EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Overview of paediatric legislation for pharmaceuticals

EU Regulatory Workshop
"Paediatric Investigation Plans in Ophthalmology"

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An agency of the European Union

Overview - outline

- Paediatric legislation operating to address unmet needs of children
  - Paediatric Committee (PDCO)
  - Paediatric investigation plans (PIPs)
- Clarifying
  - Waiver
  - Deferral
- Working with stakeholders
  - Pharmaceutical companies
  - Health care professionals
  - Patient organisations and the public

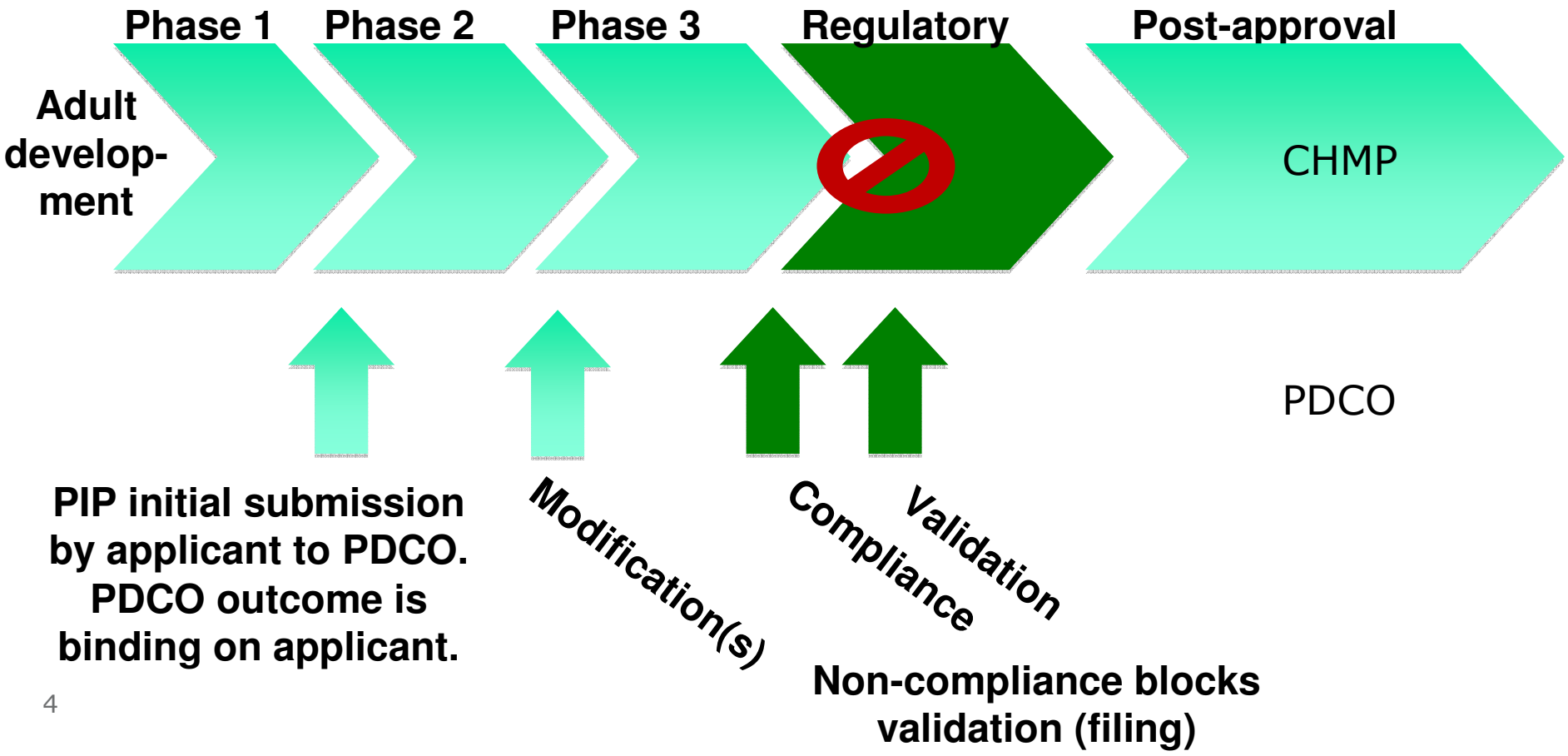
What is the paediatric legislation for pharmaceuticals?

