European Medicines Agency

Doc. Ref. EMEA/620398/2008
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EUROPEAN MEDICINES AGENCY DECISION

of 1 December 2008

on the application for agreement of a Paediatric Investigation Plan for Alipogene tiparvovec, EMEA-000292-PIP01-08 in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council as amended

(ONLY THE ENGLISH TEXT IS AUTHENTIC)

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THE EUROPEAN MEDICINES AGENCY,

Having regard to the Treaty establishing the European Community,

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 as amended 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 Amsterdam Molecular Therapeutics B.V on 2 May 2008 under Article 16(1) also requesting a waiver under Article 13 of said Regulation and a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 17 October 2008, in accordance with Article 18 of Regulation (EC) No 1901/2006 as amended, and Article 13 of said Regulation and Article 21 of said Regulation**,**

Having regard to Article 25 of Regulation (EC) No 1901/2006 as amended,

WHEREAS:

(1) The Paediatric Committee of the European Medicines Agency has given a positive opinion,

(2) It is therefore appropriate to adopt a Decision following the Paediatric Committee's opinion on the Paediatric Investigation Plan.

(3) It is therefore appropriate to adopt a Decision granting a waiver.

(4) 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 Alipogene tiparvovec, solution for injection, intramuscular 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 waiver for Alipogene tiparvovec, solution for injection, intramuscular 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

A deferral for Alipogene tiparvovec, solution for injection, intramuscular 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 4

This decision is addressed to Amsterdam Molecular Therapeutics B.V, .Meibergdreef 61, 1100 DA Amsterdam, Netherlands.

Done at London, 1 December 2008

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Doc. Ref.:EMEA/620398/2008
EMEA-000292-PIP01-08

POSITIVE OPINION OF THE PAEDIATRIC COMMITTEE ON A REQUEST FOR AGREEMENT OF A PAEDIATRIC INVESTIGATION PLAN FOR

Scope of the application

Active substance:
Alipogene tiparvovec

Condition(s):
Hyperchylomicronaemia

Pharmaceutical form(s):
Solution for injection

Route(s) of administration:
Intramuscular use

Name/corporate name of the PIP applicant:
Amsterdam Molecular Therapeutics B.V

Basis for opinion

Pursuant to Article 16.1 of Regulation (EC) No 1901/2006 as amended, Amsterdam Molecular Therapeutics B.V submitted for agreement to the EMEA on 02 May 2008 a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 05 June 2008.

Supplementary information was provided by the applicant on 15 August 2008.

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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 Regulation (EC) No 1901/2006 as amended,
- to grant a deferral in accordance with Article 21 of Regulation (EC) No 1901/2006 as amended
- to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of Regulation (EC) No 1901/2006 as amended and concluded in accordance with
  Article 11(1)(a) of Regulation (EC) No 1901/2006 as amended, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population

The Icelandic and the Norwegian Paediatric Committee member(s) do 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 Agency, together with its annex(es) and appendix(ces).

London, 17 October 2008

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman

ANNEX I

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

A. CONDITION(S)

Hyperchylomicronaemia

B. WAIVER

- **Condition**

Hyperchylomicronaemia

The waiver applies to:

- Paediatric population from birth to less than 2 years of age

for solution for injection for intramuscular use

C. PAEDIATRIC INVESTIGATION PLAN

- **Condition to be investigated**

Hyperchylomicronaemia

- **Proposed PIP indication**

Treatment of lipoprotein lipase deficiency

- **Subset(s) of the paediatric population concerned by the paediatric development**

From 2 to less than 18 years

- **Formulation(s)**

Solution for injection for intramuscular use

- **Studies**

Area	Number of studies	Description
Non-clinical	3	Pharmacology study of a comparison of the level and persistence of lipoprotein expression in young and mature mice Pilot study of biodistribution and germ line transmission in juvenile mice Toxicity and biodistribution study in immature mice
Clinical	1	Open label, single dose, uncontrolled trial to evaluate safety and efficacy of alipogene tiparvovec in children from 2 to less than 18 years of age diagnosed with lipoprotein lipase deficiency.

Measures to address long term follow-up of potential safety and efficacy issues in **Yes**

relation to paediatric use:	
Date of completion of the paediatric investigation plan:	**By December 2013**
Deferral for initiation of some or all studies contained in the paediatric investigation plan:	**Yes**
Deferral for completion of some or all studies contained in the paediatric investigation plan:	**Yes**