Summary of Results for Laypersons

Astellas is grateful to the patients who took part in this clinical study. Thank you.

What was the Study Called?

A Multi-center, Open-label, Non-comparative Study to Evaluate the Efficacy and Safety of Micafungin 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 mold called *Aspergillus* (invasive aspergillosis). Patients who are at risk for these types of fungal infections include patients who had a bone marrow or organ transplant, patients having chemotherapy (chemotherapy is a type of cancer treatment that uses drugs to destroy cancer cells), or patients who have very few neutrophils (a type of white blood cell).

This study was conducted in China in patients who had been diagnosed with invasive fungal infection caused by *Aspergillus*. In this study all patients were given the same study drug, micafungin. The main question this study helped answer was how well micafungin works in Asian patients with diagnosed invasive aspergillosis. The study looked at the number of patients who survived and if the symptoms of their disease were gone (complete response) and/or who survived and showed improvement in the symptoms of their disease (partial response). It was also important to find out what unwanted effects these patients had from micafungin.

The study started in August 2013 and ended in July 2015. The study was stopped earlier than planned because not enough patients joined the study. When the study ended, 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. This means that patients and study doctors knew the patients took micafungin.

This study included adult women and men who were thought to have invasive aspergillosis. They had not taken any medications for their fungal infection within 1 month before the study started. They were expected to live longer than 1 month, their liver and kidney worked sufficiently and they did not have human immunodeficiency virus (HIV) before entering the study.

Micafungin was given to the patient through a vein in the arm once a day. The dose was 50 to 300 mg, as needed. The patients who did not have hematologic (blood) diseases received micafungin for 2 weeks and up to 4 weeks, if needed. Patients with diseases of the blood received micafungin for 4 to 6 weeks and up to 12 weeks for severe infections.

This study took place at 32 clinics throughout China. 68 patients were in the study and 61 patients received at least 1 dose of micafungin. Out of these 61 patients, 42 had been diagnosed with invasive aspergillosis and could be evaluated for how well the micafungin treatment worked.

	Number of Patients
Age Group	
60 years or younger	35
Older than 60 years	7
Sex	
Men	26
Women	16

What Were the Study Results?

This study in Asian patients with invasive aspergillosis evaluated how well micafungin treatment worked. The study looked at the number of patients who survived and if the symptoms of their disease were gone (complete response) and/or who survived and showed improvement in the symptoms of their disease (partial response). Because this study stopped early, we do not fully know the answer to the question that was studied. This is a summary of what we learned while the study was open. In 19 patients out of the 42 patients who had been diagnosed with invasive aspergillosis (19/42, approximately 45%) micafungin worked well. This result in China was similar to the results reported from micafungin studies conducted in other countries.

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.

Six patients out of 61 who were given at least 1 dose of micafungin had 8 adverse reactions. The adverse reactions the patients had included drug induced liver damage, liver worked poorly, kidney damage, skin rash, decrease in the total number of white blood cells (leukocytes) and decrease in a blood protein called albumin.

An adverse reaction is considered “serious” when it is life-threatening, causes lasting problems or needs hospital care.

None of the patients experienced serious adverse reactions related to micafungin.

Six patients died during the study. None of the patients died because of micafungin.

Where Can I Learn More About This Study?

This document is a short summary of the main results from this study and reflects the information available as of June 2016. You can find this summary and more information about this study 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. This summary only shows the results of this 1 study. If you have questions about micafungin, please discuss these with your doctor.

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