

EMA/440424/2021

European Medicines Agency decision P/0377/2021

of 2 September 2021

on the agreement of a paediatric investigation plan and on the granting of a waiver for 3,5-diamino-6-chloro-N-(N-(4-(4-(2-(hexyl((2S,3R,4R,5R)-2,3,4,5,6-pentahydroxyhexyl)amino)ethoxy)phenyl)butyl)-carbamimidoyl)pyrazine-2-carboxamide/Sodium chloride solution 4.2% (P-1037 inhalation solution) (EMA-002935-PIP01-20) 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 application submitted by Parion Sciences, Inc. on 27 November 2020 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 23 July 2021, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a waiver.

¹ OJ L 378, 27.12.2006, p.1.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for 3,5-diamino-6-chloro-N-(N-(4-(4-(2-(hexyl((2S,3R,4R,5R)-2,3,4,5,6-pentahydroxyhexyl)amino)ethoxy)phenyl)butyl)-carbamimidoyl)pyrazine-2-carboxamide/Sodium chloride solution 4.2% (P-1037 inhalation solution), inhalation solution, inhalation use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A waiver for 3,5-diamino-6-chloro-N-(N-(4-(4-(2-(hexyl((2S,3R,4R,5R)-2,3,4,5,6-pentahydroxyhexyl)amino)ethoxy)phenyl)butyl)-carbamimidoyl)pyrazine-2-carboxamide/Sodium chloride solution 4.2% (P-1037 inhalation solution), inhalation solution, inhalation use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Parion Sciences, Inc., 2800 Meridian Parkway, Suite 195, 27713 - Durham, NC, United States.

EMA/PDCO/253648/2021
Amsterdam, 23 July 2021

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a waiver

EMA-002935-PIP01-20

Scope of the application

Active substance(s):

3,5-diamino-6-chloro-N-(N-(4-(4-(2-(hexyl((2S,3R,4R,5R)-2,3,4,5,6-pentahydroxyhexyl)amino)ethoxy)phenyl)butyl)-carbamimidoyl)pyrazine-2-carboxamide/Sodium chloride solution 4.2% (P-1037 inhalation solution)

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 16(1) of Regulation (EC) No 1901/2006 as amended, Parion Sciences, Inc. submitted for agreement to the European Medicines Agency on 27 November 2020 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 4 January 2021.

Supplementary information was provided by the applicant on 16 April 2021. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
- to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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

2. The measures and timelines of the agreed 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 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

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	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	3	Study 1 Randomized 2-part double-blind, placebo-controlled, incomplete block, crossover study with 28-day treatment periods to evaluate the safety and efficacy of P-1037 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 Open label, single arm study to evaluate the safety, tolerability and pharmacokinetics of P-1037 inhalation solution in 28-day study in patients with primary ciliary dyskinesia in two sequential cohorts.

		Cohort 1 will include patients from 6 years to less than 12 years of age and cohort 2 will include patients from 2 years to less than 6 years of age (PS-G102). Study 3 Randomized, double-blind, study to evaluate the safety and efficacy of P-1037 inhalation solution compared to placebo over the first 24 weeks of treatment, and to evaluate the safety of P-1037 inhalation solution over 48 weeks of treatment, in patients from 2 years to less than 18 years of age (and adults), with primary ciliary dyskinesia (PS-G301).
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 December 2025
Deferral for one or more measures contained in the paediatric investigation plan:	No