Appendix G – patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal (STA)

Enzalutamide for the treatment of metastatic hormone relapsed prostate cancer previously treated with a docetaxel-containing regimen

Thank you for agreeing to give us your views on the technology and the way it should be used in the NHS.

Patients and patient advocates can provide a unique perspective on the technology, which is not typically available from the published literature.

To help you give your views,we have provided a template. The questions are there as prompts to guide you.You do not have to answer every question. Please do not exceed the 8-page limit.

About you

Your name: [redacted]

Name of your organisation: Prostate Cancer UK

Are you (tick all that apply):

- an employee of a patient organisation that represents patients with the condition for which NICE is considering the technology?If so, give your position in the organisation where appropriate (e.g. policy officer, trustee, member, etc)

Prostate Cancer UK is the UK's leading charity for men with prostate cancer and prostate problems. We support men and provide information, find answers through funding research and lead change to raise awareness and improve care. The charity is committed to ensuring the voice of people affected by prostate disease is at the heart of all we do.

We conducted an online survey of people affected by prostate cancer about their opinions on enzalutamide for the treatment of metastatic hormone relapsed prostate cancer previously treated with a docetaxel-containing regimen and access to the drug. 63 people replied to the survey and quotes from the respondents are included in this submission. Of the 63 respondents, two men said they had been treated with enzalutamide.

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What do patients and/or carers consider to be theadvantages and disadvantages of the technology for the condition?

1. Advantages

(a) Please list the specific aspect(s) of the condition that you expect the technology to help with.For each aspect you list please describe, if possible, what difference you expect the technology to make.

As demonstrated in the Phase III trial[i], enzalutamide has been found to prolong the overall survival of men whose prostate cancer has spread to other parts of the body (advanced prostate cancer) and has stopped responding to other hormone therapy and chemotherapy treatments by an average of 4.8 months.

This treatment will make a significant difference to these men by offering the possibility of extending their lives, when there are only limited treatment options available, apart from palliative care.

Should the STA recommend enzalutamide for use for this indication it will help to provide standardised access to the drug, increase the range of clinically effective treatment options available to all patients for whom it is appropriate and provide them with greater choice and hope, possibly giving them more time with their families and improving their quality of life.

(b) Please list any short-term and/or long-term benefits that patients expect to gain from using the technology. These might include the effect of the technology on:

- the course and/or outcome of the condition
- physical symptoms
- pain
- level of disability
- mental health
- quality of life (lifestyle, work, social functioning etc.)
- other quality of life issues not listed above
- other people (for example family, friends, employers)
- other issues not listed above

Of the 63 people affected by prostate cancer who responded to our survey, 59 people (1 person did not answer this question) believe it is ‘very important’ (53) or ‘important’ (6) for enzalutamide to become a treatment option available to all men for whom it is clinically appropriate. When asked about benefits of enzalutamide, 43 people said that it was important to extend life for as long as possible. 30 said that it improved long-term control of the disease. 31 said it could provide a better quality of life, including less pain. 29 said it provided another treatment option when other treatments have failed. 20 said that an advantage of enzalutamide is that it can be administered orally.

Quotes on advantages include:

"…ultimately, if it improves quality of life, then this should be made an available option."

"It is another option to help extend someone's life - what else is there to say?"

"It would really help me if I could contribute to the work of running the household/smallholding and supporting my wife for as long as possible. I am aged 62 and do not wish to leave my wife on her own at such a young age. My pension will die with me, and the longer I live, the longer I can support my wife."

"It has been well trialled and is another welcome bullet in the armoury against prostate cancer."

"Enzalutamide would give a potential life line to all men like my husband aged only 58 (54 at advanced)who have used all treatments currently available. Our family and friends are in despair, our youngest child has two years left at uni, perhaps enzalutamide would give us that time as a family to achieve together milestones that will affect our children's future.This disease is devastating, it's not only killing the patient but his family and friends too who can do nothing"

"As someone with a limited life expectancy thanks to advanced prostate cancer I can assure you then every moment of life counts and is appreciated, even the filling in of surveys! If enzalutamide can provide an average of 5 extra months then it must be made available to people in my situation."

