Making Fair Funding Decisions for High-Cost Cancer Care: The Case of Herceptin in New Zealand

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Cancer Care: Increasing Costs

- 500-fold cost increase from 1994 to 2004
- Nothing in pipeline under US$300,000/QALY
- How much should we pay for these drugs, and for how much benefit?
- Relevant features of cancer care:
  - Very few cures
  - Very high costs
  - Uncertain proven effectiveness of drugs over time
  - “Dreaded” disease
  - Considerable social and political pressure
Case: Herceptin in New Zealand

- PHARMAC decided not to fund Herceptin beyond a certain point: 9 weeks rather than 12 months.
- Government stepped in to fund 12 month courses through separate funding stream.

1. Was PHARMAC’s decision fair?
2. Was government’s policy justified?
PHARMAC’s Herceptin Decision

- Herceptin used to treat HER2-positive breast cancer in both advanced and early stages.
- Treatment for advanced cancer already funded
- Roche application: Herceptin listed on schedule for early stage treatment
  → 12 months following completion of chemotherapy (sequential regimen).
2006: PHARMAC declines application:
- Insufficient evidence of long-term benefits
- Concerns about quality of clinical trial data
- High costs
- Commitment to continuing review of evidence

2007: Approved funding for 9-week concurrent treatment regimen.
Clinical trial evidence suggested no statistically significant difference between 12 month *sequential* regimen and 12 months of standard chemotherapy.

Evidence of comparable effectiveness of 12-month *concurrent* regimen with 9-week *concurrent* regimen.
## Trial results – Disease Free Survival

<table>
<thead>
<tr>
<th>Trial</th>
<th>DFS 3 years Control Standard chemotherapy</th>
<th>DFS 3 years Herceptin Concurrent</th>
<th>Hazard Ratio</th>
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<tbody>
<tr>
<td>B-31 and N9831 (n=3676)</td>
<td>75.4%</td>
<td>87.1%</td>
<td>0.48</td>
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<tr>
<td>FinHer (n=232)</td>
<td>77.6%</td>
<td>89.3%</td>
<td>0.42</td>
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- PHARMAC not alone in recognizing that optimal dosing regimen and treatment duration not defined for Herceptin.
- Was alone in taking 9-week FinHer trial as basis for its funding decision.
- Claimed that 9 week concurrent treatment at least four times more cost-effective than 12 month sequential:
  - NZ$14,500-16,500/QALY vs. NZ$70-80,000/QALY
- Patients who wanted 12 month treatment had to fund extra 8-9 months themselves (NZ$50 000-$120 000).
- Eight women (the “Herceptin Heroines”) took PHARMAC to court to have decision overturned.
- Judge ordered PH to review evidence and reconsider.
- **July 2008**: PH stands by original decision.
- Appealed to:
  - Absence of strong evidence of increased benefit from longer course
  - Some evidence of increased cardiotoxicity
  - Questions over quality of available evidence ("unacceptable publication bias")
  - Lack of confidence that additional expenditure for 12-month treatment would lead to greater health gains.
Criticized for relying on inadequate clinical trial evidence and falling short of international standard of care.

“Put bluntly, New Zealand women are to be denied a treatment regimen which has been robustly shown to save lives and are instead to be offered one which has not.”
“If I lived in New Zealand, I’d be dead.”

Virginia Postrel, *The Atlantic*
Chairwoman of Breast Cancer Aotearoa:
   “a cruel blow for women and their families,”
   “shameful” and “inhumane.”

Health spokeswoman from opposition National Party:
   - Breast cancer sufferers “cruelly let down” by PH
   - NP government would “free up funds” for 12 month treatment if elected.
Government steps in

- **November 2008**: NP-led government elected
- **December 2008**: announcement of funding for 12 month treatment (NZ$9m for 3 yrs) from MoH.
- Funding for 9 week treatment courses continues to come from PH budget.
Was PHARMAC’s decision unfair?

1. Yes, because provided a less effective but cheaper treatment in place of a more expensive but more effective treatment.

→ Women being deprived of a needed benefit on grounds of cost alone.

But: evidence suggests 9 weeks treatment not less effective than 12 months

→ Funding 9 weeks is cheaper, but not clearly depriving women of a benefit.
2. (i) “Weight of evidence” supports 12 month treatment courses; (ii) Without head-to-head comparison, should err on side of weight of evidence. (iii) Less cost-effective, but cost-effectiveness not only factor in decisions (iv) When lives threatened by fatal illness, cost-effectiveness should not be favoured over weight of evidence.
Calls into question central principle driving PHARMAC decisions: maximizing benefits from available resources:

“ensuring taxpayer funds allocated to health are spent in a way that produces the greatest health benefit.”

Significant moral concerns about maximizing strategy of health resource distribution.
Failure to consider distribution of benefits

Conflict between *goodness* (how much benefit) and *fairness* (how that benefit is distributed among population).

From perspective of fairness and distributive justice, it *matters* who is getting the benefits.

If treating elderly generates fewer QALYs, should we only treat young so as to maximize QALYs?
Evidence that people resist max. strategy for health: prefer to distribute resources fairly, even when fewer benefits are gained.

