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Compassionate use programmes for orphan drugs

Conclusions of EURORDIS ERTC workshop on 21 November 2011 and proposals to move forward

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Content

- The 15th Workshop
- Objectives
- Implementation EU legislation Article 83
- Experience EMA, Member States, industry associations/companies
- Proposals to move forward

The 15th Workshop

- EURORDIS Round Table of Companies (ERTC)
- “Compassionate access to rare therapies”
- 21 November 2011 (Aero-Club de France, Paris)
- 82 participants from 16 countries (EU, US, Canada)
- Representatives from the EU Commission, EMA, MSs, scientific committees members, patients organisations, academics, lawyers

Objectives

- Evaluation of the implementation of the legislation (article 83(2) Regulation (EC) N° 726/2004)
- Application to orphan drugs
- EMA experience
- Experience from MSs, industry associations, companies
- Difficulties
- Possible evolution

EU legislation

- Objective of the legislation
  - Harmonisation
  - Common approach between MSs
  - Equal treatment for patients across the EU
- Role of the EMA (*may* provision)
  - Evaluation
  - Opinion (*may* adopt an opinion)
- Role of the MSs final decision (take account)

Article 83 Regulation N° 726/2004

- Applicable to medicinal products for "chronically or seriously debilitating disease, or a life threatening disease, and who cannot be treated satisfactorily by an authorised medicinal product"
- Subject of an application for a centralised MA or undergoing clinical trials in the EU
- For a group of patients

- MSs using the provision shall notify the EMA
- Not applicable to medicinal products already authorised or not eligible to centralised procedure or under compassionate use on a named patient basis
- CHMP at the EMA *may* adopt an opinion on
  - Conditions of use
  - Conditions for distribution
  - Patients targeted

- Only MSs can request an opinion (companies cannot apply)
- Opinions are publicly available
- EMA has published a Guidance document and a Q§A document
- Practical experience
  - MSs
  - Industry associations
  - Companies

Practical experience

- EMA
  - Only 2 assessments since the implementation of the EU legislation
  - No experience at all with orphan drugs
  - What are the reasons?
  - Why the EMA is not notified?
  - Do we need a revision of the legislation?
  - Other ways forward?

- MSs/industry associations/companies
  - Compassionate use for orphan medicinal products in almost all MSs
  - Based on national legislation or on named patient basis
  - Have been authorised after compassionate use programmes which can
    - Generate data on efficacy
    - Establish safety of long term use
    - Be the basis of clinical development

- General agreement on the usefulness of compassionate use programmes
  - Facilitate access patients to new medicines
  - Opportunity for physicians to use new medicines
  - Build networking with specialists
  - Better knowledge about the medicine
  - Shorten the delay on market access
- Why article 83 is not widely used? Why MSs prefer to use national route?

Main concerns

- Differences between Mss policies difficult to understand (authorisation, documentation, prescription, assessment time, validity, follow up)
- Liability risks
- Lack of transparency
- Interference with MA procedure
- Supply and logistics, information/language
- Pressure on supply under compassionate use including off-label
- Free of charge/prices

Proposal to move forward

- Compassionate use should continue to be
  - An offer of medicines to those in need
  - Named patient basis or cohort
- Compassionate use should not be
  - A clinical trial or product development
  - An incentive or a way to market a product before the granting of marketing authorisation
  - A way to be financed

- What is needed?
  - To improve transparency, harmonisation, access and involvement of patients
  - To set up clear rules between compassionate use and development programmes and to set up guidelines based on experience
  - Integration of all data for post-marketing surveillance
  - To have an EU forum for stakeholders to facilitate common approach
  - To use article 83 of the current EU legislation (notification)

- Who can act?
  - The Commission will not revise the current legislation soon but an overview of the MSs current practices could be set up
  - MSs have rules in place that can be improved and harmonised via guidelines to be set up in common
  - Companies that have difficulties to put in place programmes should be encouraged
  - Patients association should be involved

- How to do?
  - To pursue the dialogue between MSs and between MSs and companies
  - Collaboration particularly necessary for orphans
  - Based on the experience on informal groups to set up a “Facilitation group” between MSs in order to
    - Exchange information
    - Build up common experience to set up harmonised procedures
    - Create a network which can facilitate future changes in the legislation

Conclusion

- A working group should be set up with MSs
- To improve transparency and harmonisation
- To involve patients as it’s done in most of the areas dealing with medicines
- In order to give access to new medicines for those that need particularly for orphan drugs