**Appendix G – patient/carer organisation statement template**

**NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE**

**Single Technology Appraisal (STA)**

**Axitinib for the treatment of advanced renal cell carcinoma after failure of prior systemic 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: Kidney Cancer Support Network

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)

Response: I am a ten-year renal cancer survivor (non-metastatic disease) and [redacted] of the Kidney Cancer Support Network. We are a purely voluntary group; we do not have employees or paid staff. Our Group was founded in 2006 to offer a cancer buddy scheme to help patients come to terms with a diagnosis of kidney cancer and to advocate for improved access to licensed treatments. We offer our members a safe confidential one-to-one support facility and a UK wide network of kidney cancer patients, carers and family members via our online forum and private social networking pages. Our kidney cancer patient/carer community now has many hundred members who share experiences and insights daily. We provide reliable information and offer emotional support & friendship to each other. We also advocate on behalf of our kidney cancer patient community to ensure kidney cancer patients have access to the best care and clinically effective treatment options the NHS can offer.

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We have discussed this submission for Axitinib at length across our KCSN membership to make sure we properly represent patient and carer views. We know that when N I C E appraises a cancer drug, it consults with many people: professional bodies representing clinicians, nurses, researchers, academics. Also NHS managers & commissioners, GP's and primary care funders, health economists, medicines managers, pharmacists, epidemiologists etc.

Given the unpredictable nature of this disease, it is not possible for a Patient Group like the KCSN to put forward the name of a patient with mRCC ( & eligible for Axitinib) with any certainty that he/she will be able to attend a committee meeting in Manchester in three of four months' time. We feel it is fair in our patient centred NHS, that renal cancer patients (and their primary carers) have the opportunity to talk to you direct. Here, in our submission, you can read in their own words, patients describing how they feel about the N I C E process and the effect a N I C E decision about Axitinib will have on them.

The input into this appraisal will be varied and will come from many different viewpoints and experiences; we suggest that none are as important as the thoughts and experiences of the cancer patients who are diagnosed with this difficult disease and who rely on the care and treatment they receive from the NHS for their very life. Currently patients have specialist centres for surgery and waiting time guidelines to ensure we receive timely high quality surgery from experienced surgeons; resecting metastatic disease is becoming more accepted and highly skilled surgeons carry out partial & laparoscopic nephrectomy as the new norm. New & more effective scanning techniques and regular monitoring can help take away the constant fear of recurrence. But for kidney cancer patients with aggressive high grade disease, or patients diagnosed at an advanced stage, there are far less options available to them than other more common cancers. Radiation therapy and chemotherapy are not effective. Metastatic disease in the lungs, liver or bone may be impossible to treat with anything other than targeted therapies.

And at that point, the life of a kidney cancer patient becomes precarious. Patients with difficult metastatic disease or those patients unlucky enough to be diagnosed with a rarer sub type (of which there are several) must rely on two approved 1st line only, targeted therapies to extend their lives. This despite the fact that there is now a range of clinically effective licensed treatments available. Since the first targeted therapy was approved for mRCC patients in March 2009, the outlook for this group of patients has changed dramatically. Internationally we see patients able to extend their lives by an additional 6/7/8 years, something that a decade ago would have been unheard of. To do this patients rely on sequential and individually chosen treatments, an option some cancer patient with more common cancers expect to have available as a matter of course as part of their care pathway. At the moment, there are no biomarkers available for renal cancer.

Patients find it difficult to understand a process which denies them alternative licensed targeted therapies such as Axitinib which is available at a very similar monthly cost, simply because of intolerability issues or histology. The language and methodology used in the appraisal are impenetrable to most patients. If the NHS considers the cost of a laparoscopic partial nephrectomy valuable and cost effective, why we ask, would the

NHS then refuse access to drugs which would make that investment worthwhile. It is like paying up front for a train ticket to travel from Euston all the way to Edinburgh and then kicking the patient off at Manchester!

This N I C E template (appendix G) for Patient Groups is rather prescriptive and assumes all Patient Groups can fund expert help to interpret clinical trial data & research papers. We receive £400 from N I C E to enable us to take part which does not allow us to consult with epidemiologists or health economists or carry out professional patient surveys. We know N I C E will already have the sought opinions from Clinicians, Directors of Public Health, Research Scientists, commissioners etc. We sincerely hope that the following patient statements will answer the points N I C E has suggested we should observe……

- Advantages of the technology
- Disadvantages of the technology
- Differences in opinion
- Patients who might benefit more or less
- Comparing the technology with alternative available treatments
- Advantages for patients over other current standard practice
- Disadvantages for patients compared with current standard practice
- Research evidence on patient or carer views of the technology
- Any adverse effects that were not apparent in the clinical trials
- Patient or carer views of the condition
- Availability of this technology to patients in the NHS
- Implications for patients/carers if not made available on the NHS?
- Difficulties using the technology?
- Equality
- NICE is committed to promoting equality of opportunity
- Other issues for the Appraisal Committee to consider.

