IMPLEMENTATION OF THE PAEDIATRIC REGULATION

1st EMEA Workshop for SMEs, London February 2007

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Objectives of the Regulation

entered into force 26 January 2007

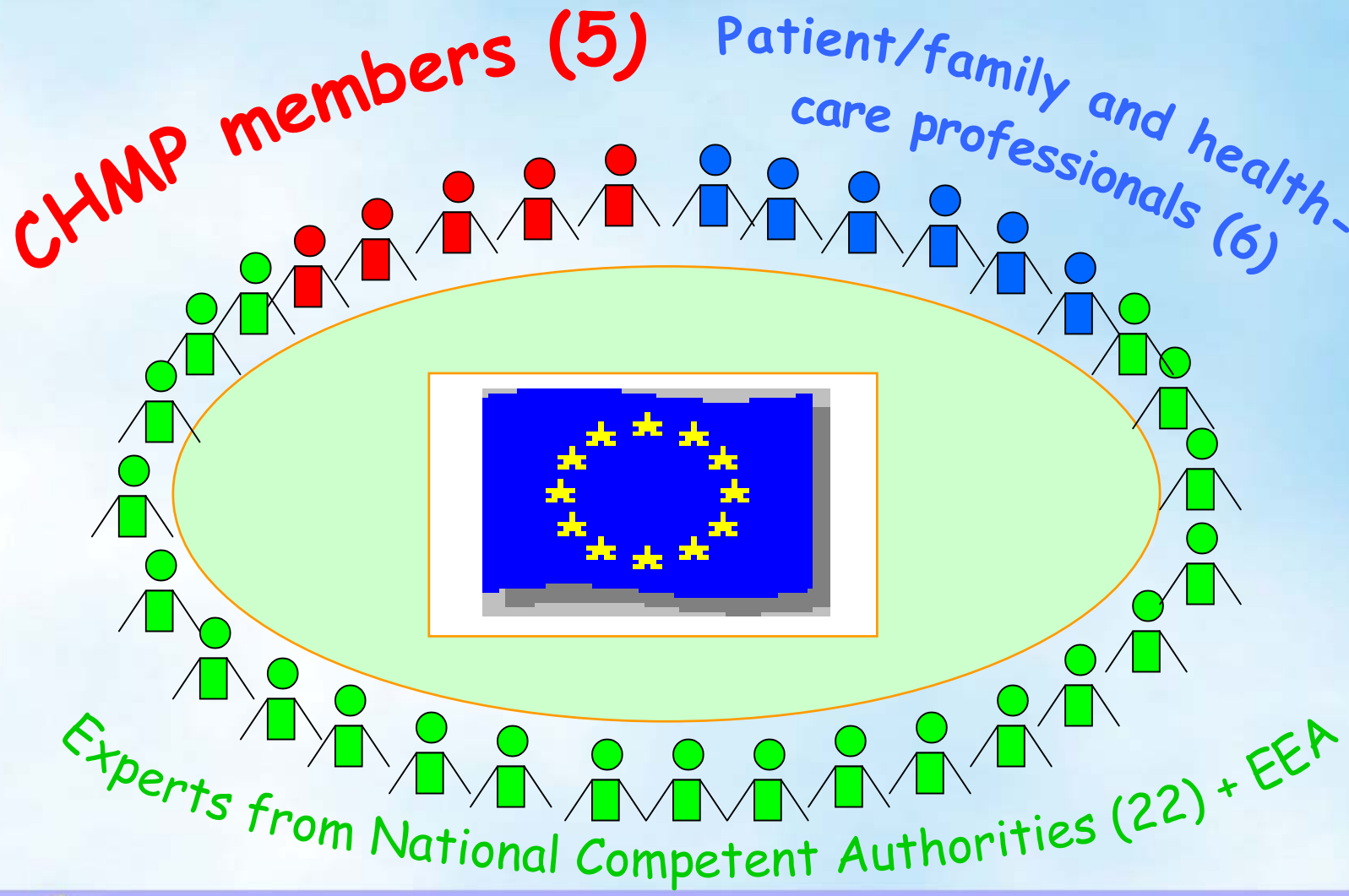
- Improve the health of children
  - Increase high quality, ethical **research** into medicines for children
  - Increase **availability** of authorised medicines for children
  - Increase **information** on medicines
- Achieve the above
  - Without unnecessary studies in children
  - Without delaying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

