EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA-EC Action Plan on Paediatrics
Progress update

5th Industry Stakeholder Platform on R&D support, 16 November 2020

Presented by Gunter Egger
Scientific officer / Paediatric medicines

Reminder: development of the EMA-EC action plan

- Nov 2017: EC 10-year-report on Paediatric Regulation
- March 2018: EMA/EC multi-stakeholder workshop
- Oct 2018: Publication of action plan
- → Ongoing work – multi-stakeholder effort

To note: progress with the implementation of the action plan has been guided by BCP considerations

Today's context for the action plan

Important now to take stock and to progress, also in the context of:

- the EC study on the paediatric regulation
- the EC pharmaceutical strategy

Scope for today:

1/ inform stakeholders about the progress with the action plan

2/ learn about Industry's initiatives/proposals

3/ agree on next steps to progress topics further

Status update – paediatric medical needs

	Action	Objectives	Progress by November 2020	Status
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: o 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) o ACCELERATE & EMA Paediatric Strategy Forum for medicinal product development for acute myeloid leukaemia in children and adolescents (04/2019), related manuscript accepted for publication in Eur J Cancer (05/2020) o ACCELERATE & EMA Paediatric Strategy Forum for epigenetic modifiers in paediatric malignancies (01/2020) • Review of the value of Paediatric Strategy Forums for regulatory decision making was published in Clin Pharmacol Ther (06/2020). • Contribution to a Multi-stakeholder workshop on paediatric unmet medical needs (12/2019), which identified the opportunity for disease-focused workshops during 2020/21 in collaboration with the IMI c4c project.	In progress.

Status overview

1	**Medical needs**
1.a	Survey on needs, focus groups
1.c	Workshops on needs
1.d	Publication of reports on needs
2	Framework for systematic assessment of needs in PIPs
3	Molecular targets / establish collaboration with FDA
2	**Cooperation of decision makers**
1	Improve dialogue between PDCO & CT assessors
2	Better integration of FDA/EMA cluster
3	Better transparency of FDA/EMA cluster discussions
4	Increase interactions of international regulators & networks

3	**Timely PIP completion**
1	Recommendations for conduct of CTs
2	Publish EU-NTC training material
3	Young people engagement
4	Extrapolation paper
5	Scientific guidelines
4	**Handling of PIP applications**
1	PIP model based on level of evidence
2	Improve opportunities for dialogue with applicants
3	Compliance check simplification
4	Summary report template review
5	Opinion key elements review
6	Website/guidance
7	Simplify submission requirements
5	**Transparency**
1	Community register
2	Transparency CT Regulation

Planned publication of interim progress report

- Published action plan to be updated with column for progress report
- By mid-December 2020

Next: Working together to progress remaining actions and to find best solutions