- REGULATION (EC) No 1901/2006
  - of the European Parliament and of the Council of 12 December 2006
  - on medicinal products for paediatric use
  - and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004
  - http://ec.europa.eu/health/documents/eudralex/vol-1/index_en.htm
- In addition, standards apply to paediatric as to adult medicines:
  - Development to take into account EMA scientific guidelines
  - Pharmaceutical quality, safety and efficacy to be demonstrated
  - Benefit – risk assessment by regulators

Objectives of the EU Paediatric Regulation

- 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 authorization for adults

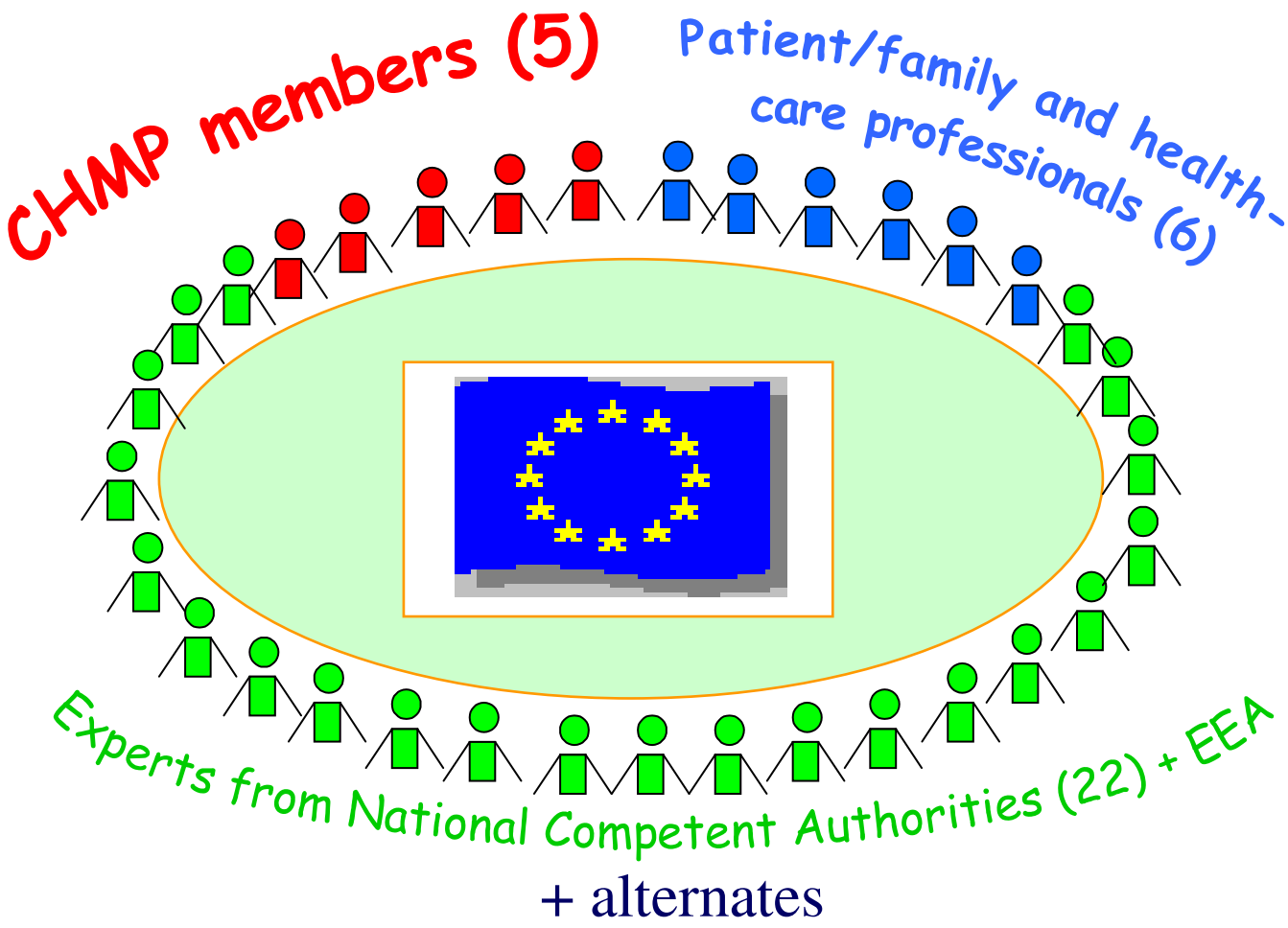
Time lines and relation of PIPs to MAAs



Pillars of the Paediatric Regulation

- Paediatric Committee
- Paediatric Investigation Plans (PIPs)
- A system of OBLIGATIONS and REWARDS
- TRANSPARENCY MEASURES
- OTHER MEASURES

