Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Axitinib for the treatment of advanced renal cell carcinoma after failure of prior systematic treatment

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: James Whale Fund for Kidney Cancer

Are you (tick all that apply):

- a patient with the condition for which NICE is considering this technology?
- a carer of a patient with the condition for which NICE is considering this technology?
- ✓ 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) Sharon Deveson Kell, Medical Publications Officer
- ✓ other? (please specify) An RCC patient who is not metastatic (Bill Savage)

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What do patients and/or carers consider to be the advantages 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.

There are three main aspects of kidney cancer that patients, carers and family members see axitinib as helping to make a difference:

1. Progression free survival. This is the main benefit of survival from a lethal disease with about 50% mortality. Although not a cure for kidney cancer, axitinib has been shown to extend progression free survival by more than 40% compared to sorafenib in a second line setting, whilst offering good quality of life.

2. Improved side effect profile. Existing first and second line drugs, such as sunitinib, pazopanib and sorafenib can have severe side effects, which affect quality of life and sometimes limit the ability of patients to tolerate the treatment. Although side effects to axitinib can also be severe, they are generally easier to manage and better tolerated than side effects to sorafenib when used as a second line treatment.

3. Access to second line treatment. For those patients unable to tolerate first line treatment with sunitinib or pazopanib due to side effects, or whose disease has stopped responding to first line treatment, a second line treatment is needed on the NHS. Patients unable to access second line treatment due to lack of funding are left without any treatment options. Axitinib offers hope to patients for a second line treatment available on the NHS.

(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

Beyond the advantages listed above, the technology offers hope to patients and carers for extended life with all the advantages that it can bring to the morale and well being of the patient, carer and family.

The technology also offers the hope of extending working life and the ability to interact socially with family and friends.

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The drug is orally administered which is huge advantage to patients, carers and the NHS since the drug can be taken at home without the need for hospital appointments.

These benefits all help to improve the quality of life for patients with advanced kidney cancer and offer them some hope of a normal life for many months or even years to come.

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 axitinib can be concerning, but the reports suggest that the side effect profile for axitinib is superior to that for sorafenib, another second line treatment for advanced kidney cancer. There is a higher incidence of high blood pressure for axitinib compared to sorafenib; however, this side effect can be well controlled with medication, significantly reducing the impact of axitinib on quality of life. Side effects, such as hand-foot syndrome, rash and alopecia are more common with sorafenib, these side effects having a greater impact on quality of life. Support Groups confirm the willingness of patients to tolerate the impact of the drug and the known methods to reduce that impact.

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

All patients want extended life, which is promised by axitinib. Side effects can be a concern but will be tolerated by the vast majority of patients and offer improved quality of life compared to alternative second line treatments, such as sorafenib.

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?

Patients with a positive commitment to new technology and the access to strong support from carer and family might benefit more from axitinib. The converse applies.

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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.

Standard practice for the treatment of advanced (metastatic) renal cell carcinoma (RCC) is surgery followed by first line treatment with sunitinib or pazopanib. These treatments have given RCC patients hope but at the cost of severe side effects and limited progression free survival. For those patients who are unable to tolerate the side effects to these first line drugs, or those for whom their disease no longer responds to treatment, there are no second line treatments available on the NHS.

Second line treatments, such as everolimus or sorafenib, are available through the Cancer Drugs Fund in England, but patients are increasingly concerned about what happens in 2013 when the Cancer Drugs Fund comes to an end. For patients in Scotland, Wales and Northern Ireland, there is no Cancer Drugs Fund to fund second line treatment and patients have to apply for funding to their PCT, a long and bureaucratic process which, for many, is too much to take during their last months of life. Alternatively, second line drugs can be accessed through participation in clinical trials, which requires a high degree of commitment from patients in terms of clinic visits and patient monitoring.

A small proportion of patients (less than 5%) respond well to the immunotherapy, interleukin-2; however, due to the toxic nature of this drug, patients are preselected for this treatment and it is only suitable for those who are relatively young and fit. Interferon-α is also still used as first line treatment for advanced RCC, but usually in patients who are not well enough to tolerate the side effects of the targeted therapies.

Other drugs for the treatment of advanced RCC include bevacizumab in combination with interferon-α and temsirolimus, which is given to patients with a poor prognostic score. These drugs are both administered intravenously and are not often used in the UK.

RCC does not respond to traditional cancer treatments such as chemotherapy or radiotherapy.

(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)

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Axitinib has been shown to be associated with improved progression-free survival compared with sorafenib while generally maintaining health-related quality of life. Progression-free survival was increased by more than 40% compared to sorafenib in a second-line setting.

