



CATAG submission to:

Pharmaceutical Benefits Advisory Committee (PBAC)

PD-1 & PD-L1 checkpoint inhibitor immunotherapies: options for subsidy consideration for multiple cancer types

June 2018

The Council of Australian Therapeutic Advisory Groups (CATAG) is an authoritative, expert, consensus-based collaboration of representatives from all Australian State and Territory Therapeutic Advisory Groups or their jurisdictional committee equivalents.

CATAG aims to standardise and improve medicines use primarily (but not exclusively) in the hospital sector across Australia through information sharing, advice and advocacy activities.

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CONTEXT

The Council of Australian Therapeutic Advisory Groups (CATAG) believes the current process of assessment undertaken by the PBAC is robust and has allowed Australian's access to medicines, which are clinically effective, safe and cost effective. This process should continue to be upheld when assessing medicines.

BACKGROUND

CATAG aims to improve medicines management and use within the framework of the National Medicines Policy as it applies to clinical practice in Australian hospitals and at the interfaces of care. It promotes the equitable, safe, cost-effective and quality use of medicines, to optimise medicines management and use in the public sector and wider community, with the objective of realising the best possible health outcomes for all Australians. CATAG supports national information sharing, advice and advocacy for the hospital sector on strategic medicines issues. CATAG has published a number of resources addressing medicines governance within public hospitals.

CATAG takes opportunities to influence the QUM in the community through appropriate governance and treatment decisions made in public hospitals.

RESPONSE TO BROAD QUESTIONS

Opening remarks

Patient centeredness is key when considering a response to the broad questions posed. Prior to enabling access to treatments payers need to make determinations about the safety, efficacy and if the treatments are available at a cost the community can afford. Patients are relying on experts to make these determinations and whether the risk of harm-benefit balance, and cost effectiveness, are sound for a patient to be undergoing the treatment. All participants in the healthcare system need to ensure patients are provided with adequate information to make informed decisions about their health and the treatments they may decide to receive.

1. Advantages of the PBAC considering PD-1 and PD-L1 checkpoint inhibitors for multi-tumour listings. As a representative body of drug and therapeutic committees in public

hospitals, if the PD-1 and PD-L1 checkpoint inhibitors were broadly listed it would alleviate some of the current inequity of access to these medicines. Determinations to access these medicines are made individually by hospital drug and therapeutic committees and therefore this can lead to variability in the decisions made. In addition there is significant duplication of effort in making determinations, which would be removed. There are significant challenges and complexities such as making cost effectiveness considerations with limited data, which DTCs are confronted with when making determinations with regard to inpatient usage requests, Medication Access Program assessments and patient-self funding requests. Regardless of whether the PBS listing is for single tumour indications or multitumour indications, if these medicines were to be listed DTCs would be alleviated from the burden of local decision making for the listed indications.

2. Disadvantages of the PBAC considering the PD-1 and PD-L1 checkpoint inhibitors. These considerations would be made with limited data and therefore there would be significant uncertainty introduced. Due to the lack of certainty of effectiveness and cost effectiveness for some indications, this would be a significant departure from the current robust review system leading to potentially future degradation of the system and cost effectiveness and expenditure creep.
3. There is significant debate internationally surrounding unmet clinical need and its constitution. The discussion concerning urgent unmet clinical need, ties into the broader context of prioritising access to medicines. In Australia access to medicines has been strengthened through the implementation of the priority review pathway by the TGA. The special access scheme is a pathway, which also facilitates access to medicines in urgent circumstances. CATAG recognises there is difficulty in defining urgent unmet clinical, however this should not enable the dispensation of the requirement for medicines to be cost effective. Urgent unmet clinical need should be defined in the context of all medicines and health conditions and not in isolation. There are a range of complexities and challenges, which need to be explored and it warrants broader national discussion with a diverse range of stakeholders.

CATAG supports a framework for appropriate medicines governance through Drug and Therapeutics Committees (DTC), to consider individual cases, which require local scrutiny that cannot be provided in the National subsidy system. DTCs have a role in continuing to manage access to medicines in public hospitals and facilitating genuine compassionate access. There needs to be acknowledgement of the existence of genuine compassionate access schemes and their availability when people have limited treatment options and the scheme is appropriately funded by industry. Concerns have arisen as these systems are currently operating inequitably depending on the healthcare organisation and the treating doctor. There are some genuine compassionate access schemes, which often occur during PBAC deliberations prior to the treatment being federally funded through the PBS. There are a significant number of 'compassionate

access' programs, which require the patient to part, pay for their treatment and these are very inequitable. CATAG repeats the need for the consideration of a National high cost medicines formulary and a cohesive approach to health technology assessment across the jurisdictions ensuring equity of access.

