What has the paediatric Regulation achieved?

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Paediatric report

- *More paediatric clinical research;*
- *More medicines for children (new medicines or new indications);*
- *More information available.*

The Paediatric Report

Challenges identified

- Differences between the various therapeutic areas (paediatric only, like many cancers);
- Completion of PIPs;
- Overlaps with the orphan legislation;
- Rewards not always "working (SPC, orphan reward, PUMA).

Next steps

- Short term actions: EMA-Commission action plan;

- Medium term vision: evaluation paediatric & orphan medicines Regulations.

EMA Commission Action plan

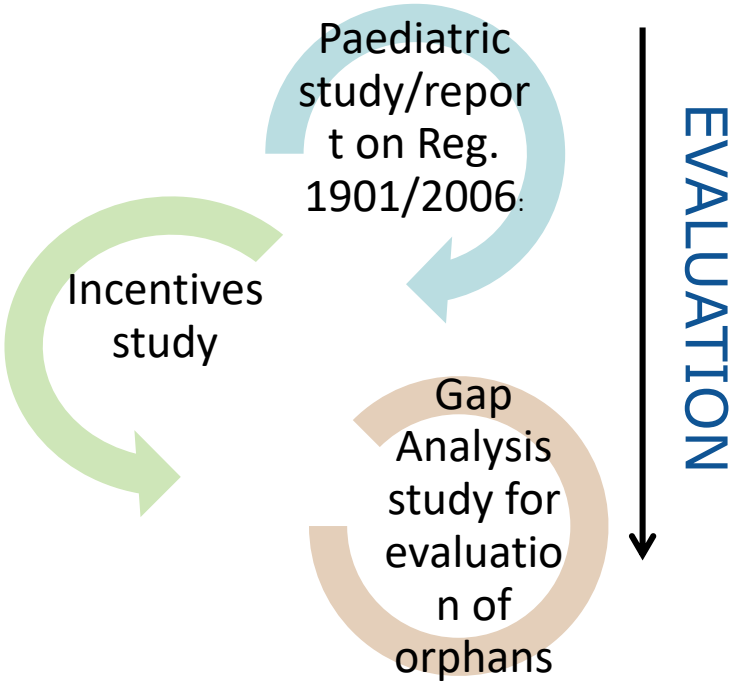
1. Identifying paediatric medical needs;
2. Strengthening of cooperation of decision makers;
3. Ensuring timely completion of paediatric investigation plans;
4. Improving the handling of PIP applications;
5. Increasing transparency around paediatric medicines

Next steps

Short term actions

- Discuss paediatric needs in an open and transparent dialogue with all interested parties;
- analyse the experience with use of deferrals; speedier completion of PIP;
- handling of PIP applications; if necessary adapt Comm. Guidelines;
- provide additional transparency of new products authorised with paediatric indications;
- deliver regular updates about development and trends of the paediatric medicines landscape fostering international cooperation and harmonisation;
- foster international cooperation.

Medium term



European Commission

Commission evaluation

- *Commission Staff working documents;*
- *Look backward to "evalaute" if the objectives of the legislations have been achieved;*
- *Assessment under 5 angles:*
  - **effectiveness;**
  - **efficiency;**
  - **relevance;**
  - **coherence;**
  - **EU added value**
- *Data collected via various sources.*

Medicines for rare diseases and children: learning from the past, looking to the future

Multistakeholder conference 17 June 2019

- *Opening session*
- *Breakout sessions*
- *Open space*

Unmet Medical needs

- *Common understanding and quantification of unmet medical need*
  - **Input "expert patients" to define unmet needs**
- *Incentives proportional to the addressing of unmet needs*
- *Global cooperation and data sharing (medicines for children)*
- *Early granting orphan designation (concept stage)*

Incentives

- Need for incentives
- Connection between reward and cost of development not always clear
- Support to real innovation/value concept
- Need for better coordination and identification of priorities
  - ➢ **Stimulation of basic research**

Medicines for children

- Development adult driven, Return on investment → important factor for paediatric developments
- Paediatric ‘Masterplans’ → all stakeholders on-board ("paediatric centers")
- ‘Orphan-like’ designation: for conditions not meeting orphan criteria (but small subpopulations with special formulation)
- Two regulations not aligned and proportionate in relation to definitions → review of rewards and incentives?

From R&D to patients

- Academia limited knowledge of regulatory requirements and incentives
- More support to basic research but avoiding duplications
- Data sharing, more transparency on outcomes of public investments
- Focus Real World Evidence on overcoming lack of consistent data/need for standardisation.

Scientific developments

- Better link needed between genetic sequencing, biological data and outcomes
- Need for multi stakeholder engagement → change of evidence standards and scientific advances
- Better cross-committee operations at EMA

Open space

- Shelved medicinal products (re-purposing possibilities);
- Avoid evergreening (generic entry and return on investments)
- Global harmonisation and cohordination
- Innovation and flying
- Continuous evidence generation
- Value based pricing
- Concept of rarity
- Targeted treatments for children

What's next?

Thank you for your attention!

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