

## Clinical Results Summary

### A clinical study to compare the plasma levels of quizartinib when taken as tablets or liquid by healthy participants

Protocol number: AC220-014

Thank You!



Daiichi Sankyo, Inc., the sponsor of this study, would like to thank the participants who took part in this study for quizartinib. Each participant helped to advance medical research and knowledge for people affected with a type of blood cancer called acute myeloid leukemia. Their contribution to medicine and healthcare is greatly appreciated.

**Important note:** This summary only shows the results of a single study. Other studies may have different findings. Researchers and health authorities look at the results of many studies to understand which treatments work and how they work. It takes a lot of people in many studies around the world to advance medical science and healthcare.

Do not use the results of this study to make health decisions. Please talk to a doctor before changing any treatment you are taking or if you have any questions about these study results.

## What was the main purpose of this study?

Quizartinib, also known as AC220, is an investigational drug that is being tested to treat people with a type of blood cancer called Acute Myeloid Leukemia, or AML.

AML is a cancer of the blood and the bone marrow. The bone marrow is found in the center of the bones, where new healthy blood cells are made. AML starts in the bone marrow and prevents it from making normal blood cells. The abnormal (cancer) cells build up in the bone marrow, so there are fewer healthy blood cells. These cancerous cells can also enter the blood stream and circulate in the blood, and go to different parts of the body.

Quizartinib was first developed in liquid form to be taken by mouth. Researchers have developed tablets for quizartinib. In this study, researchers wanted to compare the levels of quizartinib in the participants' plasma when taken as tablet(s) or liquid.

*\* Plasma is the fluid part of the blood. It contains different components of the blood that are necessary for life and health such as hormones, proteins, etc.*

### Treatment given in this study

#### Quizartinib

An investigational drug being tested for the treatment of AML

Two forms of quizartinib were studied:



Tablet



Liquid

## Main purpose of this study

The main question the researchers wanted to answer in this study was:



What are the levels of quizartinib and AC886\* in the plasma of participants when quizartinib is taken in tablet form or as a liquid?

*\*The body breaks down quizartinib into another product called AC886. AC886 has similar effects in the body to quizartinib.*

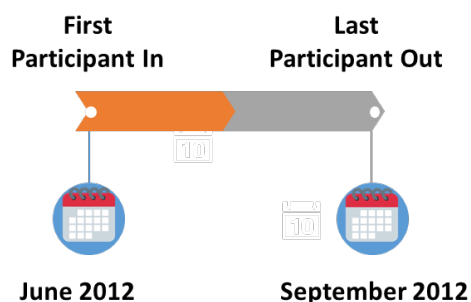
## Other purposes of this study

Other questions researchers wanted to answer in this study were:

- For the tablet form, do the plasma levels of quizartinib and AC886 increase with an increase in dose?
- What side effects could the participants develop during the study?

There were some additional questions that researchers wanted to answer but these are not discussed in this summary.

## How long was this study?



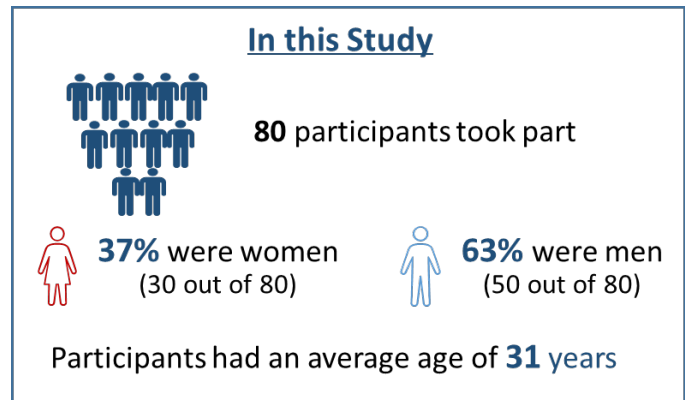
The study started in June 2012 and ended in September 2012. The study was completed as planned and a study report was created. This summary is based on that report.

## Who was in this study?

This study included 80 participants from the United States.

People could take part in the study if they:

- were healthy individuals between 18 to 55 years of age,
- were using effective birth control methods during the study or were unable to have children,
- did not have major surgery within 90 days of receiving study drug,
- agreeing to consume potassium rich foods such as bananas, oranges, cantaloupes, and yogurt for at least 24 hours before starting the study treatment (quizartinib tablet or liquid),
- had a normal weight, and healthy kidneys and liver as confirmed by blood tests.



