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-102252-PIP01-25

Scope of the Application

Active Substance(s)

mavorixafor

Condition(s)

Treatment of WHIM (Warts, Hypogammaglobulinaemia, Infections, and Myelokathexis) syndrome

Pharmaceutical Form(s)

Capsule, hard; Age appropriate solid formulation; Age appropriate liquid formulation

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Norgine Pharmaceuticals Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Norgine Pharmaceuticals Limited submitted to the licensing authority on 18/02/2026 16:19 GMT an application for a Paediatric Investigation Plan

The procedure started on 03/03/2026 17:29 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-102252-PIP01-25

Of 14/07/2026 15:25 BST

On the adopted decision for mavorixafor (MHRA-102252-PIP01-25) 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 mavorixafor, Capsule, hard; Age appropriate solid formulation; Age appropriate liquid formulation , ORAL USE .

This decision is addressed to Norgine Pharmaceuticals Limited, ARC Uxbridge, Building 01, Sanderson Road, Uxbridge, UNITED KINGDOM, UB8 1DH

ANNEX I

1. Waiver

1.1 Condition:

Treatment of WHIM (Warts, Hypogammaglobulinaemia, Infections, and Myelokathexis) syndrome 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 solid dosage formulation Age appropriate oral liquid dosage formulation Route(s) of administration: Oral use Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of WHIM (Warts, Hypogammaglobulinaemia, Infections, and Myelokathexis) syndrome

2.2 Indication(s) targeted by the PIP:

Treatment of WHIM syndrome

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 solid dosage formulation Age appropriate oral liquid dosage formulation

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Development of an age-appropriate solid formulation for oral use in paediatric patients from 6 years to less than 12 years of age. Study 2 Development of an age-appropriate liquid formulation for oral use in paediatric patients from 2 years to less than 6 years of age.
Non-Clinical Studies	2	Study 3 (X4P-001-TOX-030) Dose-range finding study in juvenile dogs. Study 4 (X4P 001 TOX 033) Definitive toxicity study in juvenile dogs.
Clinical Studies	3	Study 5 (X4P-001-103) Double-blind, randomised, placebo controlled trial to evaluate efficacy of mavorixafor in children from 12 years to less than 18 years of age (and adults) with WHIM syndrome. Study 6 (X4P-001-001-PAED) Open-label, single arm trial to evaluate pharmacokinetics, pharmacodynamics, and safety of mavorixafor in children from 6 years to less than 12 years with WHIM syndrome. Study 7 (X4P-001-002-PAED) Open-label, single arm trial to evaluate pharmacokinetics, pharmacodynamics, and safety of mavorixafor in children from 2 years to less than 6 years of age with WHIM syndrome.

Extrapolation, Modeling & Simulation Studies	3	Study 8 (X4P-001 PPK) Population pharmacokinetic analysis and paediatric scaling of mavorixafor in patients with WHIM syndrome. Study 9 (M&S-002) Population pharmacokinetic/pharmacodynamic model. Study 10 (M&S-003/ EXTRAPOL-001) Extrapolation of mavorixafor PK/PD to children, predictive PopPK/PD model in children developed based on study 9. Potential differences in PK and PD processes due to ontogeny and maturation will be incorporated into the model to provide predictive capacity.
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:	31/12/2034
Deferral of one or more studies contained in the paediatric investigation plan:	Yes