kofam

Koordinationsstelle Forschung am Menschen

CASE STUDY

# Enzalutamide monotherapy in hormone-naive prostate cancer: primary analysis of an open-label, single-arm, phase 2 study

**CATEGORISING**

**Kind**
Research project involving living people

**Type**
Clinical trial

**Subtype**
Clinical trial with medicinal products (including combinations according to Art. 2 Para. 1 Letters f and g MedDO)


**BACKGROUND**

The androgen receptor inhibitor enzalutamide (Xtandi®) was approved to treat metastatic castration-resistant prostate cancer that has progressed on docetaxel. This study assessed the activity and safety of enzalutamide monotherapy in men with hormone-naive prostate cancer.

**METHODS**

Men at least 18 years old, with hormone-naive prostate cancer, for whom hormone therapy was indicated, and who had non-castration levels of testosterone and prostate-specific antigen of 2 ng/mL or greater at screening, and an Eastern Cooperative Oncology Group score of 0, received oral enzalutamide (Xtandi®) 160 mg/day. The primary outcome, measured at week 25, was the proportion of patients in whom a prostate-specific antigen had declined 80% or more.


**QUESTIONS OF THE CATEGORISER**

**Does the research project come under the scope of application of the Human Research Act?**
Yes

**BECAUSE**
This project was based on a study protocol that defines the exact procedures to be used. It includes a relatively large number of persons and is not based on individual cases ("method-driven search for generalizable knowledge"); thus, it is classified as research according to HRA). Men 18 years or older ("persons"), with hormone-naive prostate cancer ("research concerning human diseases") received oral enzalutamide (Xtandi®) 160 mg/day.

**Is the research project a project involving living persons?**
Yes

**BECAUSE**
This research project included men aged 18 or older ("persons") with hormone-naive prostate cancer.

**Is the research project a clinical trial?**
Yes

**BECAUSE**
According to the study protocol, the investigator treated men ("persons") with hormone-naive prostate cancer with oral enzalutamide (Xtandi®) 160 mg/day ("health related intervention [therapeutic measure]"). These men were "prospectively assigned" to the intervention, and the study estimated the proportion of patients whose prostate-specific antigen had declined 80% or more by week 25 ("to investigate its effect on health").

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**Does the trial involve investigating medicinal products (including combinations according to Art. 2 Para. 1 Letters f and g Medical Device Ordinance (MedDO) from the July 1, 2020)?**
Yes

**BECAUSE**
This clinical trial investigated the effects of enzalutamide (Xtandi®) 160 mg/day ("medicinal products").

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**Is the IMP authorised in Switzerland?**
Yes

**BECAUSE**
This clinical trial investigated the effects of enzalutamide (Xtandi®) 160 mg/day. Xtandi® is "authorised" for the market in Switzerland (approval number (Xtandi®): 63040 [Swissmedic]).

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**Does the IMP administration comply with the specifications in the summary of product characteristics (SPC)?**
No

**BECAUSE**
This clinical trial investigated the effects of enzalutamide (Xtandi®) 160 mg/day in men aged 18 or older with hormone-naive prostate cancer, for whom hormone therapy was indicated and who had non-castration levels of testosterone. Enzalutamide (Xtandi®) was not given in combination with hormone therapy. Enzalutamide (Xtandi®) is approved in combination with luteinising hormone-releasing hormone analogues to treat patients with metastatic castration-resistant prostate cancer, who already received docetaxel therapy. Use of enzalutamide (Xtandi®) within this clinical trial does not correspond with approved uses.

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**Does the deviation from the specifications in the SPC concern indication or dosage?**
Yes

**BECAUSE**
This clinical trial investigated the effects of enzalutamide (Xtandi®) 160 mg/day in men aged 18 or older with hormone-naive prostate cancer, for whom hormone therapy was indicated and who had non-castration levels of testosterone. Xtandi® was not combined with hormone therapy. Xtandi® is approved in combination with luteinising hormone-releasing hormone analogues to treat patients with metastatic castration-resistant prostate cancer, who have already received docetaxel therapy. The use of Xtandi® in this clinical trial does deviates from the specifications in the Summary of Product Characteristics only in regards to required co-interventions, but not regarding indication or dosage.

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**Does the indication lie within the same disease group in the ICD-10 classification as stated in the SPC (disease group indicated by the three-digit code)?**
Yes

**BECAUSE**
This clinical trial investigates the effects of enzalutamide (Xtandi®) 160 mg/day in men aged 18 or older with hormone-naive prostate cancer, for whom hormone therapy was indicated and who had non-castration levels of testosterone. Xtandi® is approved in combination with luteinising hormone-releasing hormone analogues to treat patients with metastatic castration-resistant prostate cancer, who have already received docetaxel therapy. Hormone-naive prostate cancer falls within the same ICD-10 group as the approved indication (metastatic castration-resistant prostate cancer, for those who have previously received docetaxel therapy C61: Malignant neoplasm of prostate).

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**Is the disease for which the IMP is administered in the clinical trial a self-limiting disease or condition?**
No

**BECAUSE**
This clinical trial investigates the effects of enzalutamide (Xtandi®) 160 mg/day in men aged 18 or older with hormone-naive prostate cancer, for whom hormone therapy was indicated and who had non-castration levels of testosterone. Self-limiting diseases resolve spontaneously, with or without treatment. Untreated hormone-naive prostate cancer does not resolve spontaneously.

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**Does the IMP's administration comply with standard medical practice as defined in a treatment guideline developed in accordance with international quality critieria?**
Yes

**BECAUSE**
This clinical trial investigates the effects of enzalutamide (Xtandi®) 160 mg/day in men aged 18 or older with hormone-naive prostate cancer, for whom hormone therapy was indicated and who had non-castration levels of testosterone. Xtandi® was not be given in combination with hormone therapy. Thus it does not comply with the intended use recommended by the European Association of Urology guidelines on prostate cancer (treatment guideline developed in accordance with international quality criteria).

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**Is a placebo used in the trial, or is the original status of the medicinal product or its packaging as approved by Swissmedic modified?**
No

**BECAUSE**
This clinical trial investigated the effects of enzalutamide (Xtandi®) 160 mg/day. Xtandi® is "authorised" for the market in Switzerland, and is provided "as is". The medicinal product was provided in its original package but labelled with a trial-specific sticker (label). However, the label did not cover any pharmaceutically relevant information.

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