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

General/overall comments

Please note, comments that are beyond the scope of PD-1 and PD-L1 checkpoint inhibitor immunotherapies: options for subsidy consideration for multiple cancer types will not be considered

I was diagnosed in [redacted] with Stage 4 of Merkel cancer. As I do not have private insurance, my choices for treatment were limited.

- Chemotherapy treatment was offered as a public patient. Chemotherapy is covered by Medicare, and the treatment would have been provided to me at very little costs to me. However, the prognostic for Merkel cancer is very poor. I did not see the point in suffering the potential side effects for very little gain in survival. It also seems a waste of public money to treat Merkel patients with an ineffective treatment.

- After further discussions with my oncology doctor, immunotherapy was also offered, based on very encouraging results overseas for this particular cancer. However, I was told that the drug is not listed on the PBS for Merkel cancer (it is listed for melanoma though, and Merkel cancer is a form of skin cancer). I missed on being part of the clinical trial [redacted], which would have provided the treatment for free.

[redacted] therefore decided to use our life savings to allow me to receive immunotherapy as a private patient. The costs to me include the cost of the drug, as well as the cost of the chair as the local public hospital is not allowing me to be treated there.

In total, the cost of each treatment amounts to $5,175.00.

While I have responded well (after 5 treatments), I do not know how many treatments I will need to see the tumours disappear, or controlled. The costs are mounting rapidly.

Merkel cancer is a very rare cancer, very aggressive and with a very poor prognostic using traditional cancer treatments such as chemotherapy. Immunotherapy provides a better alternative but at a prohibitive cost for patients with no or insufficient private health cover. The costs of providing the drugs under the PBS would not add an enormous financial burden on the health system, as the drugs are already approved for melanoma treatment (Stage 4).

Specific responses

Please insert your comments against the consultation questions below.

Question 1

What do you/your organisation see as the potential advantages of the PBAC considering the PD-1 and PD-L1 checkpoint inhibitors for multi-tumour listings?

1. No chemotherapy required prior to commencement of immunotherapy
2. Less side effects than chemotherapy, shorter periods in the hospital for the treatment
3. Current cost of the drug makes the treatments very cost prohibitive for patients with no private health insurance
4. Treatment currently has got to be done at a private hospital, incurring further costs to the patient. As [redacted] resident, I was unable to access a public hospital for treatment even though I had no private health insurance and I was paying for the drug. The cost of the chair (Medicare item 13918) is 10% of the cost of the drug.

Question 2

What do you/your organisation see as the potential disadvantages of the PBAC considering the PD-1 and PD-L1 checkpoint inhibitors for multi-tumour listings?

Question 3

What is urgent unmet clinical need? How should it be established? For which patient groups?

Question 4

What is the minimum level of evidence of effectiveness that you/your organisation think should be required before a PD-1 and PD-L1 checkpoint inhibitors is considered for subsidy for a particular kind of cancer? Why?

Question 5

Do you/your organisation think it is possible for the PBAC to be able extrapolate, or apply, the evidence of effectiveness of a checkpoint inhibitor in one kind of cancer to another kind of cancer, or from late stage cancer to early stage cancer? Why? How?

Question 6

Do you/your organisation think it is possible for PBAC to satisfy itself that treatment with a PD-1 or PD-L1 checkpoint inhibitor is cost-effective without an economic model that is specific to that kind of cancer? How?

- Is it possible to group different cancer types together based on particular characteristics that are similar, and construct a single model for the group?
- Are other approaches to establishing cost-effectiveness across cancer types possible? What are those approaches and how would they operate?

With very rare cancers such as Merkel (1/100,000) and the average age of the patients (>60 years old), it seems very difficult to build an economic model which would satisfy traditional proofs of evidence.

Question 7

What do you/your organisation think is a reasonable subsidy price for Government to pay for a PD-1 or PD-L1 medicines for cancer types where the benefit is potentially very modest?

1. Merkel is a form of skin cancer so the subsidy price should reflect the cost allocated to other skin cancer treatments.
2. What does "*benefit is potentially very modest*" means? Whose benefit?

Question 8

Do you/your organisation think PD-1 and PD-L1 medicines should be made available to all patients whose cancers display a particular biomarker? Why? Which biomarker?

Question 9

Do you/your organisation think it is appropriate for the PBAC to extrapolate the evidence from one PD-1 or PD-L1 checkpoint inhibitor to other medicines in the same class(es). This could provide patients with more choice and give Government the opportunity to negotiate better subsidy prices by utilising the competition between sponsors of medicines.

Question 10

Do you/your organisation think that different evidentiary requirements are appropriate for rare cancers? How do you think cost-effectiveness should be established in this case?

Question 11

Do you/your organisation think PBAC should set aside one of its meetings each year to consider only PD-1 or PD-L1 inhibitors for cancer? (This would mean no other submissions for other medicines, including other cancer medicines, or other diseases would be considered at that meeting.)

Yes, immunotherapy will be the treatment of the future, with great outcomes for the patient.

Question 12

If limited evidence is available at the time of subsidy of a PD-1 or PD-L1 inhibitor for a type of cancer, what do you/your organisation think should happen afterwards?

- Should sponsors be required to collect more evidence?
- What should happen if the new evidence shows the medicine is less effective or has greater safety risks than expected?
- Should the medicine continue to be subsidised but at a price commensurate with its benefit? Should the sponsor be compelled to continue to make the medicine available even if it thinks the price is too low?

Question 13

(For industry/clinical groups) Clinical study information: (Please use the template provided for this information.)

- In what indications has your organisation completed clinical trials with a PD-1 and PDL1 inhibitor? Please include both positive and negative studies.
- In what indications is your organisation currently conducting or planning to conduct clinical trials with PD-1 or PD-L1 inhibitors? If usual PBAC processes were to be followed, when would you expect to make an application for subsidy for

these indications? • How does your organisation decide which indications to study and which to prioritise for registration or subsidy?
Question 14 Are there effective international models for multi-tumour subsidy that could be applied in Australia within the current regulatory framework?
Question 15 **(For Industry)** What information can you provide regarding established international agreements for multi-tumour subsidy and how could these apply in the Australian regulatory context?
Question 16 Is there anything else you/your organisation would like to add?