EMA/660447/2012

European Medicines Agency decision

P/0240/2012

of 22 October 2012

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant human N-acetylgalactosamine-6-sulfatase (EMEA-000973-PIP01-10-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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European Medicines Agency decision
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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/90/2011 issued on 8 April 2011,

Having regard to the application submitted by BioMarin Europe Limited on 18 June 2012 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 7 September 2012, 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 recombinant human N-acetylgalactosamine-6-sulfatase, concentrate for solution for infusion, intravenous 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 Europe Limited, 164 Shaftesbury Avenue, London WC2H 8HL, United Kingdom.

Done at London, 22 October 2012

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

EMA/PDCO/510007/2012

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

EMEA-000973-PIP01-10-M01

Scope of the application

Active substance(s):

Recombinant human N-acetylgalactosamine-6-sulfatase

Condition(s):

Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome)

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

BioMarin Europe Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, BioMarin Europe Limited submitted to the European Medicines Agency on 18 June 2012 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/90/2011 issued on 8 April 2011.

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

The procedure started on 11 July 2011.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

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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.

   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 annex(es) and appendix.

London, 7 September 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

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome)

2.1.1. Indication(s) targeted by the PIP

Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome).

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)

Concentrate for solution for infusion.

2.1.4. Studies

Area	Number of studies	Description
Quality	None	Not applicable.
Non-clinical	5	Study 1: Embryo-foetal development in rabbits(dose range finding). Study 2: Combined fertility and embryo-foetal developmental study in rats. Study 3: Embryo-foetal development in rabbits. Study 4: Peri- and post-natal developmental study in rats. Study 5: Repeat dose toxicity and toxicokinetics study in monkey.
Clinical	4	Study 6: Open-label, multicentre, dose-escalation safety study in children from 5 to less than 18 years. Study 7: Open-label, multicentre, extension study in adults and children from 5 years of age. Study 8: Double-blind, randomised, multicentre, 3-arm, placebo-controlled, efficacy and safety study in adults and children from 5 years of age. Study 9: Open-label, multicentre, safety and efficacy study in children under 5 years of age.

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By December 2014
Deferral for one or more studies contained in the paediatric investigation plan:	Yes