EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Human Medicines Special Areas

IPA Introductory meeting
1 February 2010

Presented by: Jordi Llinares
Section head Orphan Medicines

An agency of the European Union

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Outline

Who are we?

What do we do?

How do we do it?

Human medicines special areas

The Sector has five sections

Orphan Medicines
Paediatric Medicines
Scientific Advice
Scientific Support and Projects
SME Office

Orphan medicines

Main activities

Assessment and coordination of

- procedures for the designation of orphan medicinal products
- review of orphan designation criteria at the time of marketing authorization

Support to the Committee for Orphan Medicinal Products (COMP)

Committee for Orphan Medicinal Products

31 members + Chairman

- 1 Member per Member State
- 3 representatives from patient groups
- 3 members proposed by the EMEA

COMP Responsible for:

- opinions on designation
- advising on general EU policies
- international co-operation

Support by agency 3 secretaries and 4 scientific administrators

Orphan designation

Procedure to recognise drugs intended for development for rare diseases

And

Provide incentives for their development and marketing

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Criteria for designation

RARITY (prevalence not > 5 in 10,000) / Insufficient RETURN OF INVESTMENT

SERIOUSNESS

Life –threatening or chronically debilitating

ALTERNATIVE METHODS AUTHORISED

If satisfactory method exist the sponsor should establish that the product will be of significant benefit

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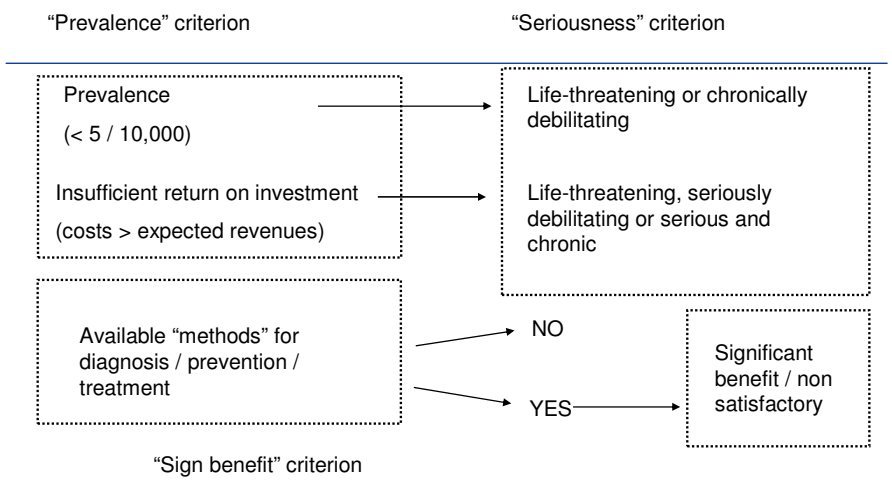

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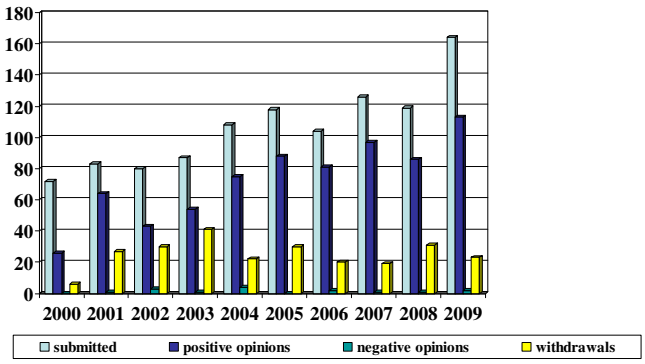
Outcome on designations



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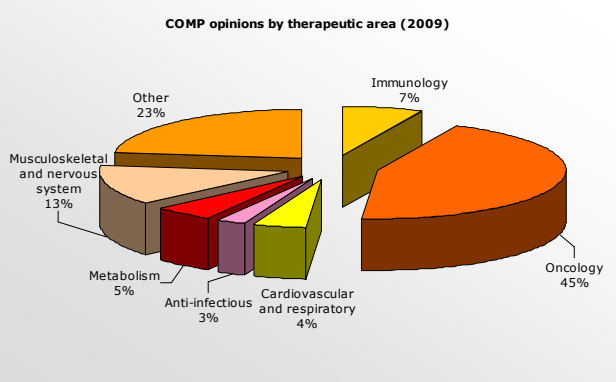
OD by therapeutic field



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Incentives (I)

Economic / marketing

- **Fee reduction / exemption**
  - **Extended incentives for SMEs (post authorisation)**
- **Market exclusivity**

Product development

- **Protocol assistance**

Community marketing authorisation

National incentives (EC inventory)

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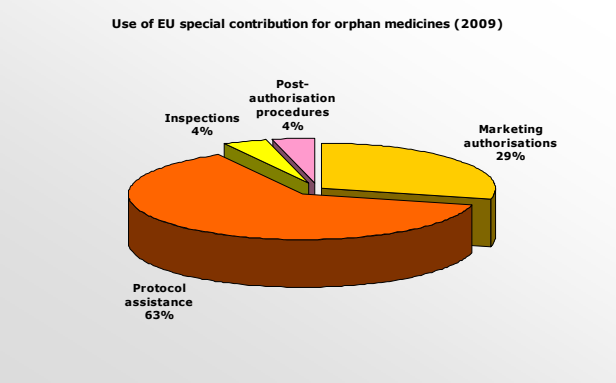
Incentives (II)

