29 April 2018
EMA/138425/2018

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: 2A and 2E

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.	European Commission - European Platform on Rare Diseases	Andri Papadopoulou	09:20-09:30
4.	Expected outcomes from the workshop	Anneliese Hilger	09:30-09:45
5.	Specific safety and efficacy considerations and follow-up in Haemophilia patients – implications for a registry	Brigitte Keller-Stanislawski	09:45-10:00

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Item	Topics for information	Presenter	Time
6.	Patients perspective	Declan Noone	10:00-10:15
7.	An overview of Haemophilia registries • PedNET Registry • EUHASS Group 3	Christine Kiepert 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 (including data entry and data exchange) **Group 2:** Use of registry data for regulatory purposes: legal and practical considerations (including data access, data verification, quality assurance processes and data format) **Group 3:** Additional data to be collected for novel products (PEGylated products, monoclonal antibodies gene therapies)	Alison Cave/Kelly Plueschke Xavier Kurz/Armin Ritzhaupt 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