Is PHARMAC’s decision-making fair, cost-effective and clinically effective? Observations from the real world

Mark J Bolland, Andrew Grey

Tumility and colleagues assessed the decision making by the Pharmaceutical Management Agency (PHARMAC). Their analysis was based upon a review of PHARMAC procedural documents and interviews with PHARMAC staff. The senior author is a member of PHARMAC’s Pharmacology and Therapeutics Advisory Committee (PTAC). In seven domains, that included clinical effectiveness, cost-effectiveness and fairness, they scored PHARMAC with almost full marks (119/125) including 5/5 for these three specific domains, concluding that PHARMAC’s decision-making framework is both fair and legitimate.

Our views as practising endocrinologists differ. While the concept of PHARMAC is admirable, in practice it generates complaints from a range of people affected by its decisions. MB has applied to PHARMAC to change funding criteria for the intravenous bisphosphonate zoledronate. AG served on the PTAC Endocrinology Advisory Sub-committee from 2016–2021, during which time he advocated for improvements in PHARMAC processes for collating, considering and applying clinical advice.

PHARMAC has a process for assessing applications for funding of medicines. However, it appears to apply a “one size fits all” to applications, irrespective of their nature, such that evaluations of minor, common-sense adjustments to existing therapies attract lengthy and inefficient processes. A consequence is that other clinically supported medication changes are delayed. We identified other aspects of PHARMAC’s interactions with clinicians, and its clinical advisors, that led us to conclude that the outcomes of PHARMAC’s decision-making is not clinically efficient, cost-effective or fair.

A) Funding criteria for intravenous bisphosphonates

For many years, PHARMAC has funded pamidronate without restriction, whereas zoledronate has been funded under special authority restriction for patients with bone metastases, early breast cancer, osteoporosis or Paget’s disease. Initially, that may have been reasonable since pamidronate was an older medication available as a generic preparation and there were marked cost differences even though the newer agent, zoledronate, is more potent, longer lasting, and more effective than pamidronate. However, generic zoledronate has now been available for several years and currently is considerably cheaper than pamidronate ($18 vs $75–80). Generic zoledronate has superseded pamidronate.

PHARMAC’s decision to fund a more expensive, less effective intravenous bisphosphonate without restriction, while limiting the use of the more effective, cheaper agent has real consequences for patients and clinicians. Intravenous bisphosphonates are a first line treatment for patients with serious hypercalcaemia. Because of the current funding criteria, such patients were treated with pamidronate when they should receive zoledronate. The fix was simple: fund zoledronate for hypercalcaemia, and to discontinue funding of pamidronate.

We brought this discrepancy to PHARMAC’s attention in May 2020. Their response was to require an application for funding of zoledronate for hypercalcaemia. This was submitted in June 2020. A year later, in May 2021, PTAC reviewed the application and gave funding zoledronate for hypercalcaemia a high priority. But then nothing happened. In December 2021, we sought an update. In February 2022, we received the update stating that consultation would be held soon. Zoledronate funding for hypercalcaemia eventually started on 1 April 2022.

Essentially, PHARMAC funded a more expensive, less effective medication for many years, and for almost two years since it was apprised of the discrepancy. What should have been a simple switch, implemented immediately if common sense applied, instead required a clinician application, a detailed assessment by PTAC, a consultation process, and then a decision. Meanwhile
patients received inferior care.

A similar situation applies to the use of zoledronate for osteoporosis treatment. Currently, 5mg zoledronate costs $60 under special authority restriction. Table 1 shows the medication cost of a standard five-year treatment course of funded osteoporosis medications. The cheapest option is 4mg zoledronate ($11/year), but even 5mg zoledronate (only available with restrictions) is cheaper than risedronate and similar to alendronate, both available without restriction. These are only medication costs: intravenous zoledronate also requires three prescription charges, three infusion charges, and likely three doctor visits and has the advantage of greater compliance. Oral bisphosphonates require 20 prescription charges and 20 doctor visits. How those costs and benefits balance out would depend on what assumptions are made, but we think they are likely to favour zoledronate.

