Compassionate use

What is compassionate use?

The term ‘compassionate use’ refers to special programmes set up to make unauthorised medicines available to patients. In particular, this may be a way of making a promising but not yet authorised medicine available to patients who:

- cannot currently be treated satisfactorily with authorised medicines,
- have a disease for which no medicine has yet been authorised,
- cannot enrol in an ongoing clinical trial.

All medicines are authorised for a specific use. When they are prescribed for use in a different way this is called ‘off-label’ use. This article deals with compassionate use.

Which medicines can be made available through compassionate use?

Compassionate use programmes should only be put in place for medicines that are expected to help patients with life-threatening, long-lasting, or seriously disabling illnesses.

Medicines in compassionate use programmes will generally have successfully been through early clinical development including toxicology and first-in-human studies. There may still be some questions about the medicine’s safety profile, and uncertainties about optimum dosage and dosing schedule.

How do compassionate use programmes work?

Compassionate use programmes are co-ordinated and implemented

nationally, who decide independently how and when to open such programmes, according to national rules and legislation.

Doctors who wish to obtain a promising medicine for seriously ill patients must contact the relevant national authority in their country and follow the corresponding procedure. The national authority keeps a register of patients treated within their compassionate use programmes, and systems are in place to record any side effects reported by the patients or their doctors.

How can a patient enter a compassionate use programme?

To enter a compassionate use programme, patients must speak to their doctor. Doctors may first advise the patient about whether there is a suitable clinical trial in their country that they can enrol in, as medicines that are not yet authorised are generally first made available to patients through clinical trials. Doctors may also advise the patient on how compassionate use programmes work in their country.

If appropriate, the doctor can speak to the national authority responsible for compassionate use programmes in their country and find out whether there is a suitable compassionate use programme available.

What is the role of the European Medicines Agency in compassionate use?

The European Medicine Agency’s (EMA) Committee for Medicinal Products for Human Use (CHMP) can provide recommendations to all EU member states on the administration, distribution and use of certain medicines in compassionate use. It also identifies which patients may benefit from compassionate use programmes.

The CHMP can provide these recommendations at the request of a member state. It may also provide these recommendations when it becomes aware that a number of compassionate use programmes with a given medicine are being set up in member states.

The recommendations from the CHMP aim to standardise compassionate use programmes across the EU, and may also help make the conditions of existing compassionate use programmes clearer. However, the CHMP recommendations do not have any legal implication and are only implemented by the member states that wish to follow them.

The EMA publishes a list of opinions adopted by the CHMP on the compassionate use of medicines on its website. This registry also includes information on the Agency's recommendations, such as how the medicine should be used and in which patients.

Apart from compassionate use, are there any other ways of obtaining medicines before authorisation?

Doctors can also obtain a promising medicine for a patient by requesting a supply of a medicine from the manufacturer, to be used for a patient under their immediate responsibility. This is often called treatment on a 'named-patient basis' and should not be confused with compassionate use programmes. In this case, the doctor responsible for the treatment will contact the manufacturer directly. While manufacturers do record what they supply, there is no central register of the patients that receive treatment in this way.

Sometimes patients can enter 'expanded access programmes'. A company that makes a promising medicine may choose to run one of these programmes to allow early access to their medicine and to widen its use to patients who can benefit from it. For

example, patients who have been treated with the medicine during a clinical trial and wish to continue treatment may be able to do so via an expanded access programme. These programmes are often authorised by national authorities in the same way as clinical trials, and patients are followed in the same way as patients in a clinical trial.

The FDA uses the terms ‘expanded access’ and ‘compassionate use’ interchangeably, this is not the case in Europe.

Much of this text has been adapted from the EMA Questions and answers on the compassionate use of medicines in the European Union, Guideline on compassionate use of medicinal products, pursuant to Article 83 of Regulation (EC) No 726/2004, and website.

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