



The TEDDY Working Group on Off Label Use in Paediatrics – how to move on

Why this Working Group?

The importance of the off-label medicines use in paediatrics is clearly recognised.

Relevant international documents on this point include:

- the European Commission Study on off-label use of medicinal products in the European Union (published in February 2017)
- the FIP–WHO technical guidelines: Points to consider in the provision by health-care professionals of children specific preparations that are not available as authorized products
- Several peer-reviewed scientific publications

The entity of the paediatric off-label use in the EU and worldwide is still high

Despite the Paediatric Regulation has achieved significant results, the lack of medicines labeled for the use in paediatric subsets is still relevant

In several therapeutic subsets, there is no alternative in paediatrics than off-label treatments

The topic is of particular interest for a network dealing with paediatric clinical research

What has been done to date?

A TEDDY 'Off-Label' WG was setup on June 30, 2016 aimed to prepare a
'European consensus statement on off-label use in children'

Program, achievements and future plans were presented by S. de Wildt during the 2016 TEDDY GA

During the first year of mandate, the group has taken in consideration the opportunity to contribute to the proposed *'Declaration on Good Off-label Practices'* released by Dr. Marc Doms and supported by many Scientific Societies and Interest Groups concluding that:

Notwithstanding its potential value in providing support to guide practitioners using medicines off-label, the GOLUP Declaration was not specific enough for the paediatric needs.



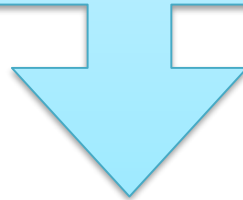
Off-Label WG Participants: S. de Wildt (chair), T. van der Zanden, A. Ceci, D. Bonifazi, M. Turner, M. Mellado, R. Piñeiro, A. Neubert, E. Jacqz-Aigrain, H. Sammons, F. Lagler, T. Sainz Costa and L. Ruggieri (scientific secretariat)

What is to be done to date?

In the TEDDY 'Off-Label' WG it was agreed that there is the need to include paediatrics as a special category in all discussions/documents dealing with off-label

and that

an ad hoc instrument could be developed covering specifically the paediatric setting



Thus, a consensus statement/policy statement on paediatric 'good off-label use' is still needed

Operational Proposal

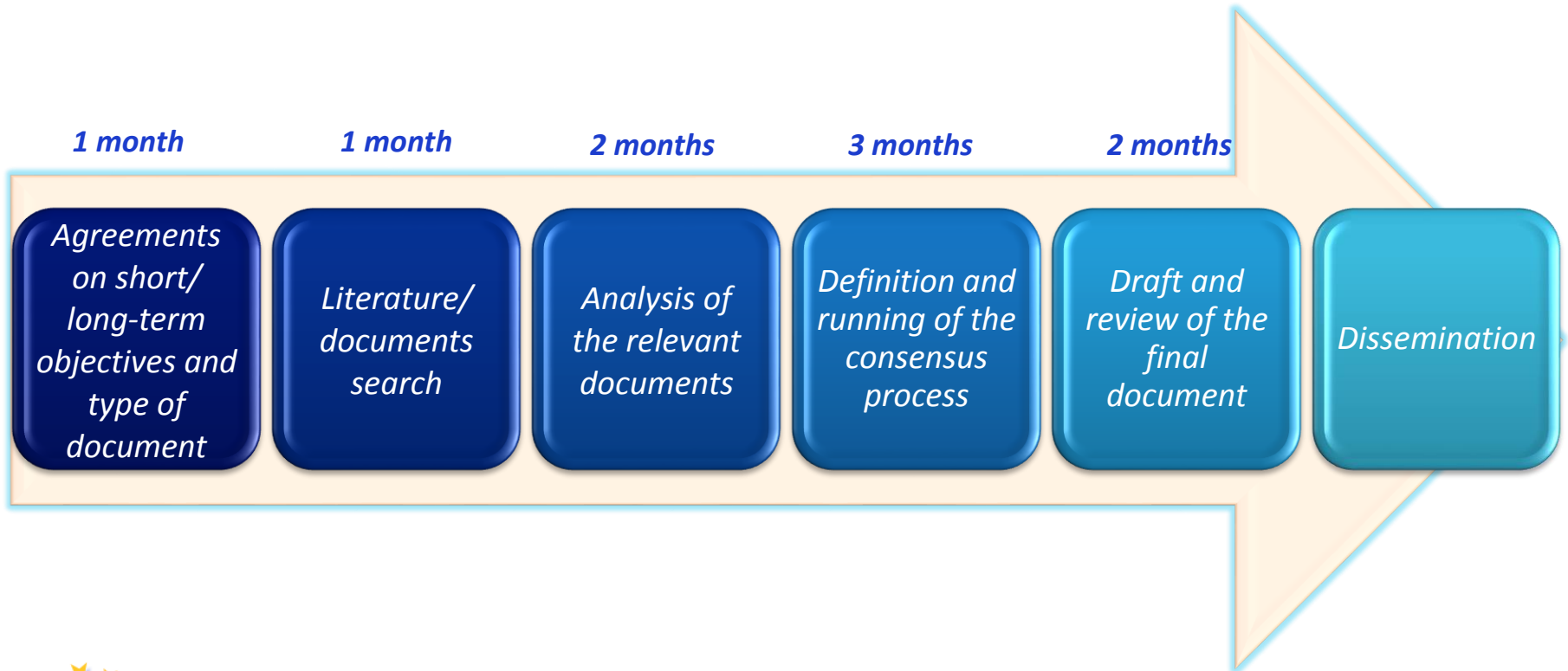
Step 1: Starting from recent literature data and documents from paediatric experts, networks, public bodies/institutions, a comprehensive overview of the existing documents and recommendations on the issue of paediatric off-label medicines use will be prepared.

Step 2: On these bases, a first draft of relevant aspects to consider will be prepared followed by 1-2 rounds of consultation using a consensus approach (Delphi or similar).

Step 3: At the end of this process, a consensus statement/policy statements will be prepared, formalised within the TEDDY Network and shared with the European and National Agencies, scientific societies and other stakeholders to receive support for its dissemination.

Possible plan of action and timeline

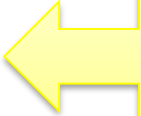
January – December 2018




Who we are?

To date the followings contributed:

- Tjitske van der Zanden (the Netherlands)
- Saskia de Wildt (the Netherlands)
- Adriana Ceci (Italy)
- Lucia Ruggieri (Italy)
- Antje Neubert (Germany)
- Florian Lagler (Austria)
- Helen Sammons (UK)
- Mark Turner (UK)
- Evelyne Jacqz-Aigrain (France)
- Maria Mellado Pena (Spain)
- Talia Sainz Costa (Spain)
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All the WG members
contributing in activities



Possibility to distinguish roles:
Members with advisory
functions (to review, provide
suggestions/indications),
Operational members (to
perform search, draft
documents...)



New members are welcome!

Comments and proposals for discussion

- Is the proposed deliverable reasonable?
- Other suggestions?
- Adequate timeline?
- To get confirmation/new adhesions
- To define roles and responsibilities for each step of activity
- Periodic update by TC