



Daiichi-Sankyo

## Clinical Results Summary

# A clinical study to learn about the effects and safety of PLX3397 in men with advanced castration-resistant prostate cancer (CRPC) that had spread to the bone

Protocol number: PLX108-06

### Thank You!



Daiichi Sankyo, Inc., the sponsor of this study, would like to thank the participants who took part in this study for PLX3397. Each participant helped to advance medical research for men affected with advanced castration-resistant prostate cancer (CRPC) that had spread to the bone. CRPC is a type of prostate cancer that keeps growing despite treatment given to reduce testosterone levels. 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?

### **Advanced castration-resistant prostate cancer (CRPC) that has spread to the bone**

Prostate cancer affects the prostate gland (a small organ in a man's pelvis). The symptoms of prostate cancer include pain, tiredness, and problems emptying the bladder and bowels. Prostate cancer that does not respond to treatment that targets the male sex hormone testosterone is known as castration-resistant prostate cancer (CRPC). CRPC is considered advanced if it spreads to other parts of the body beyond the prostate.

CRPC that spreads to the bones results in a high count of circulating tumor cells (CTCs). CTCs are individual cancer cells that have spread beyond the prostate gland and are found floating in the blood. For prostate cancer patients, CTCs are used as a measure to determine how much the cancer has spread.

PLX3397 is a drug being developed for CRPC that has spread to the bones. It acts by blocking the effect of testosterone on the prostate. In this study, researchers wanted to learn if PLX3397 could be used to reduce the size of the tumor in participants who have advanced CRPC that has spread to the bones by reducing the number of CTCs.

### **Treatment given in this study**



#### **Investigational Treatment**

##### **PLX3397**

An investigational drug being tested for the treatment of patients with CRPC whose tumor cells have spread to the bone and released CTCs in the blood.

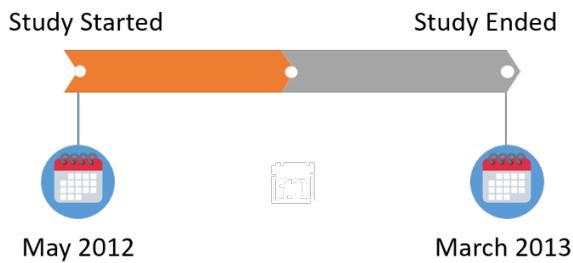
### **Main goal of this study**

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



How many participants with prostate cancer had a decrease in the count of tumor cells in the bloodstream after treatment with PLX3397?

## How long was this study?



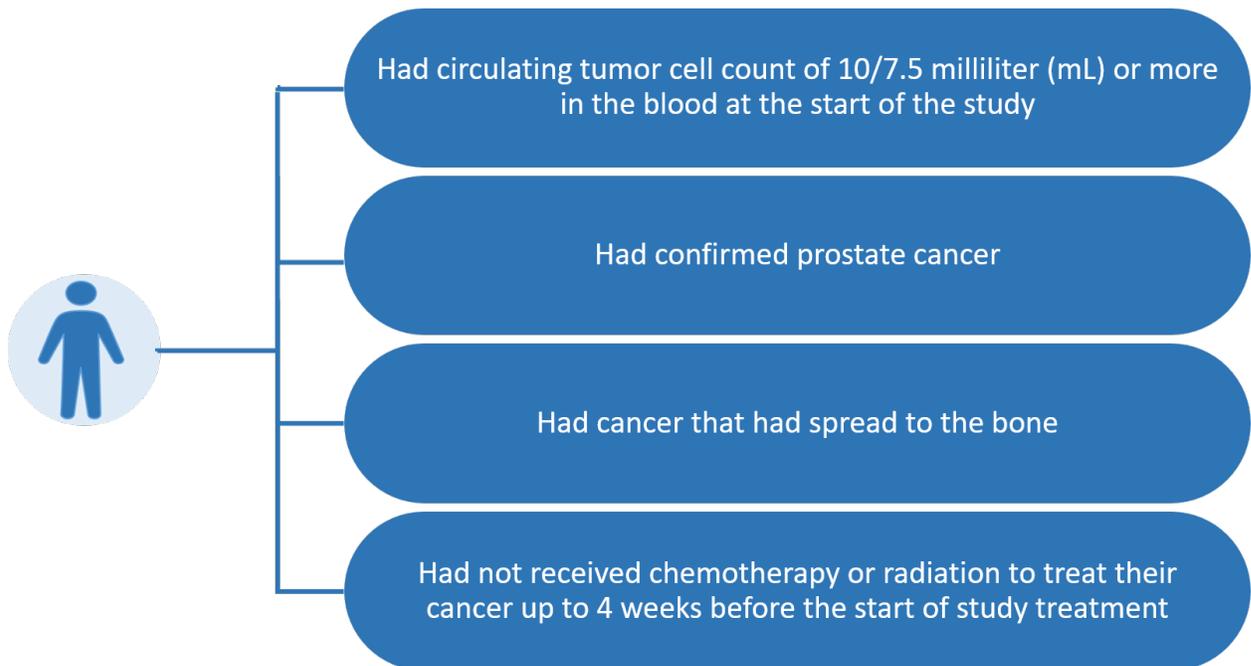
An individual participant could have been in this study for about 9 months.

