EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Perspectives from EMA Scientific Committees

Paediatric Committee - PDCO

Regulatory challenges and opportunities

PCWP/HCPWP workshop on personalised medicines

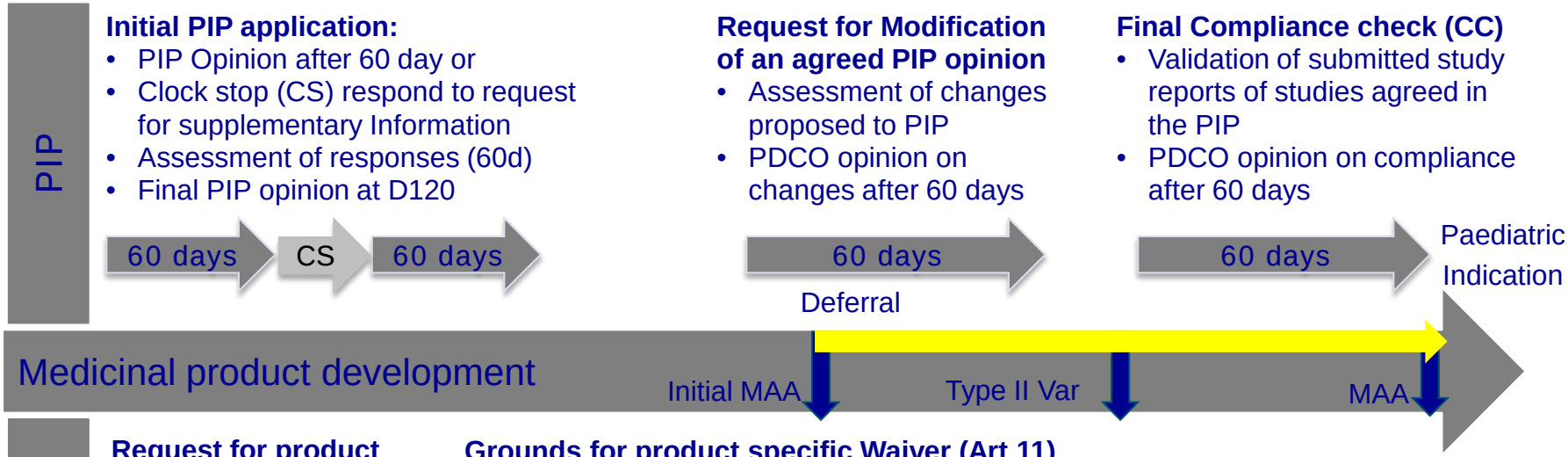
Presented by Dirk Mentzer on 14 March 2017
Chairman of Paediatric Committee

An agency of the European Union

Key developments that are shaping medicines evaluation

- Paediatric development is **mandatory** in the EU and has to follow the agreed key binding elements laid down in the opinion of the paediatric investigation plan (PIP)
- Unless a product-specific **waiver** or a class waiver is granted (which applies only for specific conditions and dosage forms)
- **Early submission** of the PIP to guarante the timely conduction of agreed paediatric clinical trial development to allow authorisation.
- **Deferrals** can also be granted (studies in children can be initiated and/or completed after applying for marketing authorisation in adults)
- **Incentive** for conducting the PIP as agreed with the PDCO in the PIP opinion.

Regulatory challenges



Grounds for product specific Waiver (Art 11)

1. The specific medicinal product or class of medicinal products is **likely to be ineffective or unsafe** in part or all of the paediatric population.
2. **The disease or condition** for which the specific medicinal product or class is intended **occurs only in adult populations**.
3. The specific medicinal product does **not represent a significant therapeutic benefit over existing treatments** for paediatric patients.

Objective of Paediatric Regulation (art. 2.2):

‘Paediatric investigation plan’ means a research and development program aiming to ensure that the necessary data are generated determining the conditions in which a medicinal product may be authorised to treat the paediatric population

Paediatric regulation plays has a key role paediatric drug development

Opportunities

EMA - Paediatric team - Scientific and regulatory support

PDCO and the power of AC/DC

- **A**ssessing - PIPs
- **C**ontributing - with expertise
  - guidelines
- **D**eciding - PIP opinion
- **C**ooperating - CHMP
  - SAWP
  - PRAC (WG)
  - COMP (WG)
  - CAT (case by case)

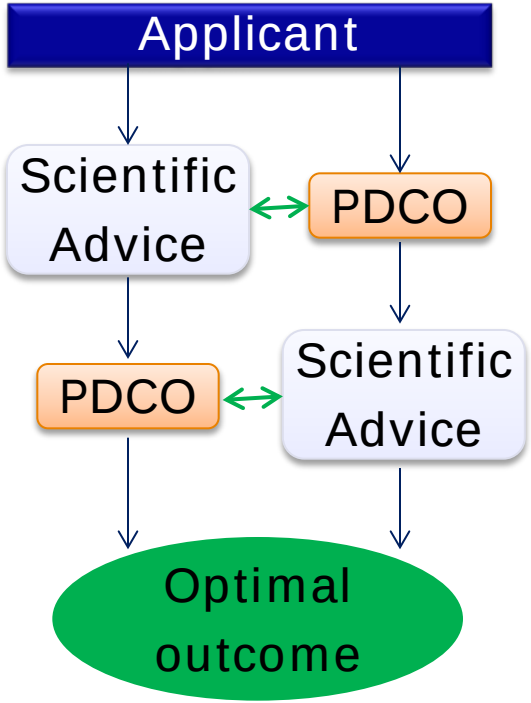
Industry: PIP application
Discussing paediatric issues
medicinal product development

Public: Input from young people into CT
EnprEMA (Networks)
Communicating awareness
Education (GRIP)

Opportunities for interaction

PDCO tries to ensure a successful PIP/waiver procedure and paediatric development by ensuring best use of existing resources efficiently and supporting use of

- extrapolation
- Modeling and simulation
- Innovative trial design
- Multi-company-multi-drug clinical trial



Thank you for your attention

Further information

Link to EU - Paediatric Regulation
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/document_listing/document_listing_000068.jsp&mid=WC0b01ac0580925c45