EMA/152080/2021

European Medicines Agency decision

P/0144/2021

of 16 April 2021

on the acceptance of a modification of an agreed paediatric investigation plan for ladarixin (EMEA-002642-PIP01-19-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.

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on the acceptance of a modification of an agreed paediatric investigation plan for ladarixin (EMEA-002642-PIP01-19-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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0124/2020 issued on 24 March 2020 and the decision P/0284/2020 issued on 12 August 2020,

Having regard to the application submitted by Dompé farmaceutici S.p.A on 27 November 2020 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 February 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 ladarixin, age-appropriate oral solid dosage form, capsule, hard, oral 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 Dompé farmaceutici S.p.A, Via Santa Lucia, 6, 20122 – Milano, Italy.

EMA/PDCO/654614/2020
Amsterdam, 26 February 2021

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

EMEA-002642-PIP01-19-M03

Scope of the application

Active substance(s):

Ladarixin

Condition(s):

Treatment of type 1 diabetes mellitus

Pharmaceutical form(s):

Age-appropriate oral solid dosage form

Capsule, hard

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Dompé farmaceutici S.p.A

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Dompé farmaceutici S.p.A submitted to the European Medicines Agency on 27 November 2020 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0124/2020 issued on 24 March 2020 and the decision P/0284/2020 issues on 12 August 2020.

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

The procedure started on 4 January 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 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

1.1. Condition:

Treatment of type 1 diabetes mellitus

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- age-appropriate oral solid dosage form, capsule, hard; oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of type 1 diabetes mellitus

2.1.1. Indication(s) targeted by the PIP

Treatment of new-onset type 1 diabetes mellitus with residual beta cell function

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

From 1 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Age-appropriate oral solid dosage form

Capsule, hard

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of a paediatric age-appropriate formulation (granules for oral solution) for use in children below 14 years of age.
Non-clinical studies	1	**Study 2** Definitive juvenile toxicity study to assess the toxic potential of ladarixin when administered daily by oral gavage to juvenile rats from post-natal day (PND) 10 to PND 45.

Clinical studies	3	**Study 3** (LDX0319) Randomized, double-blind and placebo-controlled study to assess pharmacokinetics (PK), efficacy and safety in children from 14 to less than 18 years of age (and adults) with new onset type 1 diabetes **Study 4** Randomized, double-blind and placebo-controlled study to assess the PK, efficacy and safety in children from 6 to less than 14 years of age with new onset type 1 diabetes **Study 5** Randomized, double-blind and placebo-controlled study to assess the PK, efficacy and safety in children from 1 to less than 6 years of age with new onset type 1 diabetes
Extrapolation, modelling and simulation studies	2	**Study 6** (2460250) Physiologic Based Pharmacokinetic (PBPK) modelling and simulation study to support dose finding in the paediatric target population **Study 7** Population PK modelling and simulation study to support dose finding in the paediatric target population
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:	Yes
Date of completion of the paediatric investigation plan:	By July 2028
Deferral for one or more measures contained in the paediatric investigation plan:	Yes