



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Isavuconazonium sulfate for the treatment of mucormycosis

On 4 June 2014, orphan designation (EU/03/14/1276) was granted by the European Commission to Basilea Medical Ltd, the United Kingdom, for isavuconazonium sulfate for the treatment of mucormycosis.

What is mucormycosis?

Mucormycosis is a severe infection with fungi of the order Mucorales. These fungi are commonly found in the environment, such as in soil and decaying matter, and usually only cause disease in people who have a weakened immune system, including patients receiving dialysis for kidney failure and those who have uncontrolled diabetes or acute myeloid leukaemia (a cancer of the blood). It is a serious infection that can affect the sinuses, brain, lungs, and sometimes the skin. Depending on the organ involved symptoms may include facial pain, chest pain, fever and ulcerations.

Mucormycosis is a life-threatening disease because the fungi can invade the surrounding blood vessels causing blood clots and cause death of the infected tissue in affected organs.

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

At the time of designation, mucormycosis affected approximately 0.06 in 10,000 people in the European Union (EU). This was equivalent to a total of around 3,000 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 for the treatment of mucormycosis in the EU.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with mucormycosis because preliminary clinical data showed a favourable response

*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).



to the medicine in patients who did not respond to treatment with currently authorised medicines. 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 anti-fungal 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 fungus is expected to die or is 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 the medicine in patients with mucormycosis were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 April 2014 recommending the granting of this designation.

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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Isavuconazonium sulfate	Treatment of mucormycosis
Bulgarian	Изавуконазониев сулфат	Лечение на мукормикоза
Croatian	Izavukonazonijev sulfat	Liječenje mukormikoze
Czech	Isavuconazonium sulfát	Léčba mukormykózy
Danish	Isavuconazonium-sulfat	Behandling af mukormykose
Dutch	Isavuconazoniumsulfaat	Behandeling van mucormycose
Estonian	Isavukonasoonsulfaat	Mukormükoosi ravi
Finnish	Isavukonatsoniumsulfaatti	Mukormykoosin hoito
French	Sulfate d'isavuconazonium	Traitement des mucormycoses
German	Isavuconazoniumsulfat	Behandlung der Mukormykose
Greek	Θεικό ισαβουκοναζόνιο	Θεραπεία της μουκορμικητίασης
Hungarian	Izavukonazonium szulfát	Mucormycosis kezelése
Italian	Isavuconazonio solfato	Trattamento delle mucormicosi
Latvian	Izavukonazonija sulfāts	Mukormikozes ārstēšana
Lithuanian	Izavukonazonio sulfatas	Mukormikozės gydymas
Maltese	Isavuconazonium sulfate	Kura tal-mukormikożi
Polish	Siarczan izawukonazonium	Leczenie mukormikozy
Portuguese	Sulfato de isavuconazol	Tratamento da mucormicose
Romanian	Sulfat de isavuconazon	Tratamentul mucormicozei
Slovak	Izavukonazón sulfát	Liečba mukormykózy
Slovenian	Izavukonazonijev sulfat	Zdravljenje mukormikoze
Spanish	Sulfato de isavuconazonio	Tratamiento de mucormicosis
Swedish	Isavukonazoniumsulfat	Behandling av mukormykos
Norwegian	Isavukonazonium sulfat	Behandling av mukormykose
Icelandic	Isavúkónazóníum súlfat	Meðferð á myglusveppasýkingu

¹ At the time of designation