

EMA/627334/2021

European Medicines Agency decision P/0484/2021

of 3 December 2021

on the acceptance of a modification of an agreed paediatric investigation plan for deferoxamine (mesylate) / histidine / tryptophan / aspartic acid / n-acetyl-histidine (monohydrate) / glycine / alfa-ketoglutaric acid / arginine / potassium chloride / magnesium chloride (hexahydrate) / calcium chloride (dihydrate) / sodium chloride / alanine / 3,4-dimethoxy-N-methylbenzohydroxamic acid (EMA-002735-PIP03-20-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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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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency²,

Having regard to the European Medicines Agency's decision P/0190/2021 issued on 10 May 2021,

Having regard to the application submitted by Dr. Franz Köhler Chemie GmbH on 7 July 2021 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 15 October 2021, 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 deferoxamine (mesylate) / histidine / tryptophan / aspartic acid / n-acetyl-histidine (monohydrate) / glycine / alfa-ketoglutaric acid / arginine / potassium chloride / magnesium chloride (hexahydrate) / calcium chloride (dihydrate) / sodium chloride / alanine / 3,4-dimethoxy-N-methylbenzohydroxamic acid, solution for organ preservation, 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 Dr. Franz Köhler Chemie GmbH, 14-28 Werner-von-Siemens-Str., 64625 – Bensheim, Germany.

EMA/PDCO/406120/2021
Amsterdam, 15 October 2021

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-002735-PIP03-20-M01

Scope of the application

Active substance(s):

Deferoxamine (mesylate) / histidine / tryptophan / aspartic acid / n-acetyl-histidine (monohydrate) / glycine / alfa-ketoglutaric acid / arginine / potassium chloride / magnesium chloride (hexahydrate) / calcium chloride (dihydrate) / sodium chloride / alanine / 3,4-dimethoxy-N-methylbenzohydroxamic acid

Condition(s):

Heart transplantation

Pharmaceutical form(s):

Solution for organ preservation

Route(s) of administration:

Not applicable

Name/corporate name of the PIP applicant:

Dr. Franz Köhler Chemie GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Dr. Franz Köhler Chemie GmbH submitted to the European Medicines Agency on 7 July 2021 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0190/2021 issued on 10 May 2021.

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

The procedure started on 17 August 2021.

Scope of the modification

Some measures 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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Heart transplantation

2.1.1. Indication(s) targeted by the PIP

Preservation of hearts prior to heart transplantation

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)

Solution for organ preservation

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	2	Study 1 (FKC-001_02_rat_tox_iv) Single dose intravenous toxicity study of 3,4-dimethoxy-N-methylbenzohydroxamic acid in rats Study 2 (FKC-003_01_dev_reprotox Rat) Reproductive toxicity study of 3,4-dimethoxy-N-methylbenzohydroxamic acid in rats
Clinical studies	1	Study 3 (CL-N-HTX-Paed-II/10/20) Randomised single-blind study to compare the safety of the investigational preservation solution to that of its precursor product in children from birth to less than 18 years of age undergoing heart transplantation
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:	No
Date of completion of the paediatric investigation plan:	By October 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes