Evaluation of the legislation on medicines for children and rare diseases

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Enpr-EMA Annual meeting
28 September 2020

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Evaluation of the Regulations for rare diseases and medicines for children

- Strengths and weaknesses legislation 2000-2017 (medicines for rare diseases) and 2007-2017 (medicines for children);
- Commission Staff Working Document published in August 2020;
- Part of Pharmaceutical Strategy;
- More advanced, reflects many issues of the Strategy.

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Orphan medicinal products

- Increased number of orphan medicinal products authorised
  - 18-24 could be attributed directly to the EU Regulation;
- Almost 2000 designations
  - But for many development status is unknown;
- A certain degree of indication stacking detected
  - Futute situation with personalised medicines;
- Is the prevalence criteria still adequate?
- Return on investiments criteria.

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Orphan medicinal products

- Well-established use and repurposing
  - Less than in the US but price impact;
- High cost of incentives (ME mainly):20-25 b. € (plus EU/national support to research); cost benefit slightly positive (direct impact of the Regulation);
- ME is a powerful incentive, cases of overcompensation (difficult to determine return on investment);
- Quicker on the market but not at the same time in all MS.

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Paediatric medicinal products

- Increase in paediatric trials
- High number of PIP
  - PIP procedure adequate in all situations?
  - Waivers
  - Long deferrals, possibility to follow up;
- New products/indications
  - Not a success in all therapeutic areas, some limited development;
- PUMA limited success (external factors).

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Paediatric medicinal products

- Availability linked to adult products;
- SPC complex and not uniform application (not all the products managed to benefit from it);
  - Push development when parallel adult blockbuster
  - Some cases (limited of overcompensation);
- Cost benefit of the Regulation overall positive.

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Next steps

- Inception impact assessment (public consultation Autumn/Winter)
- Impact assessment

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Main focus

- To ensure that the legislation is fit to embrace technological and scientific advances;

- To foster research and development of orphan and paediatric medicinal products especially in areas of unmet need;

- To provide effective and efficient Union procedures, for assessment and authorisation of orphan and paediatric medicinal products;

- To ensure the availability and timely access of patients to orphan and paediatric medicines.

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Thank you

For more information:

Evaluation of the medicines for rare diseases and children legislation:
https://ec.europa.eu/health/human-use/paediatric-medicines/evaluation_en

EU Pharmaceutical Strategy:
https://ec.europa.eu/health/human-use/strategy_en

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