Myeloma UK response to appraisal consultation document on bortezomib for induction therapy in myeloma before high-dose therapy and autologous stem cell transplantation

Introduction

Myeloma UK welcomes the opportunity to comment on the NICE appraisal consultation document (ACD) covering bortezomib as an induction therapy for myeloma patients prior to high-dose therapy and autologous stem cell transplantation.

We appreciate that this appraisal is not straight forward but nonetheless are disappointed by the draft decision. Our focus now is to work with stakeholders to find a way forward that will mitigate the concerns and uncertainties and to enable access to this important treatment for the appropriate group of patients eligible for high-dose therapy and stem cell transplantation.

As myeloma is a heterogeneous cancer we are very supportive of opportunities that enable appropriate wider access to new treatments for patients and prescribing flexibility for clinicians.

Myeloma UK has a good working relationship with NICE and we have absolute confidence in its appraisal methodology and processes. However, having read through the Velcade ACD, we are disappointed with the conclusions the Appraisal Committee has reached. As it currently stands, if the NICE guidance came into affect it would preclude any newly diagnosed myeloma patient from accessing Velcade on the NHS, which is unacceptable and would represent an abject failure on behalf of all the stakeholders involved.

We therefore offer the following comments to assist NICE to take forward the appraisal and to hopefully overturn the 'minded' no NICE reached after the first Appraisal Committee meeting.

The clinical case and evidence for Velcade induction

Myeloma UK understands that the comparative data between cyclophosphamide, thalidomide and dexamethasone (CTD) and Velcade and dexamethasone (with or without thalidomide) is limited, particularly as the clinical trials conducted using Velcade induction were not designed for a UK setting or with a robust HTA appraisal in mind.

However, as there is compelling clinical evidence to show that Velcade is a good therapeutic option for patients in the induction setting and that it improves progression free and overall survival, we firmly believe it is the responsibility of NICE and other stakeholders involved in the NICE appraisal to find a way to ensure that Velcade induction is made available on the NHS as a treatment option for myeloma patients who need it.

We need to work together to make the best use of the clinical evidence available and to find a rationale and a form of words to approve Velcade for an appropriate group of patients who are eligible for high-dose therapy and stem cell transplantation.

Further subgroup analysis

One opportunity that may be worth exploring in more depth is whether or not there are subgroups of myeloma patients that would be likely to benefit more than others if Velcade was approved for use as an induction therapy.

Evidence suggests that patients who attain a complete response from their initial treatment have better long term outcomes[1]. This highlights the urgent need for clinicians and their patients to have access the best

[1] Harousseau et al (2009), Blood Vol 114 no 15 3139-3146

possible treatments at diagnosis and have the ability to choose between available treatments to pick the one that is best for any given patient depending on the clinical situation at hand.

For example, as in the NICE MTA guidance covering thalidomide and Velcade in newly diagnosed patients who are not candidates for intensive therapy, it was deemed important for patients who are contraindicated or intolerant to thalidomide to be able to access an alternative but similarly effective treatment. In addition, as we highlighted in our initial response to the appraisal there is also evidence to suggest that bortezomib is better and can improve outcomes in myeloma patients with kidney impairment.

Appraisal Committee meeting

Since 2006 and the first NICE appraisal of Velcade monotherapy, Myeloma UK has been involved in a wide range of NICE appraisals of novel myeloma treatments and have always felt that they are held in a very professional manner.

However, we were very disappointed with how the NICE Appraisal Committee was conducted on 17 October and do not think that it reached the high standards that we have come to expect.

Prior to the Committee's discussion of Velcade induction there was a lengthy and difficult appraisal that had already taken place and proceedings were running over an hour and half later than anticipated. Understandably, when we were called into the meeting the members of the Appraisal Committee appeared not in the right frame of mind to conduct another complex and lengthy appraisal.

We felt that on arrival the topic was rushed through, the details and discussion were not of the depth and quality that we have experienced at previous appraisal meetings and we do not think that we were given the appropriate amount of time to discuss the complex issues associated with the data on bortezomib induction. It is difficult to see how the NICE committee could have reached any other decision, apart from a negative one, given the circumstances and frame of mind the Committee was in during the proceedings.

We therefore very much look forward to the further opportunity to discuss the Velcade induction appraisal and the associated uncertainty at the next Committee meeting.

Conclusions

As outlined above, Myeloma UK is disappointed with the negative recommendations made by NICE in the ACD on bortezomib as an induction therapy. However, we are confident that with the additional data supplied by the pharmaceutical company, further collaboration and with further consideration of how we can overcome the uncertainty associated with the data, we can find a way of making bortezomib available to myeloma patients in the UK to patients who need it.

Please contact [redacted] or call [redacted] if we can provide any further information to support this appraisal.