MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-100340-PIP01-21-M01) and to the deferral

MHRA-100340-PIP01-21-M02

Scope of the Application

Active Substance(s)

LANADELUMAB

Condition(s)

Prevention of hereditary angioedema attacks

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

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Takeda Pharmaceuticals International AG Ireland Branch submitted to the licensing authority on 09/11/2022 12:28 GMT an application for a

The procedure started on 12/01/2023 11:21 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 accept change(s) to the agreed paediatric investigation plan and to the deferral

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-100340-PIP01-21-M02

Of 24/01/2023 07:09 GMT

On the adopted decision for LANADELUMAB (MHRA-100340-PIP01-21-M02) 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 modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a for LANADELUMAB, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Takeda Pharmaceuticals International AG Ireland Branch, Block 2 Miesian Plaza, 50 – 58 Baggot Street Lower, Dublin, IRELAND, D02 HW68

ANNEX I

1. Waiver

1.1 Condition:

Prevention of hereditary angioedema attacks The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: 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(s):

Prevention of hereditary angioedema attacks

2.2 Indication(s) targeted by the PIP:

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

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):

Solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	3	Study 1 (DX-2930-03) 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). Study 2 (DX-2930-04) 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). Study 3 (SHP643-301) Open label trial to evaluate pharmacokinetics, safety and activity of DX-2930 in children from 2 to less than 12 years of age for Prevention Against Acute Attacks of Hereditary Angioedema (HAE).
Extrapolation, Modeling & Simulation Studies	2	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	0	Not applicable.
Other Measures	1	Study 6 Interim clinical study report of PIP Study 2 (DX-2930-04).

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:	30/06/2022
Deferral of one or more studies contained in the paediatric investigation plan:	Yes