Statement by [redacted] June 2008

Patient Expert for the Health Technology Appraisal of Bevacizumab, Sorafenib, Sunitinib and Temsirolimus for renal cell carcinoma

Personal Experience

Three members of my family have suffered with renal cell carcinoma. My grandmother died of the condition at the age of 51 years old in 1972 and my mother died of advanced renal cell carcinoma at the age of 59 years old in August 2007. My Great Aunt was diagnosed and treated successfully after early discovery of a small renal tumour in 2007.

My mother [redacted] was refused treatment of Sorafenib, recommended by two oncologists for advanced kidney cancer in early 2007. She was an exceptional and vibrant woman and desperate to fight for her life. I will consider her story in detail in the main statement. In summary, after a nine month battle for treatment we eventually won funding from Denbighshire Local Health Board for a two month trial of the new cancer drug Sorafenib (Nexavar). The fight for treatment took so long that she had just days to live when we finally won the treatment May 2007. However she lived for another 4 months, and died on August 12th 2007.

The [redacted] Fund

The [redacted] Fund is a patient led voluntary organisation with charitable aims, providing unique and unprecedented advocacy and support to cancer patients denied access to new drug therapies, yet to be approved by NICE. I founded the Fund in August 2007 in memory and as a tribute to Mum. It offers a bespoke one to one service to UK patients and their carers, by taking on their individual appeal for treatment and representing them at their Primary Care Trust or Local Health Board Appeal panel. I liaise with leading oncologists, who now refer patients to me who are having difficulty accessing new cancer drugs. The [redacted] Fund also works to highlight nationally lack of treatment access for cancer patients in the UK today.

This statement has been compiled from my personal experience of the disease and my communications with over 300 renal cell carcinoma patients.

The [redacted] Fund has received unrestricted educational grants from Bayer Healthcare, Roche Products Ltd and Pfizer Ltd.

Statement

The past few years have been bittersweet times for renal cell carcinoma patients. For a disease with historically such limited treatment options – finally real hope has been offered in the licensing of new targeted cancer drugs to treat their otherwise untreatable condition, once standard treatment has failed. In an age of new technology UK patients have been aware of the use of these new drugs in the rest of the western world and have been in the unenviable position of waiting for NICE appraisal until they could be made freely available in the UK.

Clinicians are rightly informing patients of these new drugs but going on to explain they cannot access them on the NHS due to financial constraints until NICE appraisal. This has caused utter devastation to families and patients dealing with a terminal diagnosis. Once all standard treatment has failed including nephrectomy and immunotherapy, patients are offered best supportive care – knowing there are effective treatments being used as standard elsewhere that they are unable access.

Patients and families have been using savings, setting up fundraising appeals, re-mortgaging their properties to access these new treatments, clinically recommended by their oncologists.

However last year a sea change occurred and through successful lobbying by patient groups, most significantly website forum members from Kidney Cancer UK and leading renal oncologists, Primary Care Trusts and Strategic Health Authorities realised that patients could not be penalised and denied effective treatment due to the timing of the NICE appraisal. Individual PCT's began agreeing to treatment on a case by case basis, by August 2007 over 70 Primary Care Trusts had agreed to fund some individual renal cell carcinoma patients with these new treatments on the basis that they are the most clinically effective treatments licensed for their condition. However this system leaves rise to the well known "postcode lottery" so regional commissioning bodies in England have been guided by their expert clinicians and now new RCC drug treatments are available immediately to some advanced renal cell carcinoma patients in the North West, North East, Pan Birmingham region, North Trent and parts of the South East.

The cost has always been high on the agenda in all decisions in regard to these treatments; however the fact remains that patients without any treatment options have a right to be given the best chance of disease stability and quality of life that is clinically available to them and equal to other patients in the western world. A patient will never be able to come to terms with a decision based on a complex mathematical equation. Patients are concerned with getting the best clinical treatment in the quickest possible time. They feel they have every right to treatments available to their counterparts in the rest of the world and that most importantly have been recommended by their clinical team.

Renal cell carcinoma is unique in its lack of response to standard chemotherapy and radiotherapy treatment. The treatment options have been extremely limited to date and have offered little hope to those patients with advanced disease. These new drugs are a tremendous breakthrough and offer such hope to patients it is absolutely vital that NICE take this on board during this appraisal. This view is endorsed by The National Cancer Research Institute - the leading hub of expertise on cancer treatments in the UK. In December 2006 the Clinical Studies Group for Renal Cancer state in their Expert Opinion document:

"Existing standard therapies for metastatic renal cell cancer are inadequate. Both sorafenib and sunitinib significantly prolong progression free survival in metastatic renal cell cancer and should now be made routinely available in the management of this disease in the UK."

Patients would ask the panel to take into account the lack of resources spent on RCC patients to date due to the lack of treatment options and would hope that this is taken into consideration during any cost analysis.

Patients also request the panel take into account the "orphan" drug status of these treatments. Orphan drugs are "medicinal products intended for the diagnosis, prevention or treatment of life-threatening or very serious diseases affecting less than five in 10 000 persons in the Community. We feel that decisions must be reached differently when evaluating such treatments for a relatively rare disease.

Case study

In April 2005, at the age of 56 my mother was given an ultrasound scan for back problems, during which a renal mass was discovered. Her mother died of kidney cancer at the age of 51 years old, so she informed the medical team of this, however the mass was contained within the kidney and not treated as urgent. She underwent a radical nephrectomy 64 days later. We had no reason to doubt that this was a fair timescale. After the operation, histology results indicated that my mother had an extremely rare and aggressive sub type of renal cell cancer – during the waiting time period for the operation the tumour had broken through the kidney and spread. When kidney tumours are excised early enough whilst still contained the prognosis is very good. Once spread, kidney cancer has one of the worst prognoses of between 3 and 10 months – tragically Mum was in the latter category.

