EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO Summary report for PIP / waiver applications – Revision of template and use in applications

2nd EMA-Industry Stakeholders Platform meeting on Paediatric medicines

15 April 2016

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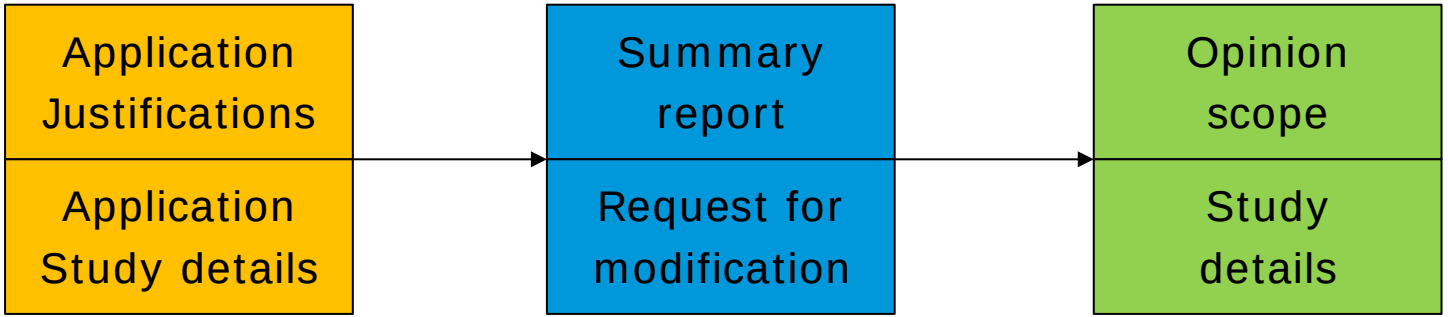
“Review and reconnect” at the Agency

- Analysis of certain areas and processes in the Human medicines areas, develop changes to allow meeting future challenges
- **Summary report:**
  - To improve focus and flow of documentation
  - To focus commenting on key elements of strategy and of studies
  - To facilitate creating the Request for modification and the Opinion