Paediatric Committee (PDCO)

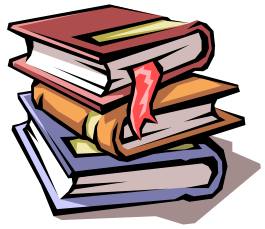


Paediatric Investigation Plans

Details of timing and of measures (i.e., non-clinical studies, paediatric trials and pharmaceutical development) necessary to obtain a paediatric indication with an age-appropriate formulation in all paediatric subsets affected by the condition

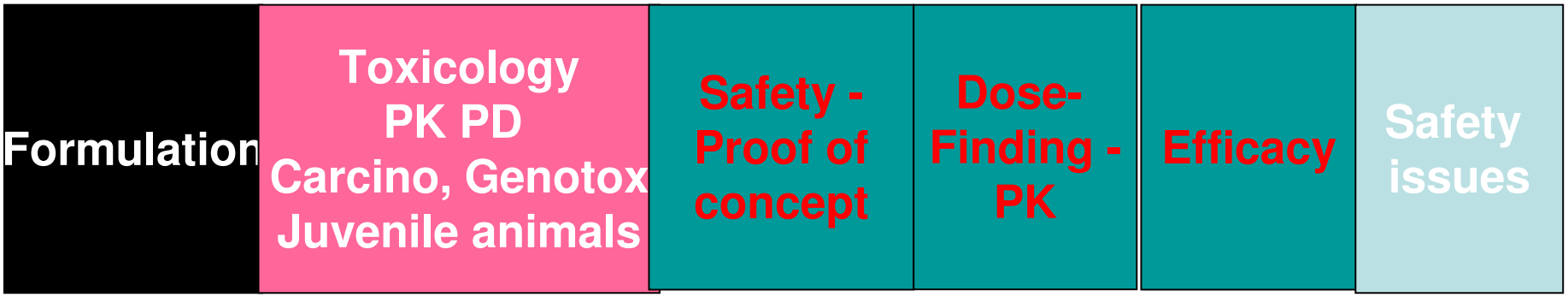
- Quality*
- Safety
- Efficacy

Marketing Authorisation criteria



* pharmaceutical quality

Paediatric Investigation Plan



- Intended to support an indication ("paediatric use") in all relevant subsets of the paediatric population
- Define required data on efficacy, safety and age-appropriate formulation (and ethical aspects)
- Timelines for study start and completion (ICH E11)
- Binding on applicants (but trial authorisation remains to be independently requested)

Waiver(s)

- Legal grounds:
  - Medicine is likely to be ineffective
  - Medicine is likely to be unsafe
  - Lack of significant therapeutic benefit
  - Disease or condition occurs only in adults population
- Three types:
  - "Full" waiver: for all conditions/indications being applied for a product
  - "Partial" waiver: one and more subset(s), indication(s), but there is a PIP!
  - Class waiver: for a class of products in a condition, or for all products aimed at a condition

Deferral(s)

- Instrument to avoid delaying marketing authorisation in adults
- "Deferred" means Marketing Authorisation Application for adults is possible before completion of measures in the PIP
- In an agreed PIP, a deferral(s):
  - Given by study (cf. US PREA "total" deferral)
  - Separately for initiation and/or completion
  - Based on dates or data needed to support study initiation (ICH E11)
  - Completion dates established

Working with stakeholders

- European network of paediatric research (Enpr-EMA)
- Workshops with academic experts as needed
- PDCO consulting frequently with external experts on medicines
- Regular interactions with organisations of pharmaceutical companies
- Patients and parent organisations integrated in discussions
- Transparency by making public
  - PIPs as soon as agreed by the PDCO (i.e., mostly before studies start), including reasoning for or against waiver or deferral
  - Clinical trial information (protocol, coming: results)
  - Consultation on paediatric guidelines

Summary

- Paediatric legislation is complex but already lead to
- Dramatic change:
  - every new medicine to be presented early for paediatric needs and data to prevent / diagnose / treat the disease (condition)
  - paediatric data maybe required for development of "old" medicines
  - first new medicines and new indications for children authorised
- Paediatric investigation plans
  - are driven by knowledge of disease and medical needs in children
  - discuss what data are required to evaluate a medicine for use in children
  - make requirements that are binding on pharmaceutical companies