PCWP/HCPWP joint meeting-Committee feedback
HCP PDCO Presentation

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Boosting the development of medicines for children: Closing report of the European Medicines Agency and European Commission (DG Health and Food Safety) action plan on pediatrics (PDF/2.17 MB)

Identifying pediatric medical needs: Workshops, Accelerate Pediatric Strategy Forums

Strengthening of cooperation of decision makers: FDA, Enpr-EMA, Human HTA bodies

Ensuring timely completion of pediatric investigation plans (PIPs): Trial preparedness, Reflection papers, Extrapolation, structured Guidance (DM)

Improving the handling of PIP applications: Stepwise PIP, pilot started, Waiver/Deferral scrutiny

Increasing transparency around pediatric medicines: Community registry for medicines and trials

Accelerate Pediatric Strategy Forums

Pediatric Oncology Strategy Forums we are organizing together with the multistakeholder platform Accelerate. Workstream of Accelerate is, EMA and participation of the FDA to organize the Pediatric Oncology Strategy Forums

Forums twice a year (on the topic of disease or class of products, alternating between the US and Europe). Sharing information in pre-competitive space, pharma presenting life assets, listening to concrete development challenges from the different perspective (including patients and parents), to take this into consideration for future actions, including the design and agreement of PIPs as you described. We have published the high value of the Forums supporting our regulatory decision making.

Outcome Strategy Forum is a scientific publication including concrete conclusions with follow up activities. Papers: Forum on epigenetic modifiers published need for prioritization recommendations was published. Forum on Pediatric AML, also on Mature B cell malignancies recommended initiation of platform trials, which were implemented, including input from us through CHMP SA qualification procedure.

Consortium C4C has organized multi stakeholder meetings, including one in IBD, require funding, did not go into prioritization of the pipeline. Role of EnprEMA, C4C

Guidance for Stepwise PIP pilot (PDF/240.73 KB) (new), First published: 06/02/2023/EMA/768685/2022

Adapting regulatory processes to better support innovation

Launch of a pilot phase for a ‘stepwise PIP’ agreement

Certain cases to agree on a partial development program, conditional on the development of a full PIP once evidence becomes available over time (partial age waivers)

Allow agreeing on PIPs for innovative medicines where crucial information is not yet available (no validated biomarkers/PD endpoints)

Planning the conditions and milestones for companies to return to EMA’s pediatric committee (PDCO) and discuss the uncertainties once more data are available

Stepwise development with fixed steps and deadlines avoiding modifications

Guidance for Stepwise PIP pilot (PDF/240.73 KB) (new), First published: 06/02/2023/EMA/768685/2022

Adapting regulatory processes to better support innovation, pilot phase for a ‘stepwise PIP’ agreement, partial development program, innovative medicines where crucial information is not yet available, companies to return to PDCO once more data are available, stepwise development, fixed steps, avoiding modifications

Soft tissue sarcoma (MDM2 inhibitor)

TP53-MDM2 signaling pathway in postnatal organogenesis

TP53mut/wt-MDM2 over/expression in pediatric tumors

Age related tumors

Allometric scaling not allowed, elimination of drug in children different age related

Stregthened focus on unmet medical needs
RWE

Multi-stakeholder strategy fora to discuss and agree the needs of children with cancer, and of children with inflammatory bowel disease

DARWIN EU: ERN Rare Liver