Clinical Study 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 medication 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:	Amplyx Pharmaceuticals, Inc. (owned by Pfizer Inc.)
Medicine Studied:	Fosmanogepix (PF-07842805 or APX001)
Protocol Number:	C4791010 (APX001-202)
Dates of Study:	04 January 2020 to 09 May 2022
Title of this Study:	A Study of Fosmanogepix in Participants With Invasive Mold Infections [A Phase 2, Open-Label Study to Evaluate the Safety and Efficacy of APX001 in the Treatment of Patients With Invasive Mold Infections Caused by Aspergillus Species or Rare Molds]
Date(s) of this Report:	27 March 2023

– Thank You –

If you participated in this study, Pfizer, the Sponsor, would like to thank you for your participation.

This summary will describe the study results. If you have any questions about the study or the results, please contact the doctor or staff at your study site.

Why was this study done?

What is invasive mold infection?

Mold infections are caused by the growth of a fungus in the body. "Invasive" means the infection has spread to one or more parts of the body where it is causing disease. Invasive mold infection (IMI) may be due to different types of molds. IMI mostly happens in people with a weakened immune system and can be deadly if not treated.

Antifungal drugs are medicines that kill or stop the growth of mold. Standard antifungal drugs can treat IMI in many cases. But, for some patients, there are few or no options to treat IMI because:

- For some molds, there is no available medicine that works. These are called "drug-resistant" molds.
- Medical problems caused by available antifungal drugs required a patient to stop taking those medicines.

IMI is often severe and can lead to death in about 30% to 80% of people with these infections.

What is fosmanogepix?

Fosmanogepix is the study medication that was being tested in this study. It was given as an injection into the vein or as a tablet taken by mouth.

Laboratory research has shown that fosmanogepix can kill *Aspergillus* and other molds that cause IMI, including many drug-resistant molds.

What was the purpose of this study?

The purpose of this study was to see the effect of fosmanogepix as a treatment for IMI in patients who could not receive other treatment options. The study measured if fosmanogepix can prevent death, and whether it is safe and well tolerated.

Researchers wanted to know:

Does fosmanogepix help to prevent death from any cause within 42 days after starting treatment?

What happened during the study?

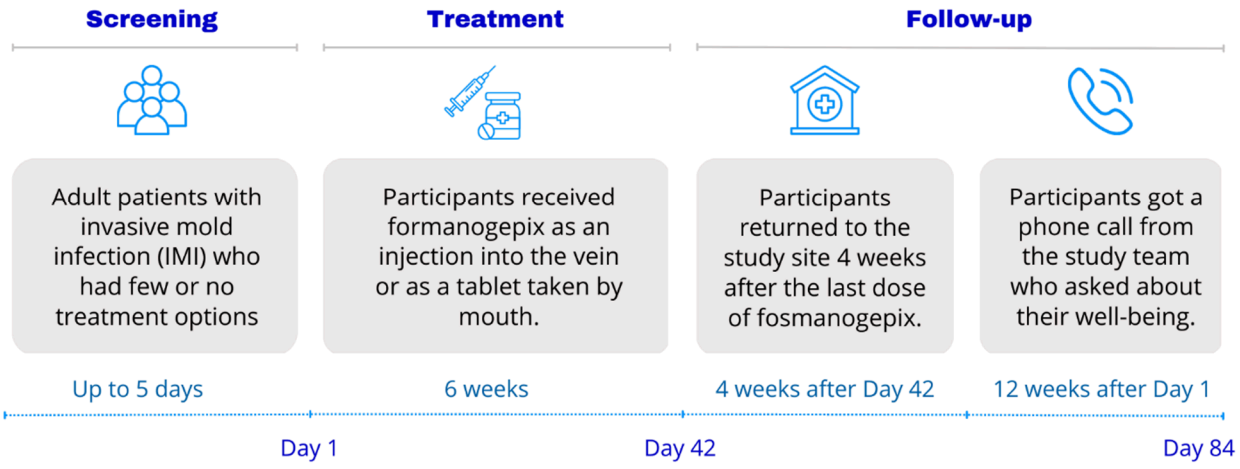
How was the study done?

Fosmanogepix was given as a treatment to a group of patients with IMI to find out if it could help prevent death.

The participants and study researchers knew the study medication given during the study. This is known as an "open-label" study.

The figure below shows what happened in this study.

What happened in this study?



The Sponsor ended the study earlier than planned to focus on a larger study of fosmanogepix. It was not because of safety concerns with fosmanogepix.

Where did this study take place?

The Sponsor ran this study at 8 locations in 4 countries (Belgium, Israel, Germany, and the USA).

When did this study take place?

It began on 04 January 2020 and ended on 09 May 2022.

Who participated in this study?

Participants in the study were adult patients with IMI who had few or no treatment options because of different reasons, such as:

- Their infecting mold was resistant to standard antifungal drugs.
- They could not tolerate standard antifungal drugs.
- Their doctor's medical opinion was that they could not receive standard antifungal drugs.
- Their IMI did not improve with standard antifungal drugs.

