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

Bladder cancer is not 'glamorous' like breast or prostate cancer and therefore does not get as much support, awareness and funding as these. People tend to put money behind cancers where they see results and media awareness so are happy to march, run, cycle and donate to these cancers. The chance to change that is to make a treatment affordable to bladder cancer patients, that is already proving itself in trial results worldwide. Being a rarer cancer, one way to gather reliable data on treatments is to apply them to patients where some similar cases show positive results.

I am [redacted]. They diagnosed me in [redacted] with Metastatic high grade urothelial carcinoma of bladder (5% micropapillary component) stage IV. The scans revealed it had spread to lymph nodes in both pelvic regions and had reached some aorta nodes. My oncologist said I was in the fifth percentile of cases because of my age, the spread and a micropapillary component in the tumour.

I had a transurethral resection of the tumour, a kidney to ureter stent and 6 cycles of Gemcitabine and Cisplatin over four months. I had the expected side effects, including lymphedema in my left leg but managed to continue working, albeit reduced hours, to support my family throughout.

Further scans, four weeks after treatment were extremely encouraging as there were no visible signs of the cancer and even my bladder biopsy was clear, however a scan in April showed cancer regrowth in my pelvic lymph nodes which means the platinum-based chemotherapy did not destroy the cancer.

I have recently undergone six weeks of abdominal pelvic consolidation radiation therapy.

Besides the obvious personal and compassionate reasons why I and others in my predicament want the opportunity to have affordable access to this treatment, there are other reasons to consider. Even if only a few of the people treated prolong and live their lives in an active and productive way, they themselves will pay for their treatment subsidy in their lifetime by contributing economically to the workforce, paying taxes and being less of a drain on the medical system by repeat attempts of treatments and deteriorating health outcomes and collect more reliable data for future patients.

I am not a medical scholar but I, and my family members have read some current research on PD-1 and PD-L1 check point inhibitors which are showing extended survival in bladder cancer patients and is already subsidised or considered being so in the UK, USA and Europe.

Considering a solution where the body's own immune system fights and prevents a disease spreading and extends life should be at the forefront of treatment options. A delay of even six months to this decision could make the difference between life and death for many of us.

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?

- For rarer cancers, such as bladder cancer, it will take longer to gather reliable data via lengthy trials.
- It is now more relevant to group cancers at a *molecular* level rather than a tissue or organ of origin for treatment because we now increasingly understand the genetic mechanisms underlying cancers.
- It is a fairer way of deciding who gets treatment
- Potential improvement in patient's longevity and tolerable side effects compared to traditional treatment and survivorship, paying back for their own treatment via health and productivity.
- Reduced treatment burden on the medical profession

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?

It will not work for everyone, but where the presence of a certain biomarker is giving positive results for PD-1 or PDL1 checkpoint inhibitors, then making genetic testing affordable for potential patients upfront will make economic sense. Everyone who is alive has one ultimate goal and that is survival

Question 3

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

- The opportunity to access affordable immunotherapy for all cancer types and a shift from perceiving cancer as a terminal illness to one characterised by survivorship.
- Immunotherapies call for a change in thinking about cancer treatment. Tackle capacity constraints in PBAC processes to ensure no delay in listing new medicines because time is critically important to cancer survivorship.
- All patient groups should have access to affordable immunotherapy especially where chemotherapy and/or radiation therapy has not eradicated their cancer, or where they cannot have chemotherapy or radiation. It should not feel like a lottery system of who gets treatment and who doesn't.

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?

- For any cancer, patients should have access to immunotherapy as an adjuvant or as a standalone option if their medical oncologist /professional proposes it.
- At a minimum, biomarker testing should be subsidised to decide on treatment subsidy or not.
- The time needed to accumulate reliable data for rarer cancers will take longer as there are fewer patients enrolled in fewer trials so any level of participation in subsidised treatment will speed up the rate of data accumulation leading to more accurate outcomes.

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?

- The processes assessing registration and reimbursement of cancer medicines need to change to adapt to the potential change in the disease path that immunotherapy brings.
- Collect comprehensive, real life data to assess the holistic benefits of cancer immunotherapy medicines. Currently clinical trial results do not do this effectively and cannot be the basis to apply assessment of effectiveness.
- A shift in how the PBAC deduces or applies this would be welcome.

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?

- As a subsidised treatment, the PBS needs radical funding reforms. In 2018, there should be a very different model than one applied even a decade ago.
- Make an agreement with the pharmaceutical manufacturers of their product to trial all patients recommended for treatment by their oncologists. If they get negative results, they withdraw the treatment from those patients and the manufacturers cover the cost. If the results are positive, and government subsidises. It helps more patients and the drug companies sell more product and the PBS only subsidises the successful treatment.

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?

- That depends on what facts the Government's 'potentially modest benefit' is based on they should also consider life-saving and compassionate access to trials.
- They should value the benefits of newer medicines not just in the health system but longevity and quality of life impacts, productivity and other impacts on patients, carers and society.

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?

N/A: I am not a medical professional

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.

N/A: I am not a medical professional

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?

- The time needed to accumulate reliable data for rarer cancers will take longer
- There are fewer patients enrolled in fewer trials so any level of participation in subsidised treatment will speed up the rate of data accumulation leading to more accurate outcomes.
- The cost-effective benefits lie in improving the health system, a reduced treatment burden on the medical profession and impacts on survivorship, quality of life and productivity

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 absolutely, the pace of medical technology on cancer therapies is moving at a faster rate than the change in the regulatory system.

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?

- Yes, sponsors should collect more evidence.
- Cancer is a 'time poor' disease. As a patient I would not want a treatment that will waste my time or give me false hope, nor would my oncologist.
- If it has some benefit for some patients, then morally they should but I'm not sure if they can be compelled to.

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?

N/A

Question 14

Are there effective international models for multi-tumour subsidy that could be applied in Australia within the current regulatory framework?

N/A

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?

N/A

Question 16

Is there anything else you/your organisation would like to add?

N/A