EMA/592153/2013

European Medicines Agency decision

P/0276/2013

of 30 October 2013

on the acceptance of a modification of an agreed paediatric investigation plan for nitisinone (Orfadin), (EMEA-000784-PIP02-11-M01) 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 nitisinone (Orfadin), (EMEA-000784-PIP02-11-M01) 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/0065/2012 issued on 28 March 2012,

Having regard to the application submitted by Swedish Orphan Biovitrum International AB on 24 June 2013 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 September 2013, 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 nitisinone (Orfadin), oral suspension, oral 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 Swedish Orphan Biovitrum International AB, Tomtebodavägen 23A, 112 76 – Stockholm, Sweden.

Done at London, 30 October 2013

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

EMA/PDCO/389530/2013

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

EMEA-000784-PIP02-11-M01

Scope of the application

Active substance(s):

Nitisinone

Invented name:

Orfadin

Condition(s):

Treatment of tyrosinemia type 1

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Swedish Orphan Biovitrum International AB

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Swedish Orphan Biovitrum International AB submitted to the European Medicines Agency on 24 June 2013 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0065/2012 issued on 28 March 2012.

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

The procedure started on 17 July 2013.

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 and to the deferral in the scope set out in the Annex I of this opinion.

   The Icelandic and the Norwegian Paediatric Committee members agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the 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 annexes and appendix.

London, 13 September 2013

On behalf of the Paediatric Committee
Dr Dirk Mentzer, 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 tyrosinemia type 1

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of tyrosinemia type 1

2.1.1. Indication(s) targeted by the PIP Treatment of tyrosinemia type 1

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)

Oral suspension, for oral use.

2.1.4. Studies

Area	Number of studies	Description
Quality	1	Study 1: Open-label, single-dose, randomised, cross-over design trial to evaluate bioequivalence between nitisinone oral suspension and capsule formulation in healthy adult volunteers.
Non-clinical	0	Not applicable.
Clinical	1	Study 2: Open-label, non-randomised, multi-dose trial to evaluate taste and palatability study of oral suspension in children diagnosed with tyrosinaemia type 1 (HT-1).

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By May 2013
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 tyrosinemia type 1

   Authorised indication(s):

   - Treatment of patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine.

Authorised pharmaceutical form(s):

Capsule, hard

Authorised route(s) of administration:

Oral use