Main pillars of the Regulation

- New EMEA Committee: the Paediatric Committee
- An agreed (evolutive) paediatric development: the Paediatric Investigation Plan (PIP)
- A mix of rewards and incentives
  - For on-patent products
  - For off-patent products
- A series of other tools for information, transparency, and stimulation of research

Paediatric Committee (PDCO)

to be established by 26 July 2007



+ alternates

Currently unauthorised products

18 months after entry into force of the Regulation, i.e 26 July 2008

- Obligation to submit **results** of agreed Paediatric Investigation Plan at time of marketing authorisation application

X If not: Invalid application for MA

- Results reported in SmPC
- Authorisation in all Member States
- **Reward**:
  - 6-month extension of the Supplementary Protection Certificate

Patent-protected authorised products

24 months after entry into force of the Regulation, i.e. 26 January 2009

- Obligation to submit **results** of agreed Paediatric Investigation Plan at time of change (variation/extension) for new indication, route of administration, or pharmaceutical form
- Results reported in SmPC
- Authorisation in all Member States
- **Reward:**
  - 6-month extension of the Supplementary Protection Certificate

Orphan drugs

- Same obligations
- Need for PIP and compliance
- **Reward:**

  2 years of market exclusivity added to existing 10 years

For off-patent products

Paediatric Use Marketing Authorisation (PUMA)

New type of MA

- Covers exclusively paediatric indication(s) and formulation(s)
- Optional but need for PIP and compliance
  - No need for MA in all Member States
  - Brand name may be retained
  - 10 years of data protection: (8+2) +1

Waivers

- Product likely to be **ineffective or unsafe** in all or part of the paediatric population
- **Disease occurs only in adults**
- **No** significant **therapeutic benefit** over existing treatments for children

→ for one or more subgroups of the paediatric population

→ for one or more specified indications

Deferrals

- Request to defer initiation or completion of some or all the measures set out in the PIP
- On initiative from applicant or Committee
- For all or part of Paediatric Investigation Plan
- Annual report to monitor deferred studies

Paediatric Investigation Plans

- Basis for the development and authorisation of a medicinal product for the paediatric population subsets
- Include details of the timing and the measures proposed (including adaptation formulation) to demonstrate:
  - Quality
  - Safety
  - Efficacy

Marketing Authorisation criteria

Paediatric Investigation Plans

- Research and development programme to ensure availability of data in the paediatric population
- Reference to ICH E11
- To be agreed and/or amended by the Paediatric Committee
- Binding on company
- EMEA Decision published

Significant studies

- Presence is basis for reward
- Significant studies need to be completed after entry into force of Regulation

