# CLINICAL TRIAL RESULTS

This summary reports the results of only one study. Researchers must look at the results of many types of studies to understand if a study medicine works, how it works, and if it is safe to prescribe to patients. The results of this study might be different than the results of other studies that the researchers review.

**Sponsor:** Pfizer, Inc.

**Medicine(s) Studied:** Ceftaroline (PF-06926068)

**Protocol Number:** C2661002

**Dates of Trial:** 04 August 2015 to 30 December 2017

**Title of this Trial:** Open-label, Multicentre Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Efficacy of Ceftaroline in Neonates and Young Infants with Late-Onset Sepsis

**Date of this Report:** 23 October 2018

## *– Thank You –*

Pfizer, the Sponsor, would like to thank you and your child for participating in this clinical trial and provide you a summary of results representing everyone who participated. If you have any questions about the study or results please contact the doctor or staff at your child's study site.

# WHY WAS THIS STUDY DONE?

"Sepsis" is the name for the body's response to a serious infection. Sepsis can be life-threatening, especially in very young babies. Babies with sepsis might have symptoms like trouble breathing, fever, or rash. Sepsis in babies is usually treated with antibiotics.

Ceftaroline is an antibiotic medicine that is used to treat certain infections in children and adults. This study was designed to learn more about using ceftaroline to treat infections in very young babies. Ceftaroline has not been approved for this use, as it is still being studied.

The main purpose of this study was to learn more about the safety of ceftaroline in very young babies with sepsis. The researchers wanted to answer this question:

- **Did the babies in this study have any medical problems? If so, what medical problems did the babies have?**

# WHAT HAPPENED DURING THE STUDY?

This study was for babies with sepsis who were at least 7 days old, but younger than 60 days old when the study started.

First, the babies were checked by the study doctor to make sure they were a good fit for the study. This was called "screening".

The babies were grouped by age:

- Group 1: Babies older than 28 days, but younger than 60 days
- Group 2: Babies between 7 days and 28 days old who were born full-term (the pregnancy lasted at least 39 weeks)
- Group 3: Babies between 7 days and 28 days old who were born prematurely (the pregnancy lasted less than 39 weeks)

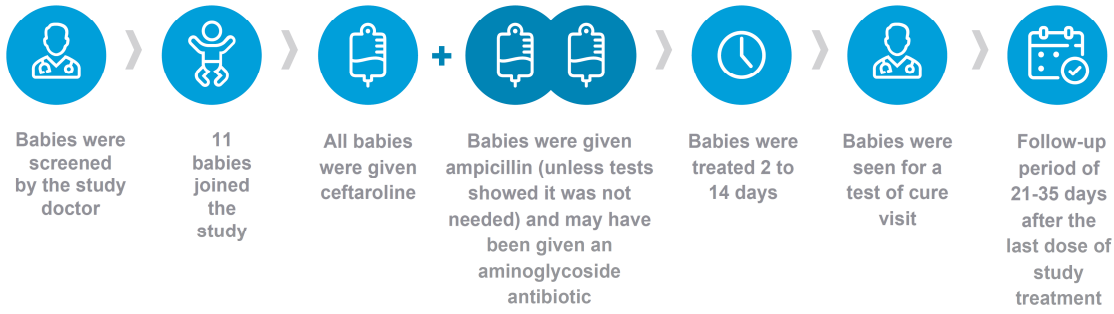
All the babies in this study were given ceftaroline **and** a standard treatment for sepsis. The standard treatment given to babies was an antibiotic called "ampicillin" (all babies in this study received ampicillin unless test results showed that it was not needed to

treat the infection). In addition, the study doctor could prescribe another antibiotic called an "aminoglycoside" if they thought it was necessary. The medicines were given by IV (a needle in the vein), and the doses were based on each baby's weight.

This was an "open-label" study, which means that both the researchers and the babies' parents/caregivers knew which medicines were being given.

Safety was carefully considered throughout the study. The study doctors examined each baby, did blood tests, and watched for any medical problems. The babies had to stay in the hospital the entire time they were receiving study treatment. The study doctors also followed up with the babies and their parents/caregivers for an additional 21 to 35 days after their last dose of study treatment.

The figure below shows what happened during this study.




While babies were only in the study for up to 49 days (treatment plus follow-up), the entire study took over 2 years to complete. Babies joined the study at 1 of 4 locations in Hungary and the United States. The first baby joined the study on 04 August 2015 and the last baby finished the study on 26 December 2017. A total of 5 girls and 6 boys joined the study.