2. Disadvantages

Please list any problems with or concerns you have about the technology.
Disadvantages might include:

- aspects of the condition that the technology cannot help with or might make worse
- difficulties in taking or using the technology
- side effects (please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)
- impact on others (for example family, friends, employers)
- financial impact on the patient and/or thier family (for example cost of travel needed to access the technology, or the cost of paying a carer)

The side effects of enzalutamide are generally mild but include:
Tiredness (fatigue)
Diarrhoea
Hot flushes
Pain in joints and muscles
An increased risk of infection due to a drop in the number of white blood cells
Headaches
Dizziness
Feeling weak
Swollen feet and ankles due to fluid build up (known as peripheral oedema)

Higher blood pressure

In the research some men taking enzalutamide had a seizure (fit). This was rare and affected less than 1 in 100 men (1%). None of the men taking the placebo had a seizure. The researchers recommend that care should be taken in men who may be at higher risk of seizures. This includes men who:

- Have a history of seizures
- Have had any kind of brain injury such as a stroke
- Have secondary cancer in the brain
- Are alcoholic[ii]

25 people responded to a question we asked about problems or concerns they may have about the efficacy of enzalutamide. Of these, 12 respondents had no concerns about enzalutamide. When asked about potential side effects 3 people were very concerned; 16 were concerned, but expect the benefits to outweigh the benefits; 39 were concerned, but no more than any other standard treatment.

Some comments received were:

“Only the fact that it is a short term extension to life like all present drugs available e.g. abiraterone.”

“It does not seem to have extended life in the trial by much.”

“The side effects mentioned appear to be perfectly manageable and not as bad as the disease. In fact, the side effects are not unlike those currently experienced with hormone therapy. My priority at this stage of my life is "quality of life" not "quantity".”

3. Are there differences in opinion between patients about the usefulness or otherwise of this technology? If so, please describe them.

As identified above, the overwhelming majority of people we surveyed agreed that for men who were in the advanced stages of prostate cancer, and for whom chemotherapy had ceased to be effective, this drug could extend life and quality time at the end of their lives.

Only a few respondents had concerns about the clinical effectiveness of enzalutamide is fairly limited, by only giving a ‘small’ extension to life.

“Only the fact that it is a short term extension to life like all present drugs available e.g. abiraterone.”

4. Are there any groups of patients who might benefit **more** from the technology than others? Are there any groups of patients who might benefit **less** from the technology than others?

Currently, abiraterone is the only treatment widely available on the NHS for men who have metastatic castration-resistant prostate cancer which has stopped responding to other hormone therapies and docetaxel chemotherapy. Cabazitaxel and denosumab are licensed for use in this setting, but have not been recommended by NICE. The only other options are palliative/best supportive care.

Comparing the technology with alternative available treatments or technologies

NICE is interested in your views on how the technology compares with existing treatments for this condition in the UK.

(i) Please list any current standard practice (alternatives if any) used in the UK.

Abiraterone is suitable for men with advanced prostate cancer that has stopped responding to other hormone therapy and chemotherapy treatments. This was approved for use on the NHS by NICE in 2012.

(ii) If you think that the new technology has any **advantages** for patients over other current standard practice, please describe them. Advantages might include:
- improvement of the condition overall
- improvement in certain aspects of the condition
- ease of use (for example tablets rather than injection)
- where the technology has to be used (for example at home rather than in hospital)
- side effects (please describe nature and number of problems, frequency, duration, severity etc)

Please see comments above.

(iii) If you think that the new technology has any **disadvantages** for patients compared with current standard practice, please describe them. Disadvantages might include:
- worsening of the condition overall
- worsening of specific aspects of the condition
- difficulty in use (for example injection rather than tablets)
- where the technology has to be used (for examplein hospital rather than at home)
- sideeffects (for examplenature or number of problems, how often, for how long, how severe).

Please see comments above.

Research evidence on patient or carer views of the technology

If you are familiar with the evidence base for the technology, please comment on whether patients' experience of using the technology as part of their routine NHS care reflects that observed under clinical trial conditions.

Two respondents to our survey said they were currently being given enzalutamide.

"If I am currently on enzalutamide then it has given me no extra side-effects beyond those of casodex which I have been on for nearly 4 years."

"I am on Enzalutamide. Fatigue is a big problem so I've been dropped to a three quarters dose to get through the day. I'd like to get back up to a full dose. Seems to be working well though."

Are there any adverse effects that were not apparent in the clinical trials but have come to light since, during routine NHS care?

