

## Package leaflet: Information for the patient

**Kalydeco 13.4 mg granules in sachet**  
**Kalydeco 25 mg granules in sachet**  
**Kalydeco 50 mg granules in sachet**  
**Kalydeco 59.5 mg granules in sachet**  
**Kalydeco 75 mg granules in sachet**  
Ivacaftor

**Read all of this leaflet carefully before your child starts taking this medicine because it contains important information for your child.**

- Keep this leaflet. You may need to read it again.
- If you have any further questions, ask your child's doctor or pharmacist.
- This medicine has been prescribed for your child only. Do not pass it on to others. It may harm them, even if their signs of illness are the same as your child's.
- If your child gets any side effects, talk to your child's doctor or pharmacist. This includes any possible side effects not listed in this leaflet. See section 4.

### What is in this leaflet

1. What Kalydeco is and what it is used for
2. What you need to know before your child takes Kalydeco
3. How to take Kalydeco
4. Possible side effects
5. How to store Kalydeco
6. Contents of the pack and other information

#### 1. What Kalydeco is and what it is used for

Kalydeco contains the active substance ivacaftor. Ivacaftor acts at the level of the cystic fibrosis transmembrane conductance regulator (CFTR), a protein that forms a channel at the cell surface that allows the movement of particles such as chloride in and out of the cell. Due to mutations in the *CFTR* gene (see below), chloride movement is reduced in those with cystic fibrosis (CF). Ivacaftor helps certain abnormal CFTR proteins open more often to improve chloride movement in and out of the cell.

Kalydeco granules are indicated:

- As monotherapy for the treatment of babies and children aged 1 month and older and weighing 3 kg to less than 25 kg with cystic fibrosis (CF) who have an *R117H* *CFTR* mutation or one of the following gating mutations in the *CFTR* gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R*.
- In combination with ivacaftor/tezacaftor/elixacaftor granules for patients aged 2 to 6 years who have CF, with at least one *F508del* mutation in the *CFTR* gene. If you have been prescribed Kalydeco to be taken with ivacaftor/tezacaftor/elixacaftor, read the package leaflet of the latter. It contains important information about how to take these two medicines.

## 2. What you need to know before your child takes Kalydeco

### Do not give your child Kalydeco

- if your child is allergic to ivacaftor or any of the other ingredients of this medicine (listed in section 6).

### Warnings and precautions

Talk to your child's doctor before your child takes Kalydeco.

- Talk to your child's doctor if your child has liver problems or has had them previously. Your child's doctor may need to adjust your child's dose.
- Increased liver enzymes in the blood have been seen in some people receiving Kalydeco. Tell your child's doctor right away if your child has any of these symptoms, which may be a sign of liver problems:
  - Pain or discomfort in the upper right stomach (abdominal) area
  - Yellowing of the skin or the white part of the eyes
  - Loss of appetite
  - Nausea or vomiting
  - Dark urine

Your child's doctor will do some blood tests to check your child's liver before and during treatment, particularly during the first year and especially if blood tests showed high liver enzymes in the past.

- Talk to your child's doctor if you have been told your child has kidney problems or has previously had them.
- Kalydeco is not recommended for patients who have undergone an organ transplant.
- Abnormality of the eye lens (cataract) without any effect on vision has been noted in some children and adolescents during treatment.

Your child's doctor may perform some eye examinations prior to and during treatment with ivacaftor.

### Children

Do not give this medicine to children under 1 month of age as it is not known if ivacaftor is safe and effective in these children.

Do not give this medicine in combination with ivacaftor/tezacaftor/elexacaftor to children under 2 years of age as it is not known if they are safe and effective for them.

### Other medicines and Kalydeco

Tell your child's doctor or pharmacist if your child is using, has recently used or might use any other medicines. Some medicines can affect how Kalydeco works or make side effects more likely. In particular, tell your child's doctor if your child is taking any of the medicines listed below. Your child's doctor may decide to adjust your child's dose or if extra check-ups are needed.

- **Antifungal medicines** (used for the treatment of fungal infections). These include fluconazole, itraconazole, ketoconazole, posaconazole, and voriconazole.

- **Antibiotic medicines** (used for the treatment of bacterial infections). These include clarithromycin, erythromycin, rifabutin, rifampicin, and telithromycin.
- **Epilepsy medicines** (used for the treatment of epileptic seizures or fits). These include carbamazepine, phenobarbital, and phenytoin.
- **Herbal medicines**. These include St. John's wort (*Hypericum perforatum*).
- **Immunosuppressants** (used after an organ transplantation). These include ciclosporin, everolimus, sirolimus, and tacrolimus.
- **Cardiac glycosides** (used for the treatment of some heart conditions). These include digoxin.
- **Anticoagulant medicines** (used to prevent blood clots). These include warfarin.
- **Medicines for diabetes**. These include glimepiride and glipizide.
- **Medicines for lowering blood pressure**. These include verapamil.

### **Kalydeco with food and drink**

Avoid giving your child food or drink containing grapefruit during treatment with Kalydeco as they may increase the side effects of Kalydeco by increasing the amount of ivacaftor in your child's body.

