



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Focus group on the concept of an 'evolutionary' PIP

Interim feedback to

6th Industry Stakeholder Platform on Research and Development support
4 June 2021





Meetings & Participants

- 3 Meetings (8 & 29 April, 31 May)
- Participants
 - Industry representatives from trade associations
 - Vice-Chair PDCO, EMA representatives



Objective & Expected outcomes

To explore a PIP model that allows, in certain cases, the paediatric development programme to become more defined over time as more evidence becomes available. The group should discuss possibilities for and limitations of such PIP model that allows to develop along with the evolution of scientific knowledge.

Mtg 1

- Reflections on a scientifically sound basis and in compliance with the regulatory requirements
- Consideration should be complemented with illustrative examples.

Criteria for the identification of paediatric developments that would qualify for a step-wise approach to agree the binding elements in the PIP

Mtg 2 (to re-visit)

Concepts for planning milestones on the basis of available evidence, with resulting binding elements and commitment for future interaction

Mtg 3 (to re-visit)

Analysis of risks inherent of such model(s), including potential delays of paediatric development, as well as resource investment

Outline of a supporting framework for dialogue in context of such PIP submissions.



Overview of criteria discussed

Criteria:

- Not fully characterised Mechanism of Action ('first in class')
- Tumour/tissue agnostic therapies
- Gene therapies
- Programmes supported by extrapolation or innovative trial designs
- "First in disease", paediatric only
- Challenges with standard of care (e.g. not existing, recently changed, heterogenous across regions)
- Programs with challenges e.g. due to patient scarcity

Discussion:

- Combination of criteria
- Case by case discussion
- To be revisited after further discussions on models/operational concepts



Overview of concepts/models discussed

- «Two-step PIP»
 - Filing of a «proof-of-concept PIP» with high level description upon completion of adult PK studies, followed later by PIP with more detailed measures based on generated data
- Better use the concept of 'duly justified' for the timing of PIP application
 - Discussion within SA or integrated dialogue, followed by PIP filing with detailed measures once this is possible based on generated data and advice input received
- Discussion
 - First highlevel discussion
 - further details, operational aspects, inherent risks and benefits to be discussed in following meetings



Outlook

- Operational: regular monthly meetings
- Inclusion of aspects discussed in parallel FG
- further details, operational aspects, inherent risks and benefits to be discussed in following meetings
- Presentation of outcome and proposal(s) at 7th Industry R&D platform in Autumn 2021



Industry's feedback

- Small, effective group
- Open dialogue, productive discussions
- Appreciate having EMA, PDCO and industry at the same table to look at risks and benefits of the proposed model(s)
- Looking forward to exploring how the model(s) can be operationalised
 - We can bring examples and test cases



Thank you for your attention

Further information

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