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Pharmacovigilance and Epidemiology Department
Inspections, Human Medicines, Pharmacovigilance and Committees Division

CAR T-cell therapy Registries Workshop

Appendix 2: Agenda and Participants

Agenda – CAR T-cell therapy Registries Workshop

9 February 2018, 08:30 to 16:30 UK time

Welcome room: 2/A

Group-work will take place in rooms: 2/C, 2/D, 2/E

Co-Chairs: June Raine, Martina Schüssler-Lenz and Tomas Salmonson.

Main Objectives of the Workshop:

- To facilitate the long-term follow up of CAR-T cell products in a real world setting and enable the generation of meaningful efficacy and safety data using haemato-oncological registries
- To agree on implementable recommendations on core data elements to be collected, patient consent, governance, quality assurance and registry interoperability.
- To agree on recommendations to optimise collaboration among registry holders, MAHs/MAAs and regulators

Item	Topics for information	Presenter	Time
1.	Welcome	June Raine Tomas Salmonson	08:30-08:35h
2.	Expected outcomes from the workshop	Martina Schüssler-Lenz	08:35-08:45h
3.	Specific safety and efficacy considerations and follow-up in patients treated with CAR T cells – implications for a registry	Pierre Demolis (efficacy)/ Brigitte Keller-Stanislawski (safety)	08:45-09:25h
4.	Value and potential of patient registries: ECFS (European Cystic Fibrosis Society) as an operational patient registry example.	Peter Mol	09:25-09:40h
5.	Haemato-oncological registries • EBMT registry • CIBMTR registry	Jürgen Kuball Marcelo Pasquini	09:40-10:10h 10:10 -10:30h
6.	Plan for the day and explanation of the Group-work activities	Patricia McGettigan	10:30-10:40h

	Break - Coffee / tea		10:40-10:55h
	Group-work	**Moderators**	10:55-12:30h
7.	*Moderators outline how the groups will operate* **Group 1:** Common data elements that are needed by stakeholders on utilisation and measures of efficacy/effectiveness of CAR-T cell products; Data verification and registry quality assurance processes needed to support regulatory decision-making **Group 2:** Common data elements that are needed by stakeholders on safety follow-up of CAR-T cell products; Data verification and registry quality assurance processes needed to support regulatory-decision making **Group 3:** Informed consents and governance, data protection, common procedures and registry interoperability, quality assurance measures to support regulatory decision-making for CAR-T cell products	Alison Cave / Kelly Plueschke Peter Arlett/ Thomas Goedecke Xavier Kurz / Mireia Castillon	
	Lunch		12:30-13:30
8.	**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		13:30-14:30h
Item	**Group Recommendations and Discussions**		
9.	Presentation of Group 1, 2 and 3 Recommendations to all the participants Discussion / Agreement on Recommendations	30 min / group	14:30-16:00h
10.	Conclusions and Next Steps	Robert Hemmings	16:00-16:30h

Participants – Work group 1

Group 1: Common data elements that are needed by stakeholders on utilisation and measures of efficacy/effectiveness of CAR-T cell products; Data verification and registry quality assurance processes needed to support regulatory decision-making	
Name	**Affiliation**
Moderators	
Alison Cave	European Medicines Agency
Kelly Plueschke	European Medicines Agency
Participants	
Giovanni Lesa	European Medicines Agency
Irene Papadouli	European Medicines Agency
Stylianos Tsigkos	European Medicines Agency
Kyriaki Tzogani	European Medicines Agency
Francesco Pignatti	European Medicines Agency
Spiros Vamvakas	European Medicines Agency
Jan Mueller-Berghaus	Paul-Ehrlich-Institute
Paolo Gasparini	Head of the Department of Advanced Diagnostics and Clinical Research
Rob Hemmings	Medicines and Healthcare Products Regulatory Agency
Tomas Salmonson	Swedish Medical Products Agency
Pierre Demolis	French National Agency for Medicines and Health Products Safety
Olli Tenhunen	Finnish Medicines Agency
Karri Penttila	Finnish Medicines Agency
Ole Weis Bjerrum	Danish Medicines Agency
Ania Urbaniak	Norwegian Medicines Agency
Stanley Frankel	Celgene
Stéphan Reynier	Cellectis
Miriam Fuchs	Novartis
Andreu Gusi Puig	European Group for Blood and Marrow Transplantation
Kuball Jürgen	European Group for Blood and Marrow Transplantation
Marcelo Pasquini	Center for International Blood and Marrow Transplant Research

Participants – Work group 2

Group 2: Common data elements that are needed by stakeholders on safety follow-up of CAR-T cell products; Data verification and registry quality assurance processes needed to support regulatory-decision making.

Name	Affiliation
Moderators	
Peter Arlett	European Medicines Agency
Thomas Goedecke	European Medicines Agency
Participants	
Zahra Hanaizi	European Medicines Agency
Caroline Voltz	European Medicines Agency
Corinne de Vries	European Medicines Agency
Hide Kondo	European Medicines Agency
Ralph Bax	European Medicines Agency
Veronique Le Ber	European Medicines Agency
June Raine	Medicines and Healthcare Products Regulatory Agency
Martina Schuessler-Lenz	Paul-Ehrlich-Institute
Brigitte Keller-Stanislawski	Paul-Ehrlich-Institute
Helga Olsen	Norwegian Medicines Agency
Koenraad Norga	University of Antwerp
Paula Boudewina van Hennik	Dutch Medicines Evaluation Board
Serena Marchetti	Dutch Medicines Evaluation Board
Rimma Berenstein	Federal Joint Committee
Laurence Adegeest	Celgene
David Chonzi	Kite Pharma
Eric Bleickardt	Novartis
Eoin Mc Grath	European Group for Blood and Marrow Transplantation
Mary M Horowitz	Center for International Blood and Marrow Transplant Research
Ulrike Holtkamp	European LeukemiaNet

Participants – Work group 3

Group 3: Informed consents and governance, data protection, common procedures and registry interoperability, quality assurance measures to support regulatory decision-making for CAR-T cell products.

Name	Affiliation
Moderators	
Mireia Castillon	European Medicines Agency
Xavier Kurz	European Medicines Agency
Participants	
Jordi Llinares	European Medicines Agency
Patrick Celis	European Medicines Agency
Ana Hidalgo-Simon	European Medicines Agency
Zaide Frias	European Medicines Agency
Ad Schuurman	European Medicines Agency
Armin Ritzhaupt	European Medicines Agency
Jane Moseley	European Medicines Agency
Marin Banovac	European Medicines Agency
Peter Mol	Dutch Medicines Evaluation Board
Doris Stenver	Danish Medicines Agency
David Olsen	Norwegian Medicines Agency
Blanca Garcia	Spanish Agency of Medicines and Medical Devices
Kieran Breen	European Parkinson's Disease Association
Helen Powell	National Institute for Care and Health Excellence
Stefan Kaehler	Celgene
Paul Wang	Kite Pharma
Sweta Shah	Novartis
Carmen Ruiz	European Group for Blood and Marrow Transplantation
Bronwen Shaw	Center for International Blood and Marrow Transplant Research
Patricia Steinert	Center for International Blood and Marrow Transplant Research