Fee reductions (50% market authorisation aplication, 100% protocol assistance, post authorisation)

10-year market exclusivity

- **protection against**
  - **similar products (structure/mech of action) for**
  - **same indication**

Use of incentives (EU contribution)



Paediatric medicines

Main activities

- administrative and scientific secretarial support to the PDCO and its working groups
- paediatric investigation plans (PIP) - waivers and deferrals
- verification of compliance with PIP
- coordination of and secretarial support to the European Network of Paediatric Research;

Paediatric Committee (PDCO)

- Members from National competent authorities (22+2)
- 5 Members from CHMP
- 6 members from health care professional and parent-patient groups
- Chair, vice chair and alternate members

Objectives of the paediatric regulation

- Increase high quality, ethical research into medicines for children
- Increase availability of authorised medicines for children
- Increase information on medicines

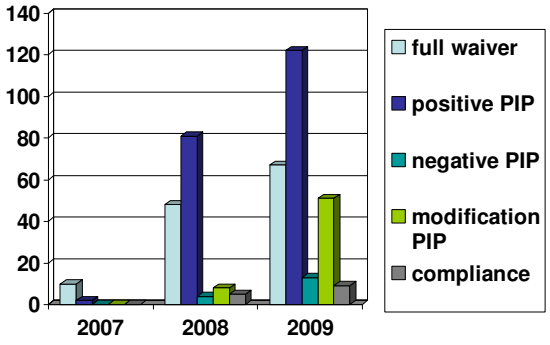
- Without unnecessary studies in children
- Without delaying authorisation for adults

Incentives from paediatric regulation

Reward for medicinal products with a Paediatric Investigation Plan (PIP)

Extension of Supplementary Protection Certificate by 6 months

Outcomes PIP / waivers



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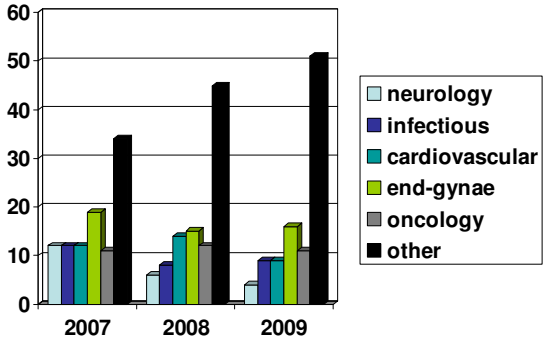
Therapeutic areas PIP/waivers



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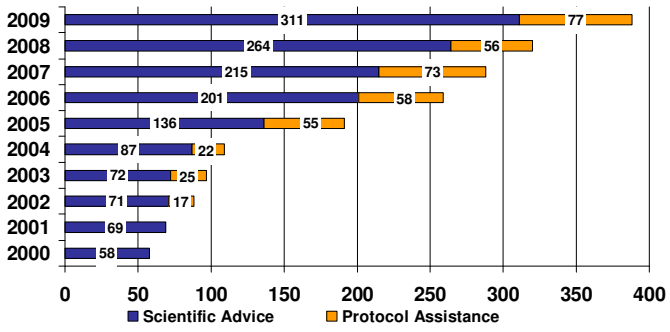
Scientific advice

- scientific advice (SA) and protocol assistance (PA) to sponsors during the phase of research and development of medicinal products;
- provision of advice on non-product specific scientific issues and guidance on the qualification of biomarkers and novel methodologies;
- administrative and scientific secretarial support to the CHMP Scientific Advice Working Party

Scientific advice working party

- Multidisciplinary Expert Group: Chairperson and 27 Members, who are experts from National Authorities or from University Clinics and Other Institutions.
- One member comes from the Committee for Advanced Therapies (CAT) and 3 Members from the Committee for Orphan Medicinal Products (COMP).
- The SAWP is supported by the SA Section: 10 medical doctors and pharmacists and 7 secretaries and administrative assistants

Scientific advice activity



Scientific support and projects

Main activities

- scientific support and coordination of scientific networks/projects within the Agency
- coordination of scientific projects
- provision of Certification for Non Clinical aspects of ATMPs (advanced medicinal products)
- contribution to the ATMPs classification;
- business pipeline activities

Small and medium size enterprises (SME) office

- assistance to SMEs through the provision of various services including (one stop shop)
- assignment of SME status,
- responding to queries,
- granting fee incentives,
- organisation of workshops and training sessions.
- Translation of product information
- Certification of quality and non-clinical data for ATM

Output

- 405 assigned SMEs from 21 countries across EEA
- 190 companies received SA

Many thanks

More information

http://www.ema.europa.eu/htms/human/mes/introduction.htm

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

http://www.ema.europa.eu/SME/SMEoverview.htm