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POSITION STATEMENT

Breakthrough therapies and the introduction of a New Zealand Early Access to Medicines Scheme (EAMS)

Introduction

It is highly unusual for the Cancer Society to get involved in any national debate around the funding of particular cancer drugs. However, in the case of the new immunotherapies for melanoma, we have made an exception for reasons of **equity, evidence** and deep concerns about existing **decision processes**.

This paper outlines the Cancer Society's concerns and recommendations for action.

SUMMARY OF RECOMMENDATIONS FOR ACTION

The Cancer Society of New Zealand RECOMMENDS:

1. **The introduction and establishment of an Early Access to Medicines Scheme (EAMS) in New Zealand.**

2. **That the EAMS Scheme be managed by PHARMAC and would be an additional fifth function for the Agency.**

3. **The EAMS Scheme be allocated a ring-fenced budget of $100 million per annum, in addition to the current pharmaceutical budget.**

4. **That PHARMAC cost-benefit analyses of medicines should capture the true costs of funding/not funding a medicine to the whole health system, not just the Pharmaceutical Budget.**

5. **A drug could be in the EAMS for a maximum period of time, prior to full submission and review under the current PHARMAC process.**

6. **EAMS could collect utilisation data to model total budgetary impact.**

7. **That PHARMAC decision-making processes (without the commercial data) are made publically available as per accepted New Zealand public administration law.**

1. Efficacy of immunotherapy drugs: the evidence

1.1 New Zealand has the highest rate of melanoma in the world, and yet there is no effective treatment for advanced/late stage melanoma.

1.2 The new immunotherapy drugs are "a game-changer", and have resulted in around 30 per cent of people's melanoma undergoing major shrinkage, and another 30 per cent undergoing significant improvement.

1.3 PHARMAC stated in 2012, that the current funded chemotherapy available to late stage melanoma patients is ineffective.

1.4 We question PHARMAC's decision to make it a "low priority" for funding based upon insufficient evidence. UK and Australia have considered the evidence strong enough to make it available to patients on their public healthcare system.

1.5 On the 5th March 2016, PHARMAC publicly stated that there is no overall survival benefit to the new immunotherapy drugs in melanoma. This is in direct contrast to several published randomised phase three studies in the literature which demonstrate survival benefit for several agents.
(http://www.ncbi.nlm.nih.gov/pubmed/25399552;
http://www.ncbi.nlm.nih.gov/pubmed/25891173;
http://www.ncbi.nlm.nih.gov/pubmed/20525992

2. Equity Issues

2.1 Immunotherapy treatment costs about $10,000 a month. Currently in New Zealand, if Kiwis can afford the drugs, they can access it by paying for the treatment privately. Doctors are having to tell their patients and their families if they don't have the money, they cannot get treated. The Cancer Society considers this unsatisfactory for a health system funded by the taxpayer.

2.2 The Cancer Society agrees that pharmaceutical companies should be challenged about the global variation in pricing demands for new pharmaceuticals in different countries. We are also of the view that, in times of high, unmet need like this, these companies should consider making such important medicines broadly accessible – this would increase market penetration of important products quickly, rather than keeping prices high and access scarce. Companies need to be challenged to price their medicines based on the value they provide rather than the profits required to return growth to shareholders.
(http://www.nejm.org/doi/full/10.1056/NEJMp1512009?rss=searchAndBrowse)

3. PHARMAC decision-making processes

The Cancer Society believes that PHARMAC does an important job. However, we feel that its functioning role and effectiveness could be strengthened and make a greater difference to both the New Zealand health system and to the health outcomes of New Zealanders.

3.1 Cost benefit and effectiveness assessment

3.1.1 Current cost-benefit assessments of new medicines appear to be both flawed and lack significant transparency. Current cost-benefit analyses of medicines are described as examining the impact upon the pharmaceutical budget. A more robust approach would be to consider the true costs of funding/not funding a medicine to the whole health system, such as end of life and palliative care, primary and secondary services, infrastructure and workforce demands as well as community supportive care. After all, our health system is a strong as the sum of all of its parts.

3.1.2 The Cancer Society RECOMMENDS that drugs should be evaluated according to robust systems like the ESMO Magnitude of Clinical Benefit scale - like a drug "scorecard". (A standardised, generic, validated approach to stratify the magnitude of clinical benefit that can be anticipated from anti-cancer therapies: the European Society for Medical Oncology Magnitude of Clinical Benefit Scale.)
http://www.esmo.org/Policy/Magnitude-of-Clinical-Benefit-Scale/Manuscript

3.1.3 The Cancer Society RECOMMENDS that effectiveness assessments are made more transparent so the New Zealand public can know whether the drug company claims of "wonder drug" hold up.

3.1.4 The alleged budgetary impact of a drug like pembrolizumab has been estimated at $30m and this has been cited as a reason not to fund. However, true likely utilisation is disputed and the EAMS could collect utilisation data to model total budgetary impact.

