Study Number: 98-0-046/FG463-21-01
EudraCT number: NA
ClinicalTrials.gov Identifier: NCT00036166

Summary of Results for Laypersons

What was the Study Called?

An Open-label, Non-comparative Study of FK463 for the Treatment of Invasive Aspergillosis

Why was this Study Needed?

Micafungin (also known as FK463 and Mycamine®) is a prescription medicine used to treat patients when a fungal infection has spread throughout their body (called an "invasive" infection). Invasive fungal infections can be caused by a yeast called *Candida* (invasive candidiasis) or a mold called *Aspergillus* (invasive aspergillosis). Patients who had a bone marrow or organ transplant, are having chemotherapy, or who have very few neutrophils (a type of white blood cell) are at risk for these types of fungal infections. Chemotherapy is a type of cancer treatment that uses drugs to destroy cancer cells.

This study was done to find out if micafungin was safe and effective in treating patients with proven or probable invasive aspergillosis infections. Also, it was important to find out what unwanted effects micafungin might cause.

This study for micafungin took place at 62 clinics in the United States, Brazil, Canada, Germany, the United Kingdom, Peru, France, South Africa, Italy, Spain and Sweden. The study took place from January 1999 to December 2002. When the study ended the sponsor (Astellas) reviewed all the study information and created a report of the results. This is a summary of that report.

What Kind of Study was This and Who Took Part in it?

This was an "open-label" study. Open-label means that the patients knew the name of the study medicine that they were given. In this study all patients took the same study medicine. Micafungin was given to the patient in a vein in the arm. The patients received micafungin daily for at least 7 days. The duration of this study was 90 days; however, it was extended further with the approval of the medical monitor. Micafungin could have been given 3 times a week if daily treatment was not possible and the patient had responded to micafungin. The starting dose for micafungin was 75 mg daily. If the starting dose was well tolerated, the dose was increased in 75 mg increments after 7 days of treatment. For patients enrolled in Europe, the dose of micafungin could not be higher than 200 mg daily.

Both men and women and adults and children took part in the study. They had proven or probable invasive aspergillosis.

Patients may have been:

- Newly diagnosed (de novo).
- Patients who failed to respond to other treatments (efficacy failures). For efficacy failure patients, micafungin either replaced their current treatment (micafungin alone) or was added to their current treatment (micafungin + other medicine).

- Patients who had to stop other treatments because of toxicity (toxicity failure).

Only patients 18 years and older who had failed treatment were enrolled in Europe.

A total of 331 patients were enrolled in the study and 326 patients received at least 1 dose of study medicine.

	Number of Patients
Age Group Aged less than 16 years Aged 16 years and older	 70 256
Men Women	210 116
EU Countries Outside EU	62 264

What Were the Study Results?

The results of the study showed that 100 mg daily, with the option of dose increases, is the best dose of micafungin to start treatment of invasive aspergillosis. Micafungin was effective, safe and able to be used in combination with other medicines to treat invasive aspergillosis.

What Adverse Reactions did Patients Have?

A lot of research is needed to know whether a medicine causes a medical problem. So when new medicines are being studied researchers keep track of all medical problems that patients have while they are in the study. These medical problems are called "adverse events" and are recorded whether or not they might be caused by the treatment taken. An "adverse reaction" is any medical problem or "adverse event" that is judged by the study doctor to be possibly caused by a medicine or treatment used in the study.

The chart below shows the most common adverse reactions experienced by patients taking part in this study.

Adverse Reaction	**De Novo (out of 23 patients)**	**Efficacy Failure Patients**		**Toxicity Failure (out of 23 patients)**	**Total (out of 326 patients)**
		Micafungin + Other Medicine (out of 257 patients)	**Micafungin Alone (out of 23 patients)**		
Increased blood level of a liver pigment (bilirubin) often a sign of liver problems	1	13	0	0	14
Nausea or the urge to vomit	0	10	0	4	14
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Adverse Reaction	De Novo (out of 23 patients)	Efficacy Failure Patients		Toxicity Failure (out of 23 patients)	Total (out of 326 patients)
		Micafungin + Other Medicine (out of 257 patients)	Micafungin Alone (out of 23 patients)		
Increased blood level of a liver or bone enzyme (alkaline phosphatase)	0	7	2	0	9
Increased blood level of a liver enzyme (ALT/SGPT)	0	8	1	0	9
Vomiting	0	6	0	3	9
High blood pressure	0	7	0	0	7
Diarrhea	0	7	0	0	7

An adverse reaction is considered "serious" when it is life-threatening, causes lasting problems or needs hospital care. Thirty-one patients experienced at least 1 serious adverse reaction:

- 2 patients in the de novo group.
- 22 patients in the efficacy failure micafungin + other medicine group.
- 5 patients in the efficacy failure micafungin alone group.
- 2 patients in the toxicity failure group.

A total of 183 patients died during the study. One patient's death (efficacy failure micafungin + other medicine group) was judged by the study doctor to be related to study medicine.

Where Can I Learn More About This Study?

Astellas may perform additional studies to better understand micafungin.

This summary of the clinical study results is available online at http://www.astellasclinicalstudyresults.com. Please remember that researchers look at the results of many studies to find out how well medicines work and which adverse reactions they might cause. If you have questions about micafungin, please discuss these with your doctor.

Sponsor contact details:

Astellas Pharma Global Development, Inc. (formerly known as Fujisawa Healthcare, Inc.)

1 Astellas Way

Northbrook, IL 60062

USA