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EUROPEAN NETWORK FOR RESEARCH ON ALTERNATING HEMIPLEGIA

Learning from each other
Member of the PDCO at the EMA

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Disclaimer

Some of the slides in this presentation are based on EMA sources which are gratefully acknowledged but the opinions are to be considered personal

Patients affected by Rare Diseases are entitled to receive drugs tested at same level of evidence as conventional patients

As for conventional drugs the Orphan Drugs evaluation should be based on quality, efficacy and safety tests

The requested design, plan and statistical approach of trials is similar for OD and conventional drugs

Children are entitled to receive drugs tested at same level of evidence as conventional patients

As for conventional drugs the Orphan Drugs evaluation should be based on quality, efficacy and safety tests

The requested design, plan and statistical approach of trials is similar for OD and conventional drugs

- Market forces alone had proven to be insufficient incentive for adequate research, development and authorisation of medicinal products for both ,
- **Patients with rare disease**
- **Children**

From protecting children against clinical research to protecting children through research

Real life experience:

- paediatric disclaimers ,‘therapeutic orphans’, off-label use in children considered a given
- 50-90% of paediatric medicines have not been tested and evaluated

New regulatory environment:

- Regulation on paediatric Medicines (Regulation - EC- No 1901/2006 + 1902/2006) came into force on the 26th January 2007 :

