Compassionate use of medicinal products in Europe: current status and perspectives

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The medical treatment of patients with chronic, life-threatening or seriously disabling diseases can be very disappointing both for the suffering patient and their physicians in cases where patients cannot be treated satisfactorily with currently authorized medicines. It may be possible to successfully treat such patients with new pharmaceuticals that have not yet been authorized by suitable clinical trials. But clinical trials are time-consuming and costly, and not every patient meets the enrolment criteria for specific clinical trials. An aggravating factor is that the marketing authorization of promising new pharmaceuticals can take several years, which is valuable time lost from the patient's perspective.

One way to tackle this problem is to allow seriously-ill patients to obtain the medicines through a "compassionate-use" programme. The term "compassionate use" is defined in Article 83 no. 2 of the Regulation (EC) no. 726/2004 of the European Parliament and of the Council[1] as "... making a medicinal product belonging to the categories referred to in Article 3 (1) and (2) available for compassionate reasons to a group of patients with a chronically or seriously debilitating disease or whose disease is considered to be life-threatening, and who cannot be treated satisfactorily by an authorized medicinal product. The medicinal product concerned must either be the subject of an application for a marketing authorization in accordance with Article 6 of this Regulation or must be undergoing clinical trials."

This regulatory framework was created by the European Legislature in 2004. Until then, France and Italy were the only two European countries with compassionate-use programmes incorporated into national law. However, the European framework does not include any binding process regulations for countries on how to introduce and implement their own compassionate-use programmes. At present, there is only a nonbinding guideline by the Committee for Medicinal Products for Human Use (CHMP),[2] the scientific committee of the European Medicines Agency (EMA). Member States should notify the EMA when they make use of the compassionate-use provision outlined in paragraph 1 of Article 83 of the Regulation (EC) no. 726/2004. The CHMP, after consulting the manufacturer or the applicant, may adopt opinions on the conditions for use and distribution and the patients targeted for compassionate use in a given therapeutic indication. The opinions will be updated on a regular basis.

In January 2010, the CHMP published its first opinions on two compassionate-use programmes in the European Union.[3] The first opinion was about the compassionate use of intravenous oseltamivir (Tamiflu®), notified to the EMA by Finland.[4] The CHMP has also published an assessment report on the compassionate use of this medicinal product.[5] The other opinion, which was notified by Sweden and published by the CHMP, was about the compassionate use of intravenous zanamivir.[6]

Due to the nonbinding nature of the European Regulation and the CHMP guideline, it is up to each Member State to adopt its own corresponding national legal procedures for the introduction and implementation of compassionate-use programmes. Germany introduced its own regulations in July 2010 and has created a quick and efficient procedure for establishing compassionate-use-programmes.[7] At time of publication, there were four notifications confirmed by the national pharmaceutical authority in Germany.[8] Other European countries, such as the Netherlands, Norway and Spain, have also established national regulations. Austria and the United Kingdom of Great Britain and Northern Ireland plan to present their new procedural rules soon.

The strict conditions of the national procedural rules mean that the use of not-yet authorized pharmaceuticals will continue to be an exception. Only a few national cases are expected to meet these conditions in any year but, in these cases, the new regulations may save valuable time for patients who have run out of treatment options. This is exactly why such regulations with clear guidelines and a fast-track procedure were long overdue. It is desirable that the other Member States of the European Union follow these examples, so that seriously ill patients throughout Europe can access promising alternative medical therapies. ■

References

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