3.2 Transparent processes

3.2.1 PHARMAC is right to consider priorities and cost, and as noted above, the cost of many new medicines is too high. However, we need much more transparency within the existing framework to be reassured that the right decisions are being made.

3.2.2 The New Zealand Law Society defines "judicial review" as "*a means to hold those who exercise public power accountable for the manner of its exercise, especially when decisions lie outside the effective control of the political process.*"

3.2.3 The Cancer Society believes that decisions on the efficacy and accessibility of medicines should not be a political decision. However Parliament has a role to ensure that processes are robust and PHARMAC can function in an optimal way.

3.2.4 To date, PHARMAC's decision-making processes have lacked significant transparency, and have not been subjected to the **judicial review** process, despite this being an exercise of executive power under New Zealand administrative law.

3.2.5 PHARMAC should be required to consult with key stakeholders, including melanoma specialists other than those they employ, and patient groups. They should also be required to justify their statements and provide evidence to support their claims.

3.2.6 PHARMAC argue that such transparency could result in reduced bargaining power. However, this does not override the need for transparency and public confidence in decision making. As an example, cost-effectiveness could be reported in price bands to maintain a degree of commercial confidence whilst allowing a degree of disclosure

3.3 A fifth function for PHARMAC

3.3.1 The Cancer Society also recommends that PHARMAC's role be extended to running an *Early Access to Medicines Scheme* is established in New Zealand.

3.3.2 This scheme should have a ring-fenced budget of an additional $100 million per annum.

3.3.3 The early access scheme would provide people with life-threatening conditions access to promising new medicines, where the evidence base is compelling but still considered incomplete. The scheme evaluates early access to break-through medicines by considering the life-threatening condition, with existing evidence and unmet need.

3.3.4 It would be a voluntary scheme for patients.

3.3.5 Evaluation processes under the Early Access to Medicine Scheme could take a similar approach to assessment and evaluation under the EAMS UK Scheme. (Outlined in the Appendix below.)

APPENDIX

A summary of the key components of the UK Early Access to Medicine Scheme and the UK Cancer Drugs Fund are outlined below:

1. THE EARLY ACCESS TO MEDICINES SCHEME EAMS (UK)

1.1 EAMS is a process that aims to give patients with life-threatening (or seriously debilitating conditions) access to promising new drugs that do not yet have a marketing authorisation, when there is a clear unmet medical need.

1.2 Under the scheme, the Medicines and Health Care Products Regulatory Agency (MHRA) will give a **scientific opinion** on the benefit/risk of the medicine based on the data available when the submission was made.

1.3 The MHRA opinion lasts for a year and can be renewed.

1.4 The scheme is voluntary and the opinion from MHRA does not replace the normal licensing procedures.

1.5 The scientific opinion is provided after a **2-step evaluation process**:

(a) **The promising innovative medicine (PIM) designation** – based on early clinical data. Three criteria must be fulfilled in order to gain a PIM designation:

(i) The condition should be life-threatening or seriously debilitating. The severity of the disease should be justified on objective and quantifiable medical or epidemiological information in terms of mortality and morbidity.
(ii) There is high unmet need (no method of treatment available) or existing methods have serious limitations
(iii) The medicine is likely to offer major advantage over methods currently used (in the UK). The potential adverse effects of the medicine are likely to be outweighed by the benefits. A positive risk balance should be based on scientific evidence, and the safety profile the product is likely to be manageable and acceptable in relation to the estimated benefits.
(Since 2014, 13 Medicines have been awarded a PIM designation in the UK.)

(b) **The early access to medicines scientific opinion** – this describes the risks and benefits of the medicine based on data gathered. It supports both clinicians and patients to make a decision on whether to use the medicine before the license is approved.

2. THE CANCER DRUGS FUND (UK)

In England, if a licensed medicine hasn't yet been approved by NICE (National Institute for Clinical Excellence) it can sometimes be obtained through the Cancer Drugs Fund.

2.1 The UK Government also sets aside funds to pay for cancer drugs that haven't already been approved by NICE and aren't available within the NHS in England. (Currently this fund is £340 million per annum.)

2.2 The fund is currently being trialled for three years. It contains a list of drugs available through the fund, usually to treat rare cancers.

2.3 The list includes what type of cancer the drug can be used for and under what circumstances.

2.4 Cancer clinicians can apply to a panel for the specialist panel to make a decision for a patient to be treated with a medicine on the Cancer Drugs Fund list.

3. ABOUT THE CANCER SOCIETY

The Cancer Society is an incorporated society established to reduce the impact and risk of cancer in New Zealand. The Cancer Society provides a broad range of support and information services for New Zealanders and their whanau affected by cancer.

In addition, we fund the largest non-government cancer research programme in New Zealand. We are an independent advocate committed to evidence-based advancement, as well as working both locally and nationally in cancer prevention and health promotion.

We receive no direct government funding, but are assisted in fulfilling our mission by the generous donations and support of New Zealanders.

Claire Austin,

Chief Executive, Cancer Society of New Zealand

8th March 2016.