EMA/627311/2020

European Medicines Agency decision

P/0476/2020

of 1 December 2020

on the agreement of a paediatric investigation plan and on the granting of a waiver for lanadelumab (Takhzyro), (EMEA-001864-PIP03-19) 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 Shire Pharmaceuticals Ireland Limited (a Takeda company) on 28 November 2019 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 16 October 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 lanadelumab, (Takhzyro), 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 lanadelumab, (Takhzyro), 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 Shire Pharmaceuticals Ireland Limited (a Takeda company), Block 2-3 Miesian Plaza, 50-58 Baggot Street Lower, 2 – Dublin, Ireland.

EMA/PDCO/682328/2019
Amsterdam, 16 October 2020

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

EMEA-001864-PIP03-19

Scope of the application

Active substance(s):

Lanadelumab

Invented name:

Takhzyro

Condition(s):

Prevention of attacks of Idiopathic non-histaminergic angioedema (INHA)

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:

Shire Pharmaceuticals Ireland Limited (a Takeda company)

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Shire Pharmaceuticals Ireland Limited (a Takeda company) submitted for agreement to the European Medicines Agency on 28 November 2019 an application for a paediatric investigation plan for the above mentioned medicinal

product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 6 January 2020.

Supplementary information was provided by the applicant on 13 July 2020. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a deferral.

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 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 attacks of Idiopathic non-histaminergic angioedema (INHA)

The waiver applies to:

- the paediatric population from birth to less than 12 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 attacks of Idiopathic non-histaminergic angioedema (INHA)

2.1.1. Indication(s) targeted by the PIP

Prevention of attacks of idiopathic non-histaminergic angioedema (INHA) in adolescent and adult patients.

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

From 12 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	0	Not applicable
Clinical studies	2	**Study 1** Randomized, placebo-controlled, double-blind study to evaluate the PK, efficacy and safety of lanadelumab in adolescents from 12 to less than 18 years of age (and adults) with idiopathic non-histaminergic angioedema, (SHP643-303)

		Study 2 Open-label extension study to evaluate the long-term safety and efficacy of repeated subcutaneous (SC) administrations of lanadelumab in adolescents from 12 to less than 18 years of age (and adults) with idiopathic non-histaminergic angioedema (TAK-743-3001)
Extrapolation, modelling and simulation studies	2	**Study 3** PopPK/PD modelling and simulation study to better inform dosing of lanadelumab in adolescents from 12 to less than 18 years of age (and adults) with idiopathic non-histaminergic angioedema **Study 4** Extrapolation study analysis of existing in house and literature data for lanadelumab in hereditary angioedema and idiopathic non-histaminergic angioedema to better inform dosing of lanadelumab in adolescents from 12 to less than 18 years of age (and adults) with idiopathic non-histaminergic angioedema
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 October 2023
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. 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