2 August 2023

CONSULTATION IS OPEN FOR THE NOVEMBER 2023 PBAC AGENDA

The agenda for the November 2023 PBAC meeting has been published and the request for Kalydeco (Ivacaftor) to be extended has been listed.

The submission for Kalydeco includes an extension of treatment access to CF patients aged 4 to 12 months and are shown to be responsive to Ivacaftor potentiation. The list of responsive mutations includes rare mutations as well as R117H and RF mutations (irrespective of the presence of an F allele).

Approximately 85 patients will gain access to a CFTR modulator for the first time.

Whilst many people will already have access to other modulators, this extension will also benefit those who may not respond well to these, as well as providing earlier access to life-changing treatment.

Consumer Comments are now being accepted by the PBAC and this is your chance to help make a difference in the lives of people living with cystic fibrosis.

Please note when providing your submission:

- Share what you know including lived experiences and be specific about symptoms, how you feel, and what your daily needs are.

- Explain the difficulties and realities of living with CF and why access is important to you.

Input can be provided via the Office of Health Technology Assessment hub at:

https://ohta-consultations.health.gov.au/ohta/pbac-nov-2023/

As part of your submission, please consider commenting on access to Trikafta, and urging Australia to align this with other countries which already have access granted to a wider range of rare gene mutations. Consumer Comments close on 20 September 2023.

PBAC Agenda

Drug Name, form(s), strength(s) and Sponsor, Submission type (Drug name, form, strength, Trade name®, Sponsor, new listing/change to listing)

IVACAFTOR
Granules 25 mg
Granules 50 mg
Granules 75 mg
Tablet 150 mg
Kalydeco®
VERTEX PHARMACEUTICALS (AUSTRALIA) PTY. LTD.
(New listing)

Drug Type and Use

(What is the drug used to treat?)

Cystic fibrosis (CF) with the CF transmembrane conductance regulator (CFTR) gene mutation

PBAC Listing/Purpose of Submission

To request a Section 100 (HSD) Authority Required (Written) listing for the treatment of CF patients aged 4 to 12 months with a gating (Class III) CFTR gene mutation; and CF patients aged 4 months or older with at least one CFTR gene mutation shown to be responsive to ivacaftor potentiation.

Table 6: *CFTR* gene mutations that produce CFTR protein and are responsive to ivacaftor

A120T	*F311del*	*I148T*	*R75Q*	*S589N*
A234D	*F311L*	*I175V*	*R117C**	*S737F*
A349V	*F508C*	*I807M*	*R117G*	*S945L**
*A455E**	*F508C;S1251N*†	*I1027T*	*R117H**	*S977F**
A1067T	*F1052V*	*I1139V*	*R117L*	*S1159F*
D110E	*F1074L*	*K1060T*	*R117P*	*S1159P*
D110H	*G178E*	*L206W**	*R170H*	*S1251N**
D192G	*G178R**	*L320V*	*R347H**	*S1255P**
*D579G**	*G194R*	*L967S*	*R347L*	*T338I*
D924N	*G314E*	*L997F*	*R352Q**	*T1053I*
*D1152H**	*G551D**	*L1480P*	*R553Q*	*V232D*
D1270N	*G551S**	*M152V*	*R668C*	*V562I*
E56K	*G576A*	*M952I*	*R792G*	*V754M*
E193K	*G970D*	*M952T*	*R933G*	*V1293G*
E822K	*G1069R*	*P67L**	*R1070Q*	*W1282R*
	*G1244E**	*Q237E*	*R1070W**	*Y1014C*
	G1249R	*Q237H*	*R1162L*	*Y1032C*
	*G1349D**	*Q359R*	*R1283M*	
	H939R	*Q1291R*	*S549N**	
	H1375P	*R74W*	*S549R**	

*Clinical data exist for these mutations [see Clinical efficacy].
† Complex/compound mutations where a single allele of the CFTR gene has multiple mutations; these exist independent of the presence of mutations on the other allele.