…….. but in our own words.

The Kidney Cancer Support Network has learned from patient experiences with targeted therapies that there are serious implementation and practicality issues with a recommendation that relies on failure of one specific treatment to which a significant number of patients are intolerant. A recommendation that is reliant on the failure or intolerability of a very specific prior treatment i.e. cytokines or sunitinib, is unfair and does not reflect the current clinical situation faced by both patients and clinicians. In practice, patients with rarer subtypes or comorbidities may have been treated at second line with additional targeted therapies made available via funding from the Cancer Drugs Fund (but not approved by N I C E for routine NHS funding). Any mRCC patient treated via the CDF route will not now meet the “eligibility criteria” for treatment with Axitinib.

To the best of our knowledge, there is no medical evidence to support conditional use of Axitinib/Inlyta only to those patients who are unable to tolerate or have a contraindication to cytokines or sunitinib. If there is an evidence base for this restriction we would like to make it available to our patient community.

It seems perverse that patients are required to have a treatment and demonstrate an

unacceptable level of toxicity so that they can progress to a drug which their clinicians feels will be of a benefit to them The current recommendation narrows options for our patients rather than facilitates ‘a basket of choice’ for individual patients in consultation with their oncologists.

For patients, having to prove that they cannot tolerate a particular treatment or that a particular treatment is not effective for their subtype, means that, by virtue of having taken even one tablet, they have “burned through” another line of therapy and may find themselves excluded from further treatments. For a patient to be told they are funded for a treatment with a drug that doesn’t work for them, but they are not funded with a drug that may work & extend their life, seems at best unreasonable and at worst iniquitous.

For rarer cancers such as renal cell carcinoma, the requirement for direct comparison trials is, we fee, unrealistic, especially in a rapidly evolving field. Until there are reliable bio markers available for renal cancer, we need to provide clinicians with an array of treatment options from which they can choose a drug which will be clinically effective for particular patients based on a particular subtype, especially if those drugs are from within a comparable price range i.e. invoiced total monthly cost.

Patients, Clinicians and Researchers know that renal cancer is not a homogenous disease. Many subtypes of this disease exist. What works well for one patient will not work for the patient sitting beside them in the clinic. Limiting the treatment options available to clinicians to treat different subtypes of the same disease is unfair and says very bluntly to the unfortunate patient who has not responded to an approved first-line treatment “You have the wrong type of kidney cancer, the NHS will not provide funding for your particular type of kidney cancer”

**<u>Patient/carer statements “In Our Own Words…”</u>**

[redacted]: I am a 36 year old mum of 3 young school age children & a RCC patient (papillary type 1/clear cell component - histology reports have not been consistant). I was diagnosed nearly 5 years ago. Last year I was totally out of options after previous drug therapies had failed, I was then fortunate enough to gain access to Axitinib which has extended my life so far by over 12 months & is still continuing to do so, with relatively easy to manage side effects.
I also run a support group for RCC patients and carers & I see first-hand what impact having Axitinib as a treatment option can offer other patients, I know patients currently receiving Axitinib who are doing very well & I have also lost dear friends to this disease who had received this treatment prior to & had their lives extended by it, in order to spend valuable time with their loved ones.
Axitinib can enable a patient who is fully aware that they are going to die from their disease, the option of living with it for a period of time & I & my family know how precious that time can be.
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[redacted]: wife of the late [redacted]: - My husband [redacted] and I were living in a beautiful 400 year old cottage in Norfolk which he had lovingly restored. He was getting treatment for kidney cancer from our local hospital. [redacted] had managed 13 cycles of sutent before being told that it was no longer working and they would be looking
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into a 2nd line treatment for him. We did a bit of research ourselves, to see what treatment options were available to him. He was told that the only drug available was Everolimus and this would have to be applied for at the local CDF. Fortunately the drug was approved and [redacted] spent the next 6 months on this drug. But the side effects were horrific. We then found out that he now had active TB. The Hospital gave up on him, said there was no more treatment that he could have and he probably only had a couple of months at best.
Meanwhile talking to other members of the Kidney Cancer Support Network, other people were doing well on a drug called Axitinib after they too had failed on Everolimus, but not in the area that we lived in. We found out that XXXX hospital was using this drug with success and we managed to get an appointment with XXXX. [redacted] was told he would have to apply to his local CDF, but he would stand a good chance. Meanwhile we had to relocate and fast! We put the cottage on the market well below the market value for a quick sale, sold and rented a house near the new Hospital.
Axitinib was approved! [redacted] had been given a lifeline. He managed the drug very well with little side effects, but unfortunately he battled on with the TB and with a weakened immune system it all took its toll and I lost him on 14th September 2013: thanks to Axitinib, I believe that my husband & I had another precious six months together.
Ending on the "what ifs" and "maybe's"... If everyone had access to Axitinib as a 2nd line treatment and it wasn't a "postcode lottery" maybe my husband would still be here today!
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[redacted] : I think it is important that NICE starts to recognize that kidney cancer is not a homogeneous condition which can be successfully treated by a single or a limited number of agents. There are many sub-types of kidney cancer as in Clear Cell, Chromophobe, Papillary type I and II, Collecting Duct Carcinoma and which may or may not be hereditary. Response for patients within each sub-type also varies considerably with each agent, from zero or stable or tumour shrinkage and for a few patients, to be in remission. To not approve Axitinib ( Inlyta) is denying some patients the opportunity of continuing to live.
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[redacted]: These comments relating to Axitinib are those of a husband who has seen his wife battle a nasty and debilitating disease for the past four years. She underwent a nephrectomy of her kidney and adrenal gland in March 2009 and initial scans were clear but the cancer had returned by December that year. Originally she was referred privately to an oncologist who put her onto Sutent as the first line treatment which started in January 2010. That was initially successful but she was taken off after ten months due to side effects severely affecting her quality of life. From then until December 2012 she was treated with four further chemotherapy drugs which either did not work or, like the Sutent, caused major issues in terms of quality of life. At that point she was put onto Axitinib as that was the last licensed treatment available, with the only remaining option being trials. Since then we have seen a constant response to the drug which has, at this time, provided stability in her condition. The biggest factor with this stability has been the reduction in side effects that she has experienced which in turn have added to her well-being.
I would argue that a drug that is effective in treating people but which has a lesser impact in terms of side effects, gives huge benefits in terms of quality of life. That is something that cannot be measured in monetary terms and something that, as a loved one, cannot