4. Minimum level of evidence of effectiveness: There should not be any difference in the level of effectiveness required for treatment subsidy in any therapeutic area. All therapeutic areas and their treatments should be considered equally, with the same level of evidentiary requirements. If sponsors seek multi-indication subsidy, then they should support the multi-indication trials required to inform decision-making. Enforcing this would drive sponsors to consider inclusion of patient groups with different response rates. Without this it would be very difficult to determine an appropriate price. CATAG supports patient access to treatments, which are effective; without the appropriate evidence we may be exposing patients to undue adverse effects and costs with minimal benefit.
5. There is limited opportunity to extrapolate data from one type of cancer to other types. As noted in the background paper treatment responses are highly variable across cancer types, age, and patient populations, therefore extrapolation would introduce a high level of uncertainty of response to treatment and therefore cost effectiveness would be difficult to determine.
6. Economic modelling: In the absence of multi tumour data the ability to construct models that are not trial based would introduce significant uncertainty. This change to the requirements for PBAC decision-making would also introduce a host of issues that extend beyond the PD1 and PDL1 medicines.
7. The subsidy price: Medication subsidy, whether by the Commonwealth or Australia's state and territory public health systems purchases health outcomes. If the benefit is modest the price should be commensurate with the benefit. If medicines with potentially very modest benefit are subsidised there should be robust risk sharing arrangements in place to manage the risk to the Commonwealth and essentially the community. The principle of trying to calibrate and construct prices according to the currency of QALYs should continue.
8. Biomarkers: Most data suggests we do not have biomarkers that can be adequately used to target therapy. The evidence suggests we should be cautious and consider the implications to other aspects of the healthcare system such as MSAC. Testing for biomarkers including PD-L1 and tumour mutational burden (TMB) may be useful in some cancers but the results are variable and research is still being undertaken to identify and validate accurate biomarkers. In relation to the extrapolation of effectiveness from one PD-L1/PD-1 inhibitor to another across different indications and/or lines of therapy the role of PD-L1 tumour proportion score testing (by immunohistochemistry) is uncertain. It is noted that for some indications pembrolizumab has regulatory approval when used in conjunction with a validated test (immunohistochemistry, see below) whereas for agents such as nivolumab regulatory approval is not predicated on TPS scores for any

indications. The significance of these differences and how they should be dealt with when considering the interchangeability or not of PD-L1/PD-1 inhibitors for different tumour types is unclear.

- a. TGA listing for pembrolizumab in relation to NSCLC: Pembrolizumab is indicated as monotherapy for the first-line treatment of patients with metastatic non-small cell lung carcinoma (NSCLC) whose tumours express PD-L1 with a greater than or equal to 50% tumour proportion score (TPS) as determined by a validated test, with no EGFR or ALK genomic tumour aberrations. *(I also note that for the registration in combo with chemotherapy there is no requirement for testing).*
- b. TGA listing for nivolumab: no mention of validated tests for any indications.

This creates uncertainty with respect to the inter-changeability of agents, and the value of specific biomarkers. When reviewing biomarker testing, consideration should be given to test validation and/or subsidisation.

9. Extrapolation of evidence: For the reasons mentioned previously and in the background paper there is a limited opportunity to extrapolate data due to treatment response variability.
10. Evidentiary evidence for rare cancers: Rare cancers are often without treatment options and there are few drivers for industry to complete trials. Rarity can be addressed through better data collection utilising other methods than clinical trials such as registries. There is a need for a National discussion regarding rarity, which is broader than rare cancers or the utilisation of PD-1 and PD-L1 check point inhibitors. There needs to be a balance between ensuring sufficient access and the requirement for evidence of efficacy in comparison to other treatments. There is some support for the facilitation of access to medicines for rarer conditions, however there needs to be sufficient safeguards in place to ensure QUM is practiced. These safeguards could be implemented through healthcare organisation governance structures such as drug and therapeutics committees to monitor outcomes and collect outcome data.
11. To be determined by the relevant parties
12. Managed entry schemes: In CATAG's opinion we would not want to depart from the existing mechanisms and decision making criteria around subsidies for medicines where there is inadequate evidence with further data foreshadowed.

OTHER COMMENTS

There are broader questions in general about insufficient data for rare diseases. This is a separate issue and should be addressed through policy debate that addresses how these treatments should be considered and subsidised.

Where these medicines and future high cost medicines are not recommended for listing by the PBAC, DTCS are then frequently required to review applications for these medicines for use in individual hospitals or healthcare organisations. In order for DTCs to make informed decisions about cost effectiveness and in the spirit of transparency, CATAG advocates and requests access to HTAs, this would promote a cohesive approach to health technology assessment.

SURVEY DATA

Methods

CATAG determined a pathway to obtain additional information to inform the PD1 and PD-L1 checkpoint inhibitor PBAC discussion was to survey hospitals through its members' networks. A survey was conducted electronically utilizing Survey Monkey™ from 28th May to 22nd June 2018. The survey template is attached in Appendix 2. The CATAG Coordinator undertook analysis and reporting. [REDACTED] Throughout the report the number of responses varies for each question; respondents were able to skip questions if they wished.

