MHRA
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United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a waiver

MHRA-100106-PIP01-21

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-carboxaminde; sodium chloride solution 4.2% (w/v)

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 the Decision

Pursuant to the Human Medicines Regulations 2012, Parion Sciences, Inc. submitted to the licensing authority on 23/07/2021 13:18 BST an application for a Paediatric Investigation Plan

The procedure started on 07/12/2021 13:01 GMT

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a waiver

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100106-PIP01-21

Of 09/03/2022 17:06 GMT

On the adopted decision 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-carboxaminde; sodium chloride solution 4.2% (w/v) (MHRA-100106-PIP01-21) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to 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-carboxaminde; sodium chloride solution 4.2% (w/v), Inhalation solution , Inhalation use .

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of primary ciliary dyskinesia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Inhalation solution Route(s) of administration: Inhalation use Reason for granting waiver: 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(s):

Treatment of primary ciliary dyskinesia

2.2 Indication(s) targeted by the PIP:

Treatment of primary ciliary dyskinesia

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Inhalation solution

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	0	Not applicable
Clinical Studies	2	Study 1 (PS-G102) A single arm, open label study to evaluate the safety, tolerability, and pharmacokinetic (PK) parameters of P-1037 inhalation solution in children from 2 years to less than 12 years of age with primary ciliary dyskinesia. Study 2 A randomised, double-blind, placebo-controlled study followed by an open-label treatment period to evaluate the efficacy and safety of P-1037 inhalation solution in the treatment of paediatric patients from 2 years to less than 18 years of age (and adults) with primary ciliary dyskinesia.
Extrapolation, Modeling & Simulation Studies	0	Not applicable
Other Studies	0	Not applicable
Other Measures	0	Not applicable

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	31/12/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes