Concept paper
17th December 2004 meeting

Compassionate Access Programmes for Orphan Products: from heterogeneity to harmonised practices?

Eurordis is organising a symposium to address the implementation of EMEA's new responsibilities as regards compassionate access programmes, as provided for in EC/EP Regulation 726/2004, recital 33[1] and article 83. This first meeting will focus on patients' expectations, and summarise the obstacles, advantages, and good practices both from competent authorities and industry perspectives.

Title	Workshop on compassionate access programmes for Orphan Products: from heterogeneity to harmonised practices?
Location	Barcelona Fundacio Dr. Robert UAB Casa Convalescència St. Antoni Maria Claret, 171 · 08041 Barcelona · Spain Tel.+ 34 93 433 50 00
Date	17 December 2004 Full day meeting
Format	Presentations by speakers, followed by debate with the audience Closed meetings; no media; report issued
Language	English
Targeted audience	European Commission European Medicines Agency Patients' organisations National Competent Authorities Pharmaceutical industry, particularly holders of an orphan product designation, Clinical Research Organisations
Number of attendees, including speakers	Maximum 50

[1] REGULATION (EC)No 726/2004 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 31 March 2004, laying down Community procedures for the authorisation and supervision of medicinal products, for human and veterinary use and establishing a European Medicines Agency

Issues to be addressed are, among others:

1. Of the 25 different national procedures for compassionate access programmes, which ones better serve the needs of the patient?
2. Are there examples of prior compassionate access programmes for an orphan drug that were successful, from the patients', the public health, and the manufacturers' perspectives?
3. For compassionate use, the Committee for Medicinal Products for Human Use (CHMP), after consulting the manufacturer or the applicant, may adopt opinions on the conditions for use, the conditions for distribution and the patients targeted. Can patients' organisations take the lead in requesting such compassionate use, and assist CHMP in defining the targeted population and conditions for use and distribution? Do we need a WGCAP (Working Group on Compassionate Access Programmes)?
4. When the CHMP adopts an opinion on a compassionate access programme, members states *"shall take account of any available opinions"* to decide whether or not to open such a programme. There is a risk of a new postal code lottery (see ovarian cancer), where patients may or may not have access to a promising new product depending on where they reside in Europe, introducing a risk of geographic discrimination. How can we avoid this situation?
5. What are the obstacles for compassionate access programmes, and what solutions can be proposed at the European level?
6. Should compassionate access programmes be different for OMP than for other medicines? For very limited populations, how can an efficient programme be organised in spite of the numerous differing national procedures? Should there be different programmes for different situations (large programmes versus smaller ones such as for orphan orphans)?