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: RARER CANCERS FOUNDATION
(incorporating the Rarer Cancers Forum)

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)

- other? (please specify)

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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.

The Rarer Cancers Foundation makes the following comments -

1) There is a recognised and unmet clinical need for a 2nd line treatment when prior systemic targeted therapy or immunotherapy has failed the patient – approval of Axitinib by N I C E for N H S funding, will extend life for many of these patients.

2) There is a recognised and unmet clinical need for a 2nd line treatment when prior 1st line treatment has been tried but found to be clinically unsuitable for individual patients - approval of Axitinib by N I C E for NHS funding, will extend the life of some of these patients.

3)The approval and funding of Axitinib as 2nd treatment for mRCC would reduce the uncertainty and distress caused to patients whose disease has progressed following prior treatment. Currently applying for further 2nd line treatment for mRCC patients via the Cancer Drugs Fund is the only option for Clinicians who believe further active treatment will be clinically beneficial for their patients. Funding by this route is only available in England and is uncertain. This leads to inequity, stress, distress and uncertainty for patients whose clinicians believe they are fit for treatment and who have the potential to have prolongation in control of their cancer and their survival by accessing a treatment that has been shown in clinical trials to be active for the second line treatment of advanced mRCC.

(b) Please list any short-term and/or long-term benefits that patients expect to gain from using the technology.

The Rarer Cancers Foundation makes the following comments -

Axitinib in 2nd line has the potential to extend the overall survival for a specific group of mRCC patients defined by the marketing authorisation.
Axitinib in 2nd line would delay the onset of palliative care and allow a specific group of mRCC patients to play a fuller role in family life and enjoy a better quality of life with their family. Some mRCC patients continue to work and although ill, some patients will be able to contribute more fully to society whilst on targeted therapy.
Axitinib in 2nd line in some patients has the potential to delay the onset of tumour pain and postpone until later the need for pain management in late stage disease.
Axitinib in 2nd line in some patients has the potential to allow patients to enjoy a longer period of active life, maintaining activities and feeling part of society before the onset of disabilities associated with palliative care and end of life care.

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Axitinib in 2nd line in some patients has the potential to extend life at this most important period of life when quality time with family and loved ones is so important. Patients will also feel better cared for and more positive in their approach to coping with their disease when they are on active treatment.

For a patient with a high performance status, to be refused active NHS treatment when there are proven, clinically effective therapies available is a terrible burden for the patient, the carer-giver, families and the wider community. A refusal to fund active effective treatment, affects kidney cancer patients throughout their last months of life and right upto the moment of death and the denial to treat patients continues to poison the lives of families and loved ones, long after death. The advent of social networking means many more kidney cancer patients within the patient communities are made aware of the distress and anguish caused to individual kidney cancer patients who are denied treatment. This obviously has a hugely negative and depressing effect on other patients on the pathway who will worry what will happen to them when they need a life extending treatment. Mentally, for patients and carers, the refusal of the NHS to actively treat patients is extremely destructive and ultimately can prevent, what is euphemistically referred to as, a "good death"

If Axitinib were to be formally approved for 2nd line mRCC, it would help prevent patients' feelings of powerlessness about treatment options and choice ; currently patients are outwith the Cancer Drugs Fund process; they are not allowed to contribute or take part in clinician-led application to the CDF for life extending drugs. If 2nd line treatment is formally approved by N I C E patients can feel their situation is recognised and dealt with compassionately and with understanding by the Health Authorities

2. Disadvantages

Please list any problems with or concerns you have about the technology.

As a patient/survivor of early stage ccRCC and Head of Patient Support at the Rarer Cancers Foundation, I am acutely aware that patients acknowledge and accept the inescapable fact that mRCC itself and management of the disease, will hugely affect how they live their lives (and how they die). Side effects of the disease itself and side effects of any subsequent treatment are matters that patients, carer-givers and families deal with every day of their lives following recurrence or after an initial mRCC diagnosis. Patients expect side effects – it is the price we have to pay. Mitigation and advice about the management of side effects, is easily accessible within the secondary care/Hospital setting and increasingly, we know patients share insights and experiences and help each other manage side effects via support groups and online forums and confidential listservs©. . The severity of side effects varies considerably between patients; some are negligible causing very little disruption to daily life, but some are more severe. However, in nearly all cases patients will confirm that side effects are infinitely preferable to no active cancer treatment and a premature death.

Oral therapies are simple to administer and there is no direct cost to the patient.

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

The Rarer Cancers Foundation makes the following comments -

There has been a positive reaction by the patient community to Axitinib. Patients recognise from the experiences of mRCC patients in America and patient experiences in the clinical trials, that Axitinib may have a lower side effects profile than other treatments currently available via the Cancer Drugs Fund.

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?

The Rarer Cancers Foundation makes the following comments - The only group of mRCC patients who will benefit from the approval of Axitinib at 2nd line, will be good performance status patients who have experienced disease progression following (one) prior treatment regime with either with Sunitinib or immunotherapy.

