



# [DITA Task Force]



**EURORDIS**  
RARE DISEASES EUROPE

by

françois houÿez  
DITA task force



## AGENDA 10-12-2019 / 9H30 TO 16H30

### LOCATION

EURORDIS headquarters, 96 rue Didot 75014 Paris  
Room Alesia 3 & 4. Call + 331 56 53 52 10 for assistance

### PARTICIPANTS

#### DITA task force members

- |                                                                |                       |
|----------------------------------------------------------------|-----------------------|
| 1. Vesna Aleskovska, National Alliance                         | Northern Makedonia    |
| 2. Zsuzsa Almasi Prader willi syndrome and other rare diseases | Romania               |
| 3. Claudie Baleyrier, Friedreich Ataxia                        | France                |
| 4. Janet Bloor, Duchenne musc. Dystrophy                       | United Kingdom        |
| 5. Anne-Mary Bodin, Eurordis                                   | France                |
| 6. Rob Camp, Eurordis                                          | Spain                 |
| 7. Tatiana Foltanova, Slovak Alliance                          | Slovak Rep.           |
| 8. Ilaria Galetti, Scleroderma                                 | Italy                 |
| 9. François Houžez, Eurordis                                   | France                |
| 10. Diana Marinello, Behcet's syndrome association             | Italy                 |
| 11. Luc Matthyssen, HTAP                                       | Belgium               |
| 12. Michela Onali, GNE myopathy                                | Italy                 |
| 13. Matteo Scarabelli, Eurordis                                | France                |
| 14. Claudia Sproedt, Cystinosis                                | Germany               |
| 15. Alan Timothy, rare lung diseases                           | United Kingdom        |
| 16. Natacha Vaz Liti, Melanoma,                                | Portugal              |
| 17. Russel Wheeler, Leber's Hereditary Optic Neuroptic Society | United Kingdom/France |

#### Guests

18. xxxxxxxx

#### Excused

- |                                          |                                          |                                                   |
|------------------------------------------|------------------------------------------|---------------------------------------------------|
| • Isabella Brambilla, Dravet, Italy      | • Antonina Waszczuk, Sanfilippo, Germany | • Jana Popova, neuro-muscular disorders, Bulgaria |
| • Danijela Szili, Rett Syndrome, Hungary |                                          |                                                   |

#### Absent

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## AGENDA

- |                                                                                                     |            |
|-----------------------------------------------------------------------------------------------------|------------|
| 1. 9h30: Welcome (François)                                                                         | 5'         |
| 2. Introduction of new members                                                                      | 20'        |
| 3. Possible action plan for 2019-2022 (see presentation 1)                                          | 20'        |
| 4. Presentation of the PCWP main objectives 2019-2022                                               | 20'        |
| Discussion: what do you think is important to you in this work plan? See attachment                 |            |
| <hr/>                                                                                               |            |
| 11h00 Coffee break                                                                                  | 15'        |
| <hr/>                                                                                               |            |
| 5. Repurposing – Presentation of the STAMP initiative                                               | 15'        |
| a. If interested: ERTC workshop 19/02/2020                                                          |            |
| 6. Medicines Shortages - Introduction                                                               | 15'        |
| Breakout sessions                                                                                   | 30'        |
| One group works on shortages                                                                        |            |
| - What do you need to know? What could be done?                                                     |            |
| One group works on drug repurposing                                                                 |            |
| - Do you have contacts with academics who have such ideas?                                          |            |
| - Do you see your role in this?                                                                     |            |
| <hr/>                                                                                               |            |
| 13:00 Lunch break                                                                                   | 45'        |
| <hr/>                                                                                               |            |
| 7. Report from breakout sessions                                                                    | 20'        |
| 8. EMA Regulatory Science Strategy 2025 and new organisation                                        | 15'        |
| 9. EMA training activities for patient advocates                                                    | 15'        |
| Breakout sessions                                                                                   | 30'        |
| One group works on EMA Regulatory Science Strategy                                                  |            |
| One group works on EMA training activities                                                          |            |
| 10. Report from breakout sessions                                                                   | 20'        |
| 11. ICH E8 guidelines on General Considerations for Clinical Trials and Critical to Quality Factors | 15'        |
| 12. Progress report – EURO CAB programme                                                            | Rob 30'    |
| 13. Advocacy for the Regulation on HTA Cooperation                                                  | Matteo 15' |
| 14. AOB                                                                                             | 10'        |
| 16:30 Meeting ends                                                                                  |            |

## DOCUMENTS AND BACKGROUND INFORMATION

DITA task force actions for 2019-2022  
PCWP Work Plan 2019-2022  
EMA Regulatory Science Strategy: see presentations

-  01- Session 1 - Overview of public consultation results.pdf
-  02- Session 2 - Rec.01\_Support developments in precision medicine, biomarkers and omics.pdf
-  03- Session 2 - Rec.02\_Support translation of advanced therapy medicinal products (ATMPs) into patient treatments.pdf
-  04. Session 3a - Rec.05\_Create an integrated evaluation pathway for the assessment.pdf
-  05. Session 3a - Rec.07\_Diversify and integrate the provision of regulatory advice along the development continuum.pdf
-  06- Session 3b - Rec.15-part11-16\_Contribute to HTAs preparedness and downstream decision making for innovative medi...
-  07- Session 3b - Rec.18\_Promote use of high-quality real-world data (RWD) in decision making.pdf
-  08- Session 4 - Rec.09\_Foster innovation in clinical trials.pdf
-  09- Session 4 - Rec.10\_Develop the regulatory framework for emerging clinical data generation.pdf
-  10- Session 4 - Rec.13\_Optimise capabilities in modelling, simulation and extrapolation.pdf
-  11- Session 5a - Rec.17\_Reinforce patient relevance in evidence generation.pdf
-  12- Session 5b - Rec.28\_Develop network-led partnerships with academia.pdf