A guide for patients about unlicensed medicines and off-label uses

Why have I been given this leaflet?
You may have been prescribed a medicine that is unlicensed or off-label. Sometimes, a medicine is prescribed in a way that is not covered by its UK marketing licence. This does not mean that it is unsafe to use. To help you understand, we have given you this leaflet. It answers some of the most frequently asked questions. If you would like to talk to anyone about your medicine, please speak to your prescriber or the person issuing or administering the medicine.

Why do medicines have a licence?
In the UK, all drug companies must have a product licence to advertise and sell a medicine. All medicines undergo a process of vigorous checks, known as clinical trials (usually in adults aged 18 to 65), to make sure that they are safe and effective. Information from the clinical trials is then given to a UK government organisation called the Medicines and Healthcare Products Regulatory Agency (MHRA) when the drug companies apply for a licence. The MHRA grants licences for medicines when they pass such checks.

The licence, which is also known as the marketing authorisation, for a medicine will be specific for treating a particular health condition or range of health conditions. The licence will also state the ages of the patients it can be used for, how much to give and how the medicine should be given. The drug company cannot advertise or make any recommendations about using a medicine outside the terms of its licence.

What is meant by unlicensed or off-label use?
An unlicensed medicine is defined as one that has no marketing authorisation for any health conditions in the UK. Some examples of the use of unlicensed medicines are:

- if a medicine is not available in the UK, it has to be imported from a country with similar high standards of licensing as the UK.
- a specially prepared liquid formulation of a medicine for someone who has difficulty swallowing the licensed tablets or capsules.
- some medicines have no licence at all, such as those for treating rare health conditions.

An off-label use of a medicine is when a medicine is being used in a way that is different to that described in the product licence. Some examples of off-label uses are:

- using a medicine for a health condition different to that stated in the licence. This is because doctors may have found that the medicine works very well for this condition. This use may be supported by previous clinical experience or by expert groups’ advice, but the drug manufacturer has not asked the MHRA to extend the licence to cover these conditions.
- using a medicine in an age group outside the licensed range (for example, children, or the elderly).
- using a medicine at a higher dose than stated in the licence. This type of off-label use may occur because some people break down the drug at a quicker rate than others and therefore need a higher dose.
If this medicine works for me, then why isn’t it licensed or why does it not get the terms of its licence changed?

There are various reasons why the medicine you have been prescribed may not be licensed or have an extended licence at this time. These include:

- the licensing process and clinical trials are very expensive. Once a drug is on the market, the drug company may decide, on cost grounds, not to try getting the original licence extended.
- the number of patients with the condition is too few. Due to statistical requirements, clinical trials need to have a certain number of patients involved to prove whether a medicine is effective or not. If there are not enough patients, the evidence will not be considered strong enough to allow the MHRA to make a decision on its effectiveness.
- It may be that the licensing process is already under way for this medicine, but it is a lengthy process and the drug may sometimes be used in clinical practice before the licence is obtained.

What does this mean for me?

The healthcare professional will take into account the relevant medical information and evidence when prescribing or issuing an unlicensed or off-label medicine. This makes sure that the medicine prescribed is appropriate for you. However, any medicine can have side effects. In the UK, all manufacturers are obliged to include an information leaflet with their medical product, which will explain the effects of the medicine. If the medicine you are given is unlicensed or off-label, you may find that the leaflet may not quite relate to you or your health condition. Your healthcare professional may tell you to take the medicine as directed on the label and to consult them if you have any concerns related to your medicine.

What if I do not want to take this medicine?

If you have any concerns about your medicine or treatment, please speak to your healthcare professional. Understanding your concerns will help them to advise you and help you make the right choices for your condition. There may be alternative options and they can discuss these with you.

Where can I find out more?

If the patient information leaflet that comes with your medicine does not relate to your condition then ask your healthcare professional for more information. If the medicine comes from another country and the leaflet is not in English, we may be able to supply one that has been translated. Please speak to the person giving you the medicine or our Medicines Information and Education Team (contact details on page three) for a translated leaflet or if you would like further information.

There are many support groups that can provide information to patients, and there may be one for your particular condition. Please speak to the person giving you the medicine if you would like more information and they may be able to direct you to an appropriate group.

How do I obtain more of this unlicensed medicine?

If you have been prescribed this medicine in hospital, when you leave the doctor at the hospital will ask your GP to prescribe the medicine for you. Your GP will then give you a prescription which you need to take to your community pharmacist in order to get your medicine. If your GP is unable to do this for any reason or if the pharmacist is unable to get hold of the unlicensed medicine then you need to contact the hospital that started the treatment for you for further advice.
Contact us
Medicines Information and Education Team
Phone: 01795 562062, 9am to 5pm, Monday to Friday
Email: kcht.medinfo@nhs.net

Write to us
Faversham Cottage Hospital
Stone Street
Faversham
Kent
ME13 8PS

Do you have feedback about our health services?
Phone: 0300 123 1807
8am to 5pm, Monday to Friday
Text: 07899 903499
Email: kentchf.PALS@nhs.net
Web: www.kentcht.nhs.uk/PALS

Patient Advice and Liaison Service (PALS)
Kent Community Health NHS Foundation Trust
Unit J, Concept Court
Shearway Business Park
Folkestone
Kent CT19 4RJ

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