FACTORS CONSIDERED IN PHARMACEUTICAL REIMBURSEMENT IN AUSTRALIA: A ROLE FOR THE RULE OF RESCUE?

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ABSTRACT

Objective: Since 1993, applications for public reimbursement in Australia of new drugs, or new indications for existing drugs, must contain an economic evaluation. The Pharmaceutical Benefits Advisory Committee (PBAC) makes recommendations on the public reimbursement of drugs largely based on its assessment of their cost-effectiveness. However, in their deliberations the PBAC may consider other relevant factors such as the rule of rescue. The role of cost minimising can be described as the impetus to act to prolong survival in a person (group) who faces imminent death. We sought to investigate and then describe how and when this rule has been applied.

Methods: Information on positive recommendations from the PBAC has been available publicly since December 1999. The number of positive recommendations related to major submissions (those containing economic evaluations) since December 1999 was recorded. Reasons for those recommendations were classified as: cost-minimising; cost-effective; rule of rescue.

Results: The PBAC has only recently stated the criteria it considers to comprise the rule of rescue. Nonetheless, it has previously made positive recommendations on the basis of that rule. From December 1999 to June 2003, the PBAC made 182 positive recommendations pertaining to major submissions: 95 (52.2%) as cost-minimising; 85 (47%) as cost-effective; and 2 (1.1%) on the basis of the rule of rescue. It is possible that more drugs were recommended on the basis of the rule of rescue but this information is not available publicly.

Conclusion: Evidence from the Australian health care system indicates that the rule of rescue has been used infrequently by the PBAC. In those instances where it has been used it was to justify the public reimbursement of drugs to treat patients with relatively rare conditions for which there is no other effective alternative treatment available. We believe that broader debate on the application of the rule of rescue and its role in priority setting is warranted.

OBJECTIVE

In Australia, the National Health Act (1953) requires that the Federal Minister of Health consider the advice (recommendations) of the Pharmaceutical Benefits Advisory Committee (PBAC) when deciding whether or not to publicly subsidise drugs via the Pharmaceutical Benefits Scheme (PBS).

In making its recommendations to the Minister, the PBAC considers the effectiveness and cost-effectiveness of the drug for which listing is requested compared to currently available alternative treatments. To this end, since 1993 applications to list drugs to the PBS must contain an economic evaluation. The PBAC may also consider other relevant factors such as a drug’s clinical place in treatment, unmet clinical need and the rule of rescue. While the latter has not been formally defined by the PBAC, it has been defined elsewhere as the impetus to act to prolong survival for a person (group) who faces imminent death. We sought to investigate and then describe how and when this rule has been applied by the PBAC.

METHODS

Since December 1999, the PBAC has posted publicly its positive recommendations (those recommending the listing of drugs on the PBS). As of the most recent meeting of the PBAC (June 2003), all outcomes from PBAC meetings (positive, negative and deferrals) are being published on its website http://www.health.gov.au/pbs/general/listing/pbacrec.

Since the PBAC has four scheduled meetings a year at which it considers applications to list drugs on the PBS, this means there were outcomes from 15 meetings for inclusion in our analysis. Our analysis pertains only to positive recommendations made between December 1999 and June 2003 which related to major submissions. Major submissions are those which constitute a relatively small number of patients, and how imminent does death have to be for the rule of rescue to apply?

We reviewed the published positive recommendations and extracted those which we ascertained related to a major submission according to the criteria above. For the purposes of this analysis, we subsequently used content analysis to classify the positive recommendations into one of three groups:

- cost-minimising – recommendations explicitly stated as being cost-minimising, or of an effectiveness which did not warrant a price premium compared to a currently listed comparator;
- cost-effective – recommendations on the basis of acceptable or acceptable but high cost-effectiveness (including cost-utility analyses); and
- rule of rescue – where the phrase “rule of rescue” appeared in the PBAC’s recommendation.

Common themes were then explored for drugs for which a positive recommendation was made on the basis of the rule of rescue.

RESULTS

Between December 1999 and June 2003, the PBAC made 182 positive recommendations pertaining to major submissions. The classification of those recommendations is shown in Table 1.

Table 1: Classification of positive recommendations by the PBAC

<table>
<thead>
<tr>
<th>Year</th>
<th>Positive recommendations</th>
<th>Cost minimising</th>
<th>Cost effective</th>
<th>Rule of rescue</th>
<th>Total # 182</th>
</tr>
</thead>
<tbody>
<tr>
<td>1999*</td>
<td>8</td>
<td>6</td>
<td>0</td>
<td>4</td>
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</tr>
<tr>
<td>2000</td>
<td>22</td>
<td>21</td>
<td>0</td>
<td>14</td>
<td></td>
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</tr>
<tr>
<td>2002</td>
<td>29</td>
<td>20</td>
<td>0</td>
<td>49</td>
<td></td>
</tr>
<tr>
<td>2003*</td>
<td>10</td>
<td>10</td>
<td>1</td>
<td>21</td>
<td></td>
</tr>
</tbody>
</table>

Note: * relates to positive recommendations from the December meeting only.

Over that period, 95 (52.2%) positive recommendations were made based on cost-minimising, 85 (46.7%) on the basis of a drug being cost-effective and 2 (1.1%) on the basis of the rule of rescue.

The two drugs for which listing was recommended under the rule of rescue were:

1. imatinib (Gleevec® in the US and Glivec® elsewhere) for the treatment of patients with chronic myeloid leukaemia in the accelerated and blast phases; and
2. riluzole (Rilutek®) for the treatment of patients with amyotrophic lateral sclerosis.

CONCLUSION

It is possible that more recommendations were made on the basis of the rule of rescue, however this cannot be discerned from the publicly available PBAC recommendations. In the case of imatinib, the published PBAC recommendation did not mention the rule of rescue, however this was stated in the brief advice provided to Novartis Pharmaceuticals Australia Pty Ltd (the sponsor of imatinib).

In its positive recommendation for imatinib (from the September 2001 meeting), the PBAC stated that:

“The PBAC judged the overall cost-effectiveness to be acceptable on the basis of the ‘rule of rescue’ for a particularly severe progressive condition that affects a very small number of patients with no effective alternative drug treatment”.

In the case of riluzole (from the March 2003 meeting), the PBAC made the following comment about its recommendation:

“While uncertainty remained about the clinical benefit of riluzole, restriction to the patient group that was expected to benefit most and the application of the ‘rule of rescue’ supported a positive recommendation to list”.

In both cases, it appears that the rule of rescue was part of the overall decision to recommend rather than being the decisive criterion per se. Furthermore, both drugs are used to treat patients with rare but severe conditions.

Information was sought from the PBAC as to the exact criteria and situations under which the rule of rescue may be invoked. While formal details of how the rule of rescue is applied by the PBAC are yet to be made public, they were able to provide information on the criteria for the application of the rule of rescue to be considered. These are depicted in Figure 1.

Some aspects of these criteria still remain unclear. For example, what is considered to constitute a relatively small number of patients, and how imminent does death have to be for a patient to be considered to have a poor prognosis for survival? In addition, it is also unclear how the rule of rescue is applied and what weight it is given in the decision making process relative to the consideration of the overall cost-effectiveness and clinical place of a drug.

REFERENCES


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A ROLE FOR THE RULE OF RESCUE?

PBAC Criteria for rule of rescue

<table>
<thead>
<tr>
<th>Candidate for rule of rescue</th>
<th>No alternative treatment available</th>
<th>Condition severe and patients face poor prognosis for survival</th>
<th>Relatively small number of patients</th>
</tr>
</thead>
</table>