EMA/236603/2023

European Medicines Agency decision

P/0213/2023

of 14 June 2023

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 (EMEA-002735-PIP03-20-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.

European Medicines Agency decision

P/0213/2023

of 14 June 2023

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 (EMEA-002735-PIP03-20-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 726/2004[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/0190/2021 issued on 10 May 2021 and the decision P/0484/2021 issued on 3 December 2021,

Having regard to the application submitted by Dr. Franz Köhler Chemie GmbH on 20 January 2023 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 26 April 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 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 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, 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 Dr. Franz Köhler Chemie GmbH, Werner-von-Siemens-Str. 14-28, 64625 Bensheim, Germany.

EMA/PDCO/44201/2023
Amsterdam, 26 April 2023

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

EMEA-002735-PIP03-20-M02

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

Invented name and authorisation status:

See Annex II

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 20 January 2023 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 and the decision P/0484/2021 issued on 3 December 2021.

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

The procedure started on 27 February 2023.

Scope of the modification

The 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:

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	Description
Quality-related studies	Not applicable
Non-clinical studies	**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	**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	Not applicable
Other studies	Not applicable
Other measures	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 August 2025
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.