Summary of Results for Laypersons

What was the Study Called?

An Open-label, Non-comparative Study of FK463 in the Treatment of Candidemia or Invasive Candidiasis

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 have had an organ transplant are at risk for these types of fungal infections. Patients with human immunodeficiency virus (HIV)/acquired immune deficiency syndrome (AIDS) and patients who have very few neutrophils (a type of white blood cell) are at risk for these types of fungal infections. Patients who have long hospital stays, have central venous catheters and have been on antibiotics, steroids and parenteral hyperalimentation are at increased risk for these yeast infections. A central venous catheter is a tube inserted in a large vein that returns blood to the heart. Parenteral hyperalimentation is when a person is fed through a vein, bypassing the usual process of eating and digestion.

This study was done to find out if micafungin was safe and effective in treating patients with candidemia or invasive candidiasis caused by both *Candida albicans* and non-*albicans Candida* species organisms. 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, Canada, Brazil, South Africa, Peru, Italy, the United Kingdom, France, Chile, Guatemala, Germany, Sweden and Poland. The study took place from February 1999 to December 2002. When the study ended the sponsor 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 5 days and up to a maximum of 6 weeks. 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 50 mg daily. If the starting dose was well tolerated, the dose could have been increased in 50 mg increments after 5 days of treatment. For patients enrolled in Europe, the dose of micafungin could not be higher than 200 mg daily. The starting dose was 100 mg for patients with a specific type of candidiasis (*Candida* infections found to be germ tube negative or identified as non-*albicans Candida* species).

Both men and women and adults and children took part in the study. They had a confirmed diagnosis of candidemia or invasive candidiasis.

Patients may have been:

- Newly diagnosed (de novo). These patients were with newly diagnosed candidiasis who received no more than 48 hours of prior systemic antifungal treatment.
- Patients who failed to respond to other treatments (efficacy failure). These were patients had confirmed candidiasis and had received more than 5 days of prior systemic antifungal therapy with no response. For efficacy failure patients, micafungin either replaced their current treatment (micafungin alone) or was added to their current treatment (micafungin + other medicine).

Only patients 18 years and older were enrolled in Europe.

A total of 357 patients were enrolled in the study and 353 patients received at least 1 dose of study medicine.

	Number of Patients
Age Group	
Aged less than 16 years	53
Aged16 years and older	300
Men	201
Women	152
EU Countries	15
Outside EU	338

What Were the Study Results?

The results of the study showed that 50 mg of micafungin daily was effective in treating patients with *Candida albicans* infections. Micafungin at a dose of 100 mg daily was effective in treating non-*albicans Candida* species infections. Micafungin can be safely increased up to 400 mg daily for up to 340 days for these patients.

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

Adverse Reaction	De Novo (out of 215 patients)	Efficacy Failure Patients		Total (out of 353 patients)
		Micafungin + Other Medicine (out of 83 patients)	Micafungin Alone (out of 55 patients)	
Increased blood level of a liver enzyme (aspartate aminotransferase)	26	1	1	28
Increased blood level of magnesium	22	0	2	24
Increased blood level of a liver enzyme (alanine aminotransferase)	22	1	0	23
Decreased number of white blood cells	21	1	0	22
Increased blood level of a liver or bone enzyme (alkaline phosphatase)	20	2	0	22
Decreased blood level of calcium	19	0	1	20
Vomiting	11	2	1	14
Nausea or the urge to vomit	4	1	4	9

An adverse reaction is considered "serious" when it is life-threatening, causes lasting problems or needs hospital care. A total of 22 patients experienced at least 1 serious adverse reaction:

- 15 patients in the de novo group.
- 3 patients in the efficacy failure micafungin + other medicine group.
- 4 patients in the efficacy failure micafungin alone group.

A total of 105 patients died during the study. None of the deaths were 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.

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