BfArM
Bundesinstitut für Arzneimittel und Medizinprodukte

Regulatorische Rahmenbedingungen für die Entwicklung und Zulassung von Orphan Medicinal Products

PMS Workshop: Arzneitherapie seltener Krankheiten – Herausforderungen und Chancen

Berlin – Brandenburgische Akademie der Wissenschaften, Berlin, 12. Mai 2009

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Europäische Orphan Designation

- Warum gibt es eine „Orphan Designation“?
- Was bedeutet „Orphan Drug“ und „Orphan Designation“?
- Unterschied zwischen „Orphan Designation“ und Arzneimittelzulassung.
- Wer entscheidet über den „Orphan Drug“ Status?
- Wie läuft die „Orphan Designation“ ab?
- Welche Vorteile bringt eine „Orphan Designation“?

Warum „Orphan Designation“? (1)

Patienten, die an einer seltenen Erkrankung leiden, haben (vorausgesetzt, dies ist wissenschaftlich möglich) den gleichen moralischen und ethischen Anspruch auf Linderung oder Heilung durch wirksame und sichere Arzneimittel wie Patienten, deren Krankheit häufiger auftritt.

Warum „Orphan Designation“? (2)

- Für pharmazeutische Unternehmen ist es jedoch aus wirtschaftlichen Gründen oft schwierig, Arzneimittel zur Behandlung seltener Erkrankungen zu entwickeln, da eine kleine Zielpopulation die Refinanzierung der Entwicklungskosten erschwert oder unmöglich macht.
- Das europäische System der „Orphan Designation“ stellt den Versuch dar, diese wirtschaftlichen Nachteile durch geldwerte Anreize zumindestens teilweise zu kompensieren.
- Bei der „Orphan Designation“ handelt es sich also um eine Subvention mit dem Ziel, die Entwicklung neuer Arzneimittel zur Diagnostik, Vorbeugung oder Therapie seltener Erkrankungen zu unterstützen und zu fördern.

Orphan International Overview

- United States ‘Orphan Drug Act’ 1983
- Japan ‘Orphan Drug Legislation’ 1993
- Singapore ‘Orphan Legislation’ 1997
- Australia ‘Orphan Legislation’ 1998
- Europe 2000
  - Regulation (EC) No 141/2000 of the European Parliament and of the Council on Orphan Medicinal Products of 16 December 1999
  - Commission Regulation (EC) No 847/2000 of 27 April 2000

Medicinal and Orphan Products
Legal Basis

Legal Basis Medicinal Products

- Council Directive 2001/83/EC amended by 2002/98/EC, 2003/24/EC and 2003/27/EC
  - Annex 1 (2003/63/EC)
- Commission Regulation (EC) 1084/2003 & 1085/2003
- Regulation (EC) 726/2004
  - Annex (obligate centralized procedure)

2003/94/EC (GMP), 87/18/EEC & 88/320/EEC (GLP)
86/609/EEC (animals), 2001/20/EC (GCP)

- European Pharmacopoeia

Legal Basis Orphan Products (in addition to the above)

- EUROPEAN PARLIAMENT and COUNCIL REGULATION (EC) No 141/2000
  - Commission Communication on orphan medicinal products 01/08/2003
- COMMISSION REGULATION (EC) No 847/2000

What is an Orphan Medicinal Product?

Orphan Medicinal Products

- medicinal products (not devices, food supplements etc.)
- for rare diseases which are life-threatening or very serious (human not vet.)
- no satisfactory methods exist or medicinal product will be of significant benefit or development costs will exceed expected return on investment

Criteria for Orphan Designation

- medically plausible condition
- life-threatening or debilitating nature of condition
- prevalence ≤ 5 in 10,000 or unlikely to generate sufficient return on investment
- no satisfactory methods exist or medicinal product will be of significant benefit

Who Decides? - COMP Composition

The Regulation establishes the Committee for Orphan Medicinal Products (COMP), within the EMEA, which is responsible for examining all applications for orphan medicinal product designation submitted to it in accordance with the Regulation (Reg. No 141/2000).

33 voting, 2 non-voting Members:

- one member nominated by each the Member State (27)
- three members nominated by the Commission to represent patient's organisations (3)
- three members nominated by the Commission on the basis of a recommendation from the Agency (3)
- two non-voting members (IS, NO) (& further observers)

Orphan Medicinal Products Designation vs. Authorization (1)

Designation - providing incentives for the development of medicinal products for the benefit of patients suffering from rare conditions

Authorization - ensuring that only medicinal products that are effective, safe and of good quality are marketed

(in general, Orphan Medicinal Products require the same level of evidence with regard to the risk / benefit for marketing authorization as non-orphan products. However, there are some special considerations possible like e.g. authorization under exceptional circumstances)

Orphan Medicinal Products
Designation vs. Authorization (2)

Who?

