28 September 2018
EMA/654423/2018
Inspections, Human Medicines, Pharmacovigilance and Committees Division

Haemophilia Registries Workshop

Appendix 1: Agenda and List of Participants

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5520
Send a question via our website www.ema.europa.eu/contact

Agenda – Haemophilia Registries Workshop

8th June 2018, 09:00 to 17:00 UK time

Welcome room: 2/A

Group-work will take place in rooms: 2/A and 2/E

Chair: Jacqueline Kerr

Main Objectives of the Workshop:

- Ensure the practical implementation of the requirements related to Registries in line with the revised FVIII Guideline on the clinical investigation of recombinant and human plasma-derived factor VIII products (link);
- Agree on processes for data access, data sharing and reporting, and clarify roles of all involved stakeholders (patients-physicians-registries-companies-regulatory authorities);
- Agree on the additional data elements to be collected for novel products (PEGylated products, monoclonal antibodies, gene therapies).

Item	Topics for information	Presenter	Time
1.	Welcome	Jacqueline Kerr	09:00-09:05
2.	Introduction	June Raine Dirk Mentzer Martina Schussler-Lenz	09:05-09:20
3.	Expected outcomes from the workshop	Anneliese Hilger	09:20-09:35
4.	Specific safety and efficacy considerations and follow-up in Haemophilia patients – implications for a registry	Brigitte Keller-Stanislawski	09:35-09:50
5.	Patients perspective	Declan Noone	09:50-10:05
6.	European Commission - European Platform on Rare Diseases	Andri Papadopoulou	10:05-10:15
7.	An overview of Haemophilia registries • PedNET Registry • EUHASS	Christine Keipert Marijke van Den Berg Mike Makris	10:15-10:30 10:30-10:40 10:40-10:50
8.	Plan and explanation of the Group-work activities	Caroline Voltz	10:50-10:55

	Break - Coffee / tea		11:00-11:15
	Group-work	**Moderators**	11:15-13:00
9.	*Moderators outline how the groups will operate* **Group 1:** Registries operation to fulfil the guideline requirements **Group 2:** Use of registry data for regulatory purposes: legal and practical considerations **Group 3:** Additional data to be collected for novel products (PEGylated products, monoclonal antibodies gene therapies)	Kelly Plueschke/Armin Ritzhaupt Xavier Kurz/Francesco Pignatti Caroline Voltz/Anna Tavridou	
	Lunch		13:00-14:00
10.	**Agreement on Recommendations** • **Group 1** • **Group 2** • **Group 3** Each Group agrees on & prepares a summary (slides / poster) of its recommendations for discussion with all of the Workshop participants		14:00-15:00
Item	**Group Recommendations and Discussions**		
11.	Presentation of Group 1, 2 and 3 Recommendations to all the participants Discussion / Agreement on Recommendations	30 min / group	15:00-16:30
12.	Conclusions and Next Steps	Jacqueline Kerr / Peter Arlett	16:30-17:00

List of participants

Name	Affiliation
Alexandre Moreau	French National Agency for Medicines and Health Products Safety (ANSM)
Andrea Laslop	Austrian Agency for Health and Food Safety (AGES)
Andrej Segec	European Medicines Agency (EMA)
Andri Papadopoulou	European Commission (EC)
Anna Tavridou	European Medicines Agency (EMA)
Anne Tsatsaris	Sanofi
Anneliese Hilger	Paul-Ehrlich-Institute (PEI)
Annemieke Willemze	Sobi
Armin Ritzhaupt	European Medicines Agency (EMA)
Artur Bauhofer	Biotest
Brigitte Brand-Staufer	Novo Nordisk
Brigitte Keller-Stanislawski	Paul-Ehrlich-Institute (PEI)
Bruce McCall	La Roche
Camelia Sima	La Roche
Carla Alonso Olmo	European Medicines Agency (EMA)
Carla Jonker	Dutch Medicines Evaluation Board (CBG-MEB)
Caroline Voltz-Girolt	European Medicines Agency (EMA)
Charles Hay	UKCHDO Registry
Christine Keipert	Paul-Ehrlich-Institute (PEI)
Christoph Male	PedNet Registry
Claire Brullé-Wohlhüter	Sanofi
Daniel Hart	UKCHDO Registry
Daniel Nogueras Zondag	European Medicines Agency (EMA)
Daniela Philadelphy	Austrian Agency for Health and Food Safety (AGES)
Declan Noone	European Haemophilia Consortium (EHC)
Dirk Mentzer	Paul-Ehrlich-Institute (PEI)

Name	Affiliation
Dominika Misztela	Plasma Protein Therapeutics Association (PPTA)
Donna Coffin	World Federation of Haemophilia (WFH)
Eileen Sawyer	uniQure
Elena Santagostino	AICE Registry
Elise Destrée	uniQure
Elke Detering	Bayer AG
Flora Peyvandi	European Association for Haemophilia and Allied Disorders (EAHAD)
Francesco Pignatti	European Medicines Agency (EMA)
Francesco Rodeghiero	European Haematology Association (EHA)
Gabriele Calizzani	National Blood Centre
Hervé Chambost	FranceCoag Registry
Hideyuki Kondo	European Medicines Agency (EMA)
Ita Walsh	Dutch Medicines Evaluation Board (CBG-MEB)
Jacqueline Kerr	Paul-Ehrlich-Institute (PEI)
Jamie Geier	Pfizer
Jane Moseley	European Medicines Agency (EMA)
Janina Karres	European Medicines Agency (EMA)
Jaume Ayguasanosa	Grifols
Jenny Goudemand	FranceCoag Registry
Johann Bichler	Octapharma AB
John Johnston	Medicines and Healthcare Products Regulatory Agency (MHRA)
Jordi Llinares	European Medicines Agency (EMA)
June Raine	Medicines and Healthcare Products Regulatory Agency (MHRA)
Karri Penttila	Finnish Medicines Agency (FIMEA)
Kathelijn Fischer	HemoNED Registry
Kelly Plueschke	European Medicines Agency (EMA)
Kristyna Marusakova	European Medicines Agency (EMA)

Name	Affiliation
Liz Holmes	BPL
Lorenzo Montrasio	Italian Medicines Agency (AIFA)
Lydie Renault	Shire
Marie Louise S. Christiansen	Danish Medicines Agency (DKMA)
Mariëtte Driessens	HemoNED Registry
Marijke van den Berg	PedNet Registry
Martina Schussler-Lenz	Paul-Ehrlich-Institute (PEI)
Mike Makris	EUHASS Registry
Nathalie Eckard	The Dental and Pharmaceutical Benefits Agency (TLV)
Pablo Rendo	Pfizer
Patricia McGettigan	European Medicines Agency (EMA)
Peter Arlett	European Medicines Agency (EMA)
Radoslaw Kaczmarek	European Haemophilia Consortium (EHC)
Rune Kjeken	Norwegian Medicines Agency (NoMA)
Sandra Nieto	Grifols
Sara Franco	French National Agency for Medicines and Health Products Safety (ANSM)
Shefali Saini	CSL Behring GmbH
Stephan Rauchensteiner	Bayer AG
Stylianos Tsigkos	European Medicines Agency (EMA)
Wing Yen Wong	Biomarin
Xavier Kurz	European Medicines Agency (EMA)
Zaide Frias	European Medicines Agency (EMA)