



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/647572/2013

European Medicines Agency decision

P/0304/2013

of 29 November 2013

on the granting of a product specific waiver for synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA (EMA-001520-PIP01-13) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Alnylam Pharmaceuticals, Inc. on 8 July 2013 under Article 13 of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 11 October 2013 in accordance with Article 13 of Regulation (EC) No 1901/2006,

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

Whereas:

- (1) The Paediatric Committee has given an opinion on the granting of a product specific waiver.
- (2) It is therefore appropriate to adopt a decision granting a waiver.

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 waiver for synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA), solution for injection, intravenous 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 2

This decision is addressed to Alnylam Pharmaceuticals, Inc., 300 Third Street, MA 02142 – Cambridge, United States.

Done at London, 29 November 2013

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

EMA/PDCO/469125/2013

Opinion of the Paediatric Committee on the granting of a product-specific waiver

EMA-001520-PIP01-13

Scope of the application

Active substance(s):

Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA

Condition(s):

Treatment of familial amyloid polyneuropathy

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Alnylam Pharmaceuticals, Inc.

Basis for opinion

Pursuant to Article 13 of Regulation (EC) No 1901/2006 as amended, Alnylam Pharmaceuticals, Inc. submitted to the European Medicines Agency on 8 July 2013 an application for a product-specific waiver on the grounds set out in Article 11 of said Regulation for the above mentioned medicinal product.

The procedure started on 15 August 2013.

Opinion

1. The Paediatric Committee, having assessed the waiver application in accordance with Article 13 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to grant a product-specific waiver for all subsets of the paediatric population and the above mentioned condition(s) in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The grounds for the granting of the waiver are set out in Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex and appendix.

London, 11 October 2013

On behalf of the Paediatric Committee
Dr Dirk Mentzer, Chairman
(Signature on file)

Annex I

Grounds for the granting of the waiver

1. Waiver

1.1. Condition: treatment of familial amyloid polyneuropathy

The waiver applies to:

- All subsets of the paediatric population from birth to less than 18 years of age;
- for solution for injection for intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.