

## **PTAC Teleconference meeting held 10 March 2011**

### **(minutes for web publishing)**

PTAC minutes are published in accordance with the *Terms of Reference for the Pharmacology and Therapeutics Advisory Committee (PTAC) and PTAC Subcommittees 2008*.

Note that this document is not necessarily a complete record of the PTAC meeting; only the relevant portions of the minutes relating to PTAC discussions about an Application or PHARMAC staff proposal that contain a recommendation are published.

PTAC may:

- (a) recommend that a pharmaceutical be listed by PHARMAC on the Pharmaceutical Schedule and the priority it gives to such a listing;
- (b) defer a final recommendation, and give reasons for the deferral (such as the supply of further information) and what is required before further review; or
- (c) recommend that PHARMAC decline to list a pharmaceutical on the Pharmaceutical Schedule.

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# **1 Widening of funded access to thalidomide and funding bortezomib for patients with multiple myeloma and AL amyloidosis**

- 1.1 The Committee considered a paper from PHARMAC staff regarding a funding proposal to widen funded access to thalidomide and to fund bortezomib that had arisen through commercial agreements with Celgene and Janssen-Cilag.
- 1.2 The Committee noted that under this proposal, bortezomib and thalidomide would be funded for any multiple myeloma (MM) and systemic AL amyloidosis patients in the first and second line settings. Members noted that under this proposal patients would be able to access funded thalidomide repeatedly but bortezomib would only be funded once i.e. as first or second line treatment.
- 1.3 Members noted that the proposal included funding that was wider than the populations considered by PTAC and/or CaTSoP to date, and for one population PTAC had recommend funding be declined. Specifically, the Committee noted that in 2009 it recommended that the funding of thalidomide for the first line treatment of transplant eligible patients be declined, and it had not previously considered the funding of bortezomib for the first line treatment of transplant eligible patients. The Committee noted it had also not considered the funding of thalidomide for patients with systemic AL amyloidosis.
- 1.4 The Committee noted that the proposal was considered by the PHARMAC Board at its 25 February 2011 meeting where the Board deferred making a decision pending further advice from PTAC regarding the safety of bortezomib and thalidomide in these wider populations.
- 1.5 The Committee considered evidence from a number of studies examining the use of stem cell transplant induction regimens containing thalidomide or bortezomib provided by PHARMAC staff.

## *Thalidomide*

- 1.6 The Committee considered that its 2009 decline recommendation for this population was principally driven by its inability to determine the benefit of thalidomide alone on longer term outcomes, such as overall survival, due to data being confounded by patients receiving a transplant and subsequent (uncontrolled) treatments. Members considered that because of these confounding factors, in the transplant induction setting it may be appropriate to focus on short term outcomes such as response rate or transplant success, rather than overall survival. Members noted that although the evidence for thalidomide in this setting was limited to mainly single arm cohort studies, it generally demonstrated that thalidomide containing induction regimens significantly (improved response rates.
- 1.7 The Committee noted that thalidomide treatment was associated with significant toxicity concerns; in particular patients were at increased risk for venous thromboembolism

- 1.8 The Committee considered that there were no specific safety concerns regarding the use of thalidomide as a first line induction treatment in transplant eligible multiple myeloma patients compared with other populations where it had previously recommended funding (i.e. first line transplant ineligible) or where it was currently funded (i.e. relapsed refractory).
- 1.9 The Committee considered that there was no reason not to fund thalidomide as a first line induction treatment in transplant eligible multiple myeloma patients as proposed.

#### *Bortezomib*

- 1.10 The Committee noted that evidence for the use of bortezomib as a transplant induction treatment in transplant eligible multiple myeloma patients was limited but it generally demonstrated that bortezomib containing induction regimens significantly improved response rates. Members noted that, like thalidomide, bortezomib was associated with significant toxicity concerns; in particular patients were at increased risk of peripheral neuropathy.
- 1.11 The Committee considered that there were no specific safety concerns regarding the use of bortezomib as a first line induction treatment in transplant eligible multiple myeloma patients compared with other populations where it had previously recommended funding (i.e. first line transplant ineligible and second line relapsed/refractory).
- 1.12 The Committee considered that there was no reason not to fund bortezomib as a first line induction treatment in transplant eligible multiple myeloma patients as proposed. The Committee considered that the proposed Special Authority criteria were appropriate.
- 1.13 The Committee noted that several haematologists, in response to consultation, had requested funding for bortezomib be extended to 3<sup>rd</sup> line (or beyond) relapsed/refractory patients. Members noted that the proposed Special Authority criteria did not include funding for this patient group. Members considered that whilst they acknowledged the desire of clinicians to have bortezomib funded for this patient group, there was currently insufficient evidence of benefit for it to support funding in this population. Members noted that funding these patients would add approximately \$2 million cost to the proposal over the first two years without any clear evidence of benefit. Members considered it highly likely that in this setting, bortezomib would be far less cost-effective than funding it in either the first or second line settings. The Committee considered it was appropriate not to fund this group at this time; however, it would welcome a third line funding application for consideration in the future..
- 1.14 The Committee also noted that one haematologist, in response to consultation and in discussion with PHARMAC staff, had requested that bortezomib re-treatment be funded (i.e. funding relapsed/refractory treatment following prior funding in the first line setting). Members noted that the proposed Special Authority criteria did not include funding for bortezomib re-treatment. The Committee considered that there was no evidence to support the use of bortezomib re-treatment as far as it was aware; therefore, it was appropriate not to fund re-treatment at this time. However, the Committee would welcome a funding application for consideration in the future.

### *Systemic AL Amyloidosis*

- 1.15 The Committee considered that because of the similarities between systemic AL amyloidosis and multiple myeloma it was reasonable that the proposed funding included both systemic AL amyloidosis and multiple myeloma patients.

### *Impact on Transplant service*

- 1.16 The Committee noted that stem cell transplantation was a resource intensive and costly procedure and considered that the proposal may increase the total number of patients receiving a transplant in New Zealand each year. However, members considered that DHB capacity and funding constraints may limit the number of transplants undertaken and although thalidomide and bortezomib funding would be available it would be at the discretion of the treating clinician and the DHB as to whether a transplant was appropriate and undertaken for an individual patient. Members considered that it may be interesting to review the number of patients undergoing transplantation to see if there is any increase if a decision to fund thalidomide and bortezomib for these patients is implemented. However, members noted that other factors also influence transplant uptake and therefore it would be difficult to attribute any changes solely to the funding of these treatments.