This study started in May 2012 but was stopped early, in March 2013, because not enough participants were enrolled. When the study was stopped, a report was created with the information that was available. This summary is based on that report.

## Who was in this study?

This study included 6 male participants from 2 study sites in the United States. The average age of the participants was 71 years.

Participants could take part in this study if they:



## What happened during this study?

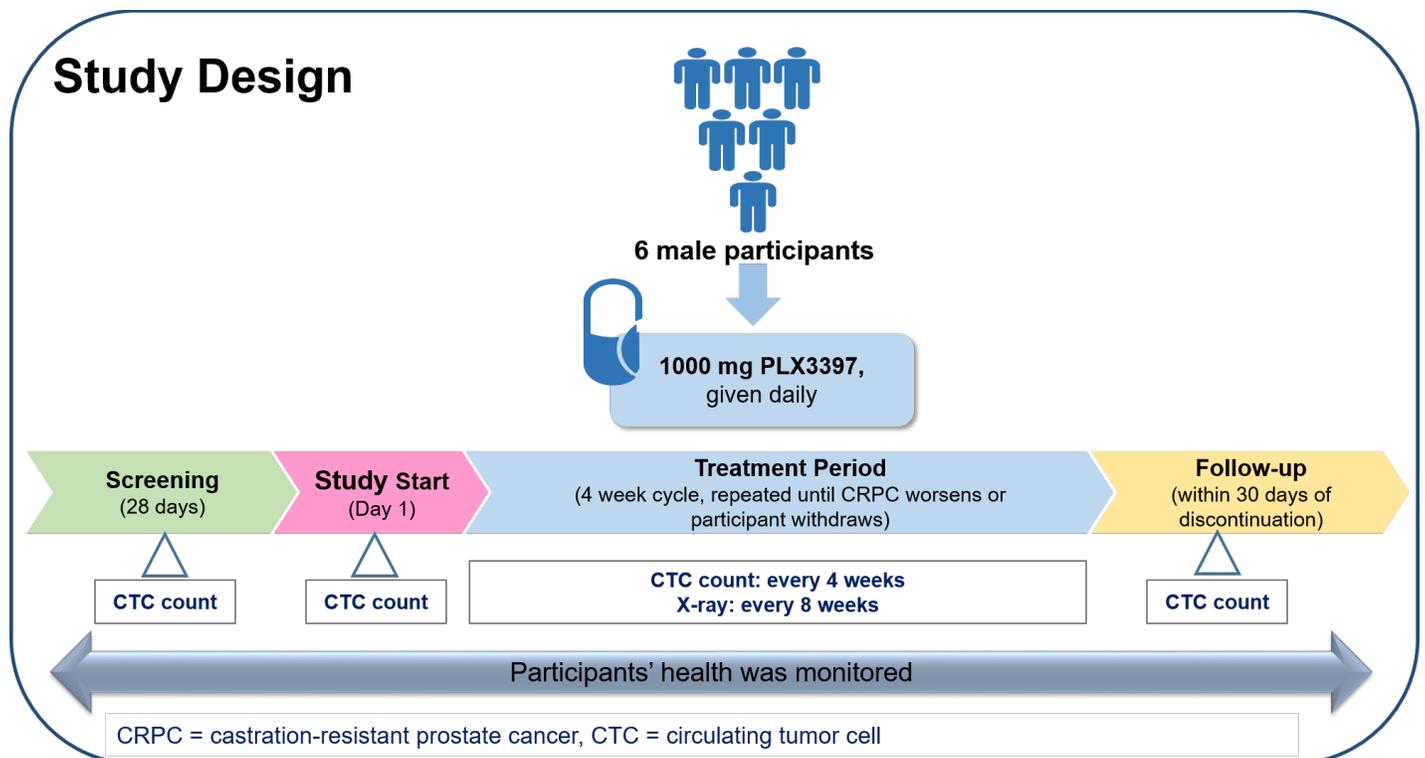
This was a Phase 2 study. In Phase 2 studies, the study treatment is given to participants with the disease condition to gather information about whether or not the study treatment treats patients as expected.

