



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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## Questions and answers on the compassionate use of medicines in the European Union

This document describes how 'compassionate use' programmes may be set up in the European Union, and the role of the European Medicines Agency in these activities, in accordance with Article 83 of Regulation (EC) No 726/2004

### What is compassionate use?

Compassionate use is a way of making available to patients with an unmet medical need a promising medicine which has not yet been authorised (licensed) for their condition.

A medicine can be marketed in the European Union (EU) only after it has been authorised. However, it is sometimes in the interest of patients to have access to medicines before authorisation. Special programmes can be set up to make these medicines available to them under defined conditions. This is known as 'compassionate use'.

### Which medicines can be made available in this way?

Compassionate use programmes can only be put in place for medicines that are expected to help patients with life-threatening, long-lasting or seriously disabling illnesses. These programmes are expected to benefit seriously ill patients who currently cannot be treated satisfactorily with authorised medicines, or who have a disease for which no medicine has yet been authorised. The compassionate use route may be a way for patients who cannot enrol in an ongoing clinical trial to obtain treatment with a potentially life-saving medicine.

At this stage in the development of the medicine, what is known of the medicine's safety may be limited. Generally, toxicology studies will have been completed and analysed, and early studies looking at how the medicine is handled by the body will have been completed. However, there may still be some uncertainties about the best way to give the medicine to patients, such as the exact dose to use, and the dose frequency, and the medicine's safety profile (which side effects it can cause) is not yet fully established.



## **How do compassionate use programmes work?**

Compassionate use programmes are co-ordinated and implemented by the EU Member States, which decide independently how and when to open such programmes according to national rules and legislation. Doctors who wish to obtain a promising medicine for one of their seriously ill patients will need to contact the relevant national authority in their respective country and follow the procedure that has been set up. The national authority keeps a register of the patients treated with the medicine within the compassionate use programme, 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. In general, medicines that have not been authorised are first made available to patients through clinical trials, and the doctor will first advise the patient on whether there is a suitable clinical trial in their country than they can enter.

The doctor will also advise the patient on how compassionate use programmes work in their country, as these rules differ from country to country. If appropriate, the doctor can speak to the authority that is responsible for compassionate use programmes in their country, and find out whether a suitable compassionate use programme is available.

## **What is the role of the European Medicines Agency regarding compassionate use?**

The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) can provide recommendations to all EU Member States on how to administer, distribute and use certain medicines for compassionate use. It does also identify which patients may benefit from compassionate use programmes.

The Committee can provide these recommendations at the request of a Member State. It can also do so when it becomes aware that compassionate use programmes with a given medicine are being set up in a number of Member States.

The recommendations complement national legislation, and do not replace it. They also do not create any legal framework in the EU Member States. The recommendations are optional, and are only implemented by the Member States that wish to use them for their patients.

The Agency's recommendations aim to standardise compassionate use programmes across the European Union. They may also help to make the conditions of existing compassionate use programmes clearer.

## **What information does the European Medicines Agency publish?**

The European Medicines Agency publishes on its website a list of opinions on the compassionate use of medicines that the CHMP has adopted. This registry also includes information on the Agency's recommendations, such as the patients in whom the medicine can be used, and how it should be used.

## **Are there any other ways of obtaining medicines before authorisation?**

Doctors can also obtain promising medicines for their patients by requesting a supply of a medicine from the manufacturer, to be used for a patient under their direct 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 group of patients that are being treated 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 list of CHMP opinions on compassionate use can be found [here](#).

Follow this [link](#) for published guideline on compassionate use of medicinal products, pursuant to Article 83 of Regulation (EC) No 726/2004.