RECORD OF CONSUMER HEARINGS

Consumer meeting with Breast Cancer Network Australia

The meeting covered the upcoming PBAC consideration of ribociclib for the treatment of patients with hormone receptor positive (HR+) and human epidermal growth factor receptor 2 negative (HER2-) advanced breast cancer (ABC). The July 2017 meeting was the PBAC's first consideration of ribociclib. Palbociclib, a medicine in the same therapeutic class as ribociclib, was not recommended by the PBAC at its March 2017 meeting. The following points provide a summary of the perspectives presented by the Breast Cancer Network Australia (BCNA) to PBAC representatives:

- The condition affects the quality of life for patients and their families, impacting on their social, economic and psychological wellbeing, with few new medicines becoming available in recent years.
- Patients with this condition and their treating doctors stated that cyclin-dependent kinase (CDK) inhibitors are available for breast cancer in many other countries. The BCNA was aware that ribociclib is under review by the TGA, but welcomed the TGA registration of palbociclib in Australia.
- Whilst acknowledging that the clinical trials for cyclin-dependent kinase (CDK) inhibitors like palbociclib and ribociclib are ongoing, and are yet to demonstrate an improvement in quality of life or an overall survival benefit, patients value the demonstrated progression-free survival outcomes, and view these medicines as well-tolerated treatment options that delay disease recurrence and the eventual onset of cytotoxic chemotherapy.
- Notwithstanding the PBAC's concerns regarding the significant toxicities associated with palbociclib treatment, patients indicated that the most common adverse events experienced with CDK inhibitors (fatigue and febrile neutropenia) are substantially less detrimental than those experienced while receiving chemotherapy (mouth ulcers, fatigue and infection). Furthermore, the adverse effects of cytotoxic chemotherapy have prevented many women suffering from this condition from remaining in the workforce or living their life with their families.
- Without PBS subsidised access, the high cost of palbociclib and ribociclib would prohibit most patients from accessing these medicines. Although sponsor-supported compassionate access programs exist, these are difficult to access, and are particularly inequitable for patients from rural areas.
- The BCNA indicated that they were willing to work together with the sponsors, consumers, PBAC and the Medical Oncology Group of Australia (MOGA) to initiate dialogue via stakeholder meetings, in order to facilitate PBS-subsidised access to CDK inhibitors for this condition.

Consumer meeting with MS Australia

Representatives of the PBAC met with MS Australia prior to the PBAC meeting to discuss ocrelizumab and glatiramer. The following points provide a summary of the perspectives presented by MS Australia to the PBAC representatives:

Ocrelizumab

- Ocrelizumab represents an additional treatment option for patients with relapsing remitting multiple sclerosis (RRMS).
- Patients and clinicians value the availability of multiple treatment options that are safe, effective, affordable and provide equitable access across the spectrum of multiple sclerosis types and severities.

Glatiramer

- Clinically isolated syndrome (CIS) is not a well-defined patient group as many patients may not be aware that they have CIS. Commonly, patients are diagnosed as likely having or not having multiple sclerosis, rather than CIS. Therefore, the experience of MS Australia is predominately with patients who have clinically definite multiple sclerosis.
- The population of patients diagnosed with CIS is small and both incidence and prevalence are difficult to assess, however the population may decrease over time as improvements in magnetic resonance imaging (MRI) technology allow earlier diagnosis of clinically definite multiple sclerosis. Some patients may never progress from CIS to clinically definite multiple sclerosis.
- Patients with clinically definite multiple sclerosis and their clinicians value early treatment to slow the disease course by minimising relapses, delaying disability progression, reducing the number of new lesions and delaying brain atrophy. However, not all patients choose to adopt pharmacological treatment, with some patients preferring non-pharmacological options to manage symptoms such as through diet or exercise.
- Early treatment is particularly valued by younger patients with clinically definite multiple sclerosis, for example, the cohort of women aged 20 – 40 years, who may be planning a family and are less willing to “watch and wait”.
- The current PBS eligibility criteria for the treatment of multiple sclerosis are not aligned with the current clinical guidelines and diagnostic tools for multiple sclerosis, such as the McDonald criteria. Overall, however, patients do not report experiencing issues with a lack of access to PBS subsidised treatments.
- Anecdotally, the manner of administration of treatments (e.g. oral versus injectable) has not been a major driver of treatment choice for patients.
- Fatigue and neuropathic pain are reported as the top two symptoms impacting on patients’ quality of life; anecdotally, treatment assists patients in managing the impact of fatigue and neuropathic pain to allow improved functioning in daily living.
- Patients and clinicians value the availability of multiple treatment options that are safe, effective, affordable and represent equitable access across the spectrum of multiple sclerosis types and severities.

Consumer meeting with Soft Bones Australia

The meeting covered the upcoming PBAC consideration of asfotase alfa for the treatment of patients with paediatric-onset hypophosphatasia (HPP). The July 2017 meeting will be the PBAC's first consideration of asfotase alfa. The following points provide a summary of the perspectives presented by the Soft Bones Australia to PBAC representatives:

- HPP can be difficult to diagnose, particularly in patients with less severe symptoms, and can sometimes be mistaken for osteoarthritis or osteomalacia. Diagnosis is based on family background, symptoms and blood test results and can be confirmed with genetic testing (usually via the Unites States) which can take several months. Accordingly, the prevalence of HPP in Australia is uncertain.
- The symptoms of HPP can affect quality of life for patients and their families/carers, impacting on their social, economic and psychological wellbeing. There is a spectrum of severity of symptoms, largely associated with limitations in physical growth, mobility, strength and stamina, both between patients and across a patient's lifetime.
    - The most severely affected patients are a small group with perinatal/infantile-onset of symptoms who present with severe respiratory issues and may require mechanical ventilation or oxygen supplementation. As these patients age, their growth may be slower than expected and they may have significantly impaired movement. Consumers are of the view that treatment with asfotase alfa improves survival and may allow these patients to come off ventilation/oxygen earlier than otherwise expected. Life-long treatment with asfotase alfa is viewed as necessary to assist in building lung capacity, strength and mobility.
    - Juvenile-onset patients may present with symptoms such as delayed growth, limitations in movement, fatigue, loss of teeth and rickets. Consumers are of the view that treatment with asfotase alfa can allow children to physically grow, improve independence in movement and build strength and stamina.
    - Patients who either have adult-onset disease, or are diagnosed later in life with a history of less severe symptoms associated with HPP during childhood, may present with a range of symptoms such as arthritis, fractures, fatigue and neurological complications (described by the consumer representatives as "brain fog"). Some of these older patients with symptoms during childhood may experience a "honeymoon period" in their 20s and 30s (which can further hamper diagnosis) but decline again in their 40s and 50s. Consumers are of the view that treatment with asfotase alfa can help to increase mobility and independence by increasing stamina, strengthening bones and healing fractures faster.