Data from the Ministry of Health Pharmaceutical data web tool show that about 50,000 people per year received a prescription for osteoporosis medications between 2016 and 2020. If all those people had been treated with 4mg zoledronate, medication costs would have been about 60% lower, an absolute saving of about $800,000–$1 million/year.

We first brought this to the attention of PHARMAC in June 2020. The response was that PHARMAC had not appreciated that 5mg zoledronate was cheaper than some of the funded alternatives and 4mg zoledronate cheaper than all of them. The Chief Executive said that PHARMAC would reconsider the special authority. The issue was considered by the PTAC endocrinology subcommittee in March 2021 who were strongly supportive, but nothing eventuated.

In response to a formal update request in January 2022, PHARMAC responded that removing the zoledronate special authority may not be cost-saving or cost-neutral because of “the expected increase in the size of the osteoporosis market”, and that the special authority would not be removed. We pointed out that use of 4mg zoledronate is about 60% cheaper than alternatives and that prescribing data show the “osteoporosis market” is static not increasing. PHARMAC have not responded, and we have not seen any public justification of the decision.

Once again, PHARMAC are funding more expensive treatment options, while applying restrictions to cheaper, arguably more effective options. PHARMAC have been aware of this for nearly two years, but nothing seems likely to change in the near future.

B) Other endocrinology therapeutics and processes for collating and considering clinical advice

The examples of the tortuous processes for intravenous bisphosphonates are not isolated: others are shown in Box 1. Notably, none of these medications are new. Each has been recommended by the Endocrinology PTAC Sub-committee as clinically useful and important, sometimes on more than one occasion. Each was initiated by members of the Endocrinology PTAC Advisory Committee. None have yet reached clinical practice.

PHARMAC advertises that its “decision-making is based on strong, objective clinical advice. The main way that we seek clinical advice is through our clinical advisory committees: PTAC and the sub-committees of PTAC”.1 If that is the case, why have simple recommendations from the Endocrinology PTAC Sub-committee not been actioned?

One possibility is that the committee hardly ever meets. During AG’s five-year tenure, the Endocrinology Committee met only four times. The first meeting took place in June 2016, but committee recommendations to fund cinacalcet (see Box 1), delist an unnecessary medication (alendronate and colecalciferol) and configure workable special authority criteria for the osteoporosis treatment denosumab were not actioned. In May 2018, PHARMAC asked the Committee to discuss a minor aspect of a specific therapeutic (denosumab), but the Committee’s concerns about the more important issue of unworkable special authority criteria were again disregarded. The last two meetings occurred in November 2020, to discuss disquiet among members at the lack of engagement by PHARMAC, and in March 2021, after repeated requests for a meeting by some Committee members.

Second, the endocrinology committee has not had a role in agenda setting, which has remained the sole provenance of PHARMAC. Thereby, PHARMAC collates advice on only those medication issues it deems important. How those medication issues are determined is not clear.

Third, communications between PHARMAC or PTAC and the Endocrinology Committee were unbalanced and erratic. Committee recommendations were discussed at PTAC meetings without a Committee member with relevant clinical expertise present, contributing to decisions not supported by the Committee or by clinicians. For example,
the special authority criteria for denosumab were applied by PHARMAC against the strong recommendations of the Endocrinology Committee and were met with bewilderment by clinicians. A new set of recommendations, endorsed by the Endocrinology Committee a year ago, has not been implemented. Decisions were communicated erratically, if at all, to the Committee, without a right of reply.

Minutes of the November 2020 meeting, at which dissatisfaction was expressed by Endocrinology Committee members about the existing PHARMAC processes, and the improvements suggested, were not made publicly available and discussion of those concerns was removed from the publicly available minutes of the March 2021 meeting after the chair had signed off the agreed record. At that meeting, the Endocrinology Committee's attempts to address its concerns were likened by PHARMAC staff to the actions of a “lobby group”.

