Pharmaceutical Benefits Advisory Committee

Reply to: PBAC@health.gov.au

Ms Nettie Burke
Chief Executive Officer
Cystic Fibrosis Australia
nettieb@cfa.org.au

Dear Ms Burke

Thank you for your letter of 27 August 2021 concerning the consideration of Trikafta® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) for patients with cystic fibrosis (CF) by the Pharmaceutical Benefits Advisory Committee (PBAC) at its July 2021 meeting. I apologise for the delay in responding.

Thank you for the additional information from the Cystic Fibrosis Research Trust in the United Kingdom and for your continued advocacy for Trikafta to be made accessible to all patients with CF.

The PBAC recognises that patients who are heterozygous for F508del in the cystic fibrosis transmembrane conductance regulator (CFTR) gene with a minimal function mutation (F/MF) do not currently have access to a CFTR modulator treatment through the Pharmaceutical Benefits Scheme (PBS) and is therefore a patient group where clinical need is high. In July 2021, to facilitate access to Trikafta® for this patient population, the PBAC recommended listing for F/MF patients aged 12 years and older, where the PBAC advice to date in relation to the cost-effectiveness and patient estimates was most closely aligned with that requested. The PBAC's recommendation was intended to cover all F/MF patients 12 years and older irrespective of the specific MF mutation.

The PBAC also recognises there is strong support from both patients and clinicians for Trikafta to be subsidised through the PBS for all patients with CF. However, the PBAC could not make a recommendation in July 2021 for listing in the broader population of CF patients aged 12 years and older, who have at least one F508del mutation in the CFTR gene. This is because the parameters that PBAC previously outlined in its advice in March 2021 and May 2021 had not been addressed by Vertex. The PBAC would welcome a proposal from Vertex that aligns with its earlier advice, and the responsibility rests with Vertex to bring forward this resubmission. As you may now be aware, the PBAC has not received an application for Trikafta for the November 2021 PBAC meeting.

The Public Summary Document, which contains more information about PBAC's July 2021 consideration of Trikafta® will be available on the PBS website at: **www.pbs.gov.au** on 29 October 2021 using the search term 'public summary document' and selecting by drug 'elexacaftor + tezacaftor + ivacaftor', or by meeting 'July 2021'.

While a positive PBAC recommendation is an essential requirement in the process of a PBS listing, Vertex must agree to list in accordance with the PBAC's advice and other processes need to be completed before the Trikafta® can be given approval for listing. These include price negotiation with the sponsoring pharmaceutical company, finalisation of costs of the listing with other government agencies, and approval by Government. The Department of Health is working to finalise these arrangements with Vertex and will proceed to listing Trikafta® for the F/MF population as quickly as possible. The PBS is released in the form of an online schedule and is updated on the first day of every month. When Trikafta® is listed on the PBS, the change will appear on the 'Summary of Changes', which can be viewed online at: **www.pbs.gov.au/browse/publications**.
You can also track progress of the PBS listing process on the PBS Medicine Status Website at: **www.pbs.gov.au/medicinestatus/home.html**.

Yours sincerely

Professor Andrew Wilson
PBAC Chair
25 October 2021