

Agenda CEF Meeting

11-12 December 2018

FIAP, 30 Rue Cabanis, 75014 Paris

Monday 10 December

19.30-21.30 *Dinner CNA & CEF, FIAP*

Tuesday 11 December - CNA & CEF Joint meeting

09.30-09.45 Welcome & introduction

09.45-10.15 ERN update: Outcome conference November & next steps, [Matt Johnson](#), [Ines Hernando](#)

10.15-10.45 Integration of ERNs into national HC systems, National Alliances and ePAGs at National level, [Matt Johnson](#), [Inés Hernando](#)

10.45-11.00 Coffee break

11.00-11.15 ePAG Training, [Lenja Wiehe](#), [Ines Hernando](#)

11.15-11.45 Social Position Paper, [Raquel Castro](#), [Dorica Dan](#)
- Presentation of the paper
- Discussion: How to make the best use of it

11.45-12.15 Rare Barometer Voices surveys: How you can get infographics for the results of your country or group of diseases, [Sandra Courbier](#) & [Erwan Berjonneau](#)

12.15-13.00 Patient Engagement in research, [Virginie Bros-Facer](#)
- European Joint Programme (EJP)
- Solve RD
- RD Connect

13.00-14.30 *Lunch*

CEF meeting

14.30-15.30 Community Advisory Boards (CAB), [Rob Camp](#)
- Progress so far
- Feedback from the CABs for Duchenne & Cystic Fibrosis
- Readiness to launch new CABs

- 15.30-16.00 *Coffee break*
- 16.00-17.30 Paradigm, *Elisa Ferrer*
 - Presentation of the project
 - Discussion on sustainability and long term patient engagement
- 17.30-18.00 Peer-to-Peer exchange
 Discussion: Medical Advisory Boards

Wednesday 12 December – CEF Training

- 09.30-10.30 Horizon scanning – Methods (Part I) *François Houÿez*

Horizon scanning systems aim to identify and prioritise new and emerging health technologies; to assess or predict their impact on health, costs, society, and the health care system; and to inform decision makers, research and budget planners. Patients' organisations should also scan their horizon to identify all technologies of interest to them in the research pipeline. This session will:

- *explain how you can use the results of your own horizon scanning*
- *learn where and how to identify health technologies of interest to you: this year focusing on pharmaceuticals*
- *organise the information you collect*
- *decide on criteria to filter those of highest interest*
- *show examples of horizon scanning already done*

- 10.30-10.45 *Coffee break*

- 10.45-11.30 Horizon scanning – Methods (Part II) *François Houÿez*

- 11.30-12.30 The HTx project - Can we inform clinicians with real world evidence in real time when prescribing a treatment? *François Houÿez, Matteo Scarabelli*

- 12.30-14.00 *Lunch*

- 14.00-14.30 Drug Repurposing, *François Houÿez*

- 14.30-15.30 EURORDIS position on Second Medical Use of Medicines/Drug Repurposing, *François Houÿez*

This is about medicines that are sometimes used to treat patients with rare diseases even if they were developed and authorised for a completely different disease. Example: Hydroxyurea first authorised to treat Myeloproliferative disorders and reimbursed for this use in many countries, and then some doctors started to use it to treat Sickle-cell disease but without marketing authorisation for this use or scientific evidence. How to make progress?

- 15.30-15.45 *Coffee break*

- 15.45-16.30 Discussion - Do you know treatments that are sometimes used for your disease without a marketing authorisation (off-label use or off-patent products)? How can we make progress?
