European Commission

Evaluation of the legislation on medicines for children and rare diseases

Pharmaceutical Committee
2 July 2020

Health and Food Safety

European Commission

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; part of Pharmaceutical Strategy
- In particular for orphans, many challenges identified mirror general challenges
- More advanced, reflects many issues of the Strategy

European Commission

Main findings evaluation

1. Unmet medical need

2. Availability and accessibility

3. Technological and scientific developments

Health and Food Safety

European Commission

Unmet medical need

- 95% of rare diseases without treatments; out of 142 authorised products → ≈30% targets disease with no alternative treatment option
- > € 1.4 billion from EU research programs spent
- Prevalence threshold still appropriate (or different method calculating prevalence?)
- Insufficient Return of Investment criterion: not used
- Paediatric pipeline strongly dependent from adult pipeline
- No efficient tools to direct paediatric development
- PIP may be waived for some products which could be effective in children (paediatric oncological products)

European Commission

Availability and accessibility

- Development and availability orphan and paediatric products ▲▲

  → calculated direct impact: 18 – 24 orphans (out of 142 products authorised)

  → over 200 new medicines for use in children + over 1000 paediatric investigations plans

- Orphan medicines on average 9 months earlier available
- Paediatric medicines dependent from "adult" product launch strategies
- Not translated into immediate *accessibility* in all Member States.
- External factors: deferred and strategic launch policies; national pricing and reimbursement decisions.

European Commission

Technological and scientific developments

- Advances in science (personalised medicine, use of biomarkers, innovative trial designs) → tools Regulations 'fit-for-purpose'?

- Widespread diseases 'split' into artificial subsets of rare diseases

European Commission

Next steps

- Publication SWD (summer)
- Dedicated Pharmaceutical Committee (after summer)