The Royal College of Pathologists
Pathology: the science behind the cure

Comments on the Appraisal Consultation Document (ACD) produced for the NICE single technology appraisal of l*enalidomide for the treatment of myelodysplastic syndromes associated with a deletion 5q cytogenic abnormality in people with red blood cell transfusion dependence [ID480]*

1. The decision of the Committee might legitimately be perceived to be discriminatory to individuals who choose not to receive blood products on religious grounds, notably Jehovah's Witnesses, which as far as I can see have not been considered as a patient group in this consultation. For such individuals with 5q- syndrome who are refractory to erythropoietin, the decision to refuse blood products leaves them with little alternative therapeutic options. The use of Lenalidomide in this situation would be justifiable both medically and morally and, if so, then withholding Lenalidomide on economic grounds from other patient groups could potentially be considered discriminatory in a court of law.

2. There is an apparent inconsistency of recommendation here between the use of Lenalidomide in 5q- syndrome and in multiple myeloma that is not clearly explained. These are both malignant haematological conditions for which Lenalidomide is a highly effective therapy and yet the few patients with 5q- syndrome are clearly being treated differently to the many patients with myeloma for whom Lenalidomide is offered in the United Kingdom as third line treatment. On face value this appears to be discriminatory by valuing patients with one form of cancer above another despite good evidence to show similar levels of efficacy and improvements in quality of life for both conditions treated with Lenalidomide. This is particularly unfair given the orphan disease status of 5q- syndrome, which is not clearly apparent in the Committees considerations.

3. The Scottish Medical Consortium has recently approved an identical Patient Access Scheme that now operates in Scotland. This decision clearly challenges the conclusion of NICE not to grant funding to Lenalidomide in this clinical setting and creates a potentially serious problem of regional geographic inequalities within the NHS.

4. The Committee question the estimate of patients continuing for more than 26 months on Lenalidomide at 27% at which point the funding of the treatment by the NHS becomes cost effective. In my professional opinion, the company Celgene has provided extremely clear evidence that this is a very reasonable (and in some regard conservative) estimate that has been arrived at using published real world data from a Spanish study. Moreover, the Scottish Medical Consortium has accepted the advice of its own consultants that this estimate is indeed representative of the clinical practice population.

5. The Committee appear to question the link between transfusion independence and overall survival in 5q- syndrome which is clearly well established and provides a good argument in favour of Lenalidomide cost effectiveness even without consideration of the enormous improvements in quality of life that are also seen in responsive patients.

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6. The current access to Lenalidomide in 5q-syndrome via the National Cancer Drugs Fund complicates and largely obviates the current NICE Appraisal Consultation Document. In light of this, a cost-sharing Patient Access Scheme, as proposed by the marketing company Celgene, appears to be a pragmatic way forward in terms of making this treatment available to eligible patients within its current marketing authorisation and along similarly lines to Lenalidomide funding in relapsed multiple myeloma. The Committee has also disregarded the fact that, according to the company's own TCS database, a significant number of patients in the UK are currently receiving Lenalidomide, some up to as many as 70 cycles of treatment, that is presently being funded by the NHS but who would have this cost covered by Celgene were the proposed Patient Access Scheme to be adopted.

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Representing the Royal College of Pathologists and the British Committee for Standards in Haematology