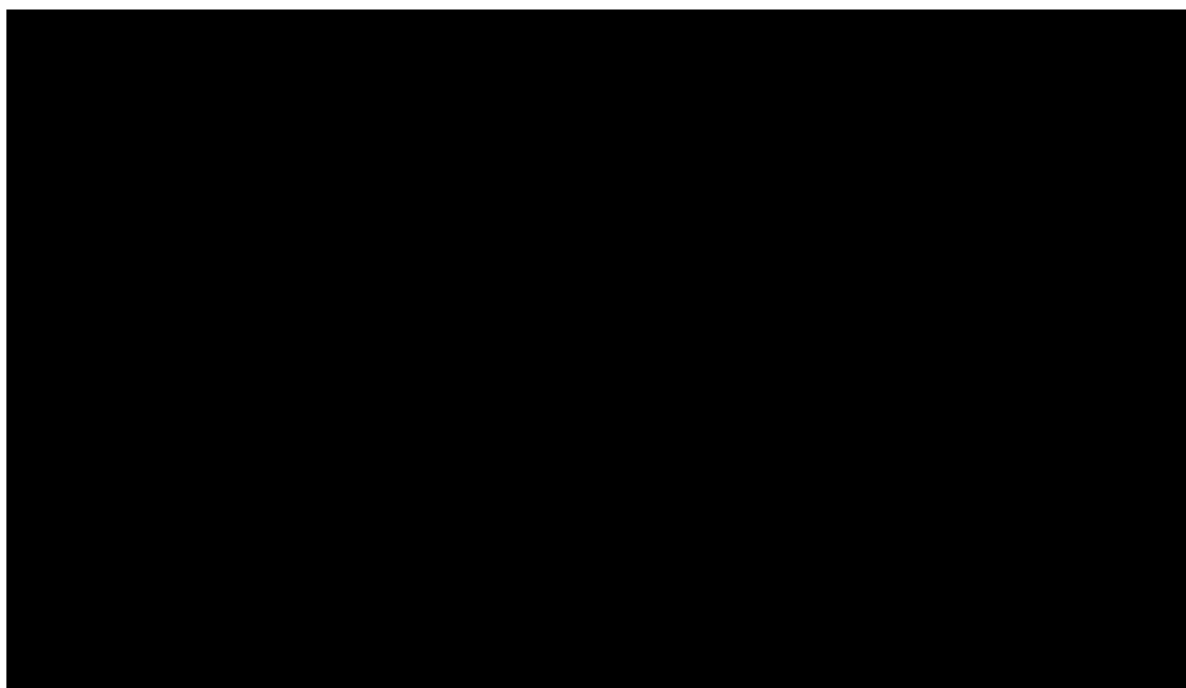
Jurisdictional, health district and institutional representation.

Participation in the survey was requested from Drug and Therapeutics Committees across Australia. Queensland, South Australia, Tasmania and Northern Territory operate state-wide formularies although hospitals within these jurisdictions also have DTCs to locally implement formulary decisions and manage medicine requests for individual patients. Table 1 indicates the types of Drug and Therapeutics Committees (DTC) that responded to the survey. All jurisdictions were represented in the survey.

Drug and Therapeutics Committee description	Jurisdiction	Response Count
State-wide	SA, NT, Tas	[REDACTED]
Metropolitan health district/network	NSW, Vic, ACT	[REDACTED]
Regional health district/network	NSW	[REDACTED]
Rural health district/network	NSW	[REDACTED]
>250 bed metropolitan hospital	WA, Qld, NSW	[REDACTED]
100-250 bed metropolitan hospital		[REDACTED]
<100 bed metropolitan hospital		[REDACTED]
>150 bed regional/rural hospital	NSW	[REDACTED]
51-150 bed regional/rural hospital	Vic	[REDACTED]
<50 bed regional/rural hospital		[REDACTED]
Specialist women's hospital		[REDACTED]
Specialist children's hospital		[REDACTED]
Specialist psychiatric hospital		[REDACTED]
Specialist rehabilitation hospital		[REDACTED]

Table 1: Jurisdictional, district and institutional Drug and Therapeutics Committee representation [REDACTED]

Collated formulary listings are displayed in Figure 1. Pembrolizumab (■■■■ DTCs) is the most commonly listed checkpoint inhibitor on DTC formularies, followed by nivolumab (■■■■ DTCs). Atezolizumab (■■■■) DTCs is most likely to be listed on formulary reflecting the PBS listed indications. Nivolumab and pembrolizumab are formulary listed but their listing sometimes differs and or expands on the PBS indications (n=■■■■ DTCs for nivolumab and ■■■■ for pembrolizumab).



Individual patient use requests¹ for PD1 and PD-L1 checkpoint inhibitors

Respondents were asked to outline the indications and the corresponding number of applications they have received since January 2016.

Atezolizumab was requested through the IPU process [REDACTED], [REDACTED] for [REDACTED]. [REDACTED] [REDACTED] IPU's were approved.

8

Avelumab was requested through the IPU process [REDACTED] times for [REDACTED] and [REDACTED] for [REDACTED] and all IPUs were approved, [REDACTED] were approved as compassionate access.

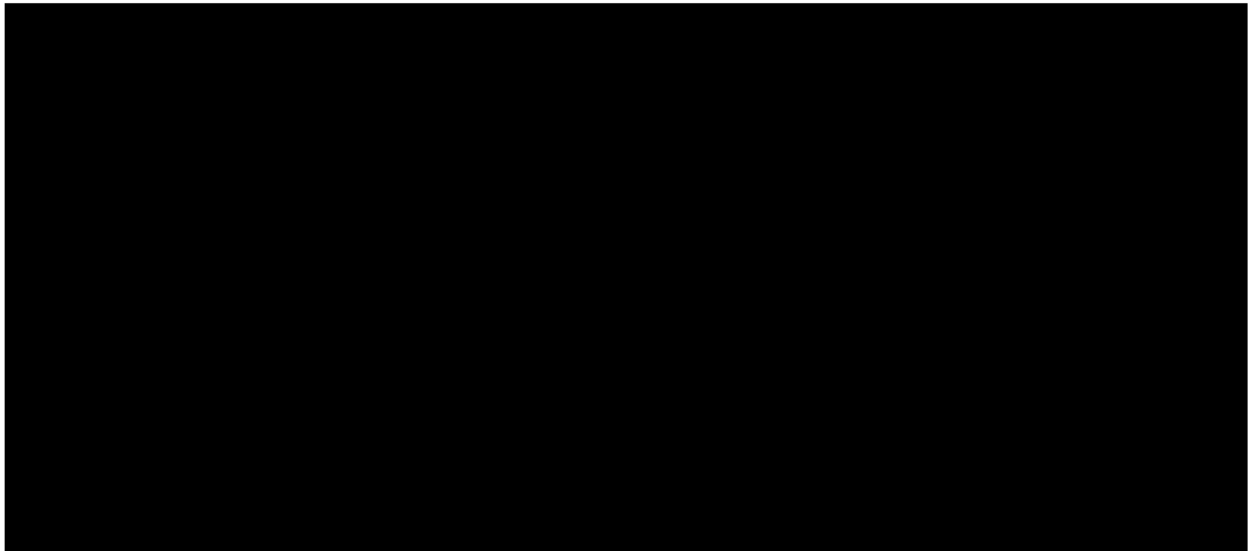


Table 2: IPUs received and approved for Nivolumab

Table 2 documents the IPU indications received by DTCs for Nivolumab and the number of requests for each indication. The most commonly requested indication is [REDACTED] [REDACTED] IPUs)

