**Consumer meeting with Crohns and Colitis Australia**

The meeting covered the upcoming PBAC consideration of separate submissions for vedolizumab for Crohn disease and Ulcerative Colitis. This was the PBAC’s first consideration for this drug for Crohn disease, and its second consideration for Ulcerative Colitis following a rejection in July 2014. The majority of the consumer comments received for these items, and the bulk of discussion in this consumer meeting, focused on Crohn disease.

The following points provide a summary of the discussion between Crohns and Colitis Australia and the representatives of the PBAC:

1. Crohn disease severely affects the quality of life of patients and their families in psychological, social and economic terms. This is in the context of a primarily younger patient population that believes there are few treatment options prior to surgery. Patients are willing to tolerate significant hardship in order to postpone or avoid surgery.
2. Patients place a high value on maximising possible treatment alternatives, as this provides a sense of empowerment and possibility in managing their disease.
3. When weighing the treatment alternatives, the patient perception may not necessarily be that the risks and benefits of vedolizumab is compared with the risks and benefits of another drug. Rather, patients may be comparing the risks and benefits of vedolizumab against the risks and benefits of surgery.
4. Vedolizumab is perceived as an additional option that will allow patients to postpone the need for surgery, and to potentially resume a normal life. Without PBS subsidy access, the cost of vedolizumab would prohibit most patients from accessing the drug.
5. The perception of the group of patients that have had access to vedolizumab for Crohn disease in clinical trials is that the adverse events are less than alternatives.

**Consumer meeting with Lymphoma Australia**

The meeting covered the upcoming PBAC consideration of separate submissions for the following drugs for lymphomas: brentuximab vedotin, bendamustine, idelalisib and obinutuzumab. The March 2015 meeting will be the PBAC’s first consideration for brentuximab vedotin, bendamustine and idelalisib for these diseases. The PBAC previously considered and rejected obinutuzumab in July 2014 for the same disease.

The following points provide a summary of the discussion between Lymphoma Australia and the representatives of the PBAC:

1. Consumers place high importance on having access to the best available treatments. The eventual goal, recognising that a cure isn’t always possible, would be to enable indolent lymphomas to be treated as chronic diseases. Ultimately patients may die of conditions unrelated to their lymphoma.
2. Patients may relapse multiple times in the course of the disease, and will be treated on relapse. As PBS subsidy may influence the choice of treatment, subsidising the most clinically effective treatments is critical to ensure the best value for the taxpayer.
3. Patients may be diagnosed at a young age and live for years after diagnosis, and therefore place a high value on Progression Free Survival (PFS). Extending the time in which the progression of their disease is held at bay, and postponing the next relapse, allows patients to resume their daily lives. In this context, the decision for the patient rests on a balance of the PFS gained against the quality of life impacts of drug toxicity. The psychological impact of patients’ fear of relapse can have a highly detrimental effect on their quality of life.
4. The PBAC is very familiar with using PFS to value the benefits of a drug – an upcoming example is bendamustine, where no survival advantage is claimed by the sponsor. A useful drug may be one that does not provide a survival advantage, but may provide quality of life benefits in terms of PFS. Some of the most informative submissions seen to date by the PBAC have presented economic models that illustrate what happens when a patient is in a PFS state. It was noted that exploring how patients could provide more input to quantify Quality of Life descriptions would be valuable in future consumer submissions.
5. It was noted that bendamustine has been available overseas for many years, and patient groups had lobbied the sponsor to make a decision to seek subsidy in Australia.
6. Consumers have a pivotal role to play, both in influencing sponsors to provide high quality clinical data that reflect the consumer experience with a drug, and in providing quantitative data on quality of life through the PBAC’s consumer input process.
7. With particular regard to brentuximab vedotin, this is seen by patients as a “bridge” to get patients to a stage where transplant is an option. However, the submission only requests use after autologous stem cell transplant (ASCT) rather than prior to ASCT. Patients do not necessarily view the drug as a curative option, but some good responses have been achieved.
8. With regard to the high cost of these drugs, the representative noted that generally patients do not feel empowered to question or challenge how sponsors arrive at their asking prices. Patients are willing to go to great lengths to self-fund treatment, and will sell or mortgage their family home, hold fundraising events and access superannuation if needed. Ultimately, patients feel that to agitate for lower prices could risk losing access to drugs they sorely need, and access options are where advocacy efforts are strongly focused. Also noted was the inequity of access to drugs through clinical trials, the availability of which is limited in rural and remote areas.
9. With regard to idelalisib and obinutuzumab, consumers view these as options for patients that are very unwell and unable to tolerate other treatments.
10. The final point noted was in reference post market surveillance in Australia, and that consumer groups would value more collection of data that could be collected and also linked across existing databases, to provide more timely understanding of utilisation and patient outcomes.

