

## JULY 2021 PBAC MEETING

### RECORD OF CONSUMER HEARINGS

#### **Consumer meeting with the Australian patient support group for Waldenström macroglobulinemia (WMozzies) (Item 5.12 zanubrutinib)**

##### **Context**

For consideration at the July 2021 PBAC meeting, BeiGene Aus Pty Ltd lodged a Category 2 submission to request a General Schedule Authority Required (Telephone) listing for zanubrutinib for the treatment of adult patients with Waldenström macroglobulinemia (WM) in two patient subpopulations: treatment-naïve patients who are 'unsuitable' for chemo-immunotherapy, and relapsed/refractory patients who have received at least one prior therapy.

##### **Record of the meeting**

A form of consumer hearing was also held with representatives from the Australian patient support group for WM (WMozzies), prior to the PBAC meeting. This was informed by a Rapid Consumer Evidence Brief prepared by the Consumer Evidence and Engagement Unit of the Department of Health, drawing on discussions held with WMozzies representatives concerning the population to have access to treatment, current challenges with existing therapies and experience of zanubrutinib.

The following is a summary of the perspectives presented to PBAC representatives:

- There are currently limited treatment options for patients with WM, and available treatment options have high toxicity. The side effects of zanubrutinib were considered relatively minor (e.g. bruising), and there was some suggestion of reduced side effects compared with ibrutinib (in terms of diarrhoea and atrial fibrillation, which were noted in international patient forums).
- Given that WM is a rare condition, the PBS restriction should ideally allow the broadest access possible, including those previously treated with a Bruton Tyrosine Kinase inhibitor (BTKi). It was considered likely that BTKi options such as zanubrutinib would be preferred to chemotherapy for both tolerability and oral formulation. Uptake was expected to be high.
- Although WM has a relapsing nature, it is the quality of life between relapsing periods that is greatly valued by those with the disease. WM makes people feel extremely tired and fatigued and the representatives noted that zanubrutinib had given individuals back their energy, allowed them to feel like themselves again and to participate in activities. It was noted that chemotherapy does not have the same benefits around quality of life and individuals continue to feel tired and unwell.
- In terms of long-term outcomes, representatives noted that the submission did not include long-term data, but explained that several WMozzies members had been treated with zanubrutinib for three to six years and remained stable, with similar experiences reported overseas.