At the November 2020 meeting, the Committee proposed some improvements to the PHARMAC processes:
1. The advisory committee meet annually
2. The agenda be set by PHARMAC AND the Committee
3. A list of Committee advice/recommendations to be produced after each meeting and reported on the PHARMAC website
4. A response provided to the Committee about each recommendation by PHARMAC/PTAC in a timely fashion
5. A facility for the Committee to respond to the PHARMAC/PTAC decisions in the event it disagrees with them
6. All recommendations and responses to be publicly available on the PHARMAC website.

As of June 2022, it was not apparent that these requests would be actioned by PHARMAC.

Collectively, these experiences belie the claims that PHARMAC highly values its clinical advisors and that its processes are “fair” (to whom?), cost-effective or clinically effective, suggesting instead that it pays lip service to clinical expertise. Perhaps, it is the chasm between PHARMAC’s decision-making and its impact on affected patients and clinicians that contributes to the discontent about PHARMAC’s performance?
Table 1: medication costs for a treatment course of osteoporosis.

<table>
<thead>
<tr>
<th>Medication</th>
<th>Cost/infusion or tablet</th>
<th>Usage</th>
<th>Total cost</th>
<th>Cost/Year</th>
</tr>
</thead>
<tbody>
<tr>
<td>zoledronate 5mg</td>
<td>$60</td>
<td>3 infusions over 5 years</td>
<td>$180</td>
<td>$36</td>
</tr>
<tr>
<td>zoledronate 4mg</td>
<td>$18</td>
<td>3 infusions over 5 years</td>
<td>$54</td>
<td>$11</td>
</tr>
<tr>
<td>alendronate 70mg</td>
<td>$0.61</td>
<td>weekly for 5 years</td>
<td>$159</td>
<td>$32</td>
</tr>
<tr>
<td>alendronate 70mg/vitamin D</td>
<td>$0.38</td>
<td>weekly for 5 years</td>
<td>$98</td>
<td>$20</td>
</tr>
<tr>
<td>risedronate 35mg</td>
<td>$0.78</td>
<td>weekly for 5 years</td>
<td>$202</td>
<td>$40</td>
</tr>
</tbody>
</table>

Box 1: examples of clinical recommendations supported by the Endocrinology PTAC Sub-committee not actioned by PHARMAC.

<table>
<thead>
<tr>
<th>Medication and recommended indication</th>
<th>Rationale</th>
</tr>
</thead>
<tbody>
<tr>
<td>Eplerenone for men with primary hyperaldosteronism who are intolerant of spironolactone³</td>
<td>No effective alternative</td>
</tr>
<tr>
<td>Cinacalcet for patients with severe primary hyperparathyroidism for whom surgery is contraindicated⁴</td>
<td>No effective alternative</td>
</tr>
<tr>
<td>Reconfigured special authority criteria for denosumab⁵ and teriparatide⁶</td>
<td>Need for second-line therapy for contraindications to, or intolerance of, bisphosphonates</td>
</tr>
<tr>
<td>Micronised progesterone for post-menopausal women⁷</td>
<td>Improved safety and tolerability</td>
</tr>
<tr>
<td>Octreotide for TSH-secreting macroadenoma⁸</td>
<td>Efficacy in tumour control</td>
</tr>
<tr>
<td>Transdermal testosterone (gels) for male hypogonadism⁹</td>
<td>Need for effective and well-tolerated non-parenteral treatment</td>
</tr>
</tbody>
</table>
COMPETING INTERESTS
None of the authors have any financial conflicts of interest. AG was a member of the PHARMAC PTAC Endocrinology Sub-committee from 2016-2021. Both authors are practising Endocrinologists.

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REFERENCES