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Open Letter to Cystic Fibrosis Community in Australia: Access for All

We wanted to update you following our intensive work with the Department of Health and PBAC over the past few months on Trikafta access.

Vertex is committed to ensuring *all* who can benefit from Trikafta® get access as quickly as possible. We have achieved broad access now in more than twenty countries around the world and believe Australians living with CF deserve the same.

At their meeting in July, the PBAC made a limited recommendation for Trikafta to a small subset of patients who are heterozygous for F508del in the CFTR gene with a minimal function (F/MF) mutation aged 12 years and older. This narrow recommendation meant 80% of the 2,200 eligible CF patients would be denied Government-funded access to Trikafta.

Following this outcome, we have had many discussions with government stakeholders in an effort to identify a path forward for not only those included in the July PBAC recommendation, but also the remaining 1,800 patients not included. We have put forward a number of solutions, which unfortunately so far have not been accepted.

Given that we want to ensure an equitable approach to access to Trikafta, last week we made a further resubmission to the PBAC, with new data across all genotypes. We believe this solution is the best opportunity to provide fast and equitable government-funded access to Trikafta for all 2,200 eligible patients.

We urge the PBAC and the Department of Health to accept the Trikafta resubmission for the December Intracycle Meeting, alongside the additional Orkambi® data requested by the PBAC at their July meeting, rather than holding it over until March next year.

Access to medicines in Australia is complex, challenging and takes time. Please be assured that the team at Vertex will continue to work tirelessly until we achieve reimbursed access to Trikafta for all eligible Australians.

Kind regards

The Vertex Team