Comments on the ACD Received from the Public through the NICE Website

Name	[redacted]
Role	NHS Professional
Other role	[redacted]
Location	Wales
Conflict	no
Notes	Also representing [redacted] ([redacted], [redacted] [redacted] [redacted] [redacted] [redacted]: [redacted] [redacted] is [redacted] [redacted] [redacted] [redacted] has provided consultancy to the manufacturer
Comments on individual sections of the ACD:	
Section 1 (Appraisal Committee's preliminary recommendations)	As [redacted] specialising in myelodysplasia and other myeloid malignancies and as representatives of the [redacted], a charity representing expert opinion and funding education and research among scientists and physicians with an interest in myelodysplasia, we wish to express our concern at the recent NICE ruling on lenalidomide. We are of the opinion that the published data from the MDS-004 and other studies strongly supports the use of lenalidomide as first line treatment in patients with transfusion dependent myelodysplasia associated with the isolated 5q deletion. Â More than two-thirds of patients will have a significant, meaningful improvement in transfusion requirements with an unprecedented improvement in quality of life. Â Patients are protected from adverse complications of blood transfusion, including infection, alloimmunisation and iron loading, and so avoid costs associated with these conditions.
Section 2 (The technology)	Primarily we are concerned that transfusion dependence and iron loading are both associated with a shortened survival outcome in patents with myelodysplasia and that this is alleviated by using lenalidomide. Â Patients responding to lenalidomide can have excess iron removed through venesection which is much cheaper and much more effective than subcutaneous or oral iron chelation therapy. Â When started sufficiently early in the patient?s journey, significant iron loading may be avoided completely. Concern has been raised regarding the number of patients reaching 26 weeks therapy. Â It is my view that sufficient numbers reach this end point as to merit an interim ruling by NICE that allows patients to be treated in a national surveillance database with a review of these data after a five year follow up, after which a further ruling could be made as to cost effectiveness. We would urge NICE to reconsider the recent interim ruling and allow patients with this malignant and crippling orphan condition access to a life changing and disease altering medication which

	has been shown, in randomised studies, to impact favourable on transfusion need, quality of life, and survival outcomes.
Section 3 (The manufacturer's submission)	
Section 4 (Consideration of the evidence)	
Section 5 (Implementation)	
Section 6 (Proposed recommendations for further research)	
Section 7 (Related NICE guidance)	With careful national surveillance, review could be undertaken between three and five years after commencement of drug availability to ascertain number of patients remaining on drug.
Section 8 (Proposed date of review of guidance)	
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