14 June 2017
EMA/648897/2017

Agenda – Registries Initiative – Cystic Fibrosis Workshop

14 June 2017, 08:30 to 16:30 UK time

Welcome room: 3-M

Group-work will take place in rooms: 3-M, 3-H, 3-J, 3-K

Co-Chairs: Peter Mol and Xavier Kurz

Main Objectives of the Workshop:

- To agree on implementable recommendations on core data elements to be collected, consents, governance, quality assurance and registry interoperability.
- To agree a workplan for the further development and finalisation of recommendations to be used by registry holders and MAHs/MAAs.

Item	Topics for information	Presenter	Time
1.	Welcome	Peter Mol	08:30-08:35h
2.	Introduction: expected outcomes from the workshop	Xavier Kurz	08:35-08:45h
3.	Update on the Patient Registries Initiative	Patricia McGettigan	08:45-09:00h
4.	Future potential for registries in regulatory processes	Irmgard Eichler	09:00 – 09:10h
4.	Value and potential of patient registries in Cystic Fibrosis	Ed McKone	09:10-09:40h
5.	Plan for the day and explanation of the Group-work activities	Patricia McGettigan	09:30-09:45
	Break - Coffee / tea		09:45-10.00h
	Group-work Facilitator: Patricia McGettigan	**Moderators**	10:00-12:00h
6.	*Moderators outline how the groups will operate* • **Group 1:** Common data elements that are needed by all stakeholders irrespective of the clinical/ academic/ regulatory/ reimbursement purposes of	Alison Cave / Kelly Plueschke	

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Item	Topics for information	Presenter	Time
	registries • **Group 2:** Informed consents and governance, data protection • **Group 3:** Common procedures and registry interoperability, quality assurance measures to support regulatory medicines evaluation	Xavier Kurz / Mireia Castillon Peter Mol / Carla Olmo Alonso	
	Lunch		12:00-13:00h
7.	**Agreement on Recommendations** • **Group 1**: Common data elements needed by all stakeholders irrespective of the clinical/ academic/ regulatory/ reimbursement purposes of registries • **Group 2**: Informed consents, governance, data protection • **Group 3**: Common procedures and registry interoperability, quality assurance measures to support regulatory medicines evaluation	Alison Cave / Kelly Plueschke Xavier Kurz / Mireia Castillon Peter Mol / Carla Olmo Alonso	13:00-14:00h
8.	Each group prepares a summary of its Recommendations. Slides / Posters with the main points to be compiled for discussion all the Workshop participants	All	14.00–14.30h
	Break – Coffee / Tea		14:30-14:45h
Item	**Group Recommendations and Discussions**		
9.	Presentation of Group 1, 2 and 3 Recommendations to all the participants Discussion / Agreement on Recommendations	Nominee from each Group	14:45-16:00h
10.	Conclusions and Next Steps	Peter Mol & Xavier Kurz	16:00-16:30h

- **Group 1**: Common data elements
    - **Expected outcomes:** Agreement on key common data elements needed by all stakeholders to fulfil their output needs
- **Group 2**: Consents and Governance
    - **Expected outcomes:** Outline agreement on consents and on a pragmatic and lean governance model adjusted for CF stakeholders' purposes and taking account of data protection requirements
- **Group 3**: Outline protocols for longitudinal data collection, registry interoperability, data verification and quality assurance for EMA qualification of CF Registries
    - **Expected outcomes:** Agreement on protocols for data collection (to facilitate registry interoperability) and on measures for data verification that would support quality assurance for EMA qualification.

Work Groups - Participants

GROUP 1 - ROOM 3/M	Common data elements needed by all stakeholders; data validation
Name	**Background**
Concepcion Prieto Yerro (AEMPS)	**Regulators**
Piotr Fiedor (Polish Agency)	**Regulators**
Hanneke van der Woude (CBG-MEB)	**Regulators**
Maria Fernandez (AEMPS)	**Regulators**
Gerhild Angyalosi (Novartis)	**Industry**
Claudia Meyer (Horizon Pharma)	**Industry**
Lutz Nährlich	**Registries Holders**
Laura Fregonese	**EMA**
Irmgard Eichler	**EMA**
Valerie Joynson (MHRA)	**Regulators**
Siobhán Carr	**Registries Holders**
Isabelle de Monestrol	**Registries Holders**
Peter Mol**- Moderator**	**Regulators**
Carla Alonso- **Moderator**	**EMA**

