As a patient with a life-long genetic condition I have been highly engaged with the health system since birth. My care requires multidisciplinary care including numerous specialists, physiotherapists, dieticians and pharmacists. My life depends on a carefully managed regime of medications including intermittent IV therapy and taking up to thirty individual medications at home on a daily basis which I access through the Pharmaceutical Benefits Scheme (PBS) and Special Access Schemes (SAS). I consider myself an educated and motivated patient and take a proactive approach in my care, including remaining up to date with the latest health technologies and treatment options that could benefit me.

I also endeavour to empower other patients and educate health professionals about the patient experience as a health coach and professional speaker. I connect and hear the experiences of patients on a daily basis through my online patient communities where I have a reach of over 3000 people.

I am very optimistic and enthusiastic about the shift from both government and health care providers towards patient-centred care. As a consumer it is important to me that healthcare pathways reflect my values and can provide me with meaningful and optimal health outcomes. This is particularly important as medications shift to a more personalised, gene focused approach which often result in personalised responses.

I have benefited from one of these personalised medications in recent years and can confidently state that improvements in my quality of life are far more relevant and impactful to me that anything that can be quantified in study endpoints. My experience on this medication has helped me understand the need for the consumer voice to be better represented in the PBAC process and other means of health technology assessment. If a medication, such as the one I have been on achieves success in clinical outcomes but not in terms of the outcomes that matter to patients (particularly quality of life), I would argue that from the patient perspective it has limited impact.

The introduction of consumer hearings to the PBAC process allowed me to share my experience on the trial medication at the beginning of March 2016. This was a very positive experience and reassured me that the depth of the outcomes that mattered to me would be considered and hold influence in the assessment process. This type of input is of the highest importance to patients and should continue to be included and expanded as part of the PBAC approval process.

I think it is important to note here that my involvement in this hearing was greatly supported by my involvement in a health forum (Room With A Patient View) a few weeks prior. The forum gave me a greater insight into what was involved in the process of evaluating

medications and what the PBAC deemed relevant. Even as an involved and motivated patient I believe it is difficult to access information about the PBAC process including the submission process in a consumer-orientated format. An example of this is the PBAC process documentation of guidelines which is available only for sponsors and fails to include consumer specific information regarding process and opportunities for contribution.

Access to information and the way information is presented is a considerable barrier to patients engaging with the PBAC process. This should not be seen as a lack of interest or willingness from patients to have their experiences represented. This was also the case in compiling this submission which I was reluctant to participate in due to my lack of technical knowledge and language and the difficulty determining, as a consumer, what would be relevant to include.

While it is undoubtedly positive to have a greater emphasis on the consumer voice, it is important that this is done in a way that is accessible and appropriate for this cohort.

There are a number of areas where the PBAC guidelines could better reflect the patient view and experience in the HTA process both in terms of the number of patients engaged and the extent and effectiveness of their voice. This would ensure Australia remains a leader in this area and continues to reflect the way involving patients in HTA ensures decisions are in touch and relevant with the needs of patients and enhances functionality of the health care they receive.

1. The submission process to the PBAC involve the consumer perspective in a greater capacity. At present the submission process largely reflects the sponsors perception of a conditional based predominantly upon clinical indicators. Integrating a greater emphasis on patient experience would allow for a critical insight into what the patient deems the impact of their disease. Provision of this information allows patients to describe the meaningful outcomes and relevance of a treatment and potentially comparison and analysis between alternative treatments. Additionally, sponsors would be able to more accurately reflect the impact their product has in an efficient and wholistic manner.

2. The PBAC process needs to systematically value and include the consumer voice. While it is commendable that there has been progress in this area with the introduction of consumer hearings and an additional consumer representative, the process is yet to formally acknowledge the relevance of the consumer perspective. The process is still largely based upon limited economic determinants and analysis of quantitative data. This approach fails to adequately evaluate the impact on quality of life – particularly

emotional wellbeing and the ability for individuals to have greater social participation and/or productivity, the greater societal impact such as the impact on care givers and service providers and the potential for a treatment to provide time/clinical improvement that would enable a patient to access further treatment pathways. These are all important indicators for patients in reflecting the burden of their disease.

3. The barriers to patient involvement in the PBAC process that stem from limitations around information flow and appropriate provision of information and education targeted at consumers need to be acknowledged and remedied by systematic and cultural change. Provision of clear, concise information to patients in a format that is worded appropriately and widely accessible (online and in hard copy through both patient advocacy groups and through healthcare providers) about the PBAC process would increase the capacity of patients to engage. This could also improve consumer confusion about the responsibility of information provision as at present there is lack of universal understanding whether this is the role of government, industry or patient advocate groups.

It is worth noting that in the patient experience, restrictions on industry as a provider of information do not limit the flow of information but reduce the quality of it, particularly through patient led online information sharing. Changes could be made around this to ensure standards of ethical behaviour were upheld yet patients or patient advocate groups received relevant information that could enhance community education and encourage consumer input.

Australia has a robust and pragmatic approach to HTA that recognises the role and importance of patient involvement. The advancement of health technology to address health at a highly targeted and personal level and the increased cost associated with therapies in particular, calls for greater depth to the assessment process. Changes to the PBAC guidelines that reflect a more collaborative approach to the HTA process and values the consumer voice and experience would support optimal health outcomes by ensuring decisions are relevant and representative of meaningful outcomes to the people who depend on them.