Summary of Results for Laypersons

What was the Study Called?

A Phase 2, Open-label, Single-arm, Efficacy and Safety Study of Enzalutamide (MDV3100) in Patients with Hormone-naïve Prostate Cancer

Why was this Study Needed?

Prostate cancer growth is dependent on male hormones or "androgens." An example of an androgen is testosterone. Enzalutamide (also known as MDV3100 and Xtandi®) is a medicine used to treat patients with prostate cancer. It blocks the effect of androgens. For a time, this stops or slows down the growth of prostate cancer and lowers the blood level of a protein produced by prostate cancer cells. That protein is called prostate specific antigen or "PSA" for short.

This was the first study to test enzalutamide in patients with prostate cancer whose testosterone level was in the normal range. The main question this study helped answer was if enzalutamide could lower patients' blood level of PSA by at least 80%. Those patients were said to have a "PSA response." It was also important to find out what unwanted effects these patients had from enzalutamide.

This study took place at 13 clinics in Belgium, Czech Republic, Denmark and Germany. The study started in May 2011 and ended in April 2017. When the study ended, the sponsor (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 all patients knew that they took enzalutamide.

Men aged 18 years or older could take part in the study if:

- Their doctor had determined that they had prostate cancer. And they were about to begin hormonal therapy on their doctor's advice.
- They did not have symptoms of prostate cancer.
- Their testosterone level was in the normal range.
- They were expected to live for at least 1 year.

Patients could not take part in this study if:

- They were taking hormonal therapy or any other type of treatment for their prostate cancer. Or they had taken those treatments in the past.
- Their cancer had (probably) spread to the brain or the skull. Or their cancer had spread to the membranes surrounding the brain and the spinal cord (the nerves of the spine).
- They had a condition that the study doctor thought would make it hard for them to take part in the study. Or their condition would make it hard to evaluate their study results.

During the study, the study doctor did a check-up of the patients at several study visits. At visit 1, patients were checked to see if they could be in the study. Patients who could be in the study took enzalutamide 160 mg (four 40-mg capsules) by mouth once a day for up to 24 weeks. Blood samples were collected during the study to test the patients' level of PSA. At week 25, the study doctor evaluated their cancer. Patients could continue taking the study medicine if the study doctor determined that they would benefit from it. After the study had ended, the study doctor determined if they would still benefit from the treatment. If so, these patients could continue the treatment in a new study.

A total of 67 patients were in the study and took at least 1 dose of enzalutamide.

	Number of Patients
Age Group	
Aged less than 65 years	11
Aged between 65 and 75 years	26
Aged 75 years or older	30
Clinic Location	
European Union Countries	67
Belgium	25
Czech Republic	9
Denmark	26
Germany	7
Outside European Union	0

What Were the Study Results?

This study was conducted in patients with prostate cancer whose testosterone level was in the normal range. The study determined if patients had a "PSA response." This meant their PSA level was at least 80% lower at the end of the enzalutamide treatment than at the start. A total of 67 patients started treatment with enzalutamide. After 24 weeks of treatment, 62 of 67 patients (93%) had a PSA response. Of the 67 patients, 42 patients took enzalutamide for 3 years. Of these 42 patients, 38 patients (91%) continued to have a PSA response.

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.

A total of 65 patients who took at least 1 dose of enzalutamide each had 1 or more adverse reactions.

The table below shows the most common adverse reactions experienced by patients who took at least 1 dose of enzalutamide.

Adverse Reaction	Enzalutamide 160 mg once a day (out of 67 patients)
Any adverse reaction	65 (97.0%)
Enlargement of a man's breasts	36 (53.7%)
Fatigue or tiredness	24 (35.8%)
Feeling hot for a brief moment	15 (22.4%)
Nipple pain	13 (19.4%)
Breast pain	6 (9.0%)
Dry skin	5 (7.5%)
Feelings of sadness, worthlessness, thoughts of suicide or death (depression)	5 (7.5%)
Nausea or the urge to vomit	5 (7.5%)
Dry mouth	4 (6.0%)
High blood pressure	4 (6.0%)
Shortness of breath	4 (6.0%)

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

Five patients experienced serious adverse reactions. The table below shows these serious adverse reactions.

Serious Adverse Reaction	Enzalutamide 160 mg once a day (out of 67 patients)
Any serious adverse reaction	5 (7.5%)
Abnormally fast irregular heartbeat involving the upper chambers of the heart (atria)	2 (3.0%)
Breast cancer	1 (1.5%)
Disorder that causes extreme fatigue	1 (1.5%)
Problem with the rate (rhythm) of the heart	1 (1.5%)
Swollen lymph nodes	1 (1.5%)

Five patients died. None of the patients died because of the study medicine.

Where Can I Learn More About This Study?

This document reflects the information available as of April 2017. This summary of the clinical study results is available online at http://www.astellasclinicalstudyresults.com.

Astellas may perform additional studies to better understand enzalutamide.

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. If you have questions about enzalutamide, please discuss these with your doctor.

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