

Unlicensed and Off-Label Medicines in Children and Neonates

This leaflet is about medicines in children and neonates.

Please read this leaflet carefully. Keep it somewhere safe so that you can read it again.

What is this leaflet about?

Medicines in the UK have a licence that says how the medicine should be used. However, this licence may not include the use of medicines in children, a particular illness, or condition. Sometimes in children it is necessary to prescribe a medicine in a way that is not covered by its UK licence when there is no suitable alternative.

This leaflet explains in more detail what unlicensed and 'off-label' use of a medicines means, why the use of many medicines in children is unlicensed, and when it is considered safe to use unlicensed medicines.

You may wish to discuss this further with your doctor or pharmacist.

What is a medicine?

Medicines are products used to prevent or treat a medical condition. They can come as tablets, capsules, liquids, injections, inhalers, creams, eye drops/ointments, suppositories, and patches.

What is a licence?

A drug company must have a product licence to advertise and sell a medicine.

The licence will state:

- Which illness(es) or condition the medicine can be used for.
- The age of patients it can be given to.

- How much medicine should be given and how to give it (e.g. by mouth, by injection etc.).

A licence is provided by a government organisation called the MHRA (Medicines and Healthcare products Regulatory Agency).

How does a drug company get a licence?

To get a licence, the drug company must use clinical trials to prove that the drug works and is safe. They do this by trying it first in clinical trials, usually in adults. To obtain a licence for use in children, clinical trials involving children are required. Information from the clinical trials is then given to the MHRA when the drug company applies for a licence.

The drug company cannot advertise or make any recommendations about using a medicine outside the terms of its licence.

Clinical trials and the licensing process are very expensive. A drug company may decide not to try to get the original licence extended if it is found to treat other symptoms once it is on the market.

What is meant by "off-label" and unlicensed use?

Unlicensed or "Off-label" use means that the medicine is being used in a way that is different to that described in the licence. Many medicines that are widely used are unlicensed or "off-label". This gives doctors more choice about which medicine to use than if they could only use licensed medicines.

Some examples are:

- Using a medicine for a different illness to that stated in the licence. Doctors may have found that the medicine works very well for this illness or condition. This use may be supported by expert groups, but the drug manufacturer has not extended the licence.
- Using a medicine in an age group outside the licensed range (usually children or the elderly).
- Using a medicine at a higher dose than stated in the licence.
- A medicine has a licence in other countries, but not the UK. It has to be imported because it is not available in the UK.
- The use of a medicine that needs to be made specially because it cannot be obtained easily; for example, a patient may not be able to swallow a tablet or capsule (which is licensed) and needs a liquid (unlicensed) version of the medicine.
- A medicine that has no licence at all. These are often medicines used for treating rare illnesses. The manufacturer may have decided that it was too expensive to carry out the clinical trials or it would be difficult to find enough patients for the clinical trials needed to get a licence.

Why is use of medicines for children often unlicensed?

To get a licence to promote the use of a medicine in children, the drug company has to perform clinical trials that involve children. There are steps in place to help make this happen, but it can be very difficult, especially when the illness is rare. After a medicine has been shown to work for some time in adults, doctors – and other prescribers – will know more about how it works and its possible side-effects and may want to use it to help other illnesses or conditions, or in other groups of patients, for example, elderly patients or children.

In many cases, doctors have to choose a medicine that is unlicensed for your child

This is because there is not a licensed medicine for the illness your child has. Another reason could be that the unlicensed medicine has some advantages over the licensed one, like being in a form your child can take more easily, because the doctor thinks that it works better for your child's illness, or because an unlicensed medicine is safer than a licensed one.

How do I know that an off-label or unlicensed medication will work?

Unlicensed and “off-label” medicines are only prescribed after careful consideration of other options available. Your doctors will have read information from medical publications supporting its use and may have taken advice from other experts. An unlicensed or “off-label” medicine will only be used if it is the most appropriate medicine for you or your child.

Is it OK to use an unlicensed medicine?

Most medicines that are prescribed for a child will have a licence for use in adults, and so clinical trials have been done to prove that they are effective and safe to use in adults. If a doctor believes a medicine is safe and effective for use in children, because they have used it many times previously, they may continue to prescribe it for a child.

Your doctor will only suggest using a medicine for your child if they think that it is the best thing to help your child.

Are there side-effects from using unlicensed medicines?

Your doctor will tell you about any expected common side-effects that your child may get.

As with all medicines, there may sometimes be other side-effects that are not known. If you notice anything unusual and are concerned, contact your doctor, nurse, pharmacist or the person who prescribed your child the medicine. You can also report any suspected side-effects to a UK safety scheme at: <http://www.mhra.gov.uk/yellowcard>.

How do I know whether a medicine is unlicensed or being used off-label?

Your doctor or pharmacist will tell you that the medicine is not licensed for use in children. The leaflet that comes with the medicine may not say anything about its use in children, or may say that the medicine is not suitable for children. This does not mean that it cannot be used safely in children – it means that the drug company does not have a licence for using the medicine in children and are not allowed to promote or give information about its use in children.

Where can I get information about unlicensed medication?

Your doctor or pharmacist will be able to tell you more about whether a medicine is unlicensed or “off-label” for children, ask your doctor, nurse, or pharmacist.

Medicines for Children (www.medicinesforchildren.org.uk) produce leaflets that are written especially for parents and carers of children. Many of these are about medicines that are unlicensed for use in children.

What else do I need to know?

Sometimes it will take longer for the pharmacist to get you a supply of an unlicensed medicine. This means that you will need to also allow one or two weeks for them to get you further supplies of your medicine.

If you need any further information about your medicine, please contact the Pharmacy ...

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