## What happened during this study?

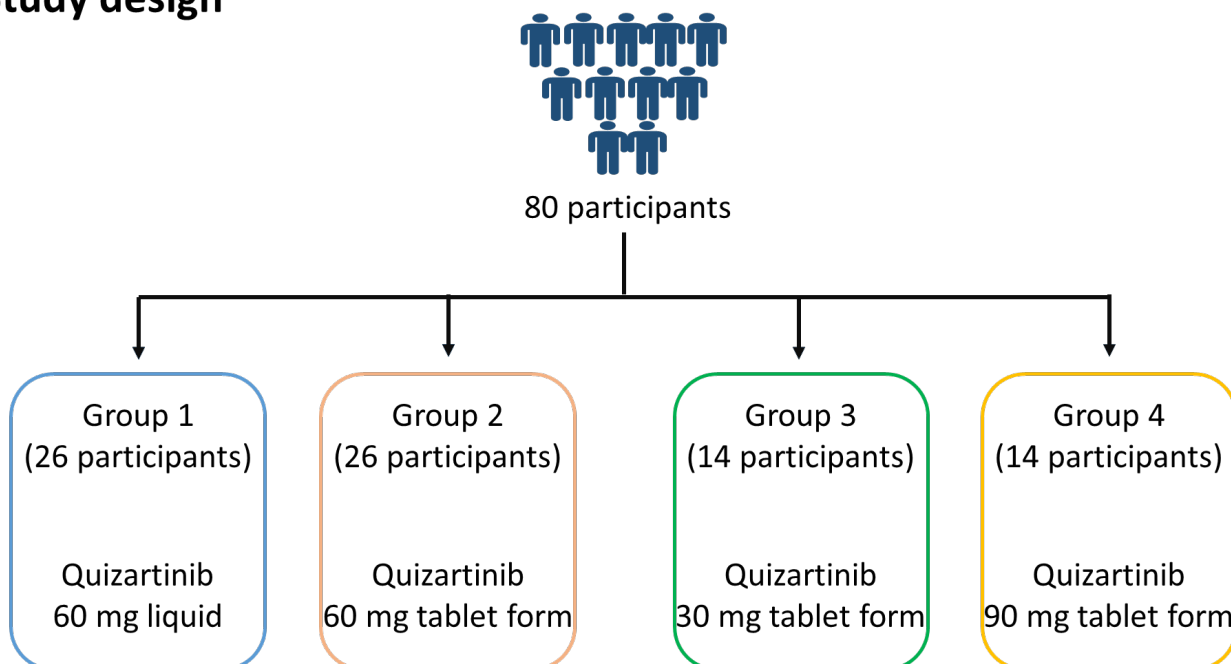
This was a Phase 1 study. Phase 1 studies are done to find out the safety of a new study treatment and how it works in a small number of participants. This helps researchers understand what happens to the study treatment in the body, and if there are any side effects.

This study was “open label”. This means that both the researchers and the participants knew which treatment was given to which participant.

Participants were screened to find out if they could take part in the study. On the day before receiving the study drug, participants were admitted to the study site for an 8-day stay. The researchers randomly assigned participants to 4 treatment groups using a computer system. This process is called randomization. It means that each participant could be assigned to any group and it helps to make sure the groups are distributed fairly.

All participants received a single dose (a tablet or liquid) of quizartinib on an empty stomach and continued fasting for 4 hours after treatment.

## Study design



The participants were discharged from the study site on the morning of Day 8. Researchers collected blood and urine samples from the participants at defined time points. Researchers monitored the participants' health up until Day 21.

