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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral.

MHRA-100992-PIP01-23-M01

Scope of the Application

Active Substance(s)

danicopan

Condition(s)

Treatment of paroxysmal nocturnal haemoglobinuria

Pharmaceutical Form(s)

Tablet

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 26/04/2023 17:09 BST an application for a Modification

The procedure started on 29/08/2023 17:18 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-100992-PIP01-23-M01

Of 25/09/2023 15:11 BST

On the adopted decision for danicopan (MHRA-100992-PIP01-23-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 danicopan, Tablet , ORAL USE .

This decision is addressed to Alexion Europe SAS, 103-105 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 12 years of age. Pharmaceutical form(s): Tablet Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

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 as add-on therapy to a C5 Inhibitor in adolescent patients with signs or symptoms of extravascular haemolysis.

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 12 years to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Tablet.

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 1 Open-label, single arm trial to evaluate the PK/PD, efficacy and, safety of danicopan as add-on therapy to a C5 inhibitor in adolescents from 12 years to less than 18 years of age with paroxysmal nocturnal haemoglobinuria (PNH) who have clinically evident extravascular haemolysis.
Extrapolation, Modeling & Simulation Studies	2	Study 2 Population PK model to establish the appropriate dose of danicopan in adolescents from 12 years to less than 18 years of age with PNH. Study 3 Extrapolation study to evaluate the efficacy, PK/PD and safety of danicopan in adolescents from 12 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:	31/10/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes