Development of fixed dose combination for the paediatric population – therapeutic need – requirements – considerations

- Specific aspects for sub-groups of paediatric population / age limit
- Reflecting potential differences in pharmacology
- Harmonising treatment guidelines
- Position of the adolescence group in the development
- Adaptation of dosing
- Age-appropriate formulation
Expert meeting on paediatric development of fixed-dose combination in treatment of HIV infection

EU Paediatric Committee

COMPOSITION

5 CHMP members

+ 3 members from Patient Groups

+ 3 members from HCP associations

Each member has an alternate
12 meetings per year
• Legal obligation for pharmaceutical industry developing new medicinal products to include paediatric development.

• Mandatory paediatric development for new products according to a PIP assessed and agreed by the PDCO (reflecting deferrals or waivers)

• The planned development (PIP) should be discussed early with PDCO

• Definition of potential needs, formulations of interest and appropriate investigational approaches

• Mandatory submission of paediatric data when filing for marketing authorisation unless waiver or deferrals have been approved by PDCO

• New Marketing Authorisation for off-patent products (PUMA)
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Initial PIP assessment

Day 30
first Discussion at PDCO
Rapporteur => Day 20
Peer Reviewer => Day 28

Day 60
second Discussion at PDCO

Discussion at Request for Modification TC

Day 90
third Discussion at PDCO

Day 120
Discussion and Opinion by PDCO (OE)

OE = Oral Explanation
TC = Telephon Conference

Clock stop

Re-start

app. 90 Days

Day 30 PIP validated by EMA

Opinion or Liste with open issues

Day 61 PIP update validated by EMA

EMEA Decision send to applicant

30 Days

60 Days

60 Days

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Product specific waiver

- Rare condition or condition occurring only in adult populations
- Lack of significant therapeutic benefit over existing treatments (Needs)
- Lack of efficacy in relation to likelihood of harm (Benefit/Risk)

Waiver effect on:
- one or more subsets of the paediatric population (Adolescence)
- one or more specified therapeutic indication
- Combination of both

No paediatric data to be provided

Waiver applied for => a specific medicine or
=> a class of medicines in a condition (Class waiver)
Deferred Paediatric Development

- scientific and technical grounds or on grounds related to public health ("urgent medicinal product development")
- commence studies in adults prior to paediatric studies due to safety concerns
- studies in the paediatric population will take longer than studies in adults (not to delay adult MAA)
- Request for additional information (e.g. non-clinical data)
- major difficulties of development age appropriate formulation(s)
Improving the health of the children of Europe, by:

• increasing **high quality research** for medicinal products for children

• **promoting the development** and authorization of such medicines at the EU level

• **improving the information** on medicines designed for children

While **avoiding unnecessary studies** in children and also not delaying the authorization of medicines for adults
Expert meeting on paediatric development of fixed-dose combination in treatment of HIV infection

What is needed – what is nice to have

- Methodology study design
- Modeling and Simulation
- Use of biomarkers (Endpoints)
- Source date for extrapolation

While avoiding unnecessary studies in children and also not delaying the authorization of medicines for adults

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Thank you for listening – let us start the discussion

…but before some house keeping

- Please introduce your self when talking
- Busy agenda, we need to stick to the response of the questions
- Aim for lunch break at 12.30h
- Wrap up and conclusion before the afternoon coffee break
- Target closure of the meeting at 16:30h