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put a price on. When my wife was diagnosed with the secondary kidney cancer we were told by the oncologist that we were lucky as, if it had been six or seven years earlier, then there was no chemotherapy available. We were fortunate because people had taken the choices to make Sutent and subsequent drugs available to those who needed it. Axitinib falls under the same category and people deserve to access to it, not because they happen to be fortunate to have private medical cover, but because it is a treatment that works and should be available to all

[redacted] ([redacted]) : I was diagnosed with kidney cancer in July 2007 and despite a nephrectomy it later spread to my lungs and to various other parts of my body including my brain. In total I have had ten tumours removed by conventional surgery, including three in my brain. A further thirty one tumours in my brain have been treated with Stereotactic Radio Surgery (Gamma Knife) by the excellent team in Sheffield on five occasions. However, I had to find £30,000 from my pension and life savings to pay for two of these. I have been prescribed Sutent (Sunitinib) for the last two and a half years which my Oncologist confirms has stabilised my cancer for the time being with minimal side effects. My Quality of Life is excellent in spite of the fact that I have to cope on my own without a carer or any help and support since the recent death of my wife from a rare form of breast cancer.
However, I am fearful that my long fight for survival will come to nought if NICE decides not to authorise the availability of Axitinib on the NHS, as it is known that Sutent like most cancer drugs has a limited efficacy after a time. It is common knowledge within the cancer fraternity that sequential treatment is the gold standard which we all must strive to attain if we are ever to win this on-going battle for the future generations. So I urge you to authorise this drug as a second line treatment for kidney cancer as I will be unable to pay for this drug without having to remortgage or sell my house. I consider I have paid enough in taxes over my lifetime to the NHS together with the above mentioned £30,000 to justify better treatment the for many thousands of cancer patients like me.
I hope to soon be celebrating my granddaughter’s first birthday and am grateful to NICE for eventually authorising Sutent which has made this possible. We cannot continue to rely on the Cancer Drugs Fund and it’s about time NICE gave cancer patients a little more support without having to fight for drugs which clearly can extend Quality of Life.
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[redacted] : I am my husband’s carer, following a brain tumour (secondary) it was found he had kidney cancer (primary). During this traumatic diagnosis my husband, for the first time in our married life of over 30 years sobbed his heart out saying he did not want to die, he wasn’t ready. He was put on a drug Pazopanib as 1st line of defence with an option of another drug as a 2nd line, this gave us both hope, the first drug shrunk his tumour and as of yet he does not need the 2nd line BUT the important thing is, the knowing it is available is a lifer saver in itself…..
It’s not just about the cost value of drugs – people making the decisions on behalf of any cancer patient should remember……….. “Every human life has a value”
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My name is [redacted]; I live with my wife and two sons and was diagnosed with Clear Cell Renal Cell Carcinoma (G4 pT2) at the age of 40 in 2010. I have never smoked, I drank alcohol rarely (now tee total) and was otherwise fit and well. A 14.5cm tumour was removed surgically along with my right kidney. In early 2013 the cancer was

