Pharma Industry point of view on paediatric medicines

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Outline

- View since 2006 – what is working well?
- Where are we now – what could work better?

Pharma Industry point of view on paediatric medicines

1. **View since 2006 – what is working well?**
2. Where are we now – what could work better?

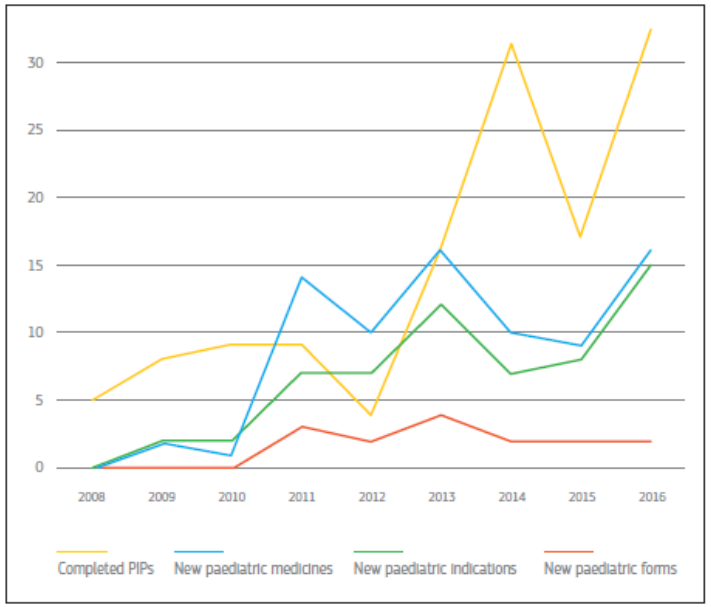
The 2006 Paediatric Regulation – aims and impact

- to encourage and enable high-quality research into the development of medicines for children;
- to ensure, over time, that most medicines used by children are specifically authorised for such use with age-appropriate forms and formulations; and
- to increase the availability of high-quality information about medicines used by children.

More paediatric trials

Source: EudraCT database

More authorised medicines



Source: EMA databases (only centrally authorised medicinal products).

260 new medicines & indications for treating children since 2007

The 2006 Paediatric Regulation – aims and impact

- Paediatric development integral to medicine development – considered globally.
- EU and US regulator coordination/ discussions to support effective development and consistency.
- Value of the EU and US frameworks recognised e.g. recent Swiss requirements.
- What next – how can we take things to the next stage?
    - Address highest unmet needs
    - Make best use of evidence to support development
    - Do this in a collaborative and pragmatic way that gets treatments to patients in a reasonable time frame
    - *Key issues not inherent in the Regulation itself.*

Pharma Industry point of view on paediatric medicines

1. View since 2006 – what is working well?
2. **Where are we now – what could work better?**

Where are we now – what could work better?

Implementation of Oct 2018 EMA/EC Action Plan on paediatrics

- Identifying paediatric medical needs
  - *Facilitate strategic development decisions to meet paediatric medical needs and focus resource into neglected areas.*
- Strengthening of cooperation of decision makers
  - *Better EMA/PDCO and MS dialogue and EMA/FDA interactions (cluster calls/ joint advice)*
- Ensuring timely completion of paediatric investigation plans (PIPs)
  - *Better trial landscape: identify key issues impeding trial conduct, improved infrastructure & national alignment (networks), drive awareness of extrapolation methodologies, training/ awareness.*
- Improving the handling of PIP applications
  - *Explore integrated scientific discussion/ advice and operational aspects including: compliance check,, submission requirements etc.*
- Increasing transparency around paediatric medicines

PIP submission timing and support

PIP submitted early*

- **OK** if well characterised mechanism/ established medicine class
- **OK** if established indication
- **OK** if stable standard of care
- **OK** if not using innovative techniques to maximise evidence (e.g. extrapolation)

- **Not OK** if new mechanism/ medicine class
- **Not OK** if new therapeutic indication
- **Not OK** if standard of care is changing
- **Not OK** if using e.g. innovative trials/ extrapolation

'Strategy' then PIP

- Examining within current legislative framework
- Key supporting aspect is integrated paediatric scientific advice

** Not later than on completion of human pharmacokinetic studies in adults*

PIP adjusted to evidence

Aim: define condition to target (inc prevalence, SoC etc.), population (age groups) & endpoints

Early interaction

Can be informed by previous discussions

Involve the right scientific & paediatric experts

Draft paediatric strategy discussion	PIP submission
Integrated sci advice, with F/U advice to adjust draft PIP based on generated adult data	Alignment with PDCO inc agreement on waivers/ deferrals (if proposed)
Quality: propose age appropriate formulation if needed, inc questions	Age appropriate formulation agreed inc description and planned completion date
Non clinical: tox and efficacy studies proposed inc questions	Tox and efficacy studies agreed with description of studies & completion dates
Clinical: study proposals inc questions e.g. prevalence calculation, what may justify delay	Detailed study designs agreed inc confirmatory with planned completion dates
M&S/ extrapolation: proposal for M&S extrapolation plan	Plan agreed based on data, full plan with planned completion date included

Questions?

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