Survey respondents were requested to provide information on the IPUs, that were not approved and the reasons for the non-approval. [REDACTED] survey respondent provided more detailed information. The IPUs not approved included: [REDACTED] for [REDACTED] [REDACTED] due to lack of evidence of benefit, and cost effectiveness ([REDACTED] application was withdrawn); and [REDACTED] application for [REDACTED] not approved due to insufficient evidence to support the safe and efficacious use of nivolumab in [REDACTED]

[REDACTED]
[REDACTED]
[REDACTED]

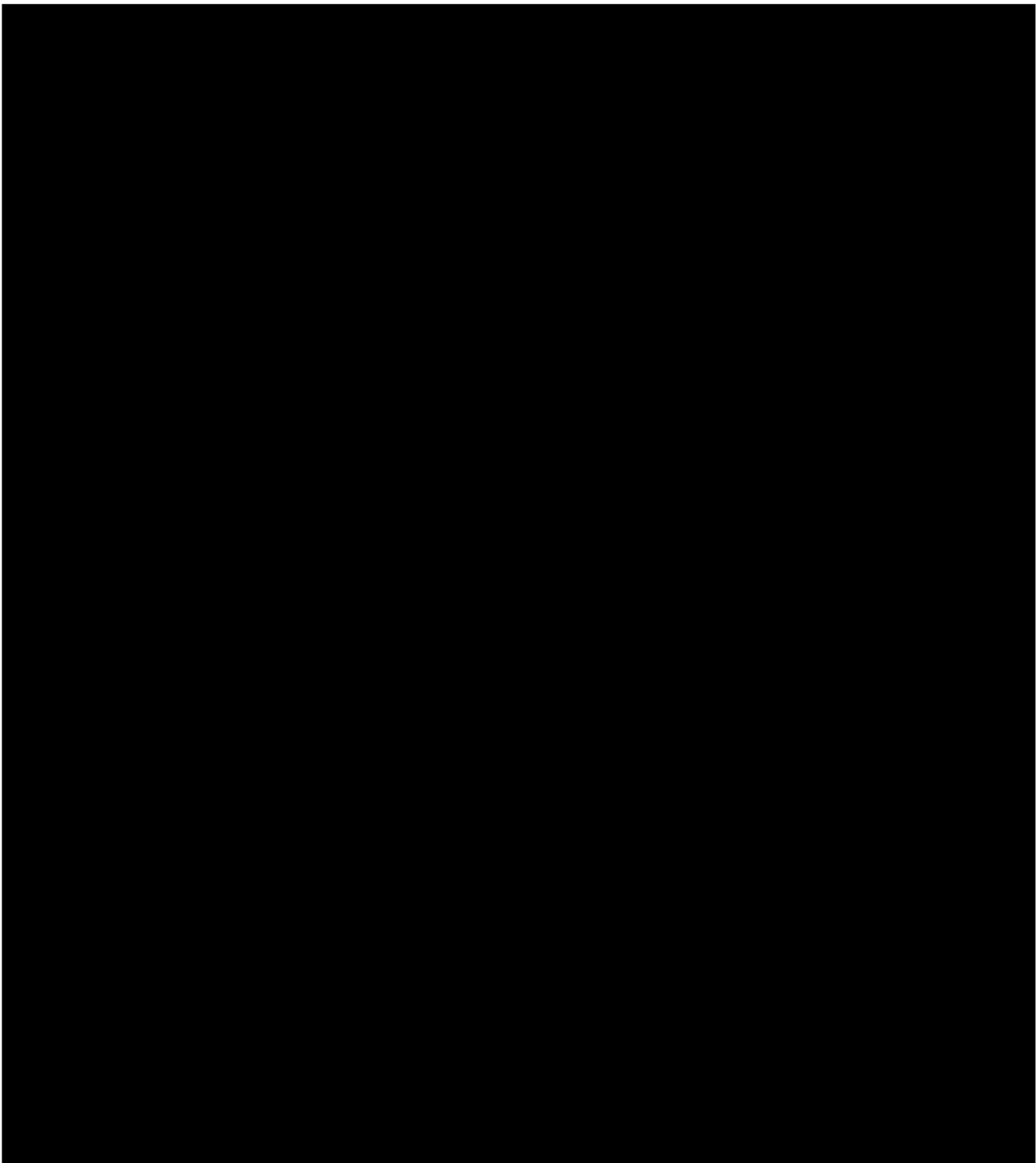


Table 3: IPU received and approved for Pembrolizumab

Table 3 documents the indications for the IPU received by DTCs and the number of requests for each indication. There are [REDACTED] separate indications for which pembrolizumab has been requested to treat, the most frequently requested indication is [REDACTED] [REDACTED] ([REDACTED] IPU requests). The IPU for pembrolizumab were not approved and reported for the following reasons. [REDACTED] IPU for [REDACTED] [REDACTED] for [REDACTED] [REDACTED] IPU for [REDACTED]

Biomarkers

Respondents were asked if any of the medicines were approved on the basis of a positive biomarker result. [REDACTED] respondent, ([REDACTED]) answered yes to approving pembrolizumab on the basis of a positive biomarker.