The babies were treated for 2 days to 14 days. All 11 babies (100%) who started the study completed it. However, only 7 babies (64%) finished their full course of study treatment. This was because the health of the other 4 babies (36%) improved enough that they were able to leave the hospital before they finished their study treatment.

This study ended earlier than planned, because there was enough information available to make ceftaroline dosing recommendations for very young babies. When the study ended in December 2017, the Sponsor began reviewing the information

collected. The Sponsor then created a report of the results. This is a summary of that report.

## WHAT WERE THE RESULTS OF THE STUDY?

Study doctors examined how well the study treatment worked on the last day the baby received study treatment and at a follow-up visit 8 to 15 days after the last dose of study treatment.

On the last day that study treatment was received, sepsis was either cured or improving in all 11 (100%) babies. At the follow-up visit, sepsis was cured in 7 out of 11 (64%) babies. Study doctors were not able to assess how well the study treatment worked in the 4 babies who were not considered cured. These 4 babies stopped their study treatment early because they were improving and received other medications so that they could be discharged home.

## WHAT MEDICAL PROBLEMS DID PATIENTS HAVE DURING THE STUDY?

The researchers recorded any medical problems the babies had during the study. Babies could have had medical problems for reasons not related to the study (for example, caused by an underlying disease or by chance). Or, medical problems could have been caused by a study treatment, or by another medicine the baby was taking. Sometimes the cause of a medical problem is unknown. By comparing medical problems across many treatment groups in many studies, doctors try to understand what the side effects of an experimental drug might be.

Out of 11 babies in this study, 5 babies (46%) had at least 1 non-serious medical problem (that means a medical problem that is not life-threatening, does not cause lasting problems, or does not need hospital care). Of the medical problems experienced by these 5 babies, diarrhea was the only medical problem that the study doctors determined may have been related to the study treatment. Diarrhea occurred in 2 babies (18%) and was considered related to the study treatment for 1 of these babies.

The table below shows the medical problems that happened during the study.

| Medical Problems | | | | |
|---|---|---|---|---|
| Medical Problem | Group 1 (4 babies) | Group 2 (5 babies) | Group 3 (2 babies) | Total (11 babies) |
| Diarrhea | 1 (25%) | 1 (20%) | 0 (0%) | 2 (18%) |
| Ear Infection | 0 (0%) | 0 (0%) | 1 (50%) | 1 (9%) |
| Fluid-filled sac in brain | 0 (0%) | 1 (20%) | 0 (0%) | 1 (9%) |
| Low number of red blood cells | 0 (0%) | 0 (0%) | 1 (50%) | 1 (9%) |
| Salmonellosis (a type of infection) | 0 (0%) | 0 (0%) | 1 (50%) | 1 (9%) |
| Skin rash | 0 (0%) | 1 (20%) | 0 (0%) | 1 (9%) |
| Stuffy nose | 0 (0%) | 0 (0%) | 1 (50%) | 1 (9%) |
| Swelling in kidneys | 1 (25%) | 0 (0%) | 0 (0%) | 1 (9%) |
| Thrush (yeast infection in the mouth) | 0 (0%) | 1 (20%) | 0 (0%) | 1 (9%) |

## WERE THERE ANY SERIOUS MEDICAL PROBLEMS?

A medical problem is considered "serious" when it is life-threatening, causes lasting problems, or needs hospital care.

One (1) baby (9%) in this study had a serious medical problem that required hospital care. This baby had salmonellosis (a type of infection in the baby's digestive system), which the study doctors determined was not related to the study treatment. No babies passed away during the study.

Overall, the medical problems reported in this study are similar to the medical problems reported in past studies with older children and adults and are not unexpected in babies with infection. No new issues related to the safety of the study treatment were found.

## WHERE CAN I LEARN MORE ABOUT THIS STUDY?

If you have questions about the results of your child's study, please speak with the doctor or staff at your child's study site.

The full scientific report of this study is available online at:

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|---|---|
| www.clinicaltrials.gov | Use the study identifier NCT02424734 |
| www.clinicaltrialsregister.eu | Use the study identifier 2014-003243-34 |

Please remember that researchers look at the results of many studies to find out which medicines work best and are safest for patients. No future studies with ceftaroline are planned at this time.

Again, **thank you** for volunteering. We do research to try to find the best ways to help patients, and you helped us to do that!