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Session Four:

Medicines Matter to the Government

During assessment, we talk about the value of medicines. What other value metrics could the government include in their assessments?

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The evaluation, value and funding of emerging technologies and new medicines in the future

What is high value care?

- Perhaps best defined with reference to low value care!

Low-value care is:

- *use of an intervention where evidence suggests it confers no or very little benefit on patients, or*
- *risk of harm exceeds likely benefit, or*
- *more broadly, the added costs of the intervention do not provide proportional added benefits*

Choosing low-value care consumes resources that could have been expended on alternative forms of care conferring greater levels of benefit, either to the patient in question or to other patients.

Source: Ian A Scott and Stephen J Duckett, *In search of professional consensus in defining and reducing low-value care*, Med J Aust 2015; 203 (4): 179-181

How do we assess the value of a technology?

- Use robust and high quality health technology assessment (HTA).
- Ensure that HTA is used across the health system.
- Ensure processes support the ongoing maintenance of value over the life cycle of the technology.

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What value metrics are used in Commonwealth HTA (PBAC and MSAC) and what should be used?

Pharmaceutical Benefits Advisory Committee (PBAC) as an example:

Key factors influencing decision making by the PBAC:

- The PBAC is established under the *National Health Act 1953.* Its primary role is to recommend to the Minister for Health which medicines should be subsidised under the PBS. The PBAC is required, under the Act, to consider the effectiveness and cost of the proposed medicine compared with existing therapies.
- In particular, the PBAC is required to consider the effectiveness and cost of the proposed medicine compared with alternative therapies. It cannot make a positive recommendation for a medicine that is substantially more costly than an alternative medicine unless it is satisfied that the proposed medicine also provides a significant improvement in health.

PBAC decision making is influenced by five quantitative factors:

- Comparative health gain. Assessed in terms of both the magnitude of effect and clinical importance of effect.
- Comparative cost-effectiveness. Includes a consideration of comparative costs, including the full spectrum of health care resources
- Patient affordability in the absence of PBS subsidy
- Predicted use in practice and financial implications for the PBS.
- Predicted use in practice and financial implications for the Australian Government health budget.

What value metrics are used in Commonwealth HTA and what should be used?

Other less-readily quantifiable factors that also influence PBAC decision making include:

- Overall confidence in the evidence and assumptions relied on in the submission.
- Equity. Implicit equity and ethical assumptions, such as age, or socioeconomic and geographical status, may vary for different submissions and need to be re-evaluated case by case.
- Presence of effective therapeutic alternatives. This helps to determine the clinical need for the proposed medicine.
- Severity of the medical condition treated.
- Ability to target therapy with the proposed medicine precisely and effectively to patients likely to benefit most.

How do we meet the challenge of new technologies and ensure they deliver value?

- Principles and practice of HTA have served us well and continue to provide a solid foundation to answer questions of value going forward.
- Challenge for new high cost technologies (eg CAR-T therapies) relate to
    1. need for national level HTA that has applicability across the health system rather than a programmatic perspective.
    2. need for national approaches to service delivery and funding
    3. need to manage clinical uncertainty through use of pricing agreements, data collection and HTA review

Case study: Evaluation and funding of Kymriah

- First of the cell therapies regulated as a Class 4 biological. FDA approved 2017 and TGA Dec 2018.
- Promising but uncertain benefit for
    1. Under 25 yos with relapsed/refractory acute lymphoblastic leukaemia (ALL)
    2. Adults with relapsed/refractory diffuse large B-cell lymphoma (DLBCL)
- Very high cost ($500,000) single infusion (manufactured offshore) delivered in tertiary public hospitals
- Not suitable for listing on the PBS

Case study: Kymriah

- HTA and appraisal conducted by Medical Services Advisory committee (MSAC)
- Positive recommendation for ALL indication (utilisation estimate 30pa).
- Uncertain clinical evidence required development of complex outcome-based, risk-sharing arrangement.
- MSAC advised conditions of use including delivery in tertiary centres of excellence (NSW and Victoria only), data collection and further HTA at 2 years.
- Commonwealth government then needed to get agreement from all states and territories re service delivery and funding models
- Currently, Kymriah is available at Peter Mac and Royal Children's in Melbourne with patients from around Australia eligible.

Kymriah: Lessons learned

- HTA is not the problem. Commonwealth level HTA through MSAC or PBAC is able to manage the appraisal of emerging, high cost therapies be they medicines, devices or biologicals.
- Implementation is challenging with need for the Commonwealth and the states to collaborate, particularly when the optimal setting for delivery of the therapy is public hospitals.
- Achieving best value depends not only on the quality of the initial investment decision but also managing uncertainty through risk share arrangements, mandated conditions of use, data collection and reappraisal.