EMA/694568/2020

European Medicines Agency decision

P/0023/2021

of 28 January 2021

on the agreement of a paediatric investigation plan and on the granting of a waiver for retinol (vitamin A) (EMEA-002790-PIP01-20) 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 application submitted by orphanix GmbH on 21 February 2020 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 11 December 2020, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a waiver.

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

(3) 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 retinol (vitamin A), solution for injection / infusion, intramuscular use, 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 agreed.

Article 2

A waiver for retinol (vitamin A), solution for injection / infusion, intramuscular use, 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 3

This decision is addressed to orphanix GmbH, Lederergasse 18/1, 4020 – Linz, Austria.

EMA/PDCO/507344/2020 Corr
Amsterdam, 11 December 2020

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a waiver

EMEA-002790-PIP01-20

Scope of the application

Active substance(s):

Retinol (vitamin A)

Condition(s):

Prevention of bronchopulmonary dysplasia

Pharmaceutical form(s):

Solution for injection / infusion

Route(s) of administration:

Intramuscular use

Intravenous use

Name/corporate name of the PIP applicant:

orphanix GmbH

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, orphanix GmbH submitted for agreement to the European Medicines Agency on 21 February 2020 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 31 March 2020.

Supplementary information was provided by the applicant on 11 September 2020. 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 agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population and 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 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 European Medicines Agency, together with its annex and appendix.

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 (PIP)

1. Waiver

1.1. Condition:

Prevention of bronchopulmonary dysplasia

The waiver applies to:

- preterm newborn infants from 30+0 weeks to 36+6 weeks of gestational age (GA);
- solution for injection / infusion, intramuscular use, intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments;

  and

- term newborn infants from birth to less than 28 days of age, and children from 28 days to less than 18 years of age;
- solution for injection / infusion, intramuscular use, intravenous use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Prevention of bronchopulmonary dysplasia

2.1.1. Indication(s) targeted by the PIP

Prevention of bronchopulmonary dysplasia

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

Preterm infants with a gestational age of less than 30+0 weeks.

2.1.3. Pharmaceutical form(s)

Intramuscular use

Intravenous use

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	1	**Study 1**

Area	Number of measures	Description
		Juvenile animal PK study
Clinical studies	1	**Study 2** Open-label study to evaluate pharmacokinetics, safety and tolerability of retinol (vitamin A) in preterm infants of less than 30+0 weeks gestational age (GA) at risk of developing bronchopulmonary dysplasia
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By August 2022
Deferral for one or more measures contained in the paediatric investigation plan:	No