

EMA/307240/2024

European Medicines Agency decision

P/0278/2024

of 14 August 2024

on the acceptance of a modification of an agreed paediatric investigation plan for crinecerfont (EMEA-002700-PIP01-19-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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on the acceptance of a modification of an agreed paediatric investigation plan for crinecerfont (EMA-002700-PIP01-19-M02) 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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0104/2021 issued on 17 March 2021, and the decision P/0097/2023 issued on 10 March 2023,

Having regard to the application submitted by Neurocrine Therapeutics Ltd on 22 March 2024 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 28 June 2024, 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 and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

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, soft, oral solution, oral use, gastric use, including changes to the deferral, 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/142422/2024
Amsterdam, 28 June 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-002700-PIP01-19-M02

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, soft

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 22 March 2024 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 and the decision P/0097/2023 issued on 10 March 2023.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral. The pharmaceutical form was amended.

The procedure started on 29 April 2024.

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 and to the deferral 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, soft

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 from study 4 (CAH2006) Study 8 Population PK analysis update with paediatric data in children less than 2 years of age Study 9 Paediatric Exposure-Response analysis with data from study 4 (CAH2006) 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 2030
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.