MHRA
10 South Colonnade
Canary Wharf
London
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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-101769-PIP01-24

Scope of the Application

Active Substance(s)

Olorofim

Condition(s)

Treatment of invasive aspergillosis, Treatment of non-Aspergillus invasive mould infections

Pharmaceutical Form(s)

Film-coated tablet, Granules

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Shionogi B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Shionogi B.V. submitted to the licensing authority on 13/02/2025 13:45 GMT an application for a Paediatric Investigation Plan

The procedure started on 05/03/2025 17:55 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-101769-PIP01-24

Of 13/06/2025 16:32 BST

On the adopted decision for Olorofim (MHRA-101769-PIP01-24) 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 Olorofim, Film-coated tablet, Granules , ORAL USE .

This decision is addressed to Shionogi B.V., Herengracht 464, Amsterdam, NETHERLANDS, 1017 CA

ANNEX I

1. Waiver

1.1 Condition:

Condition 1: Treatment of invasive aspergillosis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 28 days of age Pharmaceutical form(s): Film-coated tablet Granules 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 Condition 2: Treatment of non-Aspergillus invasive mould infections The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 28 days of age Pharmaceutical form(s): Film-coated tablet Granules 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):

Condition 1: Treatment of invasive aspergillosis Condition 2:Treatment of non-Aspergillus invasive mould infections

2.2 Indication(s) targeted by the PIP:

Condition 1:Treatment of invasive fungal infections due to olorofim-susceptible mould fungi
Condition 2:Treatment of invasive fungal infections due to olorofim-susceptible mould fungi

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

For both conditions: The paediatric population from 28 days to less than 18 years of age

2.4 Pharmaceutical Form(s):

For both conditions: Film-coated tablet Granules

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	(Same study for both conditions) Study 1 Development of an age-appropriate granule formulation for oral administration.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	(Same study for both conditions) Study 2 Single dose (Part 1) and multiple dose, (Part 2), open-label, single-arm trial to assess the pharmacokinetics and safety of olorofim in children aged 28 days to less than 18 years that are candidates to receive mould-active antifungal prophylaxis (Part 1 only) or who have possible, probable, or proven invasive mould infection (IMI).
Extrapolation, Modeling & Simulation Studies	2	(Same study /measure for both conditions) Study 3 Population Pharmacokinetic (PopPK) modelling and simulation study to confirm or modify the paediatric posology to be used in PIP study 2. Extrapolation Plan Studies 2 and 3 are part of an extrapolation plan covering the paediatric population from 28 days to less than 18 years of age.
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:	No
Date of completion of the paediatric investigation plan:	31/07/2036
Deferral of one or more studies contained in the paediatric investigation plan:	Yes