EMA/585771/2016

European Medicines Agency decision

P/0249/2016

of 9 September 2016

on the acceptance of a modification of an agreed paediatric investigation plan for sapropterin dihydrochloride (Kuvan), (EMEA-001476-PIP01-13-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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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/0232/2013 issued on 23 September 2013,

Having regard to the application submitted by BioMarin International Limited on 27 May 2016 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 19 August 2016, 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.

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

[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 sapropterin dihydrochloride (Kuvan), soluble tablet, powder for oral solution, oral use, 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 BioMarin International Limited, Shanbally, Ringaskiddy, County Cork, Ireland.

EMA/PDCO/399207/2016
London, 19 August 2016

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

EMEA-001476-PIP01-13-M01

Scope of the application

Active substance(s):

Sapropterin dihydrochloride

Invented name:

Kuvan

Condition(s):

Treatment of hyperphenylalaninemia

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Soluble tablet

Powder for oral solution

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

BioMarin International Limited

Information about the authorised medicinal product:

See Annex II

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Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, BioMarin International Limited submitted to the European Medicines Agency on 27 May 2016 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0232/2013 issued on 23 September 2013.

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

The procedure started on 21 June 2016.

Scope of the modification

A new pharmaceutical form has been added.

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 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 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.

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 hyperphenylalaninemia

The waiver applies to:

- the paediatric population from four years to less than 18 years;
- for soluble tablet, oral use;

  on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as the needs are already covered.

2. Paediatric Investigation Plan

2.1. Condition:

Treatment of hyperphenylalaninemia

2.1.1. Indication(s) targeted by the PIP

Treatment of phenylketonuria (PKU)

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

From birth to less than 4 years of age

2.1.3. Pharmaceutical form(s)

Soluble tablet

Powder for oral solution

2.1.4. Measures

Area	Number of measures	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	1	Measure 1: Open-label, randomised, multi-centre, concurrent controlled trial to evaluate, pharmacokinetics, safety and efficacy of Sapropterin Dihydrochloride as add-on to a Phenylalanine (Phe)-restricted diet compared to a Phenylalanine (Phe)-restricted diet alone, in children from birth to less than 4 years with phenylketonuria (PKU).

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By January 2014
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of hyperphenylalaninemia

   Authorised indication(s):

   - Kuvan is indicated for the treatment of hyperphenylalaninaemia (HPA) in adults and paediatric patients of all ages with phenylketonuria (PKU) who have been shown to be responsive to such treatment.
   - Kuvan is also indicated for the treatment of hyperphenylalaninaemia (HPA) in adults and paediatric patients of all ages with tetrahydrobiopterin (BH4) deficiency who have been shown to be responsive to such treatment.

Authorised pharmaceutical form(s):

Soluble tablet

Authorised route(s) of administration:

Oral use