EMA/160446/2023

European Medicines Agency decision

P/0165/2023

of 15 May 2023

on the acceptance of a modification of an agreed paediatric investigation plan for idrevloride (EMEA-002935-PIP01-20-M03) 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/0165/2023

of 15 May 2023

on the acceptance of a modification of an agreed paediatric investigation plan for idrevloride (EMEA-002935-PIP01-20-M03) 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/0377/2021 issued on 2 September 2021 and the decision P/0206/2022 issued on 10 June 2022,

Having regard to the application submitted by Parion Sciences, Inc. on 15 December 2022 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 31 March 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 idrevloride, inhalation solution, inhalation 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 Parion Sciences, Inc., 2800 Meridian Parkway, Suite 195, 27713 - Durham, NC, United States.

EMA/PDCO/954460/2022 Corr.[1]
Amsterdam, 31 March 2023

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

EMEA-002935-PIP01-20-M03

Scope of the application

Active substance(s):

Idrevloride

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of primary ciliary dyskinesia

Pharmaceutical form(s):

Inhalation solution

Route(s) of administration:

Inhalation use

Name/corporate name of the PIP applicant:

Parion Sciences, Inc.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Parion Sciences, Inc. submitted to the European Medicines Agency on 15 December 2022 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0377/2021 issued on 2 September 2021 and the decision P/0206/2022 issued on 10 June 2022.

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

The procedure started on 30 January 2023.

[1] 5 May 2023

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 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 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

Treatment of primary ciliary dyskinesia

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- inhalation solution, inhalation use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of primary ciliary dyskinesia

2.1.1. Indication(s) targeted by the PIP

Treatment of primary ciliary dyskinesia

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

From 2 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Inhalation solution

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1 (PS-G202)** Randomised 2-part double-blind, placebo-controlled, incomplete block, crossover study with 28-day treatment periods to evaluate the safety and efficacy of idrevloride inhalation solution with and without oral ivacaftor (IVA) in patients from 12 years to less than 18 years of age (and adults) with primary ciliary dyskinesia (PS-G202). **Study 2 (PS-G302)** Open label, non-controlled, single arm study to evaluate the safety, tolerability and pharmacokinetics and randomized, double-blind, study to evaluate the safety and efficacy of idrevloride inhalation solution in patients with primary ciliary dyskinesia from 2 years of age to less than 12 years of age.

	Study 3 (PS-G301) Randomised, double-blind, study to evaluate the safety and efficacy of idrevloride inhalation solution compared with placebo over 48 weeks of treatment in patients from 12 years to less than 18 years of age (and adults) with primary ciliary dyskinesia.
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 December 2029
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.