Bortezomib and Thalidomide for the first-line treatment of multiple myeloma
Appraisal Consultation Document

Response from Myeloma UK

Myeloma UK is pleased to submit this response to the Appraisal Committee's provisional recommendations on the above technologies.

Myeloma UK welcomes the provisional recommendations and considers them to be a sound and suitable basis for guidance to the NHS and of significant benefit to patients.

We have made one suggested change to paragraph 1.2 – details of this change you will find under Q2 below.

Q1. Has all of the relevant evidence been taken into account?

Myeloma UK is not aware of any evidence that has been missed which would have had an impact on the draft recommendations.

Q2. Are the summaries of clinical and cost effectiveness reasonable interpretations of the evidence?

We consider that the Appraisal Committee has reached a fair and reasonable interpretation on the evidence in its summaries of clinical and cost effectiveness.

We are satisfied that the Appraisal Committee has made valid assumptions in its modeling and produced an appropriate economic analysis.

Myeloma UK accepts the ERG's conclusion that the two thalidomide-containing regimens (MPT and CTDa) are clinically and cost effective compared with melphalan and prednisolone (MP) alone. We also accept the conclusion that the bortezomib-containing regimen (VMP) is clinically and cost effective compared with MP.

We accept that on the basis of the data reviewed that VMP appears less cost effective than MPT when both are compared with MP.

We also accept the analysis showing that when directly compared with MPT, VMP is not cost effective; however, we consider the VMP vs. MPT comparison may be unnecessary in the context of this guidance.

Myeloma UK considers that the provisional recommendations make a sound and suitable basis for guidance to the NHS, however we suggest a small change to the wording in paragraph 1.2 from its current form:

Bortezomib in combination with an alkylating agent and a corticosteroid is recommended as an option for first-line treatment of multiple myeloma in people for whom:

- *high-dose chemotherapy with stem cell transplantation is considered inappropriate* ***and***
- *the person is unable to tolerate or has contraindications to thalidomide*

to read:

Bortezomib in combination with an alkylating agent and a corticosteroid is recommended as an option for first-line treatment of multiple myeloma in people for whom:

- *high-dose chemotherapy with stem cell transplantation is considered inappropriate* ***and***
- *a thalidomide-containing regimen is considered inappropriate*

We believe that this would better reflect the Appraisal Committee's findings that (a) both thalidomide and bortezomib-containing regimens are clinically and cost effective; (b) thalidomide offers a more cost-effective option compared to melphalan and prednisolone than the bortezomib regimen offers; and (c) thalidomide is the preferred choice of clinicians for this patient group, unless considered clinically inappropriate.

We believe that the Appraisal Committee's intention is to reflect their findings that while thalidomide is generally the preferred treatment, various factors may mean it is inappropriate for individual patients, and bortezomib would offer a preferable alternative.

We are concerned that the current wording in paragraph 1.2. that "*the person is unable to tolerate or has contraindications to thalidomide*" does not accurately or fully reflect the possible reasons why thalidomide may not be considered to be appropriate.

In 4.3.2 the Committee accepted that *"the choice of treatment for an individual patient will depend on the co-morbidities present and the different mechanisms of action and side effect profiles of the treatments".* However, there may also be other important patient factors which would influence choice of treatment, such as ability to comply with taking tablets or other issues which occasionally arise.

Referring uniquely to intolerance and to the stated contraindications for thalidomide may place an unhelpful limit on clinicians' flexibility to use their judgment about what is appropriate, and could potentially cause difficulties in prescribing locally.

Myeloma UK believes a change to the wording as per our suggestion above would avoid any potential difficulties and better reflect the findings of the Appraisal Committee.