no drug development without considering children

earlier inclusion of children into the drug development process

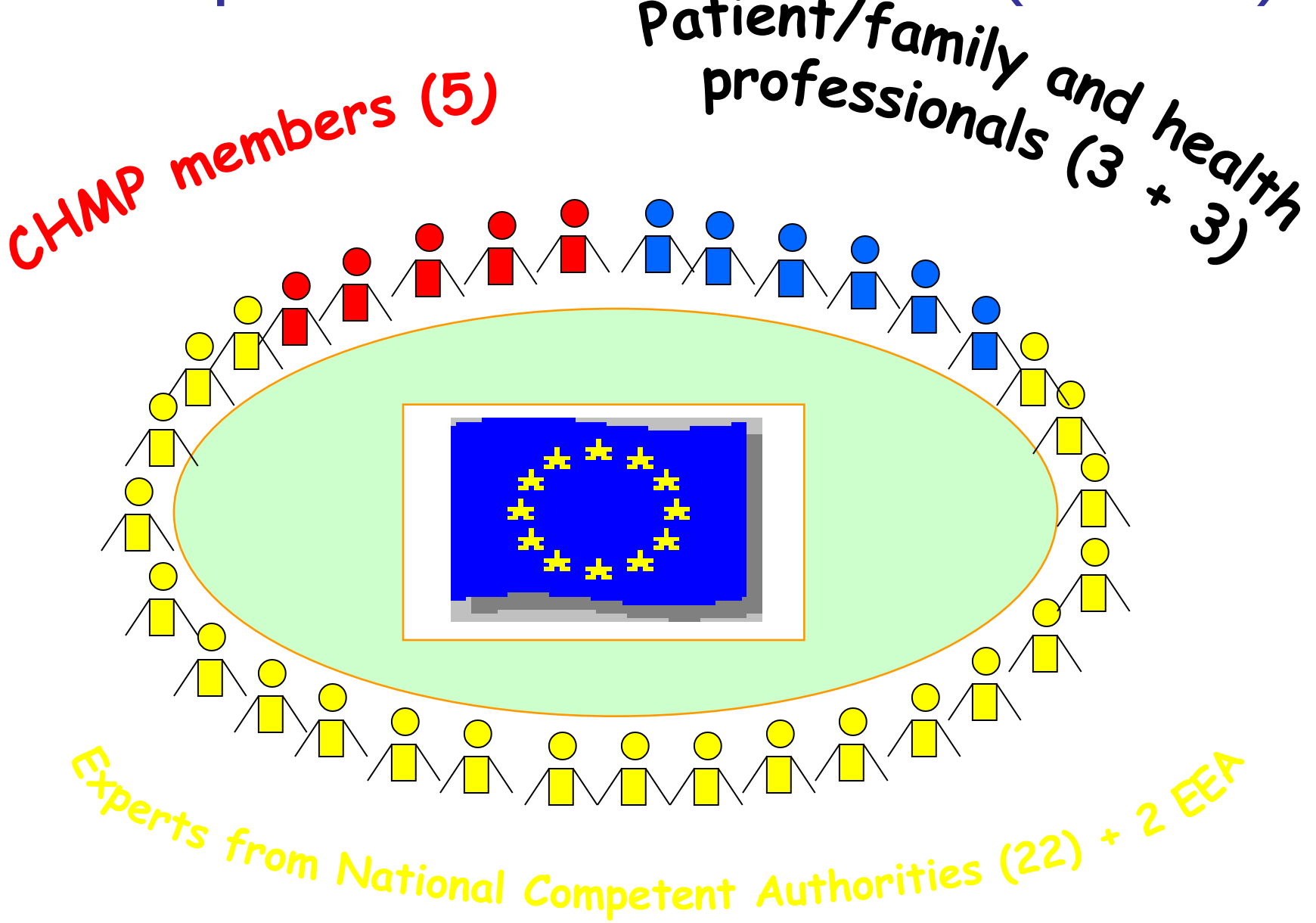
Paediatric Regulation(PR) Objectives

- Improve the health of children
  - Increase high quality, ethical research into medicines for children
  - Increase availability of authorised medicines for children
  - Increase information on medicines
- Achieve the above
  - Without unnecessary studies in children
  - Without delaying authorisation for adults

Tools of the Paediatric Regulation I.

- An expert committee: the Paediatric Committee (PDCO)
- An agreed (evolving) paediatric development: the Paediatric Investigation Plan (PIP) *Compulsory for all new drugs unless a waiver granted by PDCO*

The paediatric Committee (PDCO)



PDCO

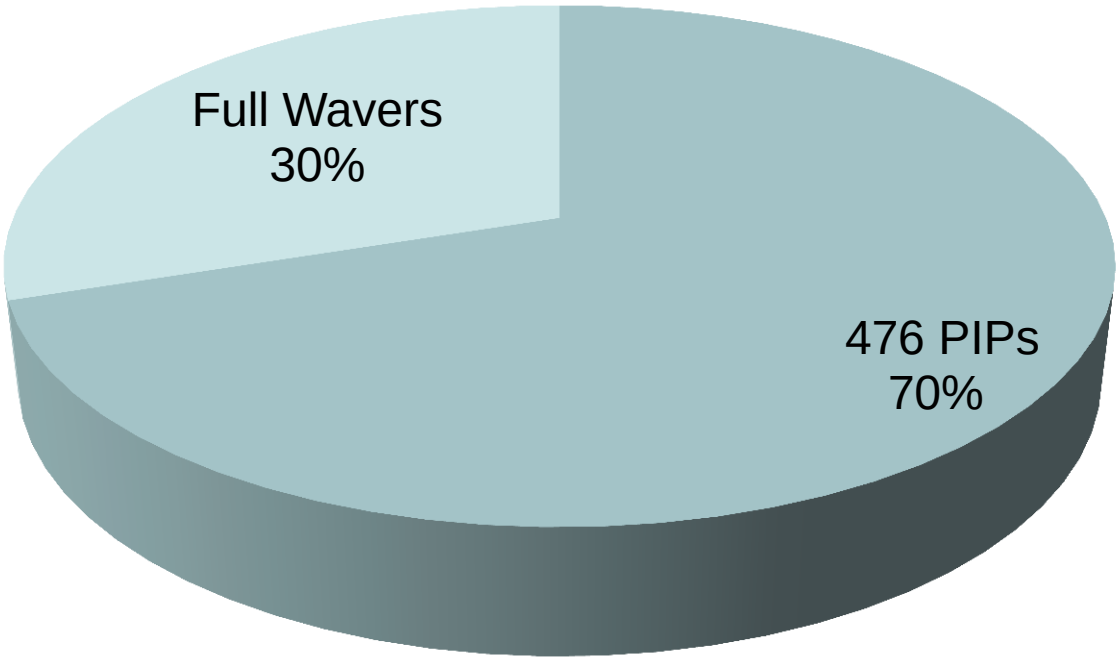
- First meeting : July 2007
- Patient and Health Professionals representatives: call 2007 appointments august 2008
- 3 years renewable appointment
- Second call: 2011
- Complimentary but also specific expertise to the PDCO

Paediatric Investigation Plan

- ✓ the basis for the development and authorisation of a medicinal product for the paediatric population subsets :General strategy for paediatric studies
- ✓ includes details of the timing and the measures proposed to demonstrate:
  - Quality
  - Safety
  - Efficacy
- ✓ to be agreed upon and/or amended by the Paediatric Committee (PDCO)
- ✓ It is binding to the company

Marketing Authorisation criteria

PDCO Opinions 682 march 2012*



Tools of the Paediatric Regulation II

- A set of rewards and incentives
  - For new and on-patent products – 6-month extension of the patent protection (Supplementary Protection Certificate)
  - For off-patent products – Paediatric Use Marketing Authorisation (PUMA)-10 years data protection
- A series of other tools for information, transparency, and stimulation of research