Studies suggest that the side effect profile for axitinib is superior to that for sorafenib. Although there is a higher incidence of high blood pressure for axitinib compared to sorafenib, this side effect can be well controlled with medication, significantly reducing the impact of axitinib on quality of life. Side effects, such as hand-foot syndrome, rash and alopecia are more common with sorafenib, these side effects having a greater impact on quality of life.

The opportunity for extended life and a more acceptable side effect profile PLUS home oral administration gives axitinib an advantage over existing second-line treatments.

(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 example in hospital rather than at home)
- side effects (for example nature or number of problems, how often, for how long, how severe).

Not known.

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.

N/A

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

N/A

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.

N/A

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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?

The main differences to patients and/or carers brought about by availability of a second line treatment for advanced RCC on the NHS include the following:

1. A second line treatment option for those patients who are no longer responding to first line treatment or who are unable to tolerate the side effects from first line treatment. Currently, this option is not available to NHS patients, unless they go through the bureaucratic process of applying for funding through either the Cancer Drugs Fund or their PCT, or participate in a clinical trial.

2. A second line treatment option offers increased progression free survival of more than 40% compared to current second line options.

3. A more acceptable side effect profile than current second line treatments resulting in improved quality of life for patients on second line treatment.

4. Oral administration of axitinib enables home administration and ease of management of second line treatment, also benefiting quality of life for the patient and their carer/family.

5. Access to an affordable treatment option for advanced RCC patients.

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

If axitinib was not made available to patients on the NHS, it would have the following implications for patients and/or carers:

1. Lack of options for second line treatment when first line treatment fails or is not tolerated by the patient. Patients will be required to apply to the Cancer Drugs Fund for funding or to their PCT when the Cancer Drugs Fund is closed, a lengthy and bureaucratic process that many patients do not have the energy or ability to go through in their last months of life. This leaves some patients without any treatment options available to them.

2. Without a second line treatment option, overall survival of advanced RCC patients will be reduced.

3. The less acceptable side effect profile of current second line treatments will impact the quality of life and compliance with medication schedules of advanced RCC patients in the last months of their lives. When compliance is reduced, drug effectiveness is sub-optimal.

4. The costs of second line treatment could potentially be passed on to the patients and their families causing stress and anxiety during an already difficult and stressful

time for the families of cancer patients. This also has a negative impact on the quality of life of advanced RCC patients.

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

Not known.

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.

Approval of axitinib as a second line treatment for advanced RCC would negate the need for clinicians and patients to apply to the Cancer Drugs Fund or their PCT for second line treatment funding. This would avoid the current perceived postcode lottery aspect of cancer drug availability and the subsequent inequalities of access to drugs brought about by this process.

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

Advanced or metastatic RCC is a terminal disease and survival beyond 5 years is rare. It is important that the committee recognises the huge boost to patient and carer morale brought about by their encouragement of new and effective drugs with more tolerable side effect profiles.

In the last year, the Friends of Renal Oncology Groups (FROG) in Oxfordshire and Buckinghamshire have witnessed many deaths from metastatic RCC. This is a depressing and upsetting experience for all. The drugs are improving and the clinicians are learning to manage the side effects. Axitinib is another large step along the road to making RCC a chronic disease rather than a death sentence.

Below is a statement from a patient carer, which summarises the feelings of patients and their families with respect to access to second line treatment for advanced RCC;

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"When my husband was first diagnosed with kidney cancer, we were told his life expectancy was probably around 12 months. Unfortunately, after having his kidney removed, we were told that suitable drug treatment was not available via the NHS. After campaigning to gain access to these drugs, unsuccessfully, we were offered the opportunity of taking part in a clinical trial, which would include my husband being given the drug we had been denied through the NHS. His body responded well to these drugs for 2 years. When we were told his body had stopped responding, we had to absorb this news and face, with trepidation, the daunting thought that we would once again have to fight to gain access to second-line treatment. When faced with the news we had been given, the last thing you need is to know that there is an alternative treatment that your body could very possibly respond well to, but you would not be able to access the drugs without a fight. Luckily, we were successfully accepted onto another clinical trial, and my husband survived for another 14 months. This meant he saw 2 grandchildren born which would not have happened if we hadn't have been given this opportunity of second-line treatment. It is crucial that second-line treatment is readily accessible when needed for kidney cancer patients. The treatment options are limited enough, without having the stress of being denied second-line treatment when the need arises. It gave us the chance to enjoy another 14 months together, and believe me, that is priceless."