Monitoring to determine continuation of treatment

Respondents were asked what is used to determine a response in order to continue treatment after initial approval of a PD-1 or PD-L1 medicine.

Atezolizumab: [REDACTED] monitor the patient response and to determine if there was disease progression. [REDACTED] computed tomography (CT) was used to determine a response and to approve continued therapy

Avelumab: [REDACTED] responses included CT. Some respondents were more specific noting CT at cycles 2 and 4. Other responses to be monitored in conjunction with CT were ECOG performance status², quality of life (QoL), and side effect profile.

Nivolumab: [REDACTED] respondents ([REDACTED]) noted CT as a tool to determine response, [REDACTED] included tumour markers in addition to CT. Other responses included positron emission tomography (PET) scans ([REDACTED]), lack of disease progression and patient tolerance and or symptoms, ECOG, QoL, side-effect profile, blood tests. [REDACTED] respondent noted occasionally, information is provided by a detailed letter

Pembrolizumab: [REDACTED] respondents ([REDACTED]) noted CT as a tool to determine response, [REDACTED] responded with mores specific times to undertake CT at cycles 2 and 4. Other responses included PET and tumour markers, clinical response, disease progression, patient tolerance, ECOG, QoL, side effect profile, blood tests. [REDACTED] respondent noted occasionally, information is provided by a detailed letter.

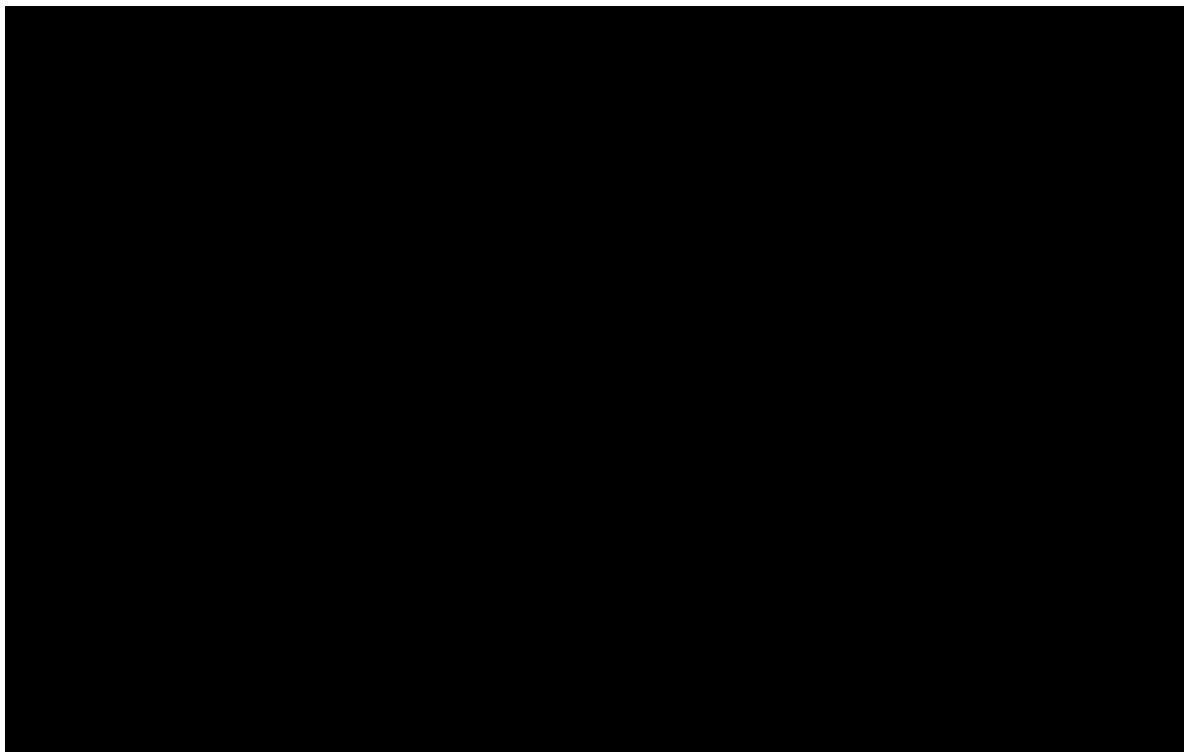
² These scales and criteria are used by doctors and researchers to assess how a patient's disease is progressing, assess how the disease affects the daily living abilities of the patient, and determine appropriate treatment and prognosis.
<https://training.seer.cancer.gov/followup/procedures/dataset/ecog.html>

Financial costs

Respondents were asked to approximate the financial cost to their organisation for supplying medicines through IPU approvals. Many respondents noted costs were limited as the medicines were supplied through compassionate access. However the costs to some organisations were in excess of \$ [REDACTED] for nivolumab and \$ [REDACTED] for pembrolizumab over a two-year period.

Clinical Trials

Respondents were asked if their organisation was conducting a clinical trial with any of the PD-1 or PD-L1 inhibitors. The most commonly reported checkpoint inhibitor being investigated through a clinical trial is [REDACTED] (respondents).

