Final Appraisal Determination: Azacitidine for the treatment of myelodysplastic syndromes, chronic myelomonocytic leukaemia and acute myeloid leukaemia

Thank you for your letter of 14 April. I have considered it carefully.

I think we may be at cross purposes. I had appreciated that your point was not that there was a procedural mistake in applying the End of Life criteria. I understand that your point is that because the extension to life is significantly over the threshold, and significantly more than some other drugs which have benefitted under the policy, it was wrong not to recommend this treatment.

However, the effect of the policy is to allow the committee to take a more generous view of what is an acceptable ICER than would otherwise be the case. How much more generous would depend on the individual treatment. And my concern is that the effect of a longer than usual life extension will already be included in the ICER (the drug is more cost effective, with a life extension of 9.5 months, than it would be with, say, 3 months). It would therefore positively be double counting if the effect of a life extension of 9.5 months not only brought the ICER down from whatever it would have been to the
actual value given, but then also required/justified the committee in recommending a higher ICER than might otherwise be the case under the End of Life policy.

I have also considered your comments on ultra orphan drugs. It remains my view that, whatever the broader picture may be, the appeal panel can only ask itself whether the committee fairly appraised the drug referred to it in accordance with its published procedures. Whether the drug should have been referred, or whether the procedures should be different, are not matters on which the committee can have a view and so not matters which it can be challenged about on appeal.

You ask that the appeal panel should consider the ultra orphan status of the drug when it considered your perversity arguments, and I feel sure it will have all the relevant features of the treatment in mind.

Conclusion

My view remains as set out in my initial letter and the institute will now make arrangements for the appeal hearing.

Yours sincerely

Appeals Committee Chair
National Institute for Health and Clinical Excellence