GROUP 2 - ROOM 3/M	Informed consents, governance, data protection
Name	**Background**
Ed McKone	**Registries Holders**
Melinda Sobor (OGYEI)	**Regulators**
Almath Spooner (HPRA)	**Regulators**
Sabine Straus (CBG-MEB)	**Regulators**
Flaminia Macchia (Vertex)	**Industry**
Anne-Ruth van Troostenburg de Bruyn (Gilead Science)	**Industry**
Carla Jonker (CBG-MEB)	**Regulators**
Frank Petavy	**EMA**
Martin Erik Nyeland (DKMA)	**Regulators**
Natividad Galiana (AEMPS)	**Regulators**
Hanne Vebert Olesen	**Registries Holders**
Jacquelin Noordhoek	**Registries Holders**
Xavier Kurz- **Moderator**	**EMA**
Mireia Castillon- **Moderator**	**EMA**

GROUP 3 - ROOM 3/K	Common procedures and registry interoperability, quality assurance measures to support regulatory medicines evaluation
Name	**Background**
Francois Meyer (HAS-SANTE)	**HTA**
Vincent Gulmans	**Registries Holders**
Lydie Lemonnier	**Registries Holders**
Robert Hemmings (MHRA)	**Regulators (DNA)**
Karolina Torneke (MPA)	**Regulators**
Nithyanandan Nagercoil (MHRA)	**Regulators**
Nils Kinnman (Vertex)	**Industry**
Saskia Dekker (Horizon Pharma)	**Industry**
Noreen Caine	**Registries Holders**
Andrea Taft (EMA)	**Regulators**
Jane Moseley (EMA)	**EMA**
Florian Gutzwiller (Novartis)	**Industry**
Alison Cave- **Moderator**	**EMA**
Kelly Plueschke- **Moderator**	**EMA**

Group oversight: Patricia McGettigan, EMA

List of participants

Cystic Fibrosis Workshop

14 June 2017, 08.30-16.30, Room 3/M

Presenter / Moderator / Rapporteur	Affiliation
Peter Mol	Medicines Evaluation Board (The Netherlands)
Xavier Kurz	European Medicines Agency
Patricia McGettigan	European Medicines Agency
Ed McKone	European Cystic Fibrosis Society Patient Registry (Ireland)
Alison Cave	European Medicines Agency
Kelly Plueschke	European Medicines Agency
Mireia Castillon	European Medicines Agency
Carla Alonso	European Medicines Agency
Registry Holders	**Affiliation**
Hanne Vebert Olesen	European Cystic Fibrosis Society (Denmark)
Jacqueline Noordhoek	Cystic Fibrosis Europe (The Netherlands)
Vincent Gulmans	European Cystic Fibrosis Society (The Netherlands)
Lydie Lemonnier	Defeating Cystic Fibrosis (France)
Isabelle de Monestrol	European Cystic Fibrosis Society (Sweden)
Lutz Nährlich	European Cystic Fibrosis Society Patient Registry (Germany)
Siobhán Carr	Cystic Fibrosis Trust (UK)
Noreen Caine	Cystic Fibrosis Trust (UK)
National Authority	**Affiliation**
François Meyer	Health Technology Assessment (France)

Patient Representative	Affiliation
Name to be confirmed	
Member States	**Affiliation**
Concepcion Prieto Yerro	Spanish Agency of Medicinal Products and Medical Devices (Spain)
Robert Hemmings	Medicines and Healthcare Products Regulatory Agency (UK)
Melinda Sobor	National Institute of Pharmacy and Nutrition (Hungary)

Patient Representative	Affiliation
Piotr Fiedor	Office for the Registration of Medicinal Products, Medical Devices and Biocidal Products (Poland)
Almath Spooner	Health Products Regulatory Authority (Ireland)
Sabine Straus	Medicines Evaluation Board (The Netherlands)
Hanneke van der Woude	Medicines Evaluation Board (The Netherlands)
Karolina Torneke	Medical Products Agency (Sweden)
Maria Fernandez	Spanish Agency of Medicinal Products and Medical Devices (Spain)
Nithyanandan Nagercoil	Medicines and Healthcare Products Regulatory Agency (UK)
Valerie Joynson	Medicines and Healthcare Products Regulatory Agency (UK)
Carla Jonker	Medicines Evaluation Board (The Netherlands)
Martin Erik Nyeland	Danish Medicines Agency (Denmark)
Natividad Galiana	Spanish Agency of Medicinal Products and Medical Devices (Spain)
EMA	
Jane Moseley	European Medicines Agency
Laura Fregonese	European Medicines Agency
Irmgard Eichler	European Medicines Agency
Corinne de Vries	European Medicines Agency
Frank Petavy	European Medicines Agency
Andrea Taft	European Medicines Agency
Valerie Muldoon	European Medicines Agency
Industry	Affiliation
Gerhild Angyalosi	Novartis
Florian Gutzwiller	Novartis
Nils Kinnman	Vertex
Flaminia Macchia	Vertex
Claudia Meyer	Horizon Pharma
Saskia Dekker	Horizon Pharma
Anne-Ruth van Troostenburg de Bruyn	Gilead Science