This leaves all other mRCC subtype patients other than clear cell, unable to access the treatment.

Perversely, this appraisal does not touch on the obvious implications for mRCC patients who are prescribed the N I C E approved alternative 1st line treatment Pazopanib). Clinicians who wish to prescribe Pazopanib on clinical grounds (rather than 1st line Sunitinib) will be hampered in the future treatment of their patients by the fact that, if they prescribe Pazopanib, they will be denying those patients an effective 2nd line Axitinib treatment in the future. We realise this is a consequence of the restrictive marketing authorisation but nevertheless there will be considerable consequences for the group of mRCC patients for whom N I C E approved Pazopanib is the correct choice of 1st line treatment if Axitinib is made available to patients 2nd line after sunitinib or immunotherapy.

The Group of patients who are deemed unsuitable (for whatever reason) to be prescribed Sunitinib or immunotherapy, have the potential to be disadvantaged by this appraisal.

We would also seek clarification from N I C E on behalf of the group of kidney cancer patients who, as part of their treatment and to benefit of other patients and the wider NHS in the future, may have taken part or be invited to take part in a clinical trial with a drug other than Sunitinib or immunotherapy. Are these patients through their actions, to be denied access to Axitinib? We understand that N I C E committees have discussed the implications of this situation, but for clarity and to keep patients fully informed and empowered, could we please request N I C E to include a statement about this particular patient group in their appraisal statement for Axitinib. We feel it is unreasonable to expect RCC patients to continue to support clinical

trials, if their actions will effectively prevent them accessing a 2nd line treatment should they need it.

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.

The Rarer Cancers Foundation makes the following comments - Currently there are only two N I C E approved 1st line therapies routinely available to mRCC patients in the UK; Sunitinib and, as an alternative in certain instances, Pazopanib.

There are currently no 2nd line N I C E approved treatments for mRCC. There is a urgent and unmet clinical need for a formal approved and effective, 2nd line treatment for mRCC patients.

Currently 2nd line treatment for mRCC patients may be available in England via an application to a regional Cancer Drugs Fund. We know some CDF committees are increasingly restrictive about funding certain treatments. This appears to impact disproportionately against rare and less common cancer patients. It is expected that the Cancer Drugs Fund will cease in 2014 when a value based pricing arrangement will be introduced. We do not know what the situation will be and which, if any, CDF funded treatments will still be available to mRCC patients post 2014.

NHS patients in Scotland, Wales and Northern Ireland do not have access to a CDF and obtaining 2nd line treatment for mRCC in these Countries can be extremely difficult without recourse to clinical trials.

(ii) If you think that the new technology has any **advantages** for patients over other current standard practice, please describe them.

The Rarer Cancers Foundation makes the following comments – The side effects profile for Axitinib appears favourable with fewer patient reported problems in online patient forums and support groups.

We understand that approx 50 patients in the UK have taken part in the AXIS clinical trial but we do have the benefit of comments from patients in other Countries where Axitinib is available. Our international patient support forums allow patients to share experiences and patients insights about disease and management of side effects.

Anonymised Patient Quotes -

1) "I participated in a clinical trial with Axitinib for 4 ½ years. Initially, it produced a 65% shrinkage in the mets in my lungs and chest. Then it kept these mets stable for the next 4 years…… I intend to go back to axitinib as soon as it is approved by the FDA, which is anticipated, perhaps, in January. The main reason is that during my 4 ½ years on this drug, I had no new brain mets. Prior to this drug, I had three episodes of brain mets, 12 mets in

all, and all successfully treated by gamma knife. Since stopping the axitinib and going on first Votrient and now Afinitor, I have had two more episodes of brain mets, 5 mets in all and treated by gamma knife.
Axitinib worked for me even though it brought some serious sides. As my cardiologist said, we know how to treat the heart complications that may develop again, but we don't know how to stop the rcc (sic) . If you have something that works, stay with it. Axitinib worked for me. I hope it works for you too.." .
2) "I have been on Axitinib for about 2 1/2 years. While my lesions have not shrunk or disappeared, there has been minimimal (sic) growth and no new growth, except for periods of time when I had to go off the medication. There are a few side effects that I have experienced but as others tell you everyone is different and the degree of side effects differ. I have experienced the high blood pressure, but is controlled with a number of anti-hypertensive drugs. I also get the diarrhea,(sic|) but that too has been tempered with Imodium or Lomotil(sic) I have had a sensitivity of my oral mucosa, but no sores or open wounds. I also get a good deal of fatigue and have learned to take a daily afternoon nap to combat that. Strangest side effect for me was an improvement in my eyesight.
With the Axitinib working for this long, I can not complain about the side effects compared to the results."
3) "I have been on Axitinib for over a year and a half as part of a clinical study. Previously I had two regimens of high dose IL-2 about 2 1/2 years apart. I have not taken Sutent to give you a comparison. I understand that the side effects of Axitnib(sic) are similar to the other anti-angiogenesis drugs and include high blood pressure, diarhea(sic) and skin rashes. For me it was an elevation of blood pressure and the GI problems but both are manageable with other meds. I have experienced some skin and mouth sensitivity, but again manageable.

