Summary of Results for Laypersons

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

What was the Study Called?

An exploratory, open label, single-arm study to evaluate the effect of Eligard® 6-month on biomarkers of disease in patients with metastatic prostate cancer. This study is also known as the EFFECT study.

Why was this Study Needed?

Prostate cancer growth is dependent on male hormones or "androgens." An example of an androgen is testosterone. Eligard® (also known as leuprorelin acetate) is a prescription medicine for the treatment of prostate cancer. Eligard keeps the testicles from making testosterone by blocking other hormones that are needed to make it. This stops or slows down the growth of prostate cancer. Prostate cancer cells produce molecules that can be found in the blood or the urine ("biomarkers"). At the time of this study, there was not yet enough information about prostate cancer biomarkers. It was not known if you could tell from their levels that anticancer treatment had an effect on the prostate cancer. Therefore, there was a need to study the effect of Eligard on biomarkers of prostate cancer.

This study was conducted in patients with prostate cancer. They received Eligard. This study looked at biomarkers that are specific for prostate cancer. Those biomarkers were testosterone, prostate-specific antigen (or PSA for short) and PCA3. The study also looked at genetic blueprints ("mRNA") for production of proteins specific for prostate cancer (PSA mRNA, PCA3 mRNA and TMPRSS2-ERG mRNA). The study was to compare the levels of those biomarkers before the Eligard injection on day 1 and 6 months thereafter.

The study started in August 2014. The sponsor (Astellas) stopped the study in August 2015. The reason was that not enough patients joined the study. When the study was stopped, 1 patient had received study medicine. Astellas reviewed all the study information and created a report of the results. This is a summary of that report.

What Kind of Study was This and Who Took Part in it?

This was an "open-label" study. This means that each patient and the study doctor knew which study medicine that patient received (Eligard).

The study included men aged 18 years or older. They had prostate cancer that had spread from the prostate to another part of the body. They had not received anticancer or hormonal treatment for the last 6 months before the start of the study. At the start of the study, their serum level of PSA was at least 5 ng/mL (5 ng of PSA in 1 mL of serum). (PSA is a protein produced by the prostate cells. An increased serum level of PSA means that the prostate cancer got worse. Serum is the fluid part of blood.) PSA mRNA could be detected in certain white blood cells in their blood. Patients were active or they could perform light daily activities. Or they were able to walk and capable of all 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. They were expected to live at least 12 months.

During the study, the study doctor did a check-up of the patients at several study visits. At the first visit, patients were checked if they could be in the study. Patients who could be in the study received a single injection of the 6-month form of Eligard beneath the skin. (The 6-month form releases medicine for 6 months.)

This study took place at 3 clinics in the Netherlands. Two patients were in the study. Out of these patients, 1 patient received study medicine. This patient was 64 years old.

What Were the Study Results?

This study in patients with prostate cancer looked at prostate cancer biomarkers. The study was to compare their levels before the Eligard injection on day 1 and 6 months thereafter.

When this study was stopped, there were not enough patients in the study to answer the study's question. This is a summary of study results for the 1 patient in the study who received study medicine.

From day 1 to day 169 (about 5.5 months after the Eligard injection), the patient's level of testosterone in serum dropped to a very low level. The level of PSA in serum also dropped. The level of PCA3 in the urine remained the same.

The tests for genetic blueprints ("mRNA") of prostate cancer biomarkers were done with PCR. PCR is a laboratory method used to make many copies of a tiny amount of mRNA, so it can be detected. The test results may not be reliable.

Prostate Cancer Biomarker	**Test Result Before the Eligard Injection (Day 1 of the Study)**	**Test Result at Day 169 of the Study**
Biomarkers in serum		
Testosterone	19.00 nmol/L (19.00 nmol of testosterone in 1 L of serum)	0.20 nmol/L
PSA	4730 µg/L (4730 µg of PSA in 1 L of serum)	362.10 µg/L
Biomarker in the urine		
PCA3	65	36
Biomarkers in certain white blood cells in the blood		
PSA mRNA	*Not detected*	40 copies/PCR
PCA3 mRNA	*Not detected*	15 copies/PCR
TMPRSS2-ERG mRNA	*Not detected*	*Not detected*

What Adverse Reactions did Patients Have?

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 patients

have while they are in the study. These medical 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.

The patient who received study medicine in this study experienced no adverse reactions.

Where Can I Learn More About This Study?

This document is a short summary of the main results from this study and reflects the information available as of March 2016. 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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