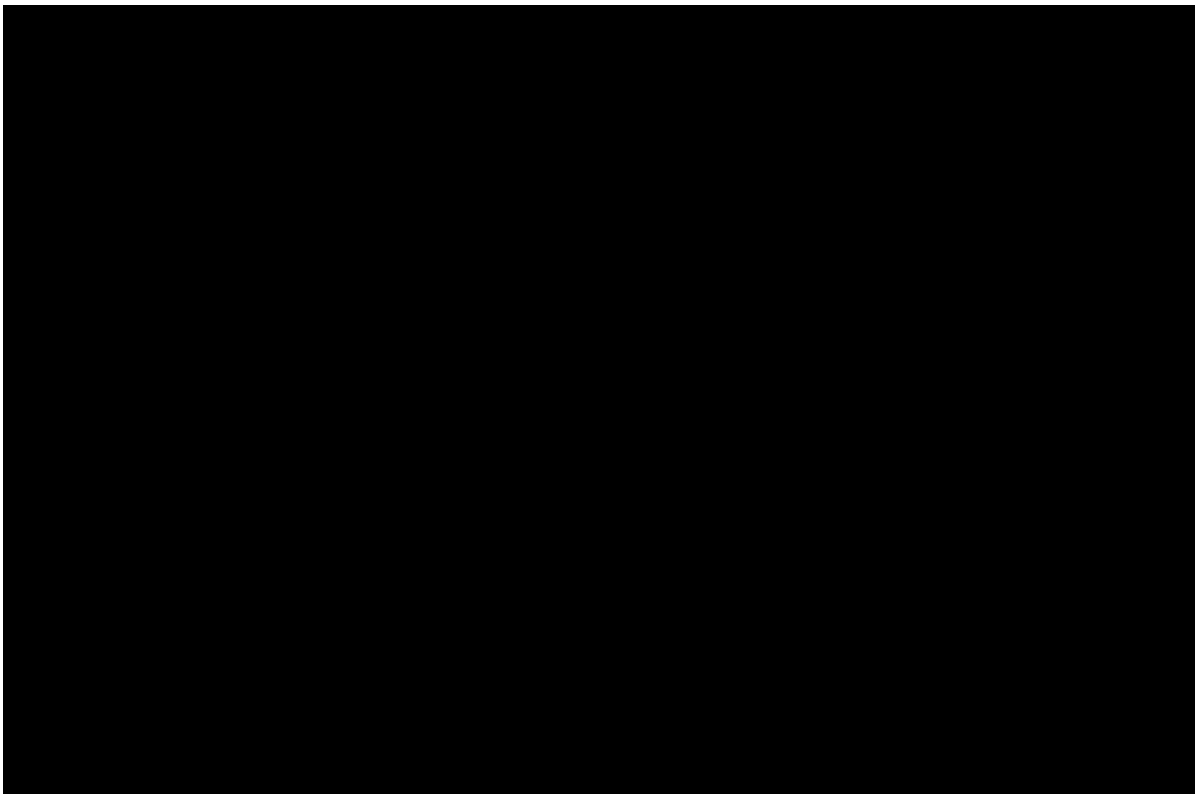


Medication Access Programs (MAP)

Sponsors may provide cost-free or subsidised mechanisms for accessing medicines outside the PBS and hospital funding mechanisms (e.g. via IPU requests). These Medicines Access Programs (MAPs) facilitate deferred cost, cost-free or subsidised medicines supply to hospital patients (usually outpatients) before the implementation of relevant funding arrangements. MAPs include, but are not limited to, compassionate use programs, expanded access programs, product familiarisation programs and cost share programs. Respondents were asked whether they were facilitating access to the PD-1 and PD-L1

inhibitors via MAP, █% (█) of respondents are facilitating access through various MAPs.

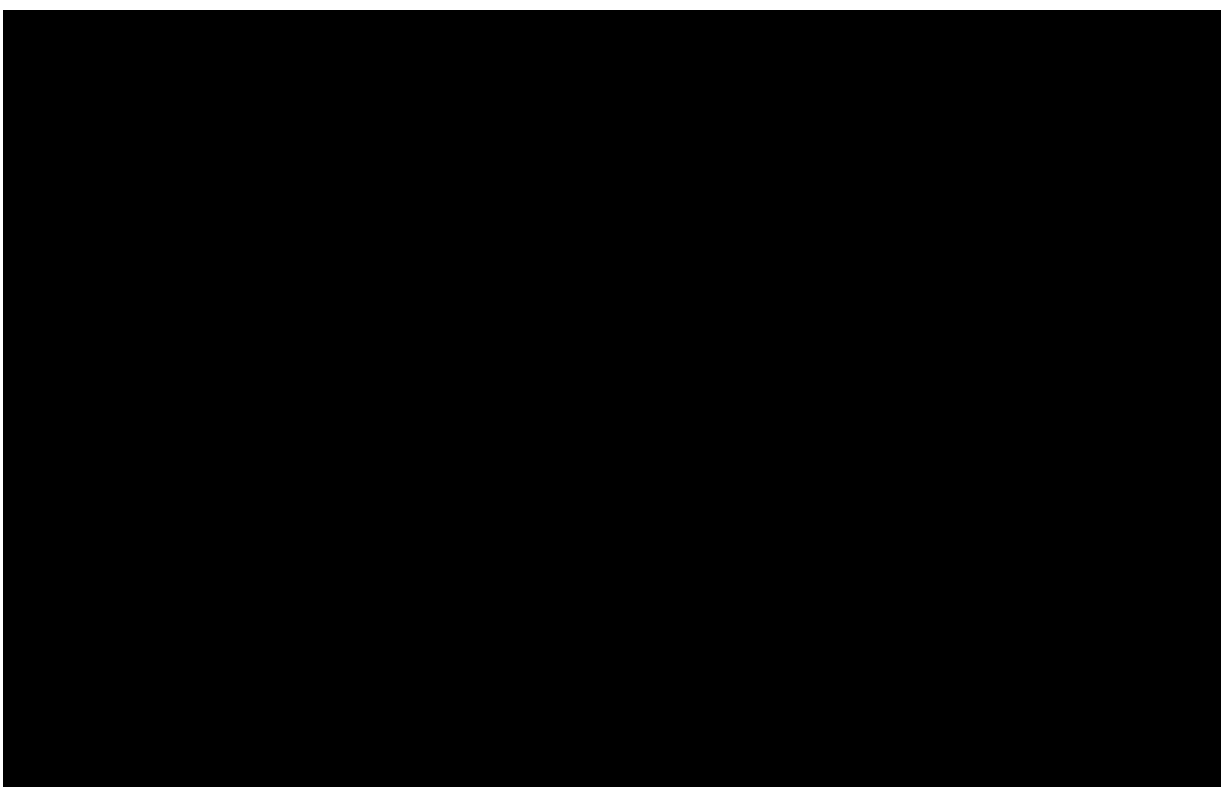
Respondents were asked the type of MAPs they were facilitating for each particular PD-1 and PD-L1 inhibitor and the number of patients enrolled in each MAP. MAPs facilitating access to █ are the █ programs operating. The █ types of MAPs operating are compassionate access programs, followed by PFPs and then cost-share programs. The most number of patients were enrolled in programs facilitating access to █. The greatest number of patients were enrolled in █ expanded access programs (█ patients), followed by nivolumab compassionate access programs (█ patients). The greatest number of patients accessing █ were through PFPs (█ patients) followed by cost share programs (█ patients).





