EMA/425115/2011

European Medicines Agency decision

P/139/2011

of 1 June 2011

on the acceptance of a modification of an agreed paediatric investigation plan for mipomersen (sodium) (EMEA-000928-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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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/41/2011 issued on 7 February 2011,

Having regard to the application submitted by Genzyme Europe B.V. on 4 April 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 20 May 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 mipomersen (sodium), solution for injection, subcutaneous 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 Genzyme Europe B.V., Gooimeer 10, 1411 DD - Naarden, The Netherlands.

Done at London, 1 June 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director

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

EMEA-000928-PIP01-10-M01

Scope of the application

Active substance(s):

Mipomersen (sodium)

Condition(s):

Treatment of heterozygous and homozygous familial hypercholesterolaemia

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Genzyme Europe B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Genzyme Europe B.V. submitted to the European Medicines Agency on 4 April 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/41/2011 issued on 7 February 2011.

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

The procedure started on 20 April 2011.

Scope of the modification

Some measures and timelines 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 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 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, 20 May 2011

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman

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 heterozygous and homozygous familial hypercholesterolaemia

The waiver applies to:

- All subsets of the paediatric population from birth to less than 12 years of age;
- for solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of heterozygous and homozygous familial hypercholesterolaemia

2.1.1. Indication(s) targeted by the PIP

Treatment of heterozygous and homozygous familial hypercholesterolaemia

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

From 12 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for injection

2.1.4. Studies

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	2	Study 1 5 week subcutaneous dose range-finding toxicity study in juvenile rats. Study 2 10 week toxicity study in the juvenile rat with a 4 week recovery period.
Clinical	1	Double-blind, placebo-controlled, multicentre randomised parallel trial to evaluate efficacy, safety and pharmacokinetics of mipomersen for treatment of paediatric patients aged from 12 to less than 18 years with heterozygous/homozygous familial hypercholesterolaemia with a long-term follow up of 2 years.

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By September 2019
Deferral for one or more studies contained in the paediatric investigation plan:	Yes