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 and to the deferral

MHRA-100340-PIP01-21-M01

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

Shire Pharmaceuticals Ireland Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Shire Pharmaceuticals Ireland Limited submitted to the licensing authority on 02/11/2021 17:13 GMT an application for a Modification

The procedure started on 29/07/2022 15:42 BST

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-M01

Of 03/10/2022 16:29 BST

On the adopted decision for LANADELUMAB (MHRA-100340-PIP01-21-M01) 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 Modification for LANADELUMAB, Solution for injection , Subcutaneous use .

This decision is addressed to Shire Pharmaceuticals Ireland Limited, Block 2 & 3 Miesian Plaza, 50 – 58 Baggot Street Lower, Dublin , Ireland, 2

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:	31/12/2021
Deferral of one or more studies contained in the paediatric investigation plan:	Yes