EMA/366000/2022

European Medicines Agency decision

P/0214/2022

of 10 June 2022

on the acceptance of a modification of an agreed paediatric investigation plan for lanadelumab (Takhzyro), (EMEA-001864-PIP01-15-M07) 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 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/0273/2016 issued on 5 October 2016, the decision P/0378/2017 issued on 1 December 2017, the decision P/0055/2018 issued on 9 March 2018, the decision P/0265/2019 issued on 23 July 2019, the decision P/0264/2021 issued on 7 July 2021 and the decision P/0022/2022 issued on 31 January 2022,

Having regard to the application submitted by Takeda Pharmaceuticals International AG Ireland Branch on 21 January 2022 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 22 April 2022, 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 lanadelumab (Takhzyro), solution for injection, subcutaneous 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 Takeda Pharmaceuticals International AG Ireland Branch, Block 3 Miesian Plaza, 50 – 58 Baggot Street Lower, D02 Y754 - Dublin 2, Ireland.

EMA/PDCO/57462/2022
Amsterdam, 22 April 2022

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

EMEA-001864-PIP01-15-M07

Scope of the application

Active substance(s):

Lanadelumab

Invented name:

Takhzyro

Condition(s):

Prevention of hereditary angioedema attacks

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Takeda Pharmaceuticals International AG Ireland Branch

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Takeda Pharmaceuticals International AG Ireland Branch submitted to the European Medicines Agency on 21 January 2022 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/0273/2016 issued on 5 October 2016, the decision P/0378/2017 issued on 1 December 2017, the decision P/0055/2018 issued on 9 March 2018, the decision P/0265/2019 issued on 23 July 2019, the decision P/0264/2021 issued on 7 July 2021 and the decision P/0022/2022 issued on 31 January 2022.

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

The procedure started on 21 February 2022.

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 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

1.1. Condition

Prevention of hereditary angioedema attacks

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- solution for injection, subcutaneous use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition

Prevention of hereditary angioedema attacks

2.1.1. Indication(s) targeted by the PIP

Prevention of angioedema attacks in patients with Types I or II hereditary angioedema

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)

Solution for injection

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable.
Non-clinical studies	Not applicable.
Clinical studies	**Study 1** Double-blind, randomised, multiple dose, placebo controlled trial to evaluate pharmacokinetics, safety, efficacy of lanadelumab (DX-2930) in children from 12 years to less than 18 years of age for Long-Term Prophylaxis Against Acute Attacks of Hereditary Angioedema (HAE), (DX-2930-03). **Study 2** Open-Label Study to Evaluate the Long-Term Safety and activity of DX-2930 in children from 12 years to less than 18 years of age for Prevention Against Acute Attacks of Hereditary Angioedema (HAE), (DX-2930-04).

	Study 3 Open label trial to evaluate the safety, pharmacokinetics (PK) and activity (clinical outcome) of lanadelumab for Prevention Against Acute Attacks of Hereditary Angioedema (HAE) in Pediatric Patients from 2 years to less than 12 years of age (SHP643-301).
Extrapolation, modelling and simulation studies	**Study 4** Modelling and simulation study to evaluate the use of DX-2930 in the Prevention Against Acute Attacks of Hereditary Angioedema (HAE) in children from 2 years to less than 18 years. **Study 5** Extrapolation study, to evaluate the use of DX-2930 in the Prevention Against Acute Attacks of Hereditary Angioedema (HAE) in children from 2 years to less than 18 years.
Other studies	Not applicable.
Other measures	**Study 6** Interim clinical study report of PIP Study 2 (DX-2930-04).

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 June 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

Prevention of hereditary angioedema attacks

Authorised indication(s):

- Takhzyro is indicated for routine prevention of recurrent attacks of hereditary angioedema (HAE) in patients aged 12 years and older

Authorised pharmaceutical form(s):

Solution for injection

Authorised route(s) of administration:

Subcutaneous use