Patient self-funding arrangements

Organisations were asked whether their DTC or equivalent reviews applications for self-funded or cost shared medicines, █% (█) of respondents reported they review self-funding or cost shared requests. █ respondent noted self-funded or cost shared options are not approved or appropriate in public hospitals. A █ only reviews cost share arrangements if the hospital has agreed to fund the cost share component. In █ █ local DTCs review self-funding applications, of which at least █ have been approved in the last 24 months. It is notable █% of DTCs are not privy to the self-funding or cost shared agreements being made.



Policies addressing patient self-funding or cost sharing agreements are implemented in █% (█) of responding organisations. The types of policies vary across organisations, respondents noting some policies only relate to self-funding and do not address cost sharing arrangements.

Benefits and challenges of PD-1 and PD-L1 medicine utilisation in Australian public hospitals

The reported benefits of these medicines were focused on increased patient access. Patients were reported as having more treatments options available than previously. This is potentially due to patients exhausting all treatment options, or the patient not being fit for traditional cytotoxic therapy and these differ and provide further options. Respondents also thought these medicines can be better tolerated than conventional chemotherapy.

The challenges noted by respondents include managing MAPs, patient expectations, off-label use, adverse effects, costs of providing treatment, inequity of access, ethics surrounding self-funding agreements, administrative burden and navigating the complexities of determining cost effectiveness, efficacy and safety.

The challenges of MAP management included administration and logistical aspects of managing PFPs, cost share and compassionate access programs. Respondents expressed their concerns with regard to:

- equity of access to the programs: they are not available at all hospitals and the way in which they operate may vary due to relationships developed by individual clinicians or departments with sponsors.
- logistics of managing MAPs, maintaining supplies which have similar requirements to clinical supplies which requiring packing lists, temperature monitoring.
- ordering of stock is more cumbersome than a standard wholesaler arrangement.
- Often the one medicine can have multiple programs and each program has a different management strategy.
- significant impact on workload for staff and may require dedicated staff to manage the programs.

Administrative and clinical burden: there are increasing burden on oncology clinics for the administration of ongoing therapy

Managing patient expectations and the complexities of the DTC processes including IPU applications, particularly for off-label use. Off-label use is increasingly used as a last line treatment for patients, there are a range of factors, which influence this, including media portrayal of success stories. Patient expectations are also enhanced as immunotherapy commonly appears in mainstream media.

Due to the complexity of the evidence for PD-1/PD-L1 checkpoint inhibitors and the high cost of the agents, patients may face inequitable access to treatment. Inequity of access stems from differing DTCs making varied decisions and the ability of their organisation to fund treatment. Some patients have the ability to self-fund, whilst in other cases the cost has been borne by the state or shared between patients, hospitals and sponsors. How best to address these complex issues is uncertain

Future concerns: questions have been raised as to whether patients who self-fund should be treated in the public sector at all and whether public hospitals charge patients for medications or the administration of medications bought privately by patients. These are complex ethical conversations, which need to be explored.

The practicalities around the compounding and reconstitution of medicines were raised by a number of respondents. The lack of guidance available regarding reconstitution and compounding of these products was noted. Some organisations do not have compounding facilities on site and therefore they outsource these needs to external pharmacies, which incurs a compounding fee per dispensing. The products also have a short expiry once compounded therefore there are implications in terms of timing treatment and wastage.

A number of educational aspects were raised by survey respondents, particularly with regard to the provision of relevant administration and monitoring information for nursing during administration, as these medicines are relatively new. Furthermore not all clinical staff may be sufficiently trained to recognise and treat immune-related adverse events associated with immune checkpoint inhibitors when admitted as inpatients. These are new drugs with new adverse effects (immune-mediated), which are treated differently to adverse effects usually experienced with conventional chemotherapy. The challenge is to ensure all staff (e.g. emergency physicians, general medical staff) are aware of these adverse effects and their management.

