EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Orphan Medicines

Note for sponsors of orphan medicinal products regarding enlargement of the European Union (EU)

Introduction

Legislation on orphan medicinal products, Regulation (EC) No 141/2000 of the European Parliament and of the Council and Commission Regulation (EC) No 847/2000, entered into force in January 2000 and April 2000 respectively. This introduced a Community procedure for the designation of medicines as orphan medicinal products and incentives for their development and placement on the market. On 29 July 2003, the European Commission adopted a Communication C178/02 setting out its interpretation of certain provisions.

In July 2013, Croatia will join the European Union. This paper is intended to provide supplementary advice to sponsors of orphan medicinal products on the practical issues arising from EU enlargement and will address: applications for orphan designation, impact on existing designations and marketing authorisations, translations and transitional arrangements prior to July 2013.

For general guidance on how to apply for orphan medicinal product designation, please refer to the *Guideline on the format and content of applications for designation as orphan medicinal products and on the transfer of designations from one sponsor to another* ENTR/6283/00 Rev. 2.

Sponsors of orphan medicinal products

The 'sponsor' of an orphan medicinal product may be an individual or a company and must be established in the EU. From the day of accession, individuals or companies from Croatia will be capable of being regarded as established in the EU (subject to the usual requirements of proof of establishment).

Application for orphan medicinal product designation

Prevalence

Where designation according to Article 3(1) (a) of Regulation (EC) No 141/2000 is sought, information on the prevalence of the condition or disease in the Community is required in accordance with the requirements laid down by Commission Regulation (EC) No 847/2000.

Prevalence (the number of persons affected by the condition in the EU at the time the application for designation is submitted) should be calculated for the condition which is the subject of the designation

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application. For applications submitted following the date of enlargement, from 1 July 2013 onwards, prevalence calculations must be based on the population[1] of the enlarged European Union, i.e. 28 Member States, plus Iceland, Liechtenstein and Norway.

Sponsors are advised to consult the *Points to consider document on calculation and reporting of the prevalence of a condition for orphan designation* COMP/436/01 prior to completing this section of an application.

Methods available for diagnosing, preventing or treating the condition

In accordance with Article 3(1) (b) of Regulation (EC) No 141/2000 and Article 2(3) of Commission Regulation (EC) No 847/2000, it is the responsibility of the sponsor to establish that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorised in the EU or, if such method exists, that the medicinal product will be of significant benefit to those affected by that condition.

For applications submitted following the date of enlargement, from 1 July 2013 onwards, sponsors will be required to include and review existing methods in the enlarged European Union, i.e 28 Member States, plus Iceland and Norway.

Translations

At the time an application for designation is made, the EMA requests translations of the following elements in the official languages of the European Union:

- the name of the substance (INN)
- the proposed orphan indication.

Decisions on orphan designation granted following accession, will require translations into all official EU languages, including Croatian, plus Icelandic and Norwegian (see template).

If an application for orphan medicinal product designation has been submitted, but not finalised before accession, the Commission decision can be adopted only if translations in all official languages, including Croatian, are available. For applications submitted for the submission deadline on 25 March 2013 or after, sponsors are requested to submit translations, in the 23 official languages of the European Union plus Icelandic and Norwegian, prior to adoption of the opinion by the Committee for Orphan Medicinal Products (COMP).

Existing orphan medicinal product designations

Decisions on orphan medicinal product designation, which have been granted by the European Commission prior to July 2013, will automatically extend to the acceding country on the day of accession.

Orphan medicinal products which have a marketing authorisation

Marketing authorisation

EU marketing authorisations that have been granted by means of a Commission decision adopted according to the provisions of Regulation (EC) No 726/2004 will automatically extend to Croatia from the date of accession.

[1] Population figures are available on Eurostat web page.

Market exclusivity

When a designated orphan medicinal product has been authorised throughout the EU and therefore benefits from market exclusivity, this market exclusivity extends automatically to the acceding country on the day of accession, encompassing the same rights as in the EU. The same 10-year market exclusivity period will apply, even if this period started before accession.

National marketing authorisations granted in the candidate country before accession do not conflict with the market exclusivity.

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