See comments above.

Are you aware of any research carried out on patient or carer views of the condition or existing treatments that is relevant to an appraisal of this technology? If yes, please provide references to the relevant studies.

As per our survey.

Availability of this technology to patients in the NHS
What key differences, if any, would it make to patients and/or carers if this technology was made available on the NHS?

Of the 63 people affected by prostate cancer that we surveyed, the most common benefits of making enzalutamide available on the NHS were identified as offering hope in extending the lives of men with advanced prostate cancer and providing an additional treatment option for these men.

Currently, abiraterone is the only non-palliative treatment option available on the NHS for men with this type of cancer. We believe all men should be offered the choice of a life-extending agents that can allow them a few extra months to spend with family and friends and should be able to access that drug on the NHS if it has been prescribed by their doctor and they make an informed choice to take it. If the STA recommends this treatment for use it would provide further hope and demonstrate to men and their carers that progress is being made in the treatment of prostate cancer, including for men with advanced disease.

"Enzalutamide would give a potential life line to all men like my husband aged only 58 (54 at advanced) who have used all treatments currently available. Our family and friends are in despair, our youngest child has two years left at university, perhaps enzalutamide would give us that time as a family to achieve together milestones that will affect our children's future. This disease is devastating, it's not only killing the patient but his family and friends too who can do nothing"

"Life would be prolonged and life is everything. End of."

"It prolongs life, nothing is more important than that"

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"It is another string to the bow in the fight against cancer and if it's the latest improved drug to prolong life with less suffering and pain, we need it for both patient and carers"

"All treatments facilitate management of the disease and options/choice are available as not every treatment suits every case so the more tools available the better lives will be for some patients"

"The cost of management options is crippling at the moment so the more that can be made available on the NHS, the better"

What implications would it have for patients and/or carers if the technology was **not** made available to patients on the NHS?

The overwhelming majority expressed disappointment or anger if enzalutamide was not made available on the NHS.

"Not happy. There is no reliable screening test and men will have advanced prostate cancer when diagnosed and we owe it to these men to give them hope."

"I would feel that they are not taking prostate cancer seriously"

"I would be devastated. It would be a bitter blow to men in this category and their families. As with Abiraterone, the average life expectancy is arbitrary - many men would live much longer and have more valuable time to spend with family and see grandchildren grow up. It would also deprive them of the "window of opportunity" for new drugs which might be in the pipeline"

"It feels slightly ridiculous to develop any drug that would benefit patients and then not make it available to them."

"I feel that to deny this treatment as in any life threatening scenario should not be an option"

Are there groups of patients that have difficulties using the technology?

No. Enzalutamide is easily administered as four capsules orally once a day.

Equality
NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that this appraisal:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which [the treatment(s)] is/are/will be licensed;
- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;
- could lead to recommendations that have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the Committee to identify and consider such impacts.

It will be important to ensure that access to this technology is equitable and discrimination does not occur inappropriately on the basis of age, ethnicity or socio-economic status. Prostate cancer is more common in men aged over 60 and African Caribbean men are three times more likely to develop prostate cancer than white men of the same age in the UK. Furthermore, men from lower socioeconomic backgrounds are less likely to survive prostate cancer than men from more affluent backgrounds. It will be important to ensure that eligible patients from these populations are not denied access to this technology (if approved) because of factors related to their age, ethnicity and socio-economic status. Information and communication strategies must also be considered and patients consulted to ensure that access can be as equitable as possible.

Other Issues

Please consider here any other issues you would like the Appraisal Committee to consider when appraising this technology.

It is important that health-related quality of life and adverse effects are considered with an equal standing to the other outcomes, such as patient-reported outcomes. Consideration of patient-reported outcomes will ensure that the agent is not only clinically effective but also improves outcomes of importance to this patient population, such as the extension of life.

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[i] Increased survival with enzalutamide in prostate cancer after chemotherapy. Scher, H et.al. The New England Journal of Medicine. September 2012.

[ii] Taken from Cancer Research UK's website (http://www.cancerresearchuk.org/cancer-help/about-cancer/cancer-questions/enzalutamide-for-prostate-cancer#sfx) – all results from AFFIRM Phase III trial: Increased survival with enzalutamide in prostate cancer after chemotherapy. Scher, H et.al. The New England Journal of Medicine.

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