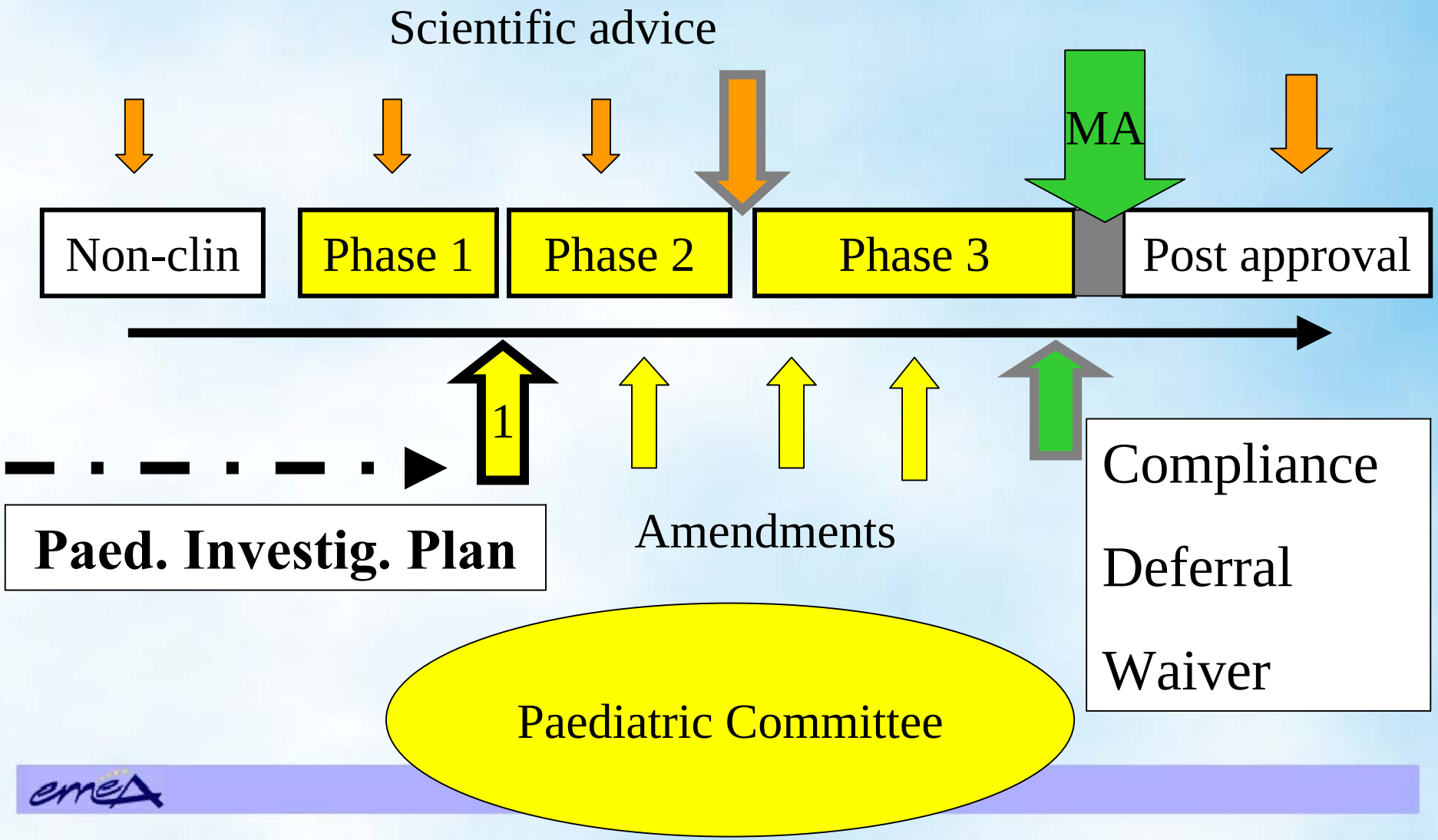
- Transitional measure

If studies already completed, not eligible for reward but data taken in account for PIP

Paediatric Scientific Advice

- Free of charge from entry into force
- Prior to submission of a PIP or during PIP implementation process
- Including advice on pharmacovigilance and risk management systems
- Not binding on Paediatric Committee
- Link Paediatric Committee / Scientific Advice Working Party

Timing Consultation of Paediatric Committee



Other measures

Paediatric needs

- Member States Survey of all existing uses of medicinal products in children, including off-label within 2 years, final EMEA inventory in third year (2009)
- Update of Paediatric needs by Paediatric Committee on basis of inventory

Community funding

- For studies into off-patent medicinal products

Other measures

Community databases

Public access to paediatric information from the European database of Clinical Trials and results (modified EUDRACT) and authorised products (EudraPharm)

Post-authorisation activities

Measures to follow efficacy and potential ADRs and when needed risk management system to be in place.

Symbol of medicines authorised for children

A European Network

EMEA paediatric research network

- To link together existing networks, investigators and centres with specific paediatric expertise
- Build up competences at a European level
- Facilitate the conduct of studies
- Avoid duplication of studies

Preparatory work at EMEA over 2005-6