Summary of Results for Laypersons

Astellas is grateful to parents and babies who took part in this clinical study. Thank you.

What was the Study Called?

A Phase 1 Open-Label Study of the Safety and Pharmacokinetics of Repeated-Dose Micafungin in Neonates

Why was this Study Needed?

When babies are in the hospital they are at risk for getting fungal infections. One reason for this risk is the use of antibiotics. Another is when doctors puncture, open or cut the skin for a procedure. There is also a risk of getting a fungal infection if the baby's immune system is not working well.

Fungal infections can be caused by a yeast called *Candida*. If the infection with *Candida* has spread throughout the body it is called systemic candidiasis. When it is in the blood it is called candidemia. There was a need to find new treatments for babies who have fungal infections.

Researchers wanted to learn about a medicine called micafungin. In this study, researchers wanted to learn how much micafungin stayed in the blood of babies. This would help researchers work out the best dose and how often micafungin should be given. In this study, babies received micafungin for treatment of candidiasis. They also received micafungin if the study doctors thought the babies might have candidemia.

Also, researchers wanted to learn if these babies had any unwanted effects from micafungin.

The study started in August 2007 and ended in October 2007. The sponsor of this study (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 each patient and a study doctor know which medicine that patient received. In this study, the parents of each baby and the study doctors knew that the baby received micafungin.

This study included babies between 2 days and 120 days old. They had systemic candidiasis or the study doctors thought the babies might have candidemia.

In this study, each baby received micafungin given slowly over 1 hour. The amount they received depended on their weight.

- Babies who weighed less than 1000 grams (2.2 pounds) received 10 milligrams (mg) of micafungin for each kilogram (kg) of their body weight. This is also known as 10 mg/kg micafungin.

- Babies who weighed 1000 grams (2.2 pounds) or more received 7 mg of micafungin for each kg of their body weight. This is also known as 7 mg/kg micafungin.

All of these babies received micafungin once a day, every day for 4 days.

On the day of the last dose (day 4) micafungin was measured in each baby's blood. A few small blood samples were taken from each baby up to 24 hours after this dose.

These babies continued to be checked for up to 3 days after their last dose of micafungin.

This study took place at 5 clinics in the US. 13 babies were in the study and received at least 1 dose of micafungin.

	Number of Babies
Age Group Aged 3 days to 119 days	 13
Sex Boys Girls	 6 7

What Were the Study Results?

In this study, researchers wanted to learn how much micafungin stayed in the blood in these babies. This helped researchers work out the best dose and how often the study medicine should be given. Researchers wanted to learn if the amount of micafungin in the babies' blood was 166.5 or more units. Researchers thought this amount of micafungin should work well in babies with systemic candidiasis or candidemia.

13 babies took part in this study.

- 6 babies weighed less than 1000 grams (2.2 pounds) and received 10 mg/kg of micafungin.
- 7 babies weighed 1000 grams (2.2 pounds) or more and received 7 mg/kg of micafungin.

All 13 received micafungin once a day, every day for 4 days.

One baby weighed more than 1000 grams (2.2 pounds) and received 10 mg micafungin by mistake, so was not included in this result.

12 babies were included in this result. After the last dose of micafungin on day 4, the following happened:

- All 6 babies that received 10 mg/kg of micafungin had 166.5 or more units of micafungin in their blood.
- Of the 6 babies that received 7 mg/kg micafungin, 5 had 166.5 or more units of micafungin in their blood. One baby had 162.6 or more units of micafungin in their blood.
- All but one baby had the required amount of micafungin in their blood.

What Adverse Reactions did Babies in the Study 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.

3 babies (23.1%, or 3 out of 13 babies) had adverse reactions in this study.

The table below shows the most common adverse reactions experienced by babies who received at least 1 dose of micafungin in this study.

Adverse Reaction	Micafungin (out of 13 babies)
Vein becomes swollen at the infusion site	1 (7.7%)
Increased blood level of a liver or bone enzyme (alkaline phosphatase)	1 (7.7%)
Decreased blood level of potassium	1 (7.7%)
Reaction that can occur during or following infusion of the drug. The reaction may include fever, chills, rash, low blood pressure, and difficulty breathing.	1 (7.7%)

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

One baby (7.7%) who received 7 mg micafungin for each kg of their body weight had a serious adverse reaction to micafungin.

Where Can I Learn More About This Study?

This document is a short summary of the main results from this study. 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. Your baby's doctor may help you understand more about the results of this study.

Sponsor contact details:

Astellas Pharma Global Development Inc.
1 Astellas Way
Northbrook IL 60062
USA