In this study, researchers wanted to learn if PLX3397 was safe and if it was effective in helping participants with CRPC whose tumor had spread to the bone.

Participants who consented to take part in this study were screened to find out if they were eligible to take part in this study. Eligible participants received 1000 milligrams of the study treatment on Day 1, which was taken by mouth. Participants continued to receive the study treatment daily, for 4 weeks. This cycle of treatment could be continued as long as participants did not show worsening of CRPC, did not have serious side effects, or asked to be removed from the study.

Researchers collected the participants' blood for CTC count at screening, at the start of the study, every 4 weeks after the first dose of treatment, and at safety follow-up. CTCs were counted by using a CTC detection system.

The researchers used X-rays to measure the size of the tumor every 8 weeks. They also monitored the health of the participants throughout the study.



## What were the key results of this study?

This study did not meet its planned enrollment goal of 20 participants by the end of 2012. Therefore, the study was stopped after 6 participants had joined. No results about how PLX3397 affects CTC count or tumor size were reported.

## 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 thought could be related to the treatments in the study. This section provides a summary of side effects related to the study treatment. The website listed at the end of this summary has more information about the medical problems that happened in this study.

Side effects are considered serious if they cause death, are life-threatening, cause lasting problems, or require hospitalization. Some participants stop study treatment because of side effects.

### How many participants had serious side effects?

In this study, serious side effects were monitored for 6 participants who took the study treatment.

None of the participants reported serious side effects related to the study treatment. No deaths were reported during the study.

### How many participants had non-serious side effects during the study?



All non-serious side effects reported by participants taking the study treatment are shown below.

Side Effects	Percentage (Number of Participants)
Feeling sick to your stomach	 67% (4 out of 6)
Feeling tired	 50% (3 out of 6)
Loss of desire to eat	 50% (3 out of 6)
Constipation	 33% (2 out of 6)
Diarrhoea	 33% (2 out of 6)
Blood glucose increased	 17% (1 out of 6)
Blood sodium increased	 17% (1 out of 6)
Difficulty breathing	 17% (1 out of 6)
Increase in liver test value of alanine aminotransferase in the blood	 17% (1 out of 6)
Increase in liver test value of aspartate aminotransferase in the blood	 17% (1 out of 6)
Increase in pancreatic test value of lipase in the blood	 17% (1 out of 6)
Increase in protein called creatine phosphokinase in the blood	 17% (1 out of 6)

Side Effects	Percentage (Number of Participants)
Inflammation of the inner lining of the body and its organs	 17% (1 out of 6)
Low blood platelet count	 17% (1 out of 6)
Low number of red blood cells	 17% (1 out of 6)
Pain in hands and feet	 17% (1 out of 6)

*Note: platelets are tiny blood cells that help the body form blood clots to stop bleeding.*

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

One participant stopped the study treatment due to an increase in the protein called creatine phosphokinase in the blood.

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

This study was planned to help researchers learn if PLX3397 was safe and if it was effective in helping participants with CRPC whose tumor had spread to the bone. However, since this study was stopped early, only information about the safety of PLX3397 was collected.

Findings from this study may be used in other studies with PLX3397. Other studies of PLX3397 are ongoing.

Please remember, this summary only shows the results of a single study. Findings from other studies may be different. 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?

You can find more information about this study on the following website:

 [www.clinicaltrials.gov](http://www.clinicaltrials.gov) Use the NCT identifier NCT01499043 in the search field.

Please remember that the results on this website may be presented in a different way. 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 Pilot Study of PLX3397 in Advanced Castration-Resistant Prostate Cancer (CRPC) Patients with Bone Metastasis and High Circulating Tumor Cell (CTC) Counts

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**Date of this summary:** 10 August 2020

This summary was prepared by Kinapse Ltd, a Syneos Health® company.