Designation	**- any natural or legal European person**
Authorization	**- pharmaceutical companies only**

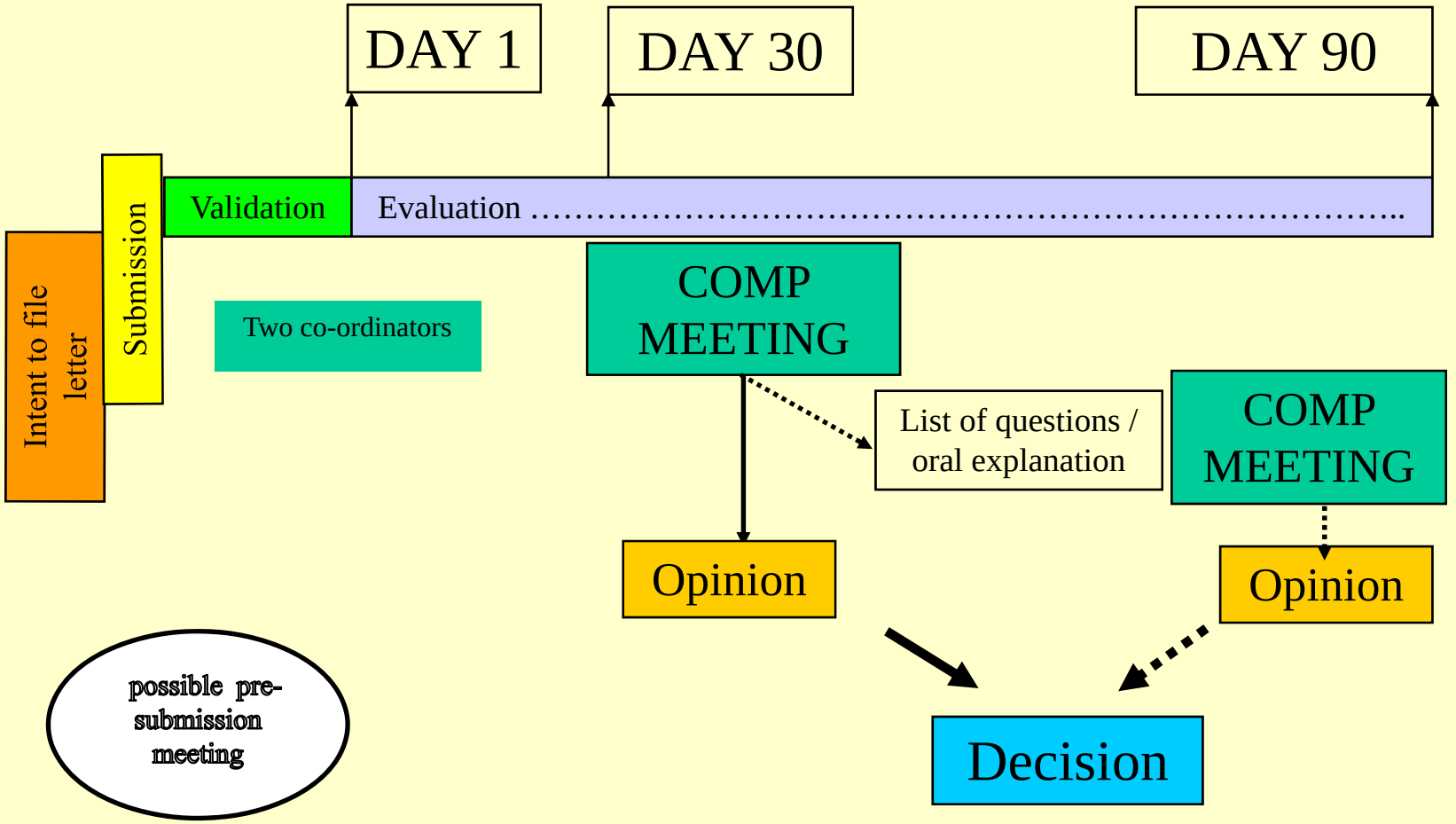
When?

Designation	**- any time during development (whenever there is sufficient data)**
Authorization	**- after finalizing clinical development**

Level of Evidence:

Designation	**- reasonable scientific assumptions**
Authorization	**- scientifically proven facts**

Outline procedure for designation



¿ Publication of public summary of opinion (lay language) on EMEA website

Orphan Medicinal Products in the EU

The Incentives (non exhaustive)

- Market exclusivity
- Protocol assistance during the development, with involvement of the CHMP
- Fee reduction for centralised applications (and marketing authorisation maintenance activities)
- Priority access to EU research programs
- National incentives (grants, tax reductions)
- (Shorter time to market)
- (To attract venture capital)

Orphan Medicinal Products in the EU

Market Exclusivity

- **Period of ten years exclusivity from the date of marketing authorisation**

- **Conditions:**

  orphan designation

  AND marketing authorisation throughout EU

- **Scope of exclusivity:**

  no market authorisation for

  similar medicinal products

  in the same indication(s)

Weitere Info

EMEA (orphan intro):

http://www.emea.europa.eu/htms/human/orphans/intro.htm

Community Register of orphan medicinal products for human use (alphabetical):

http://ec.europa.eu/enterprise/pharmaceuticals/register/alforphreg.htm


Danke für die Aufmerksamkeit!
Noch Fragen???