## What were the key results of this study?

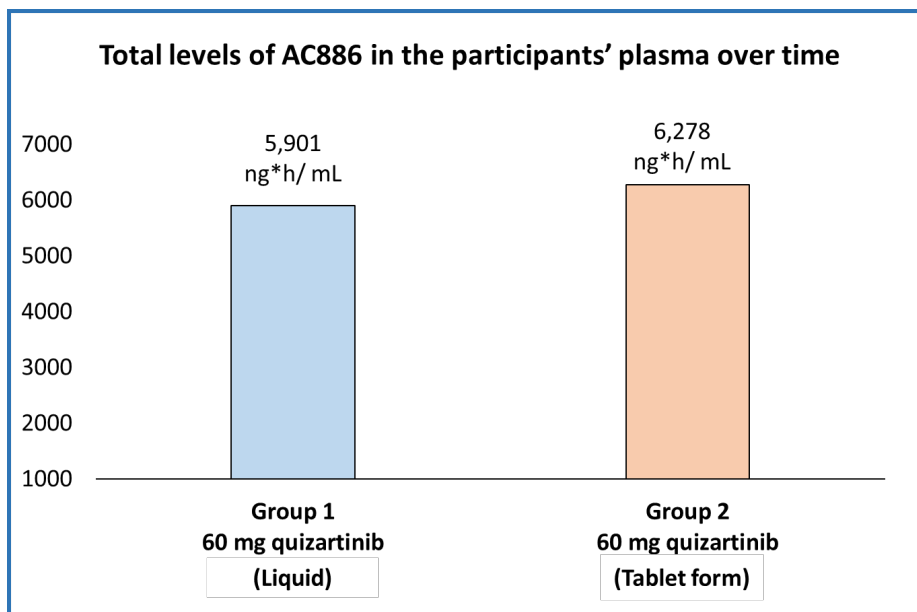
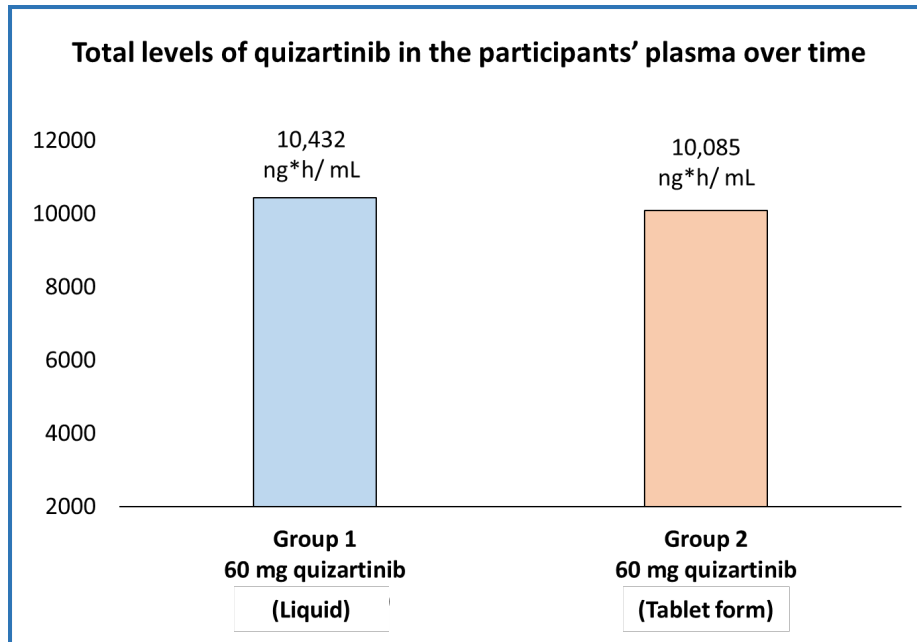
Key results from this study are shown for the total group of participants as average results. This summary does not show the results from each individual participant. An individual participant's results could be different from the total group of participants.

## What were the levels of quizartinib and AC886 in the plasma of participants when quizartinib was taken in tablet form or as a liquid?

The total plasma levels of quizartinib and AC886 measured over time were similar when quizartinib was given as in tablet form compared to when it was given as a liquid.

## How was this measured?

To answer this question, researchers compared the plasma levels of quizartinib and AC886 between Group 1 (60 mg quizartinib liquid) and Group 2 (60 mg quizartinib, tablet form). Researchers measured the total levels of quizartinib and AC886 over time from plasma samples of the participants. The measurement unit is nanograms hours per milliliter (ng\*h/mL). This shows how much of quizartinib and AC886 in nanogram (one thousand-millionth of a gram) was found in each milliliter of plasma over time.



## What were the other results of this study?

### For the tablet form, did the plasma levels of quizartinib and AC886 increase with an increase in dose?

To answer this question, researchers compared the levels of quizartinib and AC886 in the plasma of participants who took 30 mg (Group 3), 60 mg (Group 2), or 90 mg (Group 4) as a tablet(s).

