European Orphan Designation

Joint EMA / FDA / MHLW-PMDA orphan medicinal product workshop

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Background

- Lack of appropriate treatment - *"Patients suffering from rare conditions should be entitled to the same quality of treatment as other patients"*
- Market often economically not interesting
- Lack in return of investment

Legislation – Orphan Regulation (EC) No 141/2000

Criteria

1. Rare disease (not more than 5 in 10,000 persons in the EU) or not sufficient return on investment
2. Seriousness: life-threatening or chronically debilitating
3. No satisfactory method of treatment or if existing significant benefit has to be demonstrated

Committee (COMP) / procedure for designation

1. Sponsor submits an application to the European Medicines Agency (COMP) for assessment
2. European Commission Decision for designation (Orphan register)

Legislation – Orphan Regulation (EC) No 141/2000 (2/2)

Incentives

1. 10 years of market exclusivity (+2 years if studies on children),derogations if:
    1. **Consent**
    2. **Shortage of supply**
    3. **2nd product is safer, more effective or clinically superior (= clinical superiority)**
2. Protocol assistance (fee reduction for product development)
3. EU marketing autorisation
4. Eligible for national incentives

Legislation – other instruments

- Commission Regulation (EC) No 847/2000 for implementation of the criteria and definition of 'similar medicinal products' and 'clinical superiority'
- Communication from the Commission (2003/C 178/02) on the interpretation of the criteria notably "significant benefit"
    - significant benefit is based on assumption supported by data/evidence
    - e.g.claim of better efficacy or safety, benefits to a particular population sub-set,more convenient administration route
- Communication from the Commission (C2008)4077 on assessing similarity and applying derogations
- Commission Guideline (2008/C242/07) on the review of the period of market exclusivity

Legislation – other instruments

- Commission 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 *Revision ongoing*
    - Clarification on the medical plausibly and the significant benefit
    - Electronic submission and common FDA-EMA common application
    - Possible amendment to an existing designation.
- EMA recommendations on the calculation of prevalence, protocol assistance, appeal, …

Thank you

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http://ec.europa.eu/health/human-use/orphan-medicines/index_en.htm

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