EMA/202042/2012

European Medicines Agency decision

P/0065/2012

of 28 March 2012

on the agreement of a paediatric investigation plan and on the granting of a deferral for nitisinone (Orfadin) (EMEA-000784-PIP02-11) 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 Swedish Orphan Biovitrum International AB on 4 May 2011 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 10 February 2012, 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 nitisinone (Orfadin), oral suspension, oral 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 nitisinone (Orfadin), oral suspension, oral 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 Swedish Orphan Biovitrum International AB, Tomtebodavägen 23A, 112 76 Stockholm, Sweden.

Done at London, 28 March 2012

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

EMA/PDCO/40967/2012

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

EMEA-000784-PIP02-11

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

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Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Swedish Orphan Biovitrum International AB submitted for agreement to the European Medicines Agency on 4 May 2011 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 15 June 2011.

Supplementary information was provided by the applicant on 17 November 2011.

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 Icelandic and the Norwegian Paediatric Committee members agree 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(es) and appendix.

London, 10 February 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 tyrosinaemia type 1

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of tyrosinaemia type 1

2.1.1. Indication(s) targeted by the PIP Treatment of tyrosinaemia 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)

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	2	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). Study 3. Open-label, multicentre, multiple-dose trial to evaluate pharmacokinetics, efficacy and safety of once daily dosing compared to twice daily dosing 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 April 2016
Deferral for one or more studies contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition and authorised indication:

Treatment of tyrosinemia type 1.

Authorised indications:

Treatment of tyrosinemia type 1 in adult and paedaitric patients, in association with drug Orfadin.

EU Number	Invented name	Strength	Pharmaceutical Form	Route of Administration	Packaging	Package size
EU/1/04/303/001	Orfadin	2 mg	Capsule, hard	Oral use	bottle (HDPE)	60 capsules
EU/1/04/303/002	Orfadin	5 mg	Capsule, hard	Oral use	bottle (HDPE)	60 capsules
EU/1/04/303/003	Orfadin	10 mg	Capsule, hard	Oral use	bottle (HDPE)	60 capsules