EMA/688412/2011

European Medicines Agency decision

P/206/2011

of 31 August 2011

on the acceptance of a modification of an agreed paediatric investigation plan for cholic acid, (EMEA-000651-PIP01-09-M02) 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 cholic acid, (EMEA-000651-PIP01-09-M02) 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/76/2010 issued on 5 May 2010, the decision P/191/2010 issued on 15 October 2010,

Having regard to the application submitted by Special Products Ltd on 2 May 2011 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 15 July 2011, 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 cholic acid, hard capsule, age-appropriate oral formulation, 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 Special Products Ltd, Unit 16, Trade City, Avro Way, Brooklands Business Park, KT13 0YF Weybridge, Surrey, United Kingdom.

Done at London, 31 August 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director
(Signature on file)

EMA/688412/2011

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

EMEA-000651-PIP01-09-M02

Scope of the application

Active substance(s):

Cholic acid

Condition(s):

Treatment of oxysterol 7a hydrolylase deficiency and defective amidation

Treatment of other inborn errors of bile acid synthesis

Pharmaceutical form(s):

Hard capsule

Age-appropriate oral formulation

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Special Products Ltd

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Special Products Ltd submitted to the European Medicines Agency on 2 May 2011 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/76/2010 issued on 5 May 2010, the decision P/191/2010 issued on 15 October 2010.

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

The procedure started on 18 May 2011.

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Scope of the modification

Some measures of the original opinion 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 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(es) and appendix.

London, 15 July 2011

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 oxysterol 7a-hydrolylase deficiency and defective amidation

The waiver applies to:

- All subsets of the paediatric population from birth to less than 18 years of age;
- for hard capsule and age-appropriate oral formulation;
- on the grounds that the specific medicinal product is likely to be ineffective.

1.2. Condition: Treatment of other inborn errors of bile acid synthesis

The waiver applies to:

- Preterm newborn infants and term newborn infants from birth to less than 28 days of age;
- for hard capsule and age-appropriate oral formulation;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan

2.1. Condition: Treatment of other inborn errors of bile acid synthesis

2.1.1. Indication(s) targeted by the PIP

Treatment of inborn errors of bile acid synthesis responsive to treatment with cholic acid.

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

From 28 days to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Hard capsule.

Age-appropriate oral formulation (constitution needs to be agreed by the PDCO).

2.1.4. Studies

Area	Number of studies/ measures	Description
Quality	1	Development of an age-appropriate oral formulation.
Non-clinical	0	Not applicable.
Clinical	3	CCHMC study: Open-label, single-arm, non-comparative, efficacy and safety study to assess cholic acid in the treatment of inborn defects in bile acid synthesis in infants older than 1 month and children less than 18 years of age. Open-label clinical equivalence and safety study to bridge capsules used in the CCHMC study to the to-be-marketed hard capsule. Open-label continuation treatment study in infants older than 1 month and children less than 18 years of age.

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 September 2012
Deferral for one or more studies contained in the paediatric investigation plan:	Yes