PHARMAC
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Information sheet for ivacaftor (Kalydeco)

PHARMAC has announced a decision to fund ivacaftor (Kalydeco) tablets and granules for the treatment of patients with cystic fibrosis with the G551D mutation (or other class III gating mutations). Ivacaftor will be listed on the Pharmaceutical Schedule as soon as practicable following Medsafe regulatory approval.

In the meantime, from 1 March 2020 PHARMAC will consider applications for ivacaftor (Kalydeco) under the Exceptional Circumstances (EC) Framework for individual patients who meet the following criteria:

Access criteria:
Applications from respiratory specialist or paediatrician.
Approvals valid without renewal unless notified for applications meeting the following criteria:

All of the following:

1. Patient has been diagnosed with cystic fibrosis; and
2. Either:
   2.1. Patient must have G551D mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene on at least 1 allele; or
   2.2. Patient must have other gating (class III (G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N and S549R)) mutation in the CFTR gene on at least 1 allele; and
3. Patients must have a sweat chloride value of at least 60 mmol/L by quantitative pilocarpine iontophoresis or by Macroduct sweat collection system; and
4. Treatment with ivacaftor must be given concomitantly with standard therapy for this condition; and
5. Patient must not have an acute upper or lower respiratory infection, pulmonary exacerbation, or changes in therapy (including antibiotics) for pulmonary disease in the last 4 weeks prior to commencing treatment with ivacaftor; and
6. The dose of ivacaftor will not exceed one tablet or one sachet twice daily; and
7. Applicant has experience and expertise in the management of cystic fibrosis.

This process will remain in place until such time as ivacaftor is listed on the Pharmaceutical Schedule (following regulatory approval).

It is important to note that prior to regulatory approval, the supply, sale and prescribing of ivacaftor tablets and granules will need to meet the requirements in Section 25 and 29 of the Medicines Act 1981. Information about the use of unapproved medicines and the obligations of the prescriber for use of an unapproved medicine can be found on the Medsafe website.

Following Medsafe approval, ivacaftor will be listed as a PCT only-Specialist pharmaceutical in Section B of the Pharmaceutical Schedule, meaning that only DHB hospitals will be able to make subsidy claims.

Therefore, we will only consider EC applications for dispensing through a DHB hospital pharmacy.

Any EC approvals will be converted to standard Special Authority approvals following listing on the Schedule and applicants will be advised of the new approval number.

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Application form for ivacaftor (Kalydeco) for cystic fibrosis with the G551D mutation (or other class III gating mutations)

Return completed form to:
Exceptional Circumstances
PHARMAC
PO Box 10-254
WELLINGTON
Phone: 0800 023 588, option 6
Fax: 04 380 1409
Email: cftreatment@pharmac.govt.nz

Patient Details	**Details of Applying Practitioner**
Last name:	Last name:
First Name:	First name:
Address:	Address:
Gender:	Phone:
Date of Birth:	Facsimile: NZMC#:
NHI No:	Email address:

Application (check boxes where appropriate)

Patient has been diagnosed with cystic fibrosis		☐
Either	Patient must have G551D mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene on at least 1 allele	☐
Or	Patient must have other gating (class III (G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N and S549R)) mutation in the CFTR gene on at least 1 allele	☐
Patients must have a sweat chloride value of at least 60 mmol/L by quantitative pilocarpine iontophoresis or by Macroduct sweat collection system		☐
Treatment with ivacaftor must be given concomitantly with standard therapy for this condition		☐
Patient must not have an acute upper or lower respiratory infection, pulmonary exacerbation, or changes in therapy (including antibiotics) for pulmonary disease in the last 4 weeks prior to commencing treatment with ivacaftor		☐
The dose of ivacaftor will not exceed one tablet or one sachet twice daily		☐
Applicant has experience and expertise in the management of cystic fibrosis		☐

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Medicine and Dosage details:

☐ Please indicate if required	☐ Please indicate if required	☐ Please indicate if required
Form: Tablet	**Form:** Granules	**Form:** Granules
Strength: 150mg	**Strength:** 50mg, sachet	**Strength:** 75mg, sachet
Pharmacode: 2586118	**Pharmacode:** 2586134	**Pharmacode:** 2586126
Dosage required:	**Dosage required:**	**Dosage required:**

Nominated hospital pharmacy

Where will supplies be obtained if approval of this treatment is granted? (This will be a hospital pharmacy):

Name:
DHB:
Address:
Phone:

Signature of Medical Practitioner: _____

Date of Request: _____

Ivacaftor is not approved by Medsafe

The supply, sale and prescribing of ivacaftor tablets and granules need to meet the requirements in Section 25 and 29 of the Medicines Act 1981 until such time that they are Medsafe approved.

Information about the use of unapproved medicines and the obligations of the prescriber for use of an unapproved medicine can be found on the Medsafe website:
https://www.medsafe.govt.nz/profs/RIss/unapp.asp

☐ Applicant is aware of unapproved regulatory status of ivacaftor and has met the requirements of the Medicines Act 1981 in regard to prescribing ivacaftor for this patient, including patient (or legal guardian) consent.