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-100341-PIP01-21-M01

Scope of the Application

Active Substance(s)

ISAVUCONAZONIUM SULFATE

Condition(s)

Treatment of invasive aspergillosis, Treatment of mucormycosis

Pharmaceutical Form(s)

Capsules, hard, Powder for concentrate for solution for infusion

Route(s) of Administration

Oral use; Intravenous use

Name / Corporate name of the PIP applicant

Basilea Medical Ltd.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Basilea Medical Ltd. submitted to the licensing authority on 02/11/2021 14:19 GMT an application for a

The procedure started on 20/06/2022 10:34 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-100341-PIP01-21-M01

Of 11/07/2022 15:37 BST

On the adopted decision for ISAVUCONAZONIUM SULFATE (MHRA-100341-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 for ISAVUCONAZONIUM SULFATE, Capsules, hard, Powder for concentrate for solution for infusion , Oral use, Intravenous drip use .

This decision is addressed to Basilea Medical Ltd., Onslow House, Guildford, United Kingdom, GU1 4TL

ANNEX I

1. Waiver

1.1 Condition:

Treatment of invasive aspergillosis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Capsules, hard; Powder for concentrate for solution for infusion Route(s) of administration: Oral use; Intravenous use Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments. 1.2 Condition: Treatment of mucormycosis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Capsules, hard; Powder for concentrate for solution for infusion Route(s) of administration: Oral use; Intravenous use Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments

2. Paediatric Investigation Plan:

2.1 Condition(s):

Condition 1: Treatment of invasive aspergillosis; Condition 2: Treatment of mucormycosis

2.2 Indication(s) targeted by the PIP:

For Condition 1: Treatment of invasive aspergillosis For Condition 2: Treatment of mucormycosis

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

For both conditions: The paediatric population from 1 year to less than 18 years of age

2.4 Pharmaceutical Form(s):

For both conditions: Capsules, hard; Powder for concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	1	(Same study for both conditions) Study 1: 9766-TX-0066 Thirteen-week, oral gavage repeated-dose study to evaluate safety and toxicokinetics in four groups of juvenile rats treated with isavuconazonium sulfate followed by a 4-week recovery period.
Clinical Studies	2	(Same studies for both conditions) Study 2: 9766-CL-0046 Open-label, multi-centre, noncomparative study to evaluate pharmacokinetics and safety of intravenous and oral isavuconazonium sulfate in children from 1 year to less than 18 years of age with haematologic malignancy. Study 3: 9766-CL-0047 Study deleted in modification EMEA-001301-PIP02-12-M01. Study 4: 9766-CL-0048 Study deleted in modification EMEA-001301-PIP02-12-M02. Study 5: 9766-CL-0107 Open-label, multi-centre, noncomparative study to evaluate safety and tolerability of intravenous and oral isavuconazonium sulfate in children from 1 year to less than 18 years of

		age with invasive aspergillosis or invasive mucormycosis.
Extrapolation, Modeling & Simulation Studies	0	Not applicable
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:	31/01/2023
Deferral of one or more studies contained in the paediatric investigation plan:	Yes