

RECORD OF CONSUMER HEARINGS

Consumer meeting with Ovarian Cancer Australia

The meeting covered the upcoming PBAC consideration of olaparib for the maintenance treatment of women with *BRCAm* platinum-sensitive relapsed ovarian cancer. The March 2016 meeting will be the PBAC's first consideration for olaparib for this condition. The following points provide a summary of the discussion between Ovarian Cancer Australia and the representatives of the PBAC:

1. The condition severely affects the quality of life for patients and their families, impacting on their social, economic and psychological wellbeing. Patients are aware that recurrence of the disease following initial treatment is inevitable. With few new drugs becoming available in recent years, chemotherapy offered at the point of recurrence is less effective than treatment given on initial diagnosis;
2. Whilst acknowledged that olaparib treatment is not a cure for ovarian cancer, it is perceived as an important advance – it is considered to delay disease recurrence while preserving a good quality of life. It is hoped that better understanding of ovarian cancer has resulted in more effective treatments, allowing patients to avoid or postpone exposure to the detrimental side effects of cytotoxic chemotherapy.
3. Without PBS subsidised access, the cost of olaparib would prohibit most patients from accessing the drug. For many women diagnosed with ovarian cancer, it is at a time when they are no longer working full-time and are therefore severely limited in their ability to fund the treatment themselves.
4. Patient perspectives of olaparib are that the adverse events (most commonly fatigue) are less than those experienced when receiving chemotherapy. For many women the adverse effects of chemotherapy have prevented them from remaining in the workforce.

Consumer meeting with MPS Society

The meeting covered the upcoming PBAC consideration of elosulfase alfa for the treatment of Mucopolysaccharidosis type IVA (Morquio A syndrome). The March 2016 meeting will be the PBAC's second consideration for elosulfase alfa for this condition. The PBAC previously considered and rejected elosulfase alfa in November 2014 for the same condition. The following points provide a summary of the discussion between MPS Society and the representatives of the PBAC:

1. Mucopolysaccharidosis type IVA (Morquio A syndrome) is a rare and serious condition which severely affects the quality of life for patients and their families, and also other factors such as social and economic terms. Without Government subsidy or other assistance (e.g. clinical trials/compassionate access from the sponsor, assistance from the public hospital), the cost of elosulfase alfa would prohibit most patients from accessing the drug.
2. Concerns about the translation of outcome measures applied in the key clinical trials of elosulfase alfa (particularly the 6MWT) detecting the real life benefits experienced by treated patients and their carers/families.
3. Children with Morquio A syndrome experienced a significant improvement in their ability to walk and to engage in school and family activities with elosulfase alfa treatment. They also noted that a gain in height, although not universal in treated patients, was a highly significant result in an illness in which short stature is the main physical feature.
4. The Committee heard that patients with Morquio A syndrome generally deteriorate without treatment and thus even the prevention of further deterioration would be considered a significant treatment outcome.
5. It was noted that elosulfase alfa treatment can be intrusive, where the therapy schedule generally requiring weekly infusions delivered in a hospital. It was also noted that elosulfase alfa's clinical benefit varied from patient to patient being greater for younger patients. The representatives also informed the Committee that the use of the six minute walk test as a measure of elosulfase alfa's efficacy should be considered in the context of the patient's height, as the distance covered will vary due to the different heights of the patient.

Consumer meeting with Cystic Fibrosis Australia

The meeting covered the upcoming PBAC consideration of lumacaftor/ivacaftor for the treatment of cystic fibrosis (CF) in patients aged 12 years and older who are homozygous for the F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. The March 2016 meeting was the PBAC's first consideration for lumacaftor and ivacaftor for this condition.

The following points provide a summary of the discussion between Cystic Fibrosis Australia (CFA) and the representatives of the PBAC:

1. CFA and patients believe that lumacaftor/ivacaftor is an essential medicine. Without PBS subsidised access, the cost of lumacaftor/ivacaftor would prohibit most patients from accessing the drug.
2. The symptoms of cystic fibrosis and the associated treatments, tests and hospitalisations limit the ability of this population of CF patients (and their carers) to participate in school, employment and the community. The CFA highlighted the physical, emotional and financial drain on patients, families and carers and the financial burden on state and federal health care and welfare systems associated with this condition. Everyday activities such as laughing or crying can provoke severe coughing fits. CF-related issues with adequate nutrition make eating a chore for patients. In a single year CF patients can require hundreds of hours of physiotherapy and medical appointments, hundreds of insulin injections and thousands of hours using a nebuliser. For children with CF frequent school absences can isolate a child from their peers. Adults with CF find employment opportunities curtailed due to the impacts of their disease.
3. CFA and patients have a strong sense of hope that lumacaftor/ivacaftor treatment will be associated with very good health and quality of life outcomes for some patients. The outcome of most importance to patients (and their families) was "general wellness" and the impact on quality of life and mental health. Other outcomes of importance included fewer admissions to hospital, fewer exacerbations, reduction in need for intravenous antibiotics, lung function and weight gain.
4. It was acknowledged that Lumacaftor/ivacaftor treatment for CF patients homozygous for the F508del mutation does not appear to be as effective as treatment with ivacaftor monotherapy (already subsidised on the PBS) for patients with G551D mutation or other gating (class III) mutation (particularly in terms of change in FEV₁). Regardless of the effect of the combination of ivacaftor/lumacaftor on FEV₁, CF patients believe that the benefits of the treatment are significant.
5. Other treatment options were also discussed but patients deemed these not to be as effective as lumacaftor/ivacaftor treatment and are more aimed at treating the symptoms rather than the underlying cause of the disease. The burden of these treatments was discussed, including the volume of medication, nutritional feed, constant admissions to the hospital and an associated potential for long-term trauma and side effects for the patient (e.g. body aches). Patients perceived that a benefit of lumacaftor/ivacaftor would be a reduction in the need for other treatments and hospitalisations.
6. The PBAC noted that the TGA has approved the registration of lumacaftor/ivacaftor for the treatment of cystic fibrosis in patients aged 12 years and older who are homozygous

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for the F508del mutation in the CFTR gene. The PBAC noted that it is aware of trials in younger children and queried whether there would be any issues with restricting access on the basis of age, in line with the TGA registration. CFA indicated that listing lumacaftor/ivacaftor on the PBS for ages 12 and older would be for the good of the community.