Complexities for DTCs assessing cost-benefit in light of current evidence and lack of clarity for which patients are most likely to benefit

DTCs are contending with the issue of use of the PD-1 and PD-L1 checkpoint inhibitors as first or later line treatment for tumours including, but not limited to, requests for metastatic colorectal cancer (microsatellite instability high), non-small-lung cancer, metastatic hepatocellular carcinoma, pancreatic cancer, mesothelioma, metastatic pulmonary adenocarcinoma and triple negative metastatic breast cancer. These agents come at a substantial cost to health systems but are often unregistered for the proposed indication and/or are have limited evidence to support them [REDACTED]. The costs of treating the adverse effects of these medicines are often not taken into account and can be substantial.

The argument for availability on an individual patient basis is often compelling; however, consideration of such requests is complicated by the fact that biomarkers that might predict response are not binary and can have a dynamic expression. The relationship between biomarkers and treatment response to PD-1/PD-L1 checkpoint inhibitors is extremely complex and difficult to evaluate at the local level without significant resources. For selected patients it is clear that these therapies might provide significant benefit. However, the predictive value of PD-L1 may not be the same across different tumours. Additionally, a certain level of PD-1/PD-L1 expression may be more or less relevant depending on the

therapeutic agent (e.g. use of companion diagnostic with pembrolizumab but not with nivolumab).

DISCUSSION

There is considerable variation in decision-making by DTCs, evidenced through the differences in formulary listings. Despite state-wide formulary listings in some states, all jurisdictions are significantly burdened by IPU review, MAP review and governance. Although DTCs generally operate independently of each other, there are informal decision sharing networks such as CATAG and jurisdictional TAGs. Factors related to the variation in DTC decision making may include variations in DTC composition, expertise and skill, varying ability to undertake pharmaco-economic evaluation and financial resourcing. The variation in decisions can also lead to concerns regarding equity of access to medicines.

CATAG's Guiding Principles for the governance of Medicines Access Programs in Australian hospitals provides, key concepts applicable to all care settings. It is recommended approval of a MAP be delegated to, and obtained from, a committee with the required authority and expertise prior to the enrolment of any patients in the MAP. The process for a MAP being reviewed by an appropriate committee provides transparency, propriety and avoids conflicts of interest. The review process also enables a risk assessment with subsequent implementation of risk mitigation strategies, as appropriate. MAPs may provide benefits for patients, clinicians and pharmaceutical companies; however these benefits need to be clearly discussed with patients and clinicians in order that informed decisions are made regarding the utilisation of medicines through such programs. The review of MAPs depend upon DTCs and therefore there can be differing expertise and skill in their abilities to undertake these reviews.

The survey responses highlight the complexities patients and clinicians are required to navigate in order to receive and provide treatment. These medicines are often used after all other treatment options have been exhausted or where there are limited treatment options available, which may generate an emotive decision making environment. The survey highlighted, there is a gap in guidance for Australian hospitals specifically addressing patient self-funding of medicines [REDACTED] of survey respondents stated they did not have a policy for patient's self-funding medicines and [REDACTED] of respondents DTCs do not review patient self funding requests. This is of significant concern, exposing patients to the risk of financial and clinical harm, as there is no risk assessment undertaken. The benefits and harms of treatment may not be adequately addressed in order for a patient to make an informed financial and clinical decision.

LIMITATIONS

As CATAG sent the survey to its member jurisdictions to disseminate through their networks to individual public hospitals and health networks, a response rate is unable to be determined. All jurisdictions were represented in the survey, the only demographic collected was the type of DTC which was operating.

APPENDICES

Appendix 1: Definitions and terminology³

Medicines Access Programs

MAP are programs offered by pharmaceutical companies (sponsors) to facilitate deferred cost, cost-free or subsidised access to medicines for hospital patients before the implementation of relevant funding arrangements. MAP include, but are not limited to, the following:

Compassionate Use

Compassionate use is a program offered by pharmaceutical companies to provide a medicine free of charge for indications that are not already included in a funded scheme (i.e., other MAP arrangement, or eligible clinical trial). Compassionate use may be determined on an individual patient basis or as part of a wider program. Compassionate use usually involves patients with serious or life-threatening conditions or rescue treatments.

Expanded Access Programs

EAP are programs offered by pharmaceutical companies that provide an investigational product cost-free when associated with participation in a clinical trial. EAP usually involve patients with serious or life-threatening conditions. An EAP may include patients who do not meet the enrolment criteria for a clinical trial in progress, or those who have been participating in a clinical trial and require continued supply of an investigational product after its conclusion. Medicines provided under EAP are often not yet registered with the Therapeutic Goods Administration (TGA) for use within Australia.

Product Familiarisation Programs

PPF are programs offered by pharmaceutical companies that are designed to allow the prescriber to evaluate and become familiar with a product while PBS listing is being sought. Products offered under a PFP must be in accordance with the TGA-approved indications and the indication for which PBS listing is being sought.

Cost-Share Programs

CSP are programs offered by pharmaceutical companies that offer a medicine commercially at a reduced price. Use of the product either individually or as a part of a program should be considered as if the drug was simply being marketed at that reduced price. Treatment costs are shared between a pharmaceutical company and the hospital or health service organisation and/or the patient. Cost-share arrangements may include deferred cost, subsidised supply of a medicine (e.g., half price) or arrangements in which supply of a medicine at a reduced price is provided after the purchase of a specified (threshold) amount

³ Council of Australian Therapeutic Advisory Groups. Managing Medicines Access Programs. Guiding principles for the governance of Medicines Access Programs in Australian hospitals. CATAG, 2015 www.catag.org.au