The plasma levels of quizartinib and AC886 increased in proportion to the increase in dose of quizartinib for each group.

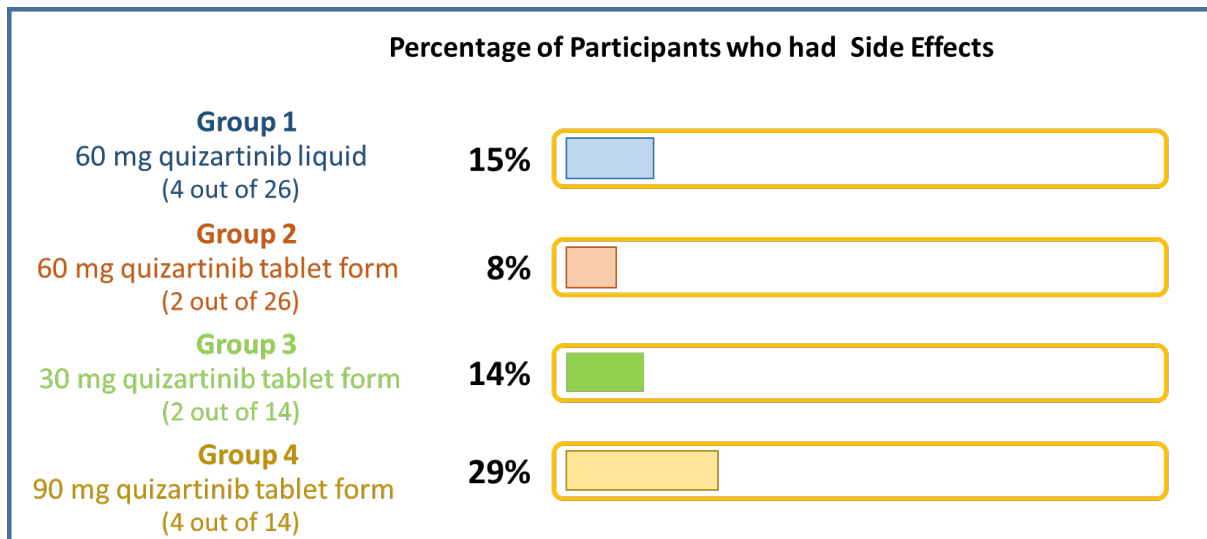
## What medical problems did the study participants have?

Side effects are medical problems (such as feeling tired) that happened during the study, which the study doctor (investigator) thought could be related to the treatments in the study. This section provides a summary of side effects related to quizartinib.

Side effects are considered serious if they cause death, are life-threatening, cause disability, cause lasting problems, cause birth defects, require hospitalization, or are otherwise considered medically important by the doctor. Some participants stop study treatment because of side effects.




Side effects other than those related to quizartinib are not reported here.

### How many participants had side effects?



## What side effects did the participants develop during the study?

The side effects experienced by 2 or more participants are reported below:

Side Effects	Group 1 (26 participants) 60 mg quizartinib Liquid	Group 2 (26 participants) 60 mg quizartinib Tablet form	Group 3 (14 participants) 30 mg quizartinib Tablet form	Group 4 (14 participants) 90 mg quizartinib Tablet form
Stomach discomfort	0	0	0	 14% (2)
Headache	 4% (1)	0	 7% (1)	0

## How many participants had serious side effects?

There were no deaths or serious side effects reported during the study that were related to quizartinib.

## How many participants had to stop treatment because of side effects?

None of the participants in the study stopped treatment early because of side effects.

## How was this study useful for patients and researchers?

This study helped researchers to understand the effects of different formulations of quizartinib, in liquid and tablet form. Researchers studied the plasma levels of quizartinib and its breakdown product AC886 in healthy participants. This study also demonstrated that as the dose of quizartinib increases, the levels of quizartinib and AC886 in the plasma also increased in a proportional way.

Findings from this study may be used in other studies. Other studies for quizartinib are ongoing.

Please remember, this summary only shows the results of a single study. Other studies may have different findings. Please talk to a doctor before changing any treatment you are taking or if you have any questions about these study results.





## Where can I learn more about this study?

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

**Full study title:** A Phase 1, Open-Label, Randomized, Single Dose, Parallel Group, Relative Bioavailability Study of Two AC220 Formulations in Healthy Volunteers

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