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 Phase 1, Open-Label, Multicenter Study to Assess the Safety, Pharmacokinetics, and Effectiveness of AGS-16C3F Monotherapy in Subjects with Renal Cell Carcinoma of Clear Cell or Papillary Histology

Why was this Study Needed?

Renal cell carcinoma is a type of kidney cancer. There are different types of renal cell carcinoma. The types of this cancer are named by how the tumor cells look under a microscope (histology). For people with clear cell renal cell carcinoma, their tumor cells look clear under a microscope. For people with papillary renal cell carcinoma, their tumor cells look like long thin tubes under a microscope.

Treatment for people with these types of renal cell carcinoma are medicines which slow the growth of their cancer. Most medicines are for people with clear cell renal cell carcinoma. There are not many medicines available for people with other types of renal cell carcinoma such as papillary renal cell carcinoma.

AGS-16C3F is new medicine being studied by researchers that could help people with clear cell or papillary renal cell carcinoma but more information is needed about this medicine.

This was a phase 1 study. The aim 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 usually include healthy people but can include people with certain health conditions.

The study started in July 2012 and ended in September 2016. The sponsor of this study (Agensys, now part of 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 the study helped answer?

- How much AGS-16C3F stays in the blood of these people over time?
- Did these people have any medical problems from AGS-16C3F?

What Kind of Study was this and Who Took Part in It?

This was an "open-label" study. That means that each person and the study doctors knew that person had received AGS-16C3F.

People with clear cell or papillary renal cell carcinoma who were 18 years or older could take part.

	Number of People
Age Group	
Aged less than 65 years	20
Aged 65 years or older	14
(2 people were aged 75 years or older)	
Sex	
Men	27
Women	7

What Happened during the Study?

This study had 2 parts.

1st PART (14 people)

People with clear cell RCC or papillary RCC could take part.

Different small groups of these people received higher to lower doses of AGS-16C3F.

This was due to medical problems from the higher doses.

The highest dose without medical problems was 3.6 mg/kg AGS-16C3F.

➡

2nd PART (20 people)

The next small group of people in the study received the following doses of AGS-16C3F:

The first 7 people taking part received 2.7 mg/kg AGS-16C3F.

The next 13 people taking part received 1.8 mg/kg AGS-16C3F.

This was due to medical problems from the higher dose.

RCC = renal cell carcinoma

Doses of AGS-16C3F are given in milligrams (mg) for each kilogram (kg) of the person's body weight. This is also known as mg/kg AGS-16C3F.

In the 1st part of the study, the different groups received AGS-16C3F in the following order:

- 4.8 mg/kg ➡ 3.6 mg/kg ➡ 2.7 mg/kg ➡ 1.8 mg/kg ➡ 1.2 mg/kg ➡ 0.6 mg/kg.

In the 2nd part of the study, the different groups received AGS-16C3F in the following order:

- 2.7 mg/kg ➡ 1.8 mg/kg.

In the 1st part of the study, the highest dose without medical problems was 3.6 mg/kg AGS-16C3F. However it was decided that in the 2nd part of the study, the next group of people in the study would start on the next lowest dose of 2.7 mg/kg AGS-16C3F.

In both parts of the study, people in the study received AGS-16C3F in the same way. They received AGS-16C3F slowly through a tube inserted into a vein. This is called an infusion.

People received AGS-16C3F once every 3 weeks. People continued with 3-week cycles at the same dose of AGS-16C3F unless they had medical problems from AGS-16C3F, their cancer got worse or the study doctor decided that person should stop treatment. The first time people received AGS-16C3F is called the 1st cycle. The second time they received AGS-16C3F (3-weeks later) is called the 2nd cycle and so on.

During both parts of the study, a blood sample was taken from people just before they received each AGS-16C3F infusion. Also during the 1st cycle and 4th cycle, a few blood samples were taken over time after they received AGS-16C3F.

Tests from blood samples taken after people received AGS-16C3F (1st and 4th cycles) showed how much AGS-16C3F stayed in the blood of these people over time.

Where Did The Study Take Place?

This study took place at 8 clinics in the USA and Canada. 34 people were in the study and received at least 1 dose of AGS-16C3F.

What Were the Study Results?

How much AGS-16C3F stays in the blood of these people over time?

During both parts of the study, a blood sample was taken from people just before they received each AGS-16C3F infusion. Also during the 1st cycle and 4th cycle, a few blood samples were taken over time after they received AGS-16C3F.

Blood tests showed how much AGS-16C3F stays in the blood of these people over time.

The blood tests showed the following:

- The amount of AGS-16C3F in the blood of people over time was as expected at the different doses.
- The amount of AGS-16C3F in the blood halved in 7.6 days.
- For people on the same repeated dose, the amount of AGS-16C3F staying in their blood did not get higher after each dose.
- The best dose to use in other studies is 1.8 mg/kg AGS-16C3F every 3 weeks.

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.

All 34 people (100% or 34 out of 34 people) had adverse reactions in this study. Of these, 8 people (23.5%, or 8 out of 34 people) had an adverse reaction due to the infusion.

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	**AGS-16C3F (out of 34 people)**
Fatigue or tiredness	20 (58.8%)
Dry eye	17 (50.0%)
Blurred vision	15 (44.1%)
Nausea or the urge to vomit	13 (38.2%)
Condition in which there is a low blood platelet count. Platelets help blood to clot.	12 (35.3%)
Decreased appetite	11 (32.4%)
Inflammation (swelling and redness) of the cornea (the transparent front cover of the eye).	11 (32.4%)
Vomiting	10 (29.4%)
Reaction that can occur during or following infusion of the study medicine. The reaction may include fever, chills, rash, low blood pressure, and difficulty breathing.	8 (23.5%)
Headache or head pain	7 (20.6%)

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

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

6 people (17.6%, or 6 out of 34 people) had a serious adverse reaction to AGS-16C3F. None of the serious adverse reactions occurred in more than one person.

2 people passed away during the study. No one passed away during the study because of AGS-16C3F.

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 online at http://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.

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