



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/EC action plan on paediatrics 2018-2022

9th Industry stakeholder platform on research and development support
5 December 2022

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An agency of the European Union





EC 10-year report on the Paediatric Regulation



1. Closure of Action plan
2. Revision of legislation



European Medicines Agency and European Commission (DG Health and Food Safety)
action plan on paediatrics



Topic areas

- Identifying paediatric medical needs
- Strengthening of cooperation of decision makers
- Ensuring timely completion of paediatric investigation plans (PIPs)
- Improving the handling of PIP applications
- Increasing transparency around paediatric medicines

To note: progress with the implementation of the action plan was intermittently guided by BCP considerations

Medical needs

- Multi-stakeholder forums on unmet needs
- Framework for systematic assessment of unmet needs in PIPs

Cooperation of decision makers

- CTCG/PDCO working group established
- International relations (FDA/EMA, Enpr-EMA)
- Collaboration EMA/PDCO and HTA bodies

Timely PIP completion

- Paediatric clinical trial preparedness framework
- Guidance on the use of extrapolation
- Paediatric addenda to scientific EMA guidelines

Handling of PIP applications

- Compliance check simplification
- Paediatric process review
- Stepwise PIP concept
- Key elements revision

Transparency

- Community register and increased transparency around paediatric medicines

In summary, the action plan has led to:



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Increased
collaboration of
stakeholders

Focus on areas of
high unmet medical
need

Adapting to
evolution of clinical
development
through utilisation of
extrapolation, M&S

Strengthened
cooperation among
regulators

Less bureaucracy

Future proof
processes adaptable
to innovative
medicine
developments (sPIP)



Planned publication of closure report

Snapshot of tabulated list:

Closure report:

- making transparent the achievements
- brief summary explaining key progress and relevance,
- followed by tabulated list of achievements on basis of interim report

Action	Objectives	Achievements
1	Develop overview of selected therapeutic areas to identify paediatric medical needs. Actions include: • Conducting public survey on criteria proposed for determining paediatric medical needs and on perceived areas of needs • Selecting therapeutic areas based on various factors, including experience with PIPs and stakeholder feedback, for further analyses by multi-stakeholder focus groups • Conducting multi-stakeholder workshops in selected therapeutic areas • Publishing reports on the paediatric therapeutic landscape related to selected areas	To raise awareness for paediatric medical needs with a view to providing a basis for strategic decision making on paediatric medicine development. • Strategies to address needs in children with malignancies were determined at multi-stakeholder forums organised by EMA together with the ACCELERATE platform: ◦ ACCELERATE & EMA Paediatric Strategy Forum for medicinal product development of checkpoint inhibitors for use in combination therapy in paediatric patients (09/2018), related publication in Eur J Cancer (11/2019) ◦ ACCELERATE & EMA Paediatric Strategy Forum for medicinal product development for acute myeloid leukaemia in children and adolescents (04/2019), related publication in Eur J Cancer (09/2020) ◦ ACCELERATE & EMA Paediatric Strategy Forum for epigenetic modifiers in paediatric malignancies (01/2020), related publication in Eur J Cancer (11/2020) ◦ ACCELERATE & EMA Paediatric Strategy Forum on CAR T-cell developments (5/2021), related publication in Eur J Cancer (11/2021) new ◦ ACCELERATE & EMA 2nd Paediatric Strategy Forum on ALK inhibitors (06/2021), related publication in Eur J Cancer (11/2021) new ◦ ACCELERATE & EMA Paediatric Strategy Forum for medicinal product development of multi-targeted kinase inhibitors in bone sarcoma (12/2021) new • Review of the value of Paediatric Strategy Forums for regulatory decision making was published in <i>Clin Pharmacol Ther</i> (06/2020).



Thank you

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