### **Driving and using machines**

Kalydeco can make your child dizzy. If your child feels dizzy, it is advised that your child does not ride his/her bike or do anything else that needs his/her full attention.

### **Kalydeco contains lactose and sodium.**

If you have been told by your child's doctor that your child has an intolerance to some sugars, contact your child's doctor before your child takes this medicine.

Kalydeco contains less than 1 mmol sodium (23 mg) per dose, that is to say essentially 'sodium-free'.

## **3. How to take Kalydeco**

Always give your child this medicine exactly as your child's doctor has told you to. Check with your child's doctor if you are not sure.

Your child's doctor will determine the correct dose for your child. Your child must keep using all other medicines, unless your child's doctor tells him/her to stop using any.

Kalydeco dosing recommendations are provided in Table 1.

**Table 1: Dosing recommendations**

<b>Age</b>	<b>Weight</b>	<b>Morning dose</b>	<b>Evening dose</b>
<b>Kalydeco as monotherapy</b>			
1 month to less than 3 months	≥ 3 kg	One sachet of ivacaftor 13.4 mg granules	One sachet of ivacaftor 13.4 mg granules
3 months to less than 6 months	≥ 3 kg	One Kalydeco sachet of 25 mg granules	One Kalydeco sachet of 25 mg granules
6 months and older	≥ 5 kg to < 7 kg	One Kalydeco sachet of 25 mg granules	One Kalydeco sachet of 25 mg granules
	7 kg to < 14 kg	One Kalydeco sachet of 50 mg granules	One Kalydeco sachet of 50 mg granules

	14 kg to < 25 kg	One Kalydeco sachet of 75 mg granules	One Kalydeco sachet of 75 mg granules
	≥ 25 kg	Please refer to Kalydeco tablets Package Leaflet	
<b>Kalydeco in combination with ivacaftor/tezacaftor/elexacaftor</b>			
2 years to less than 6 years,	< 14 kg	One sachet of ivacaftor 60 mg/tezacaftor 40 mg/elexacaftor 80 mg granules	One Kalydeco sachet of 59.5 mg granules
	≥ 14 kg	One sachet of ivacaftor 75 mg/tezacaftor 50 mg/elexacaftor 100 mg granules	One Kalydeco sachet of 75 mg granules

Give your child the morning and evening granules about 12 hours apart.

**If your child has liver problems**, your child's doctor may need to reduce the dose of Kalydeco as your child's liver will not clear the medicine as fast as in children who have normal liver function.

- **Moderate liver problems in children 6 months of age or older:** the dose may be reduced to one half of the indicated dose in the table above, that is one sachet once daily.
- **Severe liver problems in children 6 months of age or older:** the use is not recommended but your child's doctor will decide if it is appropriate for your child to use this medicine in which case the dose (as indicated in the table above) must be reduced to one sachet every other day.
- **Liver problems in children between 4 months and 6 months of age:** the use is not recommended but your child's doctor will decide if it is appropriate for your child to use and what dose your child should have.
- **Liver problems in children between 1 month and 4 months of age:** the use is not recommended.

Kalydeco is for oral use.

Each sachet is for single use only.

Giving Kalydeco to your child:

- Hold sachet of granules with cut line on top.
- Shake sachet gently to settle contents.
- Tear or cut sachet open along cut line.
- Mix the entire content of a sachet with 5 mL of age-appropriate soft food or liquid. Food or liquid should be at room temperature or below. Some examples of age-appropriate soft foods or liquids include puréed fruits or vegetables, yogurt, applesauce, water, milk, breast milk, infant formula, or juice.
- Once mixed, give the product to your child immediately. If this is not possible, give it within the following hour after mixing. Ensure that the mixture is completely and immediately consumed.
- A fat-containing meal or snack should be given to your child just before or just after dosing (some examples are provided below).

Meals or snacks that contain fat include those prepared with butter or oils or those containing eggs. Other fat-containing foods are:

- Cheese, whole milk, whole-milk dairy products, yogurt, breast milk, infant formula, chocolate

- Meats, oily fish
- Avocados, hummus, soy-based products (tofu)
- Nuts, fat-containing nutritional bars or drinks

### **If your child takes more Kalydeco than he/she should**

Your child may experience side effects, including those mentioned in section 4 below. If so, contact your child's doctor or pharmacist to ask for advice. If possible, have your child's medicine and this leaflet with you.

### **If you forget to give your child Kalydeco**

Give the missed dose if less than 6 hours have passed since the time your child missed the dose. Otherwise, wait until your child's next scheduled dose as you normally would. Do not give your child a double dose to make up for a forgotten dose.

### **If you stop giving your child Kalydeco**

Give Kalydeco to your child for as long as your child's doctor recommends. Do not stop unless your child's doctor advises you to. If you have any further questions on the use of this medicine, ask your child's doctor or pharmacist.

## **4. Possible side effects**

Like all medicines, this medicine can cause side effects, although not everybody gets them.

### **Serious side effects**

Stomach (abdominal) ache and increased liver enzymes in the blood.

