EMA/66788/2023

European Medicines Agency decision

P/0097/2023

of 10 March 2023

on the acceptance of a modification of an agreed paediatric investigation plan for crinecerfont (EMEA-002700-PIP01-19-M01) 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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on the acceptance of a modification of an agreed paediatric investigation plan for crinecerfont (EMEA-002700-PIP01-19-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0104/2021 issued on 17 March 2021,

Having regard to the application submitted by Neurocrine Therapeutics, Ltd. on 11 October 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 20 January 2023, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for crinecerfont, capsule, hard, oral solution, oral use, gastric 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 Neurocrine Therapeutics Ltd, 70 Sir John Rogerson's Quay, D02R296 – Dublin, Ireland.

EMA/PDCO/852196/2022
Amsterdam, 20 January 2023

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

EMEA-002700-PIP01-19-M01

Scope of the application

Active substance(s):

Crinecerfont

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of congenital adrenal hyperplasia

Pharmaceutical form(s):

Capsule, hard

Oral solution

Route(s) of administration:

Oral use

Gastric use

Name/corporate name of the PIP applicant:

Neurocrine Therapeutics, Ltd.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Neurocrine Therapeutics, Ltd. submitted to the European Medicines Agency on 11 October 2022 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0104/2021 issued on 17 March 2021.

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

The procedure started on 21 November 2022.

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

2. The measures and timelines of the paediatric investigation plan 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 annexes 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

Not applicable.

2. Paediatric investigation plan

2.1. Condition:

Treatment of congenital adrenal hyperplasia

2.1.1. Indication(s) targeted by the PIP

Treatment of congenital adrenal hyperplasia

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Capsule, hard

Oral solution

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Assess feasibility of administration of crinecerfont oral solution using a feeding tube.
Non-clinical studies	**Study 2 (2020-TX-212)** Determination of exposure levels and tolerability in rats after single and 4-day repeat dosing of crinecerfont. **Study 3 (2020-TX-284)** Evaluation of the effects in rats on reproductive and neurobehavioral development and function.
Clinical studies	**Study 4 (CAH2006)** Randomised trial to evaluate the PK, efficacy, and safety of crinecerfont with an initial 28-week placebo-controlled period followed by 24-week open-label treatment and an optional open-label extension (OLE) period to collect long-term safety data for up to 3 years.

	Study 5 (CAH2011) Open-label study to assess the safety, tolerability, sparse PK, and PD of crinecerfont in children from birth to 2 years of age including an optional open-label extension period to collect long-term safety data for up to 3 years. **Study 6 (CAH3008)** Study deleted in procedure EMEA-002700-PIP01-19-M01
Extrapolation, modelling and simulation studies	**Study 7** Population PK analysis update with paediatric data in children 2 to less than 18 years **Study 8** Population PK analysis update with paediatric data in children less than 2 years **Study 9** Paediatric Exposure-Response analysis with data from adult and paediatric study in ages 2 years to less than 18 years of age **Study 10** Paediatric Exposure-Response analysis update with data from paediatric study in ages less than 2 years of age combined with data from adults and older paediatric ages

3. Follow-up, completion and deferral of PIP

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

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.