Summary of Results for Laypersons

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

What was the Study Called?

Determination of Plasmatic and CSF Levels of High Doses of Micafungin in Neonates Suffering from Systemic Candidiasis and/or *Candida* Meningitis

Why was this Study Needed?

When neonates (infants aged 1 month or less) are in the hospital they are at risk for getting fungal infections. One reason for this risk is the use of antibiotics. And there is risk when doctors puncture, open or cut the skin for a procedure. Fungal infections can be caused by yeast called *Candida*. If the infection with *Candida* has spread throughout the body it is called systemic candidiasis. Micafungin (also known as FK463 and Mycamine®) is a prescription medicine given through a vein to treat fungal infections. Other studies have looked at dosing in infants with micafungin. More information was needed on higher doses of micafungin in infants and neonates.

This study was conducted in infants and neonates with systemic candidiasis. The children received 8 mg of micafungin for each kg of their body weight per day. The question this study helped answer was what was the amount of micafungin in the child's plasma with this dose. Plasma is fluid found in the blood. It was also important to find out what unwanted effects these patients had from the study medicine.

The study started in May 2015 and ended in April 2018. When the study ended, 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. This means that the parents of each child and the study doctors knew which study medicine the children received (micafungin).

Infants and neonates who had systemic candidiasis were in the study. They may or may not have had *Candida* meningitis. *Candida* meningitis is inflammation (swelling and redness) of the connective tissue surrounding the brain and spinal cord as a result of infection with *Candida*. And they may or may not have had excess fluid in the brain. They were expected to survive for at least 3 days.

Each child in the study received micafungin through a vein. They received 8 mg for each kg of their body weight every day, for at least 2 weeks or longer. Between the 3rd and the 10^{th} days of dosing, micafungin was measured in the child's plasma. Blood was obtained from a small blood vessel (capillary) in the child's heel. It was measured at 1 hour, 3 hours and 8 hours after they had received their dose.

This study took place at 2 clinics in Italy. 35 children were in the study.

	Number of Children (Out of 35)
Age Group	
Aged 0 to 4 weeks	8
Aged 4 weeks to 4 months	20
Aged 4 months to 6 months	3
Aged 6 months to less than 2 years	4
Sex	
Boys	20
Girls	15

What Were the Study Results?

This study was in infants and neonates with systemic candidiasis. The question this study helped answer was what was the amount of micafungin in the child's plasma after the child received 8 mg of micafungin per kg of weight.

In this study 34 children had micafungin in their plasma measured after treatment.

	Time After Micafungin IV Infusion (out of 34 patients)		
	1 hour after infusion	3 hours after infusion	8 hours after infusion
Average amount of micafungin in plasma in µg/mL (µg/mL is the µg of micafungin in each mL of the child's plasma)	17.233	15.591	10.273

The results in this study were similar to earlier studies at similar doses. Micafungin can be safely given to neonates and infants with systemic candidiasis at a dose of 8 mg per kg of body weight per day.

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.

One child (2.9%, or 1 out of 35 patients) had an adverse reaction of increased blood levels of liver enzymes in this study.

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

None of the children experienced a serious adverse reaction in this study.

Five children died during this study. None of the children 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 January 2019. 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 child's doctor may help you understand more about the results of this study.

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