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European Medicines Agency decision

P/0038/2019

of 29 January 2019

on the refusal of a paediatric investigation plan and on the refusal of a deferral and on the granting of a waiver for givosiran (EMEA-002048-PIP02-18) 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 726/2004[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 Alnylam UK Limited on 26 March 2018 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 14 December 2018, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and on its own motion in accordance with Article 12 of said Regulation,

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

Whereas:

(1) The Paediatric Committee of the European Medicines has given an opinion on the refusal of a paediatric investigation plan and on the refusal of a deferral and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision refusing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision refusing a deferral.

(4) 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 paediatric investigation plan for givosiran, solution for injection, subcutaneous 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 refused.

Article 2

A deferral for givosiran, solution for injection, subcutaneous 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 refused.

Article 3

A product-specific waiver for givosiran, solution for injection, subcutaneous 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 Alnylam UK Limited, Braywick Gate, Braywick Road, SL6 1DA – Maidenhead, United Kingdom.

EMA/PDCO/645010/2018
London, 14 December 2018

Opinion of the Paediatric Committee on the refusal of a Paediatric Investigation Plan and a deferral and on the granting of a product-specific waiver

EMEA-002048-PIP02-18

Scope of the application

Active substance(s):

Givosiran

Condition(s):

Treatment of Acute Hepatic Porphyria

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Alnylam UK Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Alnylam UK Limited submitted for agreement to the European Medicines Agency on 26 March 2018 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 2 May 2018.

Supplementary information was provided by the applicant on 10 September 2018. The applicant proposed modifications to the paediatric investigation plan.

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 refuse the paediatric investigation plan in accordance with Article 17(1) of said Regulation as the measures and the timelines are not appropriate to ensure the generation of the necessary data determining the conditions in which the medicinal product may be used to treat the paediatric population or some subsets, nor to adapt a paediatric formulation, or do not bring expected significant therapeutic benefit;
   - to refuse a deferral in accordance with Article 21 of said Regulation;
   - to grant a product-specific waiver for all subsets of the paediatric population on its own motion in accordance with Article 12 of said Regulation and concluded 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.

Annex I

Grounds for the granting of the waiver

1. Waiver

1.1. Condition:

Treatment of Acute Hepatic Porphyria (AHP)

The waiver applies to:

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