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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100179-PIP01-21

Scope of the Application

Active Substance(s)

vemircopan

Condition(s)

Treatment of paroxysmal nocturnal haemoglobinuria

Pharmaceutical Form(s)

Capsule, hard; Age appropriate oral dosage form

Route(s) of Administration

Oral use

Name / Corporate name of the PIP applicant

Alexion Europe SAS

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Alexion Europe SAS submitted to the licensing authority on 30/09/2021 12:23 BST an application for a Paediatric Investigation Plan

The procedure started on 04/02/2022 16:06 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 agree a paediatric investigation plan and grant a deferral and grant a waiver

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-100179-PIP01-21

Of 21/02/2022 14:55 GMT

On the adopted decision for vemircopan (MHRA-100179-PIP01-21) 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 a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for vemircopan , Capsule, hard; Age appropriate oral dosage form , Oral use .

This decision is addressed to Alexion Europe SAS, 103-105 Rue Anatole France , Levallois-Perret, France, 92300

ANNEX I

1. Waiver

1.1 Condition:

Treatment of paroxysmal nocturnal haemoglobinuria The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Capsule, hard; Age appropriate oral dosage form Route(s) of administration: Oral use Reason for granting waiver: on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of paroxysmal nocturnal haemoglobinuria

2.2 Indication(s) targeted by the PIP:

Treatment of paroxysmal nocturnal haemoglobinuria

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):

Capsule, hard; Age appropriate oral dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age appropriate oral form
Non-Clinical Studies	2	Study 2 Exploratory toxicity/efficacy study Study 3 Dose range-finding juvenile toxicity study
Clinical Studies	1	Study 4 Open label single arm study to evaluate the pharmacokinetics, pharmacodynamics, efficacy and safety of ALXN2050 (vemircopan) in children from 2 years to less than 18 year with paroxysmal nocturnal haemoglobinuria (PNH)
Extrapolation, Modeling & Simulation Studies	2	Study 5 Modelling and simulation study to derive dosing of ALXN2050 for use in children from 2 years to less than 18 years to age with paroxysmal nocturnal haemoglobinuria (PNH) Study 6 Extrapolation study to evaluate ALXN2050 for use in children from 2 years to less than 18 years of age with PNH.
Other Studies	0	Not applicable
Other Measures	0	Not applicable

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	30/06/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes