EMA/233140/2020

European Medicines Agency decision

P/0192/2020

of 15 May 2020

on the acceptance of a modification of an agreed paediatric investigation plan for 2-iminobiotin (EMEA-001070-PIP01-10-M02) 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/0035/2012 issued on 3 February 2012,

Having regard to the application submitted by Neurophyxia B.V. on 13 December 2019 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 27 March 2020, 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 2-iminobiotin, solution for infusion, intravenous 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 Neurophyxia BV, Onderwijsboulevard 225, 5223 DE - ’s-Hertogenbosch, The Netherlands.

EMA/PDCO/15548/2020
Amsterdam, 27 March 2020

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

EMEA-001070-PIP01-10-M02

Scope of the application

Active substance(s):

2-iminobiotin

Condition(s):

Treatment of perinatal asphyxia

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Neurophyxia B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Neurophyxia B.V.submitted to the European Medicines Agency on 13 December 2019 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0035/2012 issued on 3 February 2012.

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

The procedure started on 28 January 2020.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan 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 Norwegian Paediatric Committee member agrees 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 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:

Treatment of perinatal asphyxia

The waiver applies to:

- all subsets of the paediatric population from 44 weeks of gestational age to less than 18 years of age;
- solution for infusion, 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:

Treatment of perinatal asphyxia

2.1.1. Indication(s) targeted by the PIP

Prevention of perinatal asphyxia

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

Newborns from 33 weeks to less than 44 weeks of gestational age.

2.1.3. Pharmaceutical form(s)

Solution for infusion

2.1.4. Studies

Area	Number of studies	Description
Quality	0	Not applicable
Non-clinical	2	**Study 1** Randomised, placebo-controlled dose range finding study of 2-Iminobiotin after moderate to severe perinatal hypoxia-ischemia in the newborn piglet. **Study 2** Randomised, placebo-controlled study of combination treatment of 2-Iminobiotin with hypothermia after moderate to severe perinatal hypoxia-ischemia in the newborn piglet.

Clinical	6	**Study 3 (NEU-01-02-01)** Multi-centre, non-randomised, open-label, non-controlled pilot study to evaluate safety, tolerability and pharmacokinetics of 2-Iminobiotin for the treatment of moderate to severe perinatal asphyxia in neonates with a gestational age from 36 to less than 44 weeks. **Study 4 (NEU-01-02-02)** Pilot open-label study to evaluate safety, tolerability and pharmacokinetics of 2-Iminobiotin for the treatment of moderate to severe perinatal asphyxia in neonates from 33 to less than 35 weeks of gestational age. **Study 5 (NEU-01-02-03)** Single centre open label study to investigate the pharmacokinetics and short-term safety of 2-iminobiotin for the treatment of moderate to severe perinatal asphyxia in neonates with a gestational age from 36 to less than 44 weeks receiving hypothermia treatment. **Study 6 (NEU-01-02-04)** Pilot, open-label study to evaluate safety, tolerability and pharmacokinetics of 2-Iminobiotin for the treatment of moderate to severe perinatal asphyxia in neonates from 33 to less than 35 weeks of gestation receiving hypothermia treatment. **Study 7** *Deleted during procedure EMEA-001070-PIP01-10-M02* **Study 8 (NEU-01-03-02)** Double blind, randomised, placebo-controlled multicentre study to evaluate efficacy at 24 months of age, safety and tolerability of 2-Iminobiotin for treatment of moderate to severe perinatal asphyxia in neonates with a gestational age from 35 weeks to less than 44 weeks receiving hypothermia treatment. **Study 9 (NEU-01-02-05)** *Added during procedure EMEA-001070-PIP01-10-M02* Double-blind, randomised, placebo-controlled multicentre study to evaluate the efficacy of the combination of hypothermia and 2 iminobiotin (2-IB) after birth asphyxia in neonates from 35 to less than 44 weeks of gestational age.

3. Follow-up, completion and deferral of PIP

Concerns on potential long-term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By March 2029
Deferral for one or more studies contained in the paediatric investigation plan:	No