COMMON EMEA/FDA APPLICATION FORM FOR ORPHAN MEDICINAL PRODUCT DESIGNATION

K. Westermark

COMP chairperson

Project

- Based on EU application form
  - US did not have form before
- Merge common requirements respecting particularities
- Annex exclusive requirements
  - E.g. Significant benefit

What is the common application form?

- A common form to simplify administrative process
- The first step in getting “closer but not the same”
- An invitation to sponsors and regulators to think about patients beyond markets and administrations
  - If you have a product get designations and “attract development”
  - Then benefit patients wherever they are

What is it not?

- Sharing the assessment
  - Different criteria/requirements (sign benefit, prevalence threshold)
  - Different approaches (definition of condition)
- Single opinion on designation EU and US
- A change on the criteria or procedure for designation
- A new common guideline
- A replacement of sections A to E (remainder)

Conclusions

- EMEA and FDA worked on a common application for submission of orphan designation requests
- Procedure started in April 2007 and finished November 2007
- First step in harmonising administrative practices
- Different regulations and procedures
- Common application will not deliver a common opinion

BUT

EU-EURORDIS/FDA comparison 2000-2005:
90% of applications accepted in the EU also accepted by the FDA (unpublished data)

GROUNDS FOR FURTHER HARMONISATION

EMEA/COMP-FDA/OOPD Next steps:

Common application:

- Encouragement of submissions
- Regular contacts – monthly teleconferences EMEA-FDA

Annual reports on development:

- Information on procedures from both Agencies
- Timeline and structure harmonisation

Guidelines on orphan designation:

- Existing EMEA/COMP guidelines
- Proposal for future discussion and harmonisation of common terms