A total of 21 participants got at least 1 dose of fosmanogepix.

- There were 19 men and 2 women in this study.
- Participants were between the ages of 40 and 82 years.
- All but 1 participant had cancer before joining this study.
- IMI was found in the lungs (17 participants), sinuses (1 participant), and other parts of the body (3 participants).

An independent group of infectious disease doctors was asked to confirm the type of IMI at the start of the study. They found that:

- 20 participants had likely IMI (also known as probable IMI) or proven IMI.
- 1 participant did not likely have IMI.

Participants received fosmanogepix for up to 6 weeks. Of the 21 participants:

- 11 finished the treatment period.
- 10 did not finish the treatment period because of 1 or more reasons listed below:
    - They had a medical problem during this period.
    - They died.
    - They left before the study was over by their choice.

How long did the study last?

Each study participant was in the study for about 12 weeks. In total, the study took about 2 years and 4 months to complete.

On 21 April 2022, the Sponsor ended the study earlier than planned to focus on a larger study of fosmanogepix. It was not because of safety concerns with fosmanogepix.

Participants who had already signed up finished the study, even though the study was stopped early.

After the study ended in May 2022, 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?

Does fosmanogepix help to prevent death from any cause within 42 days after starting treatment?

To answer this question, researchers checked the records of all 20 participants with probable or proven IMI.

Researchers counted how many participants died from any cause between the start (Day 1) and end (Day 42) of fosmanogepix treatment.

Researchers found that:

- 5 out of 20 participants (25%) died from any cause within 42 days after starting fosmanogepix.
- Participants in this study had a lower rate of death from any cause within 42 days after starting fosmanogepix compared to the expected rate (45%) of death based on other studies with other antifungal drugs.

Based on these results, researchers consider that fosmanogepix could be a treatment option for IMI. More studies are needed to confirm this.

This is a summary of just some of the main results of this study. Other studies may have different results.

What medical problems did participants have during the study?

The researchers recorded any medical problems the participants had during the study. Participants 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 also have been caused by a study treatment or by another medicine the participant was taking. Sometimes the cause of a medical problem is unknown. By comparing medical problems across many treatment groups in many studies, researchers try to understand what effects a study medication might have on a participant.

All 21 participants (100%) had at least 1 medical problem during the study.
No participant left the study because of medical problems.

The table below shows the most common medical problems that happened in 5 or more participants in this study.

Below are instructions on how to read Table 1.

Instructions for Understanding Table 1.

- The **1st** column lists the most common medical problems during the study. It lists all medical problems seen in 5 or more participants.
- The **2nd** column tells how many of the 21 participants taking fosmanogepix had each medical problem. Next to this number is the percentage of the 21 participants who had this medical problem.

  For example, 13 out of 21 participants (62%) taking fosmanogepix had a queasy feeling (nausea).

Table 1. What were the most common medical problems in the study?	
Medical Problem	**Fosmanogepix (21 Participants)**
Queasy feeling (nausea)	13 out of 21 participants (62%)
Loose, watery bowel movements (diarrhea)	10 out of 21 participants (48%)
Throwing up (vomiting)	9 out of 21 participants (43%)
Not feeling hungry or not having the desire to eat (lack of appetite)	7 out of 21 participants (33%)
Swelling in the legs or arms (edema)	6 out of 21 participants (29%)
Fever	5 out of 21 participants (24%)

Did study participants have any serious medical problems?

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

A total of 13 out of 21 participants (62%) had at least 1 serious medical problem during the study.

The table below shows the most common serious medical problems that happened in 2 or more participants in this study.

The instructions on how to read Table 2 are the same as for Table 1.

Table 2. What were the most common serious medical problems in the study?	
Medical Problem	**Fosmanogepix (21 Participants)**
Fever with a low white blood cell count	3 out of 21 participants (14%)
Blood infection	2 out of 21 participants (10%)
Heart suddenly stopped beating	2 out of 21 participants (10%)
Loose, watery bowel movements (diarrhea)	2 out of 21 participants (10%)
Lungs failed and were unable to maintain gas exchange	2 out of 21 participants (10%)

An independent group of experts called the Data and Safety Monitoring Board (DSMB) kept track of the participants' safety throughout the study. They reviewed all the safety information collected, including deaths.

Overall, 9 participants died between Day 1 and Day 84 of the study. The DSMB did not think fosmanogepix caused any of the deaths.

Where can I learn more about this study?

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

For more details on your study protocol, please visit:

www.pfizer.com/research/research_clinical_trials/trial_results — Use the protocol number **C4791010**

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

www.clinicaltrials.gov — Use the study identifier **NCT04240886**

www.clinicaltrialsregister.eu — Use the study identifier **2019-001386-33**

Please remember that researchers look at the results of many studies to find out which medicines can work and are safe for patients.

Again, if you participated in this study,
thank you for volunteering.
We do research to try to find the
best ways to help patients, and you helped
us to do that!