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found to have metastasised and a further operation was carried out to remove a tumour from my right lung. So far my treatment has not involved taking any drugs.
Whilst the two major surgical procedures I have undergone have taken their toll physically, it is the psychological impact of living with the prospect of further metastases that is most difficult for me, my family, friends and colleagues. I have been told that having had such a large primary tumour and an instance of spread puts me at a high risk of recurrence.
I have continued to work full time as a Wholesale Director working for a magazine marketing and distribution company and apart for a couple of months off to recover from the two surgeries I have continued to work full time and like many cancer patients I continue to make a full contribution to society .
I have become very active with Kidney Cancer support groups since my diagnosis, partly in an effort to better understand my prognosis and to learn from others affected the various treatment options that I may have to consider if the worst was to happen. It is from seeing the incredibly effective impact drugs such as Axitinib is having on some of the people around me where life is extended and crucially quality of life is significantly improved that helps me and those around me cope with the uncertainty ahead. Without access to these drugs my prospects may be severely damaged greatly amplifying my already high levels of stress.
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[redacted] : Please find here my intended email in support of Axitinib. I have Kidney Cancer. In 2011 my kidney and a very large tumour was removed. On review I was told my cancer was very aggressive, but no further treatment at that point was available. I did however enter the Sorce Sorafenib trial. After two years nodules were found and removed.
No further treatment is on offer. So I have a situation where, I will most likely need, later in my prognosis, further assistance, and I would pray that some will be available. I would on this basis implore you to make available Axitinib, and other drugs or treatments that are available at this time.
We are at present on the fulcrum of great advances with this disease and its treatment/cure. The [redacted] name is synonymous with fighting Cancer. Therefore grant the right to use what has been found.
Part of the Hippocratic Oath (Modern version) states: "I will remember that I do not treat a fever chart, a cancerous growth, but a sick human being, whose illness may affect the person's family and economic stability. My responsibility includes these related problems, if I am to care adequately for the sick. "
In short that means we treat humans not illnesses. Please be human. Give us the tools.
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[redacted]: Started Axitinib in January 2013, after failure of Sutent and disease progression in spinal and lung metastases. Still taking it. Dosage: 10 mg per day (5mg twice a day) for the first 9 months, 12 mg per day since end of September (6mg twice a day).
Efficacy: Mixed. 1st CT, after 8 weeks, showed stability in all mets. Subsequent scans have shown stability in spinal met (however, also having monthly infusions of Zometa) but small though steady increases in lung nodules.
Effects: Easy to tolerate to start with, but became increasingly difficult to live with. We are beyond delighted that the spinal tumour is keeping nice and quiet, even if that's down to the combined effects of Axitinib plus Zometa. We also feel that, as mRCC is such an

unpredictable and tricky beast, there cannot be too many drugs to combat it in the armoury and it's well worth having Axitinib amongst what are really quite limited options.

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[redacted]: The decision as to whether Axitinib should be available as a second line drug for Kidney Cancer is, to many, a matter of life and death. Not only life and death but also, sadly, an example of the ever increasing schism between health care available to the rich and the poor.

I discovered I had Kidney cancer 10 years ago and have been fighting against the disease ever since. I've had one Kidney, 2 lung lobes, an adrenal gland removed and the other ablated plus half a rib removed to date. I've won every battle so far. Two years ago we discovered inoperable mets in my both lungs and pancreas and we took the hard decision to start on a course of Sunitinib. Trust me, no one WANTS to take these drugs, it's a matter of need. I have 2 miserable weeks in every cycle but it's worth it for the 4 good weeks and the ever improving scan results. My last CT Scan showed no apparent evidence of disease.

My daughter and her husband are trying for a baby and having Sutent available to me means I will be able to meet my first grandchild. But what happens if Sutent stops working? Should I be written off after all that the NHS has invested in my treatment and which has been so worthwhile? Should I remortgage my house to pay for the drugs that would only be available to me privately? With a 10 year history of cancer, health insurance is certainly no longer an option to me. Patients are in no doubt about the true value of Axitinib

The NHS has a limited budget, that is a clear and unchangeable fact, and why should you make continuing care available to cancer patients? The CDF includes funding for Axitinib at 2nd line. We should be in a position to use CDF data and reassess the true cost of Axitinib to patients and the NHS, based on this new evidence.

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Thank you for the opportunity to take part in this appraisal.

[redacted]

For and On Behalf of the Membership of the Kidney Cancer Support Network

www.kidneycancersupportnetwork.co.uk
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