The treatment options were extremely limited, an immunotherapy drug which subsequently we found out has a less then 7% response rate was the only active treatment offered to her. This was unsuccessful. It was then I began researching other options and contacted specialist renal oncologists and was told of two new cancer drugs that were used as standard treatment in the rest of the western world and had just received European Licence to treat advanced kidney cancer. Our nearest renal oncology expert was at the Christie Hospital Manchester, He assessed the only treatment option viable for my mother were the new targeted therapy drugs Sunitinib or Sorafenib.

My mother's oncologist in Wales stated that he could not put an application in for treatment with Sorafenib to Conwy & Denbighshire NHS Trust as "they would not pay for it". My mother had dealt with her diagnosis and subsequent illness with such courage and the thought that she would not get the chance to fight her disease with this treatment took a huge toll on her emotionally and physically. She withdrew from us and became depressed and was consumed with fear that she would have to wait for the cancer "to kill her" without any active treatment to halt it. By this stage she had endured two major operations, palliative radiotherapy and immunotherapy. She had lost the use of her right arm and her legs and had an open wound on her back where the tumour had surfaced and was infected. Her quality of life was deteriorating rapidly and the need for active treatment was urgent.

Understandably her focus was now accessing the treatment recommended by her oncologist. She arranged for an estate agent to value the house and researched selling her home to fund the treatment. We immediately discounted that option and me and my husband agreed to remortgage our home to raise the funds. The psychological anguish for my mother was as distressing as her disease – as a family we were all consumed with this battle for treatment. My sister and I took unpaid leave from work to help and support her and I worked until 3am most mornings trying to find a way to access the treatment on the NHS. In May 2007 the Local Health Board agreed to a trial period of two months Sorafenib for her. The fight for treatment had taken nine months and she was gravely ill. However, she transformed psychologically and was full of optimism and hope for the future. She was admitted to St Kentigern Hospice and we were informed she would never walk again; never use her arm again and her back wound would never heal and she was days from death. My mother religiously took her Sorafenib and did walk again; did recover the partial use of her arm; the tumour on her back reduced and wound healed and lived with a good quality of life for a further four months. I am convinced if she had received the treatment eight months earlier she may still be here now.

Mum's story is sadly not unique. I have heard the same story from literally hundreds of patients during my subsequent work with The [redacted] Fund. The patients below are just some of the cases I have encountered.

Story 1

[redacted] was diagnosed with renal cell carcinoma in June 2006.
His left kidney removed in August 2006.
Several secondary tumours were discovered in both lungs.
In September 2006 he was prescribed Interferon to self inject 3 times a week.
After 3 weeks he became very ill and could not tolerate the Interferon- he was taken off the drug immediately.
His oncologist applied for funding for Sorafenib in October 2006.
In December he was told that the funding had been refused.
He appealed, and an appeal case was set for January 10th – he won the appeal
The P.C.T. would only fund for 12 weeks and then review following a scan.
His first scan in May 2007 confirmed he had a remarkable response and there were no visible tumours.
The P.C.T. then agreed to continue funding.
His last scan in February 2008 showed no sign of the cancer.
[redacted] is back working full time.
His oncologist has stated that without these drugs he would be dead now.

Story 2

[redacted] was diagnosed with renal cell carcinoma in October 2007.
He underwent an immediate nephrectomy.
An MRI scan after surgery discovered secondary cancer in his liver and spine.
[redacted] oncologist recommended Sunitinib as the most clinically effective treatment
In December the Primary Care trust rejected this application.
The [redacted] Fund submitted an appeal for [redacted] in January 2008 with the full backing of his oncologist.
The panel rejected the appeal and though conceding this was the only available active treatment for his disease, refused to pay for treatment.
[redacted] was offered best supportive care to for symptom control only.
[redacted] died in March 2008 at the age of 52 years old.

Conclusion

Patients request that the panel takes into account these vital points when appraising these new technologies:

- Renal cell carcinoma patients have been to date disadvantaged by suffering from a less common cancer with limited treatment options.
- The disease does not respond to standard chemotherapy and radiotherapy and once metastasised has a poor prognosis.
- The standard immunotherapy treatment has a low response rate and has serious and debilitating side effects.
- These new technologies offer the only real hope of clinical stability, improved quality of life and an extension of life to renal cell carcinoma patients.
- Patients feel strongly that by withholding these new technologies from them, they are effectively sentenced to a premature death.
- Patients believe that they must have equal access to these treatments alongside renal cell carcinoma patients throughout the rest of the western world.
- Patients feel the cost analysis must reflect the fact that they currently have extremely limited treatment options and thus to date their disease has caused little financial burden to the NHS.
- Alongside the obvious negative clinical impact of being denied these treatments, the psychological effects on the patient and the family are devastating.
- Renal cell carcinoma patients with advanced disease have no alternative treatments if these drugs are not approved by the panel. They will be offered palliative care only to help with symptom control and no further active treatment will be offered.

My aim for this submission is to offer the panel an outline of the experience of some UK patients today living with renal cell carcinoma. It is a great privilege to represent their views. I am also grateful to have the opportunity to tell Mum's story in the hope that her incredible battle may contribute to fair and equitable access to these treatments for all renal cell carcinoma patients in the UK.

[redacted]