28 August 2020

TRACKING TRIKAFTA

Drug access is high on the agenda for all people with cystic fibrosis (CF) and Cystic Fibrosis Australia (CFA) is pleased to provide an update on the status of the Trikafta approval process.

In February this year Vertex submitted Trikafta to the Therapeutics Goods Administration (TGA) for approval. CFA can confirm that the submission parameters included all people over the age of 12 years with at least one copy of the F508del gene mutation.

The TGA is part of the Department of Health and is responsible for regulating prescription medicines, vaccines, vitamins and minerals, medical devices, blood and blood products. The TGA does this through pre-market assessment and reviews of clinical trial data, post-market monitoring and the enforcement of standards to evaluate the safety and performance of a product and manage any risks to consumers.

Generally, the TGA's safety and efficacy process takes approximately 12 months and once approval is secured a drug can proceed to the Pharmaceutical Benefits Advisory Committee (PBAC). The PBAC is an independent body of doctors, health professionals, health economists and consumer representatives.

The PBAC's main role is to recommend new medicines for listing on the Pharmaceutical Benefits Scheme (PBS). No new medicine can be listed unless the Committee makes a positive recommendation. The PBAC meets three times a year and we hope that Trikafta will be listed on the March 2021 Agenda. We will know if this is the case on 23 December 2020 when the PBAC Agenda is published. From that point our consumer comments will all need to be submitted by mid February 2021.

The PBAC considers four key issues …

1. the medical conditions the medicine will benefit
2. its clinical effectiveness
3. safety and side effects, and
4. cost-effectiveness or 'value for money' compared to other treatments.

Six weeks after the PBAC meeting its 'outcomes' are published in a Public Summary Document. There are three potential 'outcomes' – Recommendation, Deferral and Rejection – and each of these then takes a different drug access pathway.

A 'Recommendation' is of course what we are all hoping for and this allows the price negotiations to begin in earnest. CFA hopes that Trikafta is approved at the March 2021 PBAC meeting and a commercial deal can be struck within six months.

CFA constantly monitors the Heath Technology Assessment (HTA) process and we have plans for a strong advocacy campaign to support the Trikafta submission. If you would like to be involved in the campaign please email Nicki at CFA on nickim@cfa.org.au. By contributing your thoughts, concerns and constructive advice you will be a valued member of a dedicated team.

The more campaigners, the louder our voice and in the past we have proven our clarion calls are not to be ignored!

Kind regards

Nettie Burke
CEO
Cystic Fibrosis Australia
0404034294
nettieb@cfa.org.au