Overview of assessment and documentation of paediatric medicine procedure



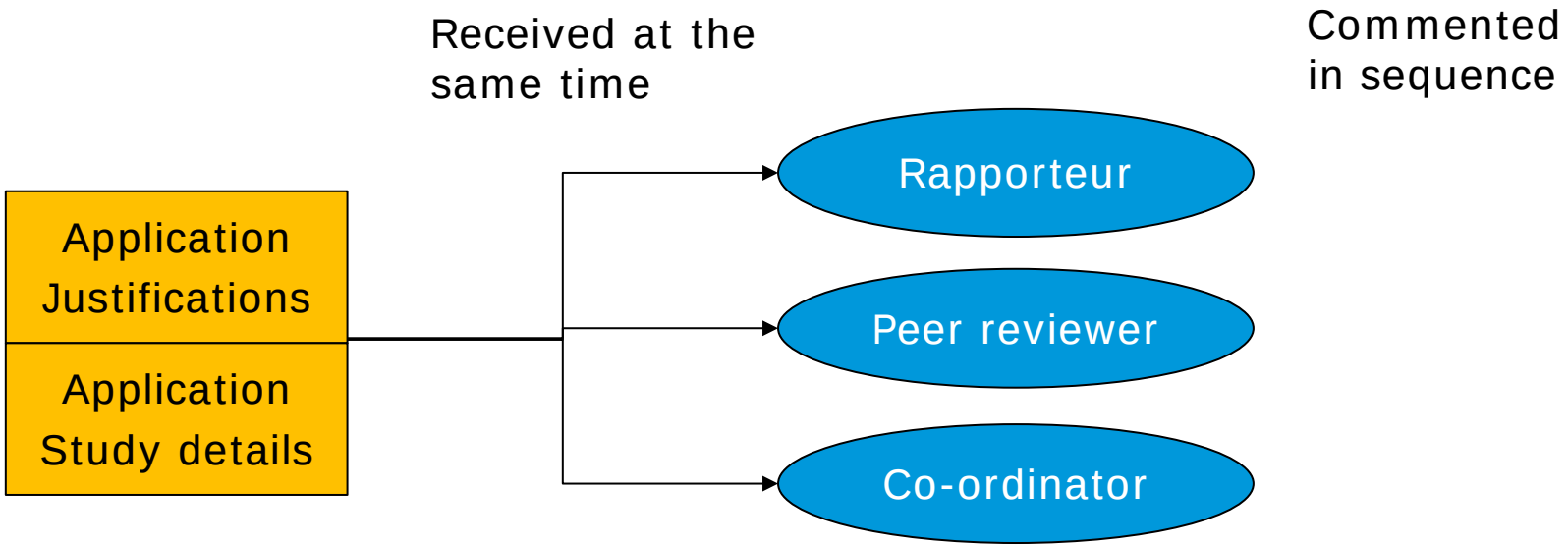
Strengths of current summary report

- Transparency of range and depth of discussion
- Transparency of decision-making
- Template is used by applicants and EMA
- Includes guidance for assessors to inform applicants what is expected
- Template permits to include many details
- Objective of template includes to ensure application quality
- Logical train of thought from disease to study details
- Clear link to regulatory aspects (e.g. deferral, waiver)
- Implements Commission guideline

Revised summary report template

- Fewer comments needed in parts A, B and C
- Part A simplified, more informative
- Parts B and D partly restructured
- Part C simplified
- Part D (=PIP strategy and studies) to better inform understanding of proposals, to lead more directly to elements in Opinion and / or request for modification
- Improved guidance to applicants about what is expected
- Structuring involvement of other expert(s) and groups

Application handling



4.1.2. Overall paediatric development plan

Summary of applicant position

Domain of data to be generated	Studies in domain	Ages, time lines, subject number, important interrelation	Comments contributing to a request for modification
Pharmaceutical quality	<automatically filled from form>		
Non-clinical safety	<automatically filled from form>		
Non-clinical efficacy / disease model(s)	<automatically filled from form>		
Clinical pharmaco-logy (dose-finding)	<automatically filled from form>		
Clinical safety and efficacy	<automatically filled from form>		
Clinical supportive (extrapol., model)	<automatically filled from form>		
Clinical long-term effects	<automatically filled from form>		

Example: overview

4.1.3. Strategy summary of proposed paediatric development generating data addressing quality, safety and efficacy

Summary of applicant position

Guidance to applicants:

- *Provide strategic rationale (linking disease and type of medicine), but not specific or detailed justifications.*
- *Applicants are invited to produce a single graphical representation / figure showing the studies on a timeline and showing which data inform which study as well as showing regulatory submission time-points (to visualise deferred initiations and deferred completions).*

<Application text>

<Graphical representation, about half a page>

4.1.4. Summary of use of early interaction(s) for paediatric development, received scientific advice and guidelines for paediatric development

Summary of applicant position

How were recommendations of early paediatric interaction, scientific advice (EMA, FDA, others) and/or EMA guidelines implemented? Why could the recommendations not be implemented, which new data were considered?

<Application text>

4.2. Evaluation team comments on overall plan and strategy

Guidance for evaluation team - please comment on overarching questions:

- *Is the development plan understandable?*
- *Compared to relevant other PIPs agreed by the PDCO, is this proposal similar?*
- *Are there obvious missing elements?*
- *Is the proposed overall plan agreeable?*
- *Are timelines = are deferrals (vis-à-vis adults) agreeable (conclusion needed on deferrals)?*
- *(Feasibility?)*

Example comment box

Co-ordinator (context, issues, handling options):

<text>

Rapporteur (acceptance or issues / objections, solutions / counterproposals):

<text>

Peer reviewer (missing aspects, clarity):

<text>

***Evaluation team suggestions for request for modification or opinion (e.g., additional measures necessary, changes to timelines, deferrals, waivers; data needed for PDCO discussion)*:**

<text>

4.5.4. Explanation of key elements of the clinical studies (up to 1 page per study) [Modules: FWG, NcWG, SAWP, CHMP]

<Repeat all of the following per study>

Study n - explanations of relevant design and conduct features

Guidance to applicants: address only main / key / selected
potentially controversial elements. Include graphical repr
any slightly complex design.

<Justification for relevant elements>

***Study n - tabulated synopsis** (for re-use in opinion):*

Binding element	Proposal	Edited proposal if likely to be acceptable	Comments contributing to a request for modification
Main design features	<automatically filled from PDF study elements form>		
Population	<automatically filled from PDF study elements form>		
Number of participants	<automatically filled from PDF study elements form>		
Primary endpoint (time of assessment)	<automatically filled from PDF study elements form>		
...			

Example commenting:
- By design element
- To modify element

Plans for introducing revised template

- Pilot phase
  - EMA to offer revised Summary template
  - to applicants identified based on letter of intent
  - for submitting PIP applications
  - from June to October 2016 inclusive.
- Followed by review with PDCO and finalisation

Thank you