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EMA/COMP/306060/2014  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Isavuconazonium sulfate for the treatment of invasive aspergillosis

On 4 July 2014 orphan designation (EU/3/14/1284) was granted by the European Commission to Basilea Medical Ltd., United Kingdom, for isavuconazonium sulfate for the treatment of invasive aspergillosis.

#### What is invasive aspergillosis?

Invasive aspergillosis is an infection caused by species of fungi known as *Aspergillus*. These fungi are very widely present in the air, but normally do not cause disease. In some people, usually when the immune system (the body's natural defences) is weakened, the fungus can attack and invade through the lungs and spread throughout the body via the bloodstream, damaging other organs and tissues including the heart, kidney, liver, brain, and bones.

Invasive aspergillosis is a life-threatening disease that can be fatal due to damage to the lungs and other organs.

#### What is the estimated number of patients affected by the condition?

At the time of designation, invasive aspergillosis affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 102,000 people\*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

#### What treatments are available?

At the time of designation, several medicines were authorised in the EU for the treatment of invasive aspergillosis, including voriconazole, posaconazole and amphotericin B.

The sponsor has provided sufficient information to show that isavuconazonium sulfate might be of significant benefit for patients with invasive aspergillosis because clinical studies have shown that it is as effective as authorised treatments but may have an improved safety profile, particularly with

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\*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 511,100,000 (Eurostat 2014).

respect to effects on the liver. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

## **How is this medicine expected to work?**

Isavuconazonium sulfate is a product that belongs to the 'triazole' class of antifungal medicines. Once in the body, it is converted into its active form, isavuconazole, which is thought to work by blocking the actions of enzymes that are involved in making ergosterol, an important component of fungal cell membranes. Without a functional cell membrane, the *Aspergillus* fungus is expected to die or prevented from spreading.

## **What is the stage of development of this medicine?**

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with isavuconazonium sulfate in patients with invasive aspergillosis were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for invasive aspergillosis. Orphan designation of isavuconazonium sulfate had been granted in the United States for treatment of this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 14 May 2014 recommending the granting of this designation.

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Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

## For more information

Sponsor's contact details:

Basilea Medical Ltd  
c/o Cox Costello & Horne Limited  
Langwood House, 63-81 High Street  
Rickmansworth  
Hertfordshire WD3 1EQ  
United Kingdom  
Tel. +44 203 281 7645  
Fax +44 192 377 1988  
E-mail: [medical.information@basilea.com](mailto:medical.information@basilea.com)

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- [Orphanet](#), a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- [European Organisation for Rare Diseases \(EURORDIS\)](#), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

## Translations of the active ingredient and indication in all official EU languages<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Isavuconazonium sulfate	Treatment of invasive aspergillosis
Bulgarian	Изавуконазониев сулфат	Лечение на инвазивна аспергилоза
Croatian	Izavukonazonijev sulfat	Liječenje invazivne aspergiloze
Czech	Isavuconazonium sulfát	Léčba invazivní aspergilózy
Danish	Isavuconazonium-sulfat	Behandling af invasiv aspergillose
Dutch	Isavuconazoniumsulfaat	Behandeling van invasieve aspergillose
Estonian	Isavukonasoonisulfaat	Invasiivse aspergilloosi ravi
Finnish	Isavukonatsoniumsulfaatti	Invasiivisen aspergilloosin hoito
French	Sulfate d'isavuconazonium	Traitement des aspergilloses invasives
German	Isavuconazoniumsulfat	Behandlung der invasiven Aspergillose
Greek	Θειικό ισαβουκοναζόνιο	Θεραπεία της εν τω βάθει ασπεργίλλωσης
Hungarian	Izavukonazonium szulfát	Invazív aspergillosis kezelése
Italian	Isavuconazonio solfato	Trattamento dell'aspergillosi invasiva
Latvian	Izavukonazonija sulfāts	Invazīvas aspergilozes ārstēšana
Lithuanian	Izavukonazonio sulfatas	Invazinės aspergiliozės gydymas
Maltese	Isavuconazonium sulfate	Kura tal-aspergilloosi li tinxtered mal-ġisem
Polish	Siarczan izawukonazonium	Leczenie inwazyjnej aspergilozy
Portuguese	Sulfato de isavuconazol	Tratamento da aspergilose invasiva
Romanian	Sulfat de isavuconazon	Tratamentul aspergilozei invazive
Slovak	Izavukonazón sulfát	Liečba invazívnej aspergilózy
Slovenian	Izavukonazonijev sulfat	zdravljenje invazivne aspergiloze
Spanish	Sulfato de isavuconazonio	Tratamiento de aspergilosis invasiva
Swedish	Isavukonazoniumsulfat	Behandling av invasiv aspergillos
Norwegian	Isavukonazonium sulfate	Behandling av invasiv aspergillose
Icelandic	Isavúkónazóníum sulfate	Meðferð á ífarandi aspergillosis

<sup>1</sup> At the time of designation