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New guidance on compassionate use programmes published

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Legal framework

Article 6 of the EU Directive on the Community Code relating to Medicinal Products for Human Use (2001/83/EC) provides that a medicinal product may not be placed on the market of a member state unless:

- a marketing authorisation has been issued by the competent authority of that member state; or
- an authorisation has been granted in accordance with the EU Regulation on Centralised Procedure (2309/93).

EU Regulation 726/2004, which lays down procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishes the European Medicines Agency, provides an exception to this requirement under defined circumstances, within the framework of so-called 'compassionate use programmes'.

According to Article 83 of the regulation, 'compassionate use' is defined as making a medicinal product belonging to the categories referred to in Articles 3(1) and (2) of Regulation 726/2004 available for compassionate reasons to a group of patients with a chronically or seriously debilitating or life-threatening disease which cannot be treated satisfactorily by an authorised medicinal product. The medicinal product concerned must be:

- the subject of an application for a marketing authorisation in accordance with Article 6 of Regulation 726/2004; or
- undergoing clinical trials.

Austria has made use of this option and incorporated the possibility of compassionate use into the Act on Medicinal Products. Section 8a of the act provides that medicinal products falling within the definition of Article 83 of Regulation 726/2004 do not require marketing authorisation, provided that the Federal Office for Safety in Healthcare (BASG) has approved the marketing of such medicinal product within the framework of a compassionate use programme. Such a programme must be established for a defined group of patients - those suffering from a chronically or seriously debilitating or life-threatening disease - that cannot be treated satisfactorily by an authorised medicinal product.

The application for approval may be submitted by:

- a manufacturer that sponsors an approved clinical trial for the product in question; or
- the applicant for an authorisation according to Article 6 of Regulation 726/2004.

Guidance on compassionate use programmes

On November 21 2012 the BASG issued its guidance for applications for compassionate use programmes.(1) Applications must be submitted electronically to the BASG, accompanied by documentation detailing:

- the contact details of the person responsible and, if applicable, the legal representative located within the European Union or a member state of the European Economic Area;
- the name of the medicinal product, a declaration of the active substance and

composition and the mode of application, dosing and therapeutic application;

- a description of the disease and justification that the patients for whom the medicinal product is intended suffer from a chronically or seriously debilitating or life-threatening disease;
- the criteria for patient selection and an estimate of patient numbers;
- confirmation that no adequate treatment with a medicinal product authorised in Austria is available;
- justification that the patient group cannot be included in an active clinical trial;
- documentation that the medicinal product has been manufactured according to good manufacturing practice standards;
- justification and data illustrating the safety and efficacy of the medicinal product for the proposed indication;
- the criteria for the interruption or early termination of the compassionate use programme;
- details of:
  - an approved pivotal clinical trial referenced by its EudraCT number or an approved pivotal clinical trial of the medicinal product in a non-EU country, and proof that the trial has been conducted according to the internationally recognised requirements of good clinical practice; or
  - the marketing authorisation application submitted to the European Medicines Agency, the BASG or the competent authority of another member state;
- the current investigator's brochure or a proposed draft summary of product characteristics;
- justification of the therapeutic use of the medicinal product in a compassionate use programme:
  - that has received a negative opinion in an application for marketing authorisation;
  - that has been withdrawn from the market; or
  - whose marketing has temporarily been suspended; or
  - for which the conduct of a clinical trial has been:
    - refused;
    - withdrawn after approval;
    - suspended; or
    - approved only under condition of specific commitments (in each case the grounds for the decision must be outlined);
- information and documentation provided to patients (in German) and a description of the procedure followed to obtain patients' informed consent;
- requirements for the medicinal facilities and the qualifications of participating physicians;
- participation of an approved compassionate use programme in another member state; and
- the treatment protocol, including summaries of:
  - the principles of the compassionate use programme;
  - product characteristics;
  - patient information;
  - provisions for patient treatment;
  - an outline of the data to be collected; and
  - a description of pharmacovigilance measures.

Approved compassionate use programmes must be listed on the BASG homepage. The BASG will inform the European Medicines Agency on approval. Approvals are valid for one year, unless terminated early. A request for an extension can be submitted one month before the expiry of the programme.

Medicinal products that are shipped to or imported into Austria within the context of an approved compassionate use programme are exempt from shipment notifications or import applications under the Medicinal Products Importation Act 2010. Advertising of medicinal products in the context of a compassionate use programme is therefore not permitted in Austria. Promotional material for a compassionate use programme must not advertise the medicinal product, but be limited to promoting the recruitment of patients.

On granting of marketing authorisation for a medicinal product that is under an active compassionate use programme in Austria, the BASG will set the date of the end of the programme with respect to the actual availability of the medicinal product on the Austrian market in collaboration with the holder of the programme or marketing approval.

Comment

The BASG's guidance on compassionate use programmes provides manufacturers of medicinal products with useful guidance for gathering the necessary documentation to make a successful application for such programmes in Austria. It makes available new medicinal products to patients in need and demarcates the borderline between compassionate use programmes and clinical trials.

For further information on this topic please contact Rainer Herzig at Preslmayr Attorneys at Law by telephone (+43 1 533 16 95), fax (+43 1 535 56 86) or email (herzig@preslmayr.at).

Endnotes

(1) The guidance is available both in English and German on BASG's website www.basg.gv.at/uploads/tx_basginfobox/L_Z24_compassionate_use_at_en_01.pdf.