(iii) If you think that the new technology has any **disadvantages** for patients compared with current standard practice, please describe them. Disadvantages might include:

The Rarer Cancers Foundation makes the following comments – Please see previous comments with reference to side effects from disease and treatments.

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.

The Rarer Cancers Foundation makes the following comments –We do not have any evidence from patients taking Axitinib as part of their routine NHS care.

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

The Rarer Cancers Foundation makes the following comments - See above comment .

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.

The Rarer Cancers Foundation makes the following comments – I attach a link (pdf to follow under separate cover as per guidance) to the study relating to depression and kidney cancer patients; this is relevant to the situation of patients who are denied active treatment and could also inform the data relating to the overall survival of mRCC to whom the only available regime is best supportive care .

http://www.plosone.org/article/info%3Adoi%2F10.1371%2Fjournal.pone.0042324

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 Rarer Cancers Foundation makes the following comments – There is a recognised and unmet clinical need for a 2nd line treatment when prior systemic targeted therapy or immunotherapy has failed the patient – approval of Axitinib by N I C E for N H S funding, will extend the life of mRCC patients.

There is a recognised and unmet clinical need for a 2nd line treatment when prior 1st line treatment has been found to be clinically unsuitable for individual patients - approval of Axitinib by N I C E for NHS funding, will extend the life of mRCC patients.

The approval and funding of Axitinib as 2nd treatment for mRCC would reduce the uncertainty and distress caused to patients whose disease has progressed following prior systemic treatment and for whom, currently, there is no routine follow on treatment.

During the period of treatment with Axitinib, for other clinical trials may report and newer treatments may to become available, allowing for sequential treatments and a further extension of life. This happened when N I C E approved Sunitinib in 2009 since when a number of new and innovative drugs have been licensed thus increasing the overall survival of mRCC patients.

The Rarer Cancer Foundation is aware of the November 2011 article in The Lancet, which reports the Phase III trial (AXIS) for Axitinib and endorses the findings of the

trial, which reported a significant extension of life for patients treated 2nd line with Axitinib. (pdf file of article to follow separately as per N I C E guidance)

What implications would it have for patients and/or carers if the technology was **not** made available to patients on the NHS?
The Rarer Cancers Foundation makes the following comments –

1) Many patients would face a premature death.

2) Patients may fear a lengthy dying process, depression, additional stress, feelings of worthlessness, disappointment & anger towards a health system they see as treating them unfairly and letting them down when they most need it.

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

The Rarer Cancers Foundation makes the following comments – see comments regarding patients who have taken part in clinical trials and those who have been prescribed Pazopanib, an approved alternative to sunitinib; the N I C E approved 1st line

There may be difficulties for individual patients with swallowing difficulties or poor absorption.

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

The Rarer Cancers Foundation makes the following comments – 2nd line treatments for kidney cancer patients diagnosed with a rare or less common cancers such as kidney cancer do not have equity of access to the number of NHS funded treatments, which are made available to more common cancer patients. The RCF would suggest there should be action to redress this balance so that cancer patients diagnosed with a rarer cancer type do not feel they are unfairly treated. Kidney Cancer patients have few funded treatment options when compared to other treatments available to cancer patients diagnosed with cancers that are more common. The Committee could review the methods used to judge the clinical and cost effectiveness and numbers of cancer treatments licensed for cancer patients with less common cancers.

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

The Rarer Cancers Foundation makes the following comments – We do not recognise the comparator described in this scope which is detailed as BSC (best supportive care) . We are aware that N I C E has been “advised” by the Department

of Health that N I C E should not include, as comparators, drugs, which are funded via the Cancer drugs Fund. We do not feel this is a realistic or a patient centred approach.

We have day-to-day contact with kidney cancer patients throughout the UK and internationally. We support kidney cancer patients who are in active treatment and those out of treatment. Based on our experience in the real world providing day by day patient support for patients with metastatic disease, we find the assumption that an average mRCC patient would survive for 10+months following progression on a targeted therapy, to be totally unrealistic. If this figure is to be used to extrapolate the cost effectiveness of Axitinib, then it is our view as a respected patient support organisation, that a cost analysis by N I C E will result in an unrealistic cost to the NHS and could lead to this appraisal being refused.
There is robust data i.e. the Cochrane meta analysis, to inform the committee of a realistic estimate for overall survival for mRCC patients of good performance status who have immunotherapy alone 1st line and no further treatment. Without nephrectomy this was 7 months and with nephrectomy 12-15 months. An average survival of 10+ months for a patient whose first line treatment has stopped working therefore seems excessive and does not accord with our experience.