**Consumer meeting with Australian Melanoma Consumer Alliance (AMCA), Melanoma Patients Australia (MPA) and CanSpeak Australia.**

The meeting covered the upcoming PBAC consideration of pembrolizumab. The March 2015 meeting will be the PBAC’s first consideration for this drug for metastatic melanoma.

The following points provide a summary of the discussion between AMCA, MPA, CanSpeak Australia and the representatives of the PBAC:

1. Patients have a high level of belief in the effectiveness of pembrolizumab, and are extremely eager for early access to the drug. Perception of the drug is that it is seen as a significant advance, with better safety than alternatives. This is in the context of a predominantly younger patient group who are dealing with a very serious prognosis – these patients are willing to take risks with new treatments, and to find ways to pay for the drug if needed. In addition, these patients are very willing to participate in clinical trials but not necessarily in *comparative* clinical trials – they are not willing to risk not receiving the trial drug.
2. Consumers have a high level of concern about the equity of rules governing patient eligibility – ultimately, patients with melanoma would expect to have access to the drug regardless of any prior treatment they may have received.
3. The Department and the PBAC have accommodated to a significant extent the sponsor’s requests to be able to provide the latest data at several points outside the standard process. This has included submission of hitherto-unseen clinical data and a revised economic model within days of the March PBAC meeting commencing.
4. The representatives informed PBAC that there is a perception that up to 90% of patients could expect to respond to pembrolizumab, even if this is not reflected in the data presented by the sponsor to the PBAC. Consumers also believe that the response to pembrolizumab is “durable and sustained”. The PBAC was extremely concerned at the substantial mismatch between the public expectations of the drug and the data submitted in support of its subsidy application.
5. With regard to the high cost of this drug, consumers do not feel empowered to question or challenge how sponsors arrive at their asking prices. Patients are willing to go to great lengths to self-fund treatment, and will sell or mortgage their family home, hold fundraising events and access superannuation if needed.
6. The future of other treatments for melanoma, including ipilimumab, would need to be considered. It is not clear whether patients would expect to be able to access ipilimumab should pembrolizumab fail – patients believe that the development of these drugs represents new options that simply were not available until recently.
7. It was noted that patients believe that the risks associated with early access to this drug was accepted by many, because of the perception of gaining a life-saving response.

**Consumer meeting with Hepatitis Australia, Hepatitis NSW and the Australian Injecting and the Illicit Drug User’s League.**

The meeting covered the upcoming PBAC consideration of submissions for sofosbuvir, sofosbuvir with ledipasvir, asunepravir, daclatasvir and ribavirin. The March 2015 meeting will be the PBAC’s second consideration for sofosbuvir, having rejected the drug in July 2014. For the other four submissions, the March 2015 meeting will be the PBAC’s first time to consider them,

The following points provide a summary of the discussion between Hepatitis Australia, Hepatitis NSW, the Illicit Drug User’s League and the representatives of the PBAC:

1. The high burden of disease associated with Hepatitis C Virus (HCV) infection was noted, and the urgent need for new treatments acknowledged. The significant adverse reactions associated with interferon-based therapies effectively eliminate these regimens as an option for some patients.
2. Concern about not having any treatment (the so-called “warehousing” practice adopted by clinicians), lack of access to fibroscans and the lack of adequate follow-up for patients that are “warehoused”. A complex referral system does not work for many groups of Hepatitis C patients – for example, it was quoted in the meeting that in the ACT only 28 patients have had access to treatment to date.
3. Community expectation with regard to the new drugs for Hepatitis C is high, and there is a high level of anticipation with patients keenly aware that these drugs are available in markets outside Australia. It was noted that these expectations were in place for a significant time before the sponsors chose to make reimbursement submissions to the PBAC.
4. Co-ordinated treatment of HCV, particularly in moving towards the control (and potentially eradication) of the virus, would require health-system-wide approaches that are outside the remit of the PBAC.
5. As the PBAC is empowered to determine the circumstances under which PBS subsidy may be granted, elements such as whether to limit prescribing to specialists would be considered in potentially widening access. The PBAC particularly noted the advice of consumer groups that a PBS listing limiting access based on a specified disease severity would not be supported, rather a broad-based access system was favoured.
6. It was also noted that representatives felt that these drugs should be assessed for their capacity for providing a cure within a 12 week period, not as longer term treatment strategies.