### **Possible signs of liver problems**

Increased liver enzymes in the blood are common in patients with CF. These may be signs of liver problems:

- Pain or discomfort in the upper right area of the stomach (abdominal) area
- Yellowing of the skin or white part of the eyes
- Loss of appetite
- Nausea or vomiting
- Dark urine

Tell your child's doctor straight away if he/she gets any of these.

### **Very common side effects (may affect more than 1 in 10 people)**

- Upper respiratory tract infection (the common cold), including sore throat and nasal congestion
- Headache
- Dizziness
- Diarrhoea
- Stomach or abdominal pain
- Changes in the type of bacteria in mucus
- Increased liver enzymes (signs of stress on the liver)
- Rash

**Common** side effects (may affect up to 1 in 10 people)

- Runny nose
- Ear pain, ear discomfort
- Ringing in the ears
- Redness inside the ear
- Inner ear disorder (feeling dizzy or spinning)
- Sinus problems (Sinus congestion)
- Redness in the throat
- Breast mass
- Flu
- Low blood sugar (hypoglycaemia)
- Abnormal breathing (shortness of breath or difficulty breathing)
- Wind (flatulence)
- Spots (Acne)
- Itchy skin
- Increased creatine phosphokinase (sign of muscle breakdown) seen in blood tests

**Uncommon** side effects (may affect up to 1 in 100 people)

- Ear congestion
- Breast inflammation
- Enlargement of the breast in males
- Nipple changes or pain
- Wheezing
- Increased blood pressure

**Additional side effects in children and adolescents**

Side effects seen in children and adolescents are similar to those observed in adults. However, increased liver enzymes in the blood are more frequently seen in young children.

**Reporting of side effects**

If your child gets any side effects, talk to your child's doctor or pharmacist. This includes any possible side effects not listed in this leaflet. You can also report side effects directly via:

Yellow Card Scheme

Website: [www.mhra.gov.uk/yellowcard](http://www.mhra.gov.uk/yellowcard) or search for MHRA Yellow Card in the Google Play or Apple App Store.

By reporting side effects you can help provide more information on the safety of this medicine.

**5. How to store Kalydeco**

Keep this medicine out of the sight and reach of children.

Do not use this medicine after the expiry date which is stated on the carton, wallet and sachet after EXP. The expiry date refers to the last day of that month.

This medicine does not require any special storage conditions.

Once mixed, the mixture has been shown to be stable for one hour.

Do not throw away any medicines via wastewater or household waste. Ask your pharmacist how to throw away medicines you no longer use. These measures will help to protect the environment.

## **6. Contents of the pack and other information**

### **What Kalydeco contains**

#### Kalydeco 13.4 mg granules in sachet:

The active substance is ivacaftor. Each sachet contains 13.4 mg of ivacaftor.

#### Kalydeco 25 mg granules in sachet:

The active substance is ivacaftor. Each sachet contains 25 mg of ivacaftor.

#### Kalydeco 50 mg granules in sachet:

The active substance is ivacaftor. Each sachet contains 50 mg of ivacaftor.

#### Kalydeco 59.5 mg granules in sachet:

The active substance is ivacaftor. Each sachet contains 59.5 mg of ivacaftor.

#### Kalydeco 75 mg granules in sachet:

The active substance is ivacaftor. Each sachet contains 75 mg of ivacaftor.

The other ingredients are: silica, colloidal anhydrous, croscarmellose sodium, hypromellose acetate succinate, lactose monohydrate, magnesium stearate, mannitol, sucralose and sodium laurilsulfate (E487).

See the end of section 2 - Kalydeco contains lactose and sodium.

### **What Kalydeco looks like and contents of the pack**

Kalydeco 13.4 mg granules in sachet are white to off-white granules.

Kalydeco 25 mg granules in sachet are white to off-white granules.

Kalydeco 50 mg granules in sachet are white to off-white granules.

Kalydeco 59.5 mg granules in sachet are white to off-white granules.

Kalydeco 75 mg granules in sachet are white to off-white granules.

The granules are supplied in sachets.

Kalydeco 13.4 mg granules in sachet, Kalydeco 25 mg granules in sachet, Kalydeco 50 mg granules in sachet, and Kalydeco 75 mg granules in sachet:

- Pack size of 56 sachets (contains 4 individual wallets with 14 sachets per wallet)

Kalydeco 59.5 mg granules in sachet and Kalydeco 75 mg granules in sachet:

- Pack size of 28 sachets (contains 4 individual wallets with 7 sachets per wallet)

### **Marketing Authorisation Holder**

Vertex Pharmaceuticals (Europe) Limited  
2 Kingdom Street  
London, W2 6BD  
United Kingdom  
Tel: +44 (0)203 204 5100

### **Manufacturer**

Almac Pharma Services (Ireland) Limited  
Finnabair Industrial Estate  
Dundalk  
Co. Louth  
A91 P9KD  
Ireland

Almac Pharma Services Limited  
Seagoe Industrial Estate  
Craigavon  
Northern Ireland  
BT63 5UA  
United Kingdom

**This leaflet was last revised in March 2024**

### **Other sources of information**

Detailed information on this medicine is available on the Medicines and Healthcare products Regulatory Agency website: <http://www.mhra.gov.uk>.