

17 June 2021

## Stakeholders Unite

For one hour yesterday the key stakeholders in the Trikafta negotiations met in Canberra to consider the hurdles and challenges blocking a positive recommendation by the PBAC.

Senior representatives from the Department of Health and the Pharmaceutical Benefits Advisory Committee (PBAC); three members of the Vertex team and Professor Peter Wark were present. Also in attendance was Michelle Philpotts, parent of two children with CF; Pia Sappl who lives with cystic fibrosis (CF) every day and myself. This stakeholder assembly met at The Rex Hotel for a frank and honest exchange of ideas.

Everyone was committed to an open dialogue and to exploring new options – after all, we share a deep urgency about the fate of Trikafta in Australia. The complexity of the issue meant that no firm and unanimous resolutions could be arrived at, but the key holdups can now be clearly outlined.

The three issues still hampering a positive recommendation focus are long term data; patient numbers and the details of Managed Access Program (MAP).

Professor Peter Wark spoke about data and research comparing Trikafta to other modulators and he also shared information on international health outcomes. It was clear to all that Trikafta will change lives for the better and extend lives so that people with CF can have a fulfilling future.

Cystic Fibrosis Australia (CFA), supported by Prof. Wark, made the following commitment to the Department of Health, the PBAC and to Vertex ... using Australian Cystic Fibrosis Data Registry (ACFDR) data, CFA will provide regular information on patient numbers and Trikafta onboarding and discontinuations.

Michelle and Pia were outstanding as our CF community representatives, and their compelling life experience constitutes real world evidence for the impact of CF of individuals and families.

Pia and Michelle asked for immediate compassionate access once a PBAC positive recommendation was given and reminded the group that even delays of ‘days’ not ‘months’ negatively impacted people’s health and life expectancy.

Our consumer representatives, together with CFA, implored Vertex to resubmit to the July PBAC meeting and to be creative and innovative when considering MAP criteria. Vertex needs to make this work. We will wait and see if our pleas are heard and acknowledged.

Tomorrow the minutes from the May Intracycle PBAC meeting will be made public. CFA will be sharing more analysis of these important results next week.

Trikafta is still not in our hands, but this week we have moved even closer to broad access. All parties at the Stakeholder Meeting are campaigning strategically and wholeheartedly for a swift resolution.

Kind regards

A handwritten signature in black ink, appearing to read "Nettie Burke". The signature is fluid and cursive, with the first name "Nettie" being more prominent than the last name "Burke".

Nettie Burke  
CEO  
Cystic Fibrosis Australia  
0404034294  
[nettieb@cfa.org.au](mailto:nettieb@cfa.org.au)