent patients with Niemann-Pick disease, type C

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	8	**Study 1:** Natural History Study of patients with Niemann-Pick disease, type C (NPC) **Study 2:** Open-label, single-centre, multiple-dose, dose escalation study in children and adolescents from 2 to less than 18 years of age with NPC (and adults up to 25 years of age), to assess safety, tolerability and pharmacokinetics of 2-hydroxypropyl-ß-cyclodextrin (HP-ß-CD) **Study 3:** Randomized, double-blind, sham-controlled, 3-part, efficacy and safety trial of HP-ß-CD, in patients with NPC disease from 4 to less than 18 years of age (and adults up to 21 years of age)

		Study 4: Open-label, safety and pharmacokinetic trial of HP-ß-CD, administered by the lumbar intrathecal route (IT) via an implanted delivery device, in patients with NPC disease from 4 to less than 18 years of age (and adults up to 21 years of age) **Study 5:** Open-label, safety and efficacy trial of HP-ß-CD in children from 2 to less than 4 years of age with NPC disease **Study 6:** Open-label, safety and efficacy trial of HP-ß-CD in children from birth to less than 2 years of age with NPC disease **Study 7:** Open-label, safety and pharmacokinetic trial of HP-ß-CD, administered by the lumbar intrathecal route (IT) via an implanted delivery device, in children from 2 to less than 4 years of age with NPC disease **Study 8:** Open-label, safety and pharmacokinetic trial of HP-ß-CD, administered by the lumbar intrathecal route (IT) via an implanted delivery device, in children from birth to less than 2 years of age with NPC disease
Extrapolation, modelling and simulation studies	0	Not applicable
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:	No
Date of completion of the paediatric investigation plan:	By April 2019
Deferral for one or more measures contained in the paediatric investigation plan:	Yes