



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

EMA/627435/2021 Corr

## European Medicines Agency decision P/0479/2021

of 3 December 2021

on the acceptance of a modification of an agreed paediatric investigation plan for isavuconazonium (sulfate) (Cresemba), (EMA-001301-PIP02-12-M04) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

### **Disclaimer**

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

**Only the English text is authentic.**



# European Medicines Agency decision

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004<sup>1</sup>,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency<sup>2</sup>,

Having regard to the European Medicines Agency's decision P/0135/2013 issued on 14 June 2013, decision P/0151/2017 issued on 7 June 2017, decision P/0314/2018 issued on 12 September 2018, and decision P/0100/2019 issued on 22 March 2019,

Having regard to the application submitted by Basilea Pharmaceutica International Ltd on 28 June 2021 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 15 October 2021, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

- (1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

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<sup>1</sup> OJ L 378, 27.12.2006, p.1.

<sup>2</sup> OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

**Article 1**

Changes to the agreed paediatric investigation plan for isavuconazonium (sulfate) (Cresemba), powder for solution for infusion, capsule, hard, intravenous use, oral use, including changes to the deferral, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

**Article 2**

This decision is addressed to Basilea Pharmaceutica International Ltd, Grenzacherstrasse 487, 4005 – Basel, Switzerland.



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

EMA/PDCO/420055/2021  
Amsterdam, 15 October 2021

## Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001301-PIP02-12-M04

### Scope of the application

**Active substance(s):**

Isavuconazonium (sulfate)

**Invented name:**

Cresemba

**Condition(s):**

Treatment of invasive aspergillosis

Treatment of mucormycosis

**Authorised indication(s):**

See Annex II

**Pharmaceutical form(s):**

Powder for solution for infusion

Capsule, hard

**Route(s) of administration:**

Intravenous use

Oral use

**Name/corporate name of the PIP applicant:**

Basilea Pharmaceutica International Ltd

**Information about the authorised medicinal product:**

See Annex II



## **Basis for opinion**

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Basilea Pharmaceutica International Ltd submitted to the European Medicines Agency on 28 June 2021 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0135/2013 issued on 14 June 2013, decision P/0151/2017 issued on 7 June 2017, decision P/0314/2018 issued on 12 September 2018, and decision P/0100/2019 issued on 22 March 2019.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 17 August 2021.

## **Scope of the modification**

Some timelines of the Paediatric Investigation Plan have been modified.

## **Opinion**

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
  - to agree to changes to the paediatric investigation plan and to the deferral in the scope set out in the Annex I of this opinion.

The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

## **Annex I**

**The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)**

# 1. Waiver

## 1.1. Condition

Treatment of invasive aspergillosis

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- powder for solution for infusion, capsule, hard, intravenous use, oral use;
- 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 to:

- the paediatric population from birth to less than 1 year of age;
- powder for solution for infusion, capsule, hard, intravenous use, oral use;
- 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

Treatment of invasive aspergillosis

### 2.1.1. Indication(s) targeted by the PIP

Treatment of invasive aspergillosis

### 2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 1 year to less than 18 years of age

### 2.1.3. Pharmaceutical form(s)

Powder for solution for infusion

Capsule, hard

#### 2.1.4. Measures

Area	Number of measures	Description
Quality	0	Not applicable
Non-clinical	1	<b>Study 1: 9766-TX-0066</b> 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	2	<b>Study 2: 9766-CL-0046</b> Open-label, multi-center, 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 <b>Study 3: 9766-CL-0047</b> Study deleted in modification EMEA-001301-PIP02-12-M01. <b>Study 4: 9766-CL-0048</b> Study deleted in modification EMEA-001301-PIP02-12-M02. <b>Study 5: 9766-CL-0107</b> Open-label, multi-center, noncomparative study to evaluate safety and tolerability of intravenous isavuconazonium (sulfate) in children from 1 year to less than 18 years of age with invasive aspergillosis or invasive mucormycosis

#### 2.2. Condition

Treatment of mucormycosis

##### 2.2.1. Indication(s) targeted by the PIP

Treatment of mucormycosis

##### 2.2.2. Subset(s) of the paediatric population concerned by the paediatric development

From 1 year to less than 18 years of age

##### 2.2.3. Pharmaceutical form(s)

Powder for solution for infusion

Capsule, hard



#### 2.2.4. Measures

Area	Number of measures	Description
Quality	0	Not applicable
Non-clinical	1	<b>Study 1</b> Same measure as for condition Treatment of invasive aspergillosis.
Clinical	2	<b>Study 2</b> Same measure as for condition Treatment of invasive aspergillosis. <b>Study 3</b> Study deleted in modification EMEA-001301-PIP02-12-M01. <b>Study 4</b> Study deleted in modification EMEA-001301-PIP02-12-M02. <b>Study 5</b> Same measure as for condition Treatment of invasive aspergillosis.

### 3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By January 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

## **Annex II**

### **Information about the authorised medicinal product**

**Condition(s) and authorised indication(s):**

1. Treatment of invasive aspergillosis

Authorised indication(s):

- Cresemba is indicated in adults for the treatment of invasive aspergillosis.

2. Treatment of mucormycosis

Authorised indication(s):

- Cresemba is indicated in adults for the treatment of mucormycosis in patients for whom amphotericin B is inappropriate.

**Authorised pharmaceutical form(s):**

Capsule, hard

Powder for concentrate for solution for infusion

**Authorised route(s) of administration:**

Oral use

Intravenous use