This submission presents the case that the PBAC Guidelines review should include:

(1) A review of how data from patients and their carers (consumers) are collected and presented to PBAC, why (the purpose), and by what means (the mechanisms)

(2) A chapter on methods to systematically research patient provided data to be included in the guidance, with considerations of 'Can research methods capture 'patient value' to the satisfaction of patients and their representatives?' and whether scientific/social science research fulfils the purposes of consumer involvement or is additional to?'

(3) Whether a disease and its management is more a population or public health issue than an issue for individual consumers or consumer support groups, as with type 2 diabetes? In which case, it should be treated as such, with quality data collection (including registries as demonstrated in Italy) and pooling of datasets to enable effective evidence based monitoring and management to inform HTA decisions about quality use of medicines – in line with the National Medicines Policy.

The environment

At a health system level the importance of the consumer voice in healthcare decision-making is well recognised. Consumer experts on advisory committees are a well-established and important inclusion. Internationally consumer engagement in health technology assessment processes (HTA) is shown to add value to the decision making, where regulatory and HTA bodies are moving beyond a more traditional engagement with individuals who serve as spokespersons for a health condition and are able to represent the views of others and ask key question to include a more evidence-based means of understanding the range of patients' experiences. This is at different stages of a disease or condition, addressing issues such as unmet medical needs, meaningful treatment benefits, risk tolerance (risk-benefit considerations), and outcome preferences.

Consumers and consumer groups now have the expectation that they will be more meaningfully and responsibly engaged in medical treatment and product decision-making. A strengthened vision of knowledge or evidence for decision-makers includes clinical, economic and patient-based knowledge.

(1) A review of how data from patients and their carers (consumers) are collected and presented to PBAC, why (the purpose), and by what means (the mechanisms)

Greater clarity and explanation is called for on consumer engagement activities and consumer input to address agenda items in PBAC.

(a) The purpose of enabling consumer input into the PBAC agenda should be clearly defined, to inform all stakeholders. For example, is it to legitimise decision making, improve implementation of the decisions, or add value to the health technology assessment (HTA) processes and inform decision making.

Consumers have unique experience of living with a health condition and its management, so they are in a position to contribute important knowledge on effectiveness, acceptability and appropriateness from the consumer perspective. They can provide data on functional, social and psychological and mental issues. Furthermore, a typical patient story, done and used well, can provide a concrete meaning to what are somewhat abstract ideas for researchers, economists and policy makers as well as clinicians.

Consumer participation activities that are transparent, have a well-defined purpose, and a clear plan for how information will be shared among PBAC members and staff and utilised are required.

(b) Assuming that this is more than a socio-political gesture and the input is really wanted, then comprehensive input from a broad spectrum of affected consumers from all walks of life should be encouraged.

(i) Robust, well-structured tools and methodologies should be developed to support such activities, and made readilly available. For example, a mandated mutually agreed template for providing the information can assist in the collation and presentation to committee members. In this way the input is available to assist in decision making and recommendations. Social media should be added to the tools for disseminating calls for input and research carried out on best use of this medium.

(ii) Provision of quality background information on the new drug by the sponsor is required so that consumer groups are well-informed and not placed at a disadvantage to all other stakeholders. By provision of clinical trial data, consumer groups can address research design, populations studied, outcomes of interest in their input, making it richer in content, usefulness and applicability.

In summary, procedural fairness, with well-defined processes for receiving, processing and presenting submissions; encouraging submissions to be representative of the affected population; and working toward efficient and effective use of time and resources (from the perspective of all involved), is required. Consumer participation activities should capture perspectives that are representative of the range of consumer experiences, and be accessible to those wishing to engage. Use of technologies and other approaches should be investigated, both by PBAC and consumer groups.

The present online template was developed in association with seeking consumer input but is very restrictive and few use the template. This creates variation in the materials received; and testimonies and multiple copies of similar letters asking for medicines to be made available. The multiple ways of receiving the information makes the input more difficult to summarise in a meaningful, concise manner for the meeting; and some submissions may be given greater weighting than others because of presentation and language, for example, making it a far from equitable situation.

(c) Providing consumer input is resource intensive so clear and transparent processes are required for collating the input and for presentation to PBAC. Feedback should be provided to those who make submissions as a matter of course, and to inform and improve future input.

(d) Mechanisms for training and support to provide patient input should be reviewed as part of this process, for example by the PBAC Secretariat, as consumers are important stakeholders in the HTA process.

Consumer groups should be encouraged to run their own surveys and interviews of members, and develop skills in these activities. They should be encouraged to seek information on, for example, consumer attitudes to benefit-risk assessments, patient preferences and the outcomes measured in clinical trials, size of effect and timeframes. Consumer groups can also consider variability between individual patient perspectives and its importance.

Consumers can be partners for example to corroborate knowledge obtained from the literature or the sponsors in their submissions, provide input on benefit-risk considerations and willingness to accept trade-offs (patient preferences) in the Australian environment.
We need to expand the capacity of all stakeholders – industry, academia, government and consumer organisations – to work in partnership with consumers.

(2) Methods to systematically research patient provided data in the literature

As more studies report on patient preferences and risk-benefit assessments, techniques are needed to identify, assess and, eventually, synthesise results from a diverse set of methodologies for patient perspectives to be more formally elicited and included in HTA.

Preferences for outcomes can be used to inform decision and cost-effectiveness models, while preferences for treatments can inform clinical decisions.

(3) Whether a disease and its management is more a population or public health issue than an issue for individual consumers or consumer support groups, as with type 2 diabetes?

As a population health issue, quality data collection (including registries as demonstrated in Italy) and pooling of datasets to enable effective evidence-based monitoring and management to inform HTA decisions about medicines are required.

Consumer groups can work with other stakeholders on the interpretation of the data collected. Consumer groups can work to identify common beliefs and the assumptions made of daily life experiences with a disease or taking certain drugs, for example looking at commonly used measurements or outcomes, so that awareness and diagnosis increase.

Overall
Multi-stakeholder outreach is one of the process tools of HTA. A clear vision of what productive, meaningful consumer participation and engagement looks like will best enable all stakeholders to contribute to the value of PBAC recommendations and the transparency of processes used.