EMA/233424/2020

European Medicines Agency decision

P/0176/2020

of 13 May 2020

on the agreement of a paediatric investigation plan and on the granting of a waiver for livoletide (EMEA-002455-PIP01-18) 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 726/2004[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 application submitted by Millendo Therapeutics SAS on 10 September 2018 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 27 March 2020, 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 livoletide, solution for injection, subcutaneous 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 livoletide, solution for injection, subcutaneous 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 Millendo Therapeutics SAS, 15G Chemin du Saquin, Building G, 60130 – Écully, France.

EMA/PDCO/77010/2020
Amsterdam, 27 March 2020

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

EMEA-002455-PIP01-18

Scope of the application

Active substance(s):

Livoletide

Condition(s):

Treatment of Prader-Willi syndrome

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Millendo Therapeutics SAS

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Millendo Therapeutics SAS submitted for agreement to the European Medicines Agency on 10 September 2018 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 16 October 2018.

Supplementary information was provided by the applicant on 20 December 2019. 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

1.1. Condition:

Treatment of Prader-Willi Syndrome.

The waiver applies to:

- the paediatric population from birth to less than 4 years of age;
- solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Treatment of Prader-Willi Syndrome.

2.1.1. Indication(s) targeted by the PIP

Treatment of hyperphagia and food-related behaviours in paediatric patients ≥ 4 years of age with Prader-Willi Syndrome (PWS).

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

From 4 to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for injection.

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	1	Study 1 Definitive juvenile toxicity study in rats (AZP01-TOX-031).
Clinical studies	3	Study 2 Double-blind, placebo-controlled safety and tolerability study of livoletide in patients with genetically confirmed Prader-Willi syndrome (PWS) in paediatric patients from 12 to less than 18 years of age (and adults) (AZP01-CLI-002).

		Study 3 Double-blind, placebo-controlled study to evaluate the safety and efficacy of livoletide on food-related behaviours in paediatric patients from 4 to less than 18 years of age (and adults) with PWS after 3 months of treatment (AZP01-CLI-003 (ZEPHYR) Phase 2b). Study 4 Double-blind, placebo-controlled study to evaluate the safety and efficacy of livoletide on food-related behaviours in paediatric patients from 4 to less than 18 years of age (and adults) with PWS after 6 months of treatment (AZP01-CLI-003 (ZEPHYR) Phase 3).
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 March 2025
Deferral for one or more measures contained in the paediatric investigation plan:	No