James Whale Fund for Kidney Cancer

Response to the Appraisal Consultation Document: Axitinib for treating advanced renal cell carcinoma after failure of prior systemic treatment

James Whale Fund for Kidney Cancer wishes to comment on the NICE Appraisal Consultation Document (ACD), which does not support the use of axitinib for treating advanced renal cell carcinoma after failure of prior systemic treatment. The Fund represents the views of patients and families who are affected by kidney cancer.

1. **Clinical Effectiveness**

   The Committee have recommended that the drug axitinib (Inlyta®) should not be considered a good use of NHS resources for advanced renal cell carcinoma patients after failure of prior systemic treatment. This is despite axitinib's effectiveness at prolonging the life of kidney cancer patients compared to sorafenib in the AXIS trial and best supportive care in the simulated treatment comparison (STC).

   The decision by the Committee to not recommend axitinib for advanced renal cell carcinoma patients after failure of prior systemic treatment means that terminally ill kidney cancer patients are again denied access to effective, licensed second-line treatment on the NHS after failing on sunitinib (Sutent®) or cytokines. The Committee has acknowledged that axitinib meets the end-of-life criteria but yet still recommends that axitinib is not a good use of NHS resources.

   As noted in the ACD, the use of cytokines is diminishing with the recent advances in targeted therapies, and is currently only prescribed for about 10% of advanced kidney cancer patients. The majority of patients receive either sunitinib or pazopanib (Votrient®) as first line treatment; however, the axitinib marketing authorisation only allows for patients previously treated with cytokines or sunitinib, which does not reflect current clinical practice.

   The Committee has not taken into consideration the probability that axitinib could one day (in the near future) be used in combination with other cancer drugs to further extend the life expectancy of advanced renal cell carcinoma patients.

2. **Health Economic Assessments**

   We are disappointed that yet again another drug for the treatment of advanced renal cell carcinoma has been declined on the basis of the use of an unsuitable health economic assessment for small patient groups: Incremental Cost Effectiveness Ratio (ICER) per Quality Adjusted Life Year (QALY) is used in assessment of cost effectiveness for all cancer drugs and is based on a

threshold of an ICER per QALY of £30,000, set in 1999 (although recently a threshold of £50,000 has been quoted). These assessments have time and again been shown to be unfair to many rare cancer patient groups, denying patients access to life-prolonging treatments during a difficult time for both themselves and their families.

3. **Sub-optimal Treatments Available on the NHS**

   It has been shown that advanced renal cell carcinoma patients given sequential drug treatment with targeted therapies have the best prognosis for survival. The Committee's recommendation could deny patients this treatment option, which offers hope and comfort to patients and their families trying to come to terms with a terminal illness. The UK's cancer death rate is currently 6% higher than the European average; NICE's decisions are having a profound effect on the way we treat our cancer patients and the quality of health care available to our citizens. It leaves UK renal cell carcinoma patients at a major disadvantage in terms of the availability of state-of-the-art cancer drugs, meaning that these patients are likely to die prematurely compared to the rest of Western Europe and the United States of America.

4. **Patient Benefits**

   The Committee do not seem to have consulted the patient experts to any great extent for the ACD and any evidence of patient benefits has been given little weight in the recommendation compared to the discussion of evidence on costs. We feel that the patient perspective must be included in the Final Appraisal Document (FAD) and given due weight if the Committee wish to present a balanced and rounded appraisal.

5. **Equalities Statement**

   Patients for whom sunitinib or pazopanib are not a therapeutic option because of intolerance or co-morbidities (e.g. congestive heart failure, poor nutritional state, impaired mobility, hypertension) and patients who are unsuitable for immunotherapy (due to e.g. organ impairment, presence of hepatic metastases, and contraindications such as liver dysfunction or brain metastases) are discriminated against and will not have any therapeutic option under the NHS. The equalities statement in the Appraisal Consultation Document is, therefore, untrue since not all patients are affected by the guidance in the same way.

Conclusions

Kidney cancer accounts for approximately 2% of all new cancers in the UK (approximately 9,000 people per year), and the incidence of kidney cancer is increasing. Advanced renal cell carcinoma affects about 4,000 people annually. Renal cell carcinoma is particularly difficult to treat and does not respond well to conventional cancer treatments, such as chemotherapy and radiotherapy. Once renal cell carcinoma spreads, targeted therapies, such as axitinib, are the only hope for these patients. The Committee's recommendation leaves clinicians with the choice of only two drugs (sunitinib and pazopanib) with which to treat terminally ill kidney cancer patients. If first line treatment is not effective or the patient is unable to tolerate it's

side effects, patients are left with three choices; pay for a different drug themselves, appeal for funding through the Cancer Drugs Fund (which continues until March 2014) or Individual Funding Requests (which are invariably rejected by the local funding bodies who follow the lead of NICE), or palliative care while they wait to die. Appeals for funding can take anything up to 6 months to complete, during which time patients are receiving no active treatment, their cancer is progressing and their quality of life deteriorating.

In the light of the issues raised above the James Whale Fund for Kidney Cancer is of the view that the Committee's recommendation in relation to the patient who has no therapeutic option is a breach of Human Rights (Article 2-the right to life).