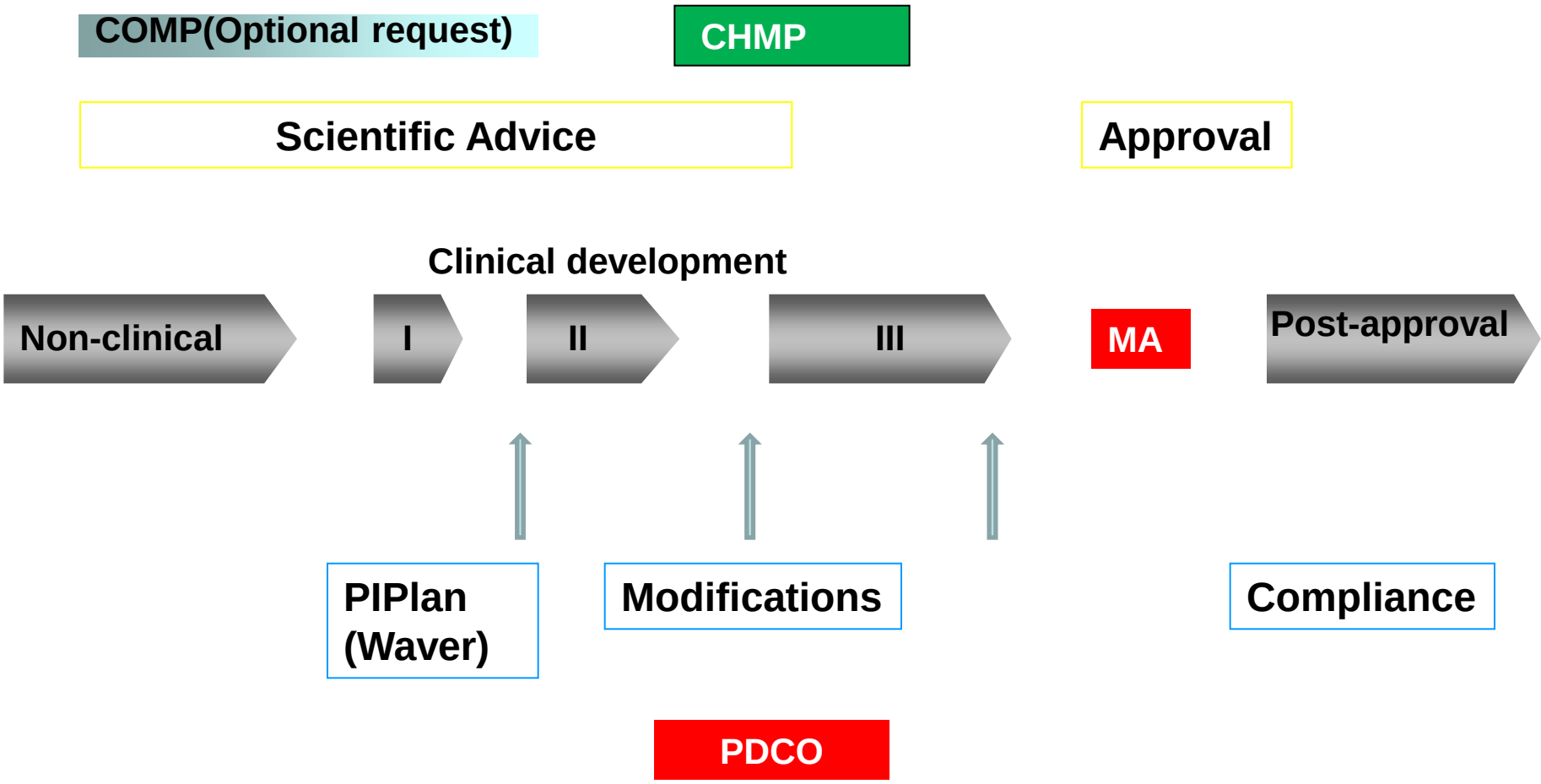
EU Paediatric Regulation: Obligations and Incentives

Type of MP	Obligation	Incentive	Comments
New MP	Paediatric Investigation Plan or Waiver	6 months extension of SPC (patent)	Necessary for validation of application
On Patent and authorized Medicine	Paediatric Investigation Plan or Waiver	6 months extension of SPC (patent)*	When new indication or new route or new pharmaceutical form: necessary for validation
Orphan Medicine	Paediatric Investigation Plan or Waiver	2 additional years of market exclusivity	**10+2** years of market exclusivity
Off patent Medicine	None (voluntary PIP possible for PUMA)	10 years of data protection	Research funds Paed. Use MA (PUMA)

The main tools to achieve them III

- A series of other tools for information, transparency, and stimulation of research:
  - ✓Database of Paediatric Trials (EudraCT)
    - Protocols, Results and Studies previously performed (+/- published
    - Open in 2010
  - ✓Database of authorised Products in EU (EudraPharm)
  - ✓Medicinal Product information (including results)
  - ✓Report on the 5 Years experience with the PR

Role of the PDCO in the regulatory Process



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Thank you !