Expert meeting on paediatric development of fixed- dose combination in treatment of HIV infection

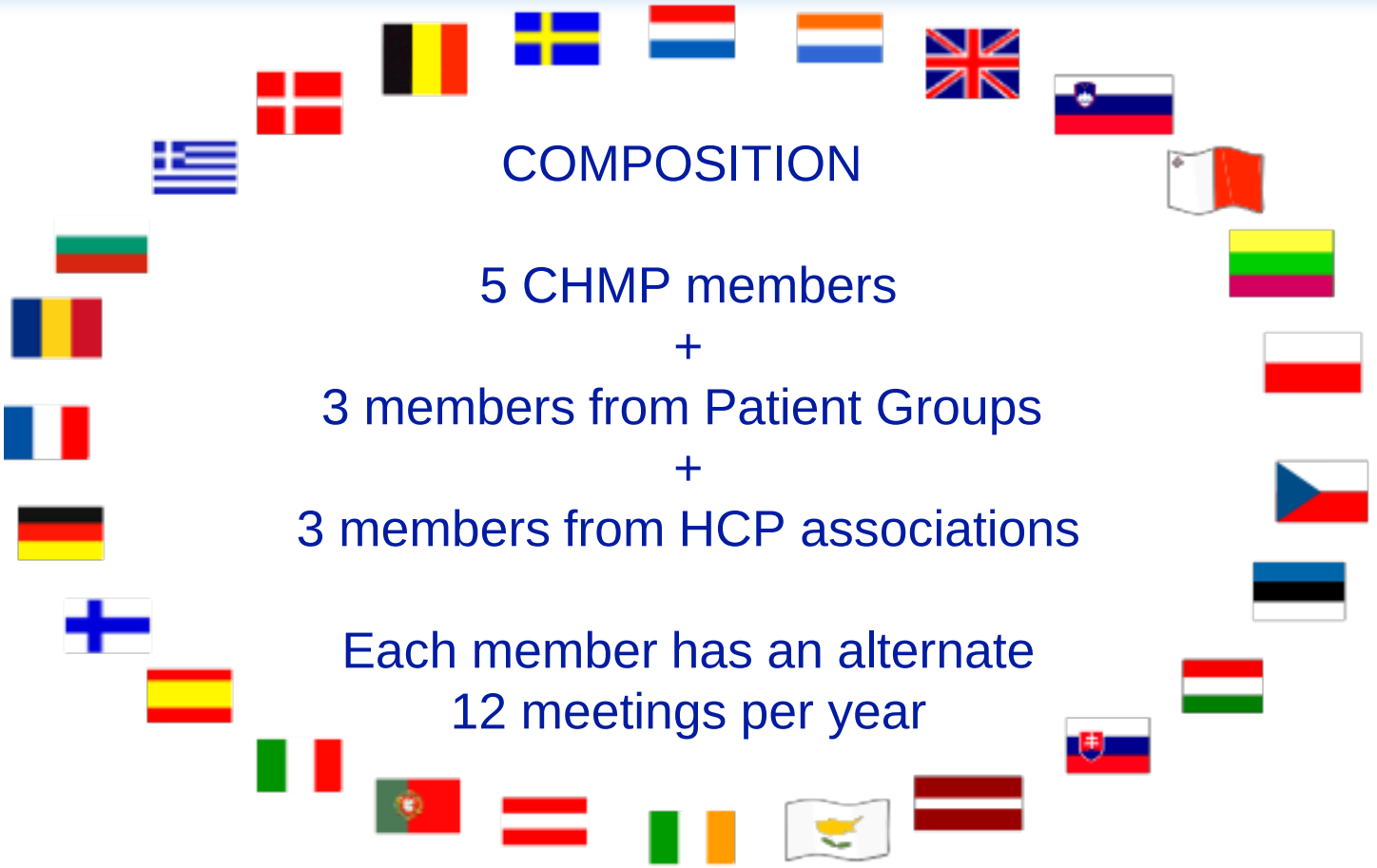
Meeting Objectives

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

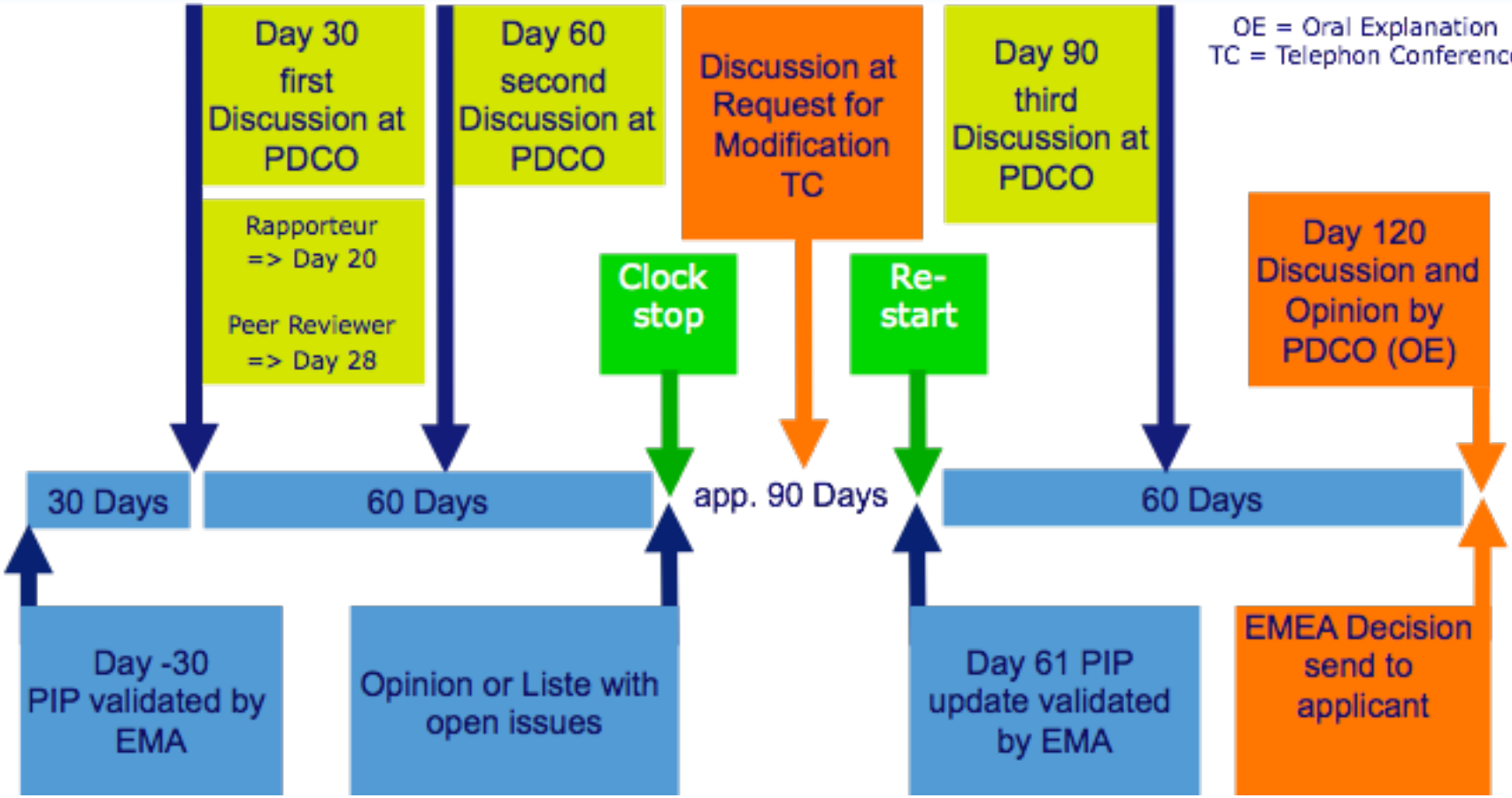
Expert meeting on paediatric development of fixed- dose combination in treatment of HIV infection

Back bone of EU Paediatric Regulation

- **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)**

Expert meeting on paediatric development of fixed- dose combination in treatment of HIV infection

Initial PIP assessment



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)

Aim of the Paediatric Regulation

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

Expert meeting on paediatric development of fixed- dose combination in treatment of HIV infection

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