Plain Language Summary of Study Results

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

What was the study called?

A study of dosing schedules of ASP1235 for people with acute myeloid leukemia (AML)

Why was the study needed?

Acute myeloid leukemia is a type of cancer when bone marrow makes too many white blood cells which are not normal. The main treatment offered for people with acute myeloid leukemia is chemotherapy. However, in some people the cancer can come back after it has disappeared with earlier therapy. This is called relapsed cancer. And in some people, cancer does not go away at all with treatment. This is called refractory cancer.

Researchers in this study were interested in finding better ways to treat acute myeloid leukemia. The study medicine, ASP1235, was studied for treating people with acute myeloid leukemia.

This was a phase 1 study. The aim of a phase 1 study is to learn the best dose for a study medicine. This is usually the dose that people can receive without getting medical problems from the medicine. Phase 1 studies may include healthy people but can include people with certain health conditions.

The study started in November 2016. The sponsor (Astellas) stopped the study in September 2020. Astellas reviewed all the study information and created a report of the results. This is a summary of that report.

What were the main questions this study helped answer?

- Did the people in the study have any medical problems from ASP1235?
- How much of ASP1235 could people with leukemia receive without having medical problems from ASP1235?

What other questions did this study helped answer?

- How did ASP1235 act in the body and what was the best dosing schedule for future studies?
- How did ASP1235 effect the people's leukemia in this study?

What kind of study was this and who took part in it?

This was an open-label study. That means that each person in the study and the study doctors knew that person received ASP1235.

The study included men and women with confirmed acute myeloid leukemia. To be in the study, people had the following:

- They had failed at least 1 treatment.
- Their cancer had come back after it had disappeared with earlier therapy. Or their cancer did not go away with a prior treatment.
- These people were not able to try other treatments.
- They were active or they could perform light daily activities. Or they were able to walk and were capable of self-care, but unable to carry out any work activities. And they were up and about more than half of the time that they were awake.

The table below shows some information about the people who received the study medicine. 54 people were in the study. Out of these, 43 people received at least 1 dose of ASP1235.

	Number of People
Age Group	
Less than 65 years	20
65 years to less than 75 years	12
75 years or more	11
Sex	
Men	26
Women	17

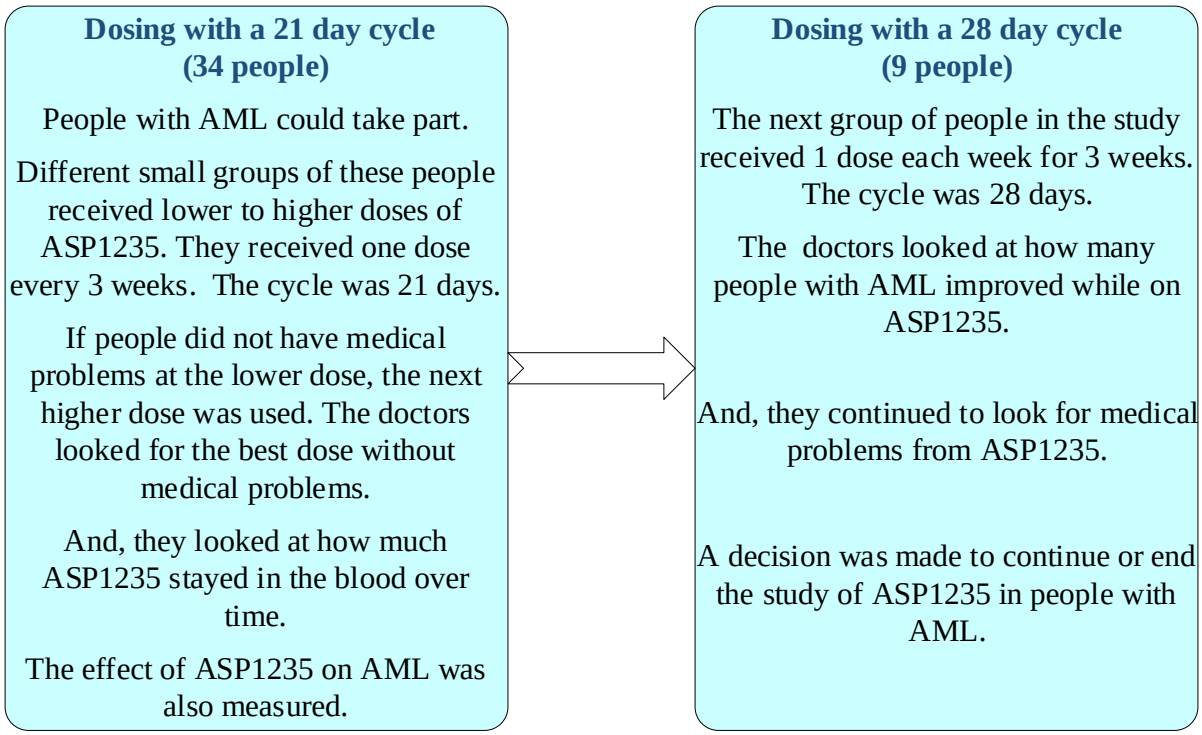
Where did the study take place?

This study took place at 6 clinics in the US and Canada.

What happened during the study?

The study doctors did a check-up of the people at several study visits. At the first visit, people were checked to see if they could be in the study. People chosen to be in the study received different doses of ASP1235. And the timing of their doses was different. People gave blood samples often during the study. Tests on these samples showed how much ASP1235 stayed in their blood over time. Other tests measured the effect of ASP1235 on AML.

ASP1235 was given through a vein in the arm. The dosing schedule is also called a treatment cycle. It means how often a dose is given and the length of time between doses. The following diagram shows what happened during the study.



What medical problems did these people have during the study?

What adverse reactions did people have in this study?

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 people have while they are in the study. These 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.

33 people (76.7% or 33 out of 43 people) had adverse reactions in this study.

The table below shows the most common adverse reactions experienced by people who took at least 1 dose of study medicine in this study.

Adverse Reaction	ASP1235 (out of 43 people)
Blurred vision	10 (23.3%)
Decreased number of a type of blood cell that helps to clot blood (platelet)	5 (11.6%)
Dry eye	5 (11.6%)
Inflammation (swelling and redness) of the cornea (the transparent front cover of the eye)	5 (11.6%)

Did any of the people in this study have serious adverse reactions?

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

12 people (27.9%, or 12 out of 43 people) had serious adverse reactions in this study.

28 people passed away during the study. None of the deaths were thought to be caused by ASP1235.

What were the study results?

The other question that this study helped answer:

How much of ASP1235 could people in this study receive without having medical problems from ASP1235?

ASP1235 had unacceptable medical problems with the eye at higher dose levels. At lower dose levels there were still medical problems with the eye. They were not as bad as at the higher dose levels.

What were the other study results?

The other questions that this study helped answer:

How did ASP1235 act in the body and what was the best dosing schedule for ASP1235?

The level of ASP1235 went up in the blood stream as the dose was increased. However, test results varied greatly among people in the study. This variation was moderate to high. Also, there were no clear trends between the levels of ASP1235 in the blood and how well their leukemia responded to it.

The number of people in the study and the information from their tests were not enough to answer this question.

How did ASP1235 affect the people's leukemia in this study?

Not enough people saw their leukemia slow or end after receiving ASP1235. 3 people had remission of their leukemia. Researchers could not identify specific characteristics to explain why ASP1235 worked in these 3 people. The study sponsor (Astellas) decided to end the study earlier than planned.

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 at https://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 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