European Federation of Pharmaceutical Industries and Associations

EMA/EC multi-stakeholder workshop to further improve the implementation of the paediatric regulation
20 March 2018

Topic 1
Identification of paediatric medical needs

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Identification of paediatric medical needs – methodology
Industry's perspective

- Which ongoing initiatives to identify paediatric medical needs are you working on or aware of?

- Which criteria and methodology would you suggest to prioritise diseases/conditions of unmet paediatric needs?

Ongoing initiatives to identify paediatric medical needs

- **At company's level: opinions from KOL/experts, academic societies, patients, caregivers and networks on their view on medical needs**
  - based on clinical experience of disease registers or national quality hospital registers
  - contributes to knowledge of disease
  - informs decision on scope of PIP

- **Across companies:**
  - Disease-specific strategy forum: example of Accelerate/EMA forum on mature B cell malignancies in children (London-November 2017) and the PAH forum (June 2017)

    - input of multistakeholder contribution (regulators (EMA [PDCO, COMP, CHMP], FDA) and academics, patient representatives, industry

    - to address medical needs, specify specific populations (high risk, neonates, oncology)
  - Additionally IMI projects e.g. IMI2 ITTC-P4 to define and provide clear guidance for use

Methodology – Industry's position (1)

Disease classification

There should be an agreement on the disease classification(s) that are used to organise the paediatric conditions according specific categories defined in classification system

Data attached to each paediatric medical need:

In addition to the data required under Article 43 (**prevalence** of the conditions in the paediatric population, the **seriousness** of the conditions to be treated, and the availability and suitability of **alternative treatments** for the conditions in the paediatric population), there should also be an indication of:

- **Type of need**
  - Need for treatment
  - Need for better treatment (eg. improved safety and/or efficacy)
- **Existing supporting data**, including ongoing clinical trials, basic research to increase scientific knowledge (e.g etiology of disease) or other research (e.g. to refine target)

Methodology – industry's position (2)

- **The list of paediatric medical needs should be agreed by all stakeholders**

  For industry, it is key to direct paediatric development for the products in their pipeline to the most appropriate area

- **Need for regular updates of the list of paediatric medical needs**
  - multistakeholders contribution
  - frequency to be determined
  - information on status of research
  - global alignement
- **Need to discuss how the list of paediatric medical need is leveraged in decision-making (commitments versus waivers and/or deferrals)**

To conclude

- We believe that defining paediatric medical needs has benefits well beyond PIPs in paediatric research
- We support a multistakeholder review to clarify paediatric medical needs
- We agree that ‘specialist groups’ should be tasked, e.g. as done for paediatric oncology and for PAH
- Paediatric networks have a key role to play
- And that the regular review of the needs is a way of ensuring that research continues to be directed towards areas of unmet need to enable better research in children’s diseases