Preference for directing resources to those who are worse off; resistance to discriminating against those who benefit less (elderly, chronically ill, disabled).

Suspictions of CEA as tool of resource allocation reflect concerns about maximizing
Priority to the worse off

- Principle of maximization must be constrained by fairness considerations
- Important consideration is special concern for worse off – urgency, severity, age
- Giving priority to the worse off means directing resources to where they will have a bigger relative improvement
- Improvement matters more to worse off
Prioritarianism: benefiting people matters more, morally, the worse off those people are.

At odds with maximization: it does matter where the benefits go, and not just how big the benefits are.

A QALY has more or less moral significance depending on how well off the person is who receives it.
Impact on decision-making:

- Give more weight to QALYs that accrue to worse off
- Give preference to treatments that help the worse off *even if less cost-effective*
Argument in favour of 12 months of funding challenges maximizing strategy

Give greater weight to fairness considerations when in tension with strategy.

Patients with HER2-positive early stage breast cancer undoubtedly among worse off.

PHARMAC made decision solely on grounds of cost-effectiveness; ignored severity of illness as reason for erring on side of “weight of evidence.”
A. Cost is not irrelevant

- Fairness does require taking factors other than cost into account – e.g., severity of illness.
- But cost not thereby irrelevant.
- Long history of cost resistance in cancer care, due to severity and fear of disease.
- “Inelasticity of demand”: demand remains the same regardless of increases in cost.
78% of oncologists believe patients should have access to effective care regardless of cost

Effectiveness often measured in terms of small survival gains

68% believe survival gain of 2-4 months justifies cost-effectiveness ratio of US$280,000/QALY

(Nadler, Eckert and Neumann, 2006)
Tolerance for high costs and distortion of benefit.

Whose perspective should determine value of benefits?

Resistance to cost, intensity of hope and fear in cancer care make patient’s perspective particularly problematic as basis of policy.

Contrary to this perspective cost is a barrier to providing these treatments.
B. Does fairness require doing everything possible for the worse off?

- If it does, then we are obliged to continually expand the health budget in order to meet demands of fairness.
- Health cannot subsume national budget at cost of other important social goods.
- Education, transportation, public safety, environmental protection also required as a matter of social justice.
- Justice demands limits on health budget
Charge of unfairness flawed

- PHARMAC did put the cost-effectiveness of the shorter treatment above the weight of evidence in its reasoning.
- Even taking into account severity of illness in this case, doing so not unfair.
- Health system that tolerates very high costs for small benefits is able to meet fewer needs
- A fair system should strive to meet as many needs as possible
Fairness *requires* efficiency

Culyer: “it is ethical to be efficient (and unethical to be inefficient).”

To spend more money than is necessary for an effective impact on health results in lower overall level of health, but is also *unfair* to those who miss out.

Providing cost-*ineffective* treatments too costly in fairness terms.
Procedural Unfairness?

- Must have a fair process so that people can accept outcomes as fair, even when they disagree about moral principles.
  - Public accessibility, transparency
  - Relevance of reasons
  - Appeals
  - Regulation.
- Initial failure to consult impugned PHARMAC’s claim to fair process.
- Overall consultation safeguards final decision
- Some public confusion about comparable effectiveness of two treatment regimens?
- Room for improvement on accessibility
- All things considered, final decision not procedurally unfair.
International comparisons

- PHARMAC different in crucial ways from other health decision-making bodies
- Both NICE in UK and PBAC in Australia issue recommendations only – not measuring opportunity costs of recommendations.
- “Weight of evidence” argument could be more compelling for these bodies.
Better comparison with NHS trusts; raised similar concerns to PHARMAC:

- NICE recommendation would cost £2.3m per year
- Significant opportunity costs: could fund if deny treatment to 563 other cancer patients.
- When opportunity costs relevant, weight of evidence must be closely scrutinized.
- NZ decision “courageous”
- Recognition of necessity of rationing built in to legislation in NZ
- NZHDA 2000: health system objectives to be pursued
  “to the extent that they are reasonably achievable within funding provided.”
- PHARMAC:
  “secure to eligible people…the best outcomes that are reasonably achievable from…within the amount of funding available.”
Strong case for not funding expensive treatments with limited effectiveness.

But for Herceptin, these principles perceived as generating an unfair outcome.

This criticism misplaced: making most cost-effective decision not necessarily unfair, even in face of severe illness.
Maximizing strategy is morally problematic

When costs high and benefits small or uncertain, failure to measure costs against benefits is also morally problematic.

PHARMAC argued, rightly, that inefficiency of paying for the longer course was itself an issue of fairness.

Lingering problem: how much weight to give priority to worse off over cost-effectiveness.
Was the government’s policy right?

- Always possible to increase health budget in face of tough allocation choices
- Sometimes it is right not to accept scarcity of resources.
- Herceptin just the tip of the iceberg: continuous expansions of health budget to accommodate cancer treatments not a fair policy option.
- Government policy sets dangerous and unsustainable precedent.