



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Inspections, Human Medicines Pharmacovigilance and Committees Division

COMP work plan 2017

adopted by the Committee on 08 December 2016

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1. Evaluation activities for human medicines

1.1. Pre-authorisation activities

1.1.1. Designation of orphan medicines

Key objectives

- Implement changes introduced in the Commission Notice on the Application of Articles 3, 5 and 7 of Regulation (EC) No 141/2000 on Orphan Medicinal Products (2016/C 424/03).
- Improve the quality of initial orphan designation applications by sharing COMP experience and issuing further recommendations
- Ensure consistency of answers to questions on significant benefit in Protocol Assistance and clarify the requirements for the demonstration of significant benefit in order to improve chances of success at the time of Orphan Designation and Marketing Authorisation
- Ensure consistency, transparency, quality and detail of the grounds of opinions given by the COMP on significant benefit at the time of marketing authorisation

Activities in 2017

- Streamline processes in line with the Commission Notice (2016/C 424/03)
 - Initial orphan designation
 - Maintenance
 - Type II variations

COMP topic leader: Lesley Greene

Other Committee participants (members of the Significant Benefit Working Group):

Member/Alternate	Name	Member State
Chair	Bruno Sepodes	
Member	Brigitte Blöchl-Daum	AT
Member	Kateřina Kopečková	CZ
Member	Frauke Naumann-Winter	DE
Member	Jens Ersbøll	DK
Member	Vallo Tillmann	EE
Member	Armando Magrelli	IT
Member	Michel Hoffmann	LU
Member	Violeta Stoyanova	NL
Member	Bożenna Dembowska-Bagińska	PL
Member	Daniel O'Connor	UK
Member	Pauline Evers	Patients' organisations representative
Member	Mario Ricciardi	Patients' organisations representative
Member	Kerstin Westermark	Nominated by EC

Member/Alternate	Name	Member State
Member	Giuseppe Capovilla	Nominated by EC
Expert	Virginie Hivert	EURORDIS*

*Affiliation

- Update “COMP recommendation on elements required to support the medical plausibility and the assumption of significant benefit for an orphan designation, EMEA/COMP/436/01” taking into account the Commission Notice (2016/C 424/03) and principles identified by the working group on significant benefit when assessing criteria for orphan designation (at designation and at marketing authorisation).

COMP topic leader: Lesley Greene

Other Committee participants (members of the Significant Benefit Working Group):

Member/Alternate	Name	Member State
Chair	Bruno Sepodes	
Member	Brigitte Blöchl-Daum	AT
Member	Kateřina Kopečková	CZ
Member	Frauke Naumann-Winter	DE
Member	Jens Ersbøll	DK
Member	Vallo Tillmann	EE
Member	Armando Magrelli	IT
Member	Michel Hoffmann	LU
Member	Violeta Stoyanova-Beninska	NL
Member	Bożenna Dembowska-Bagińska	PL
Member	Daniel O'Connor	UK
Member	Pauline Evers	Patients' organisations representative
Member	Mario Ricciardi	Patients' organisations representative
Member	Kerstin Westermark	Nominated by EC
Member	Giuseppe Capovilla	Nominated by EC
Expert	Virginie Hivert	EURORDIS*

*Affiliation

- Development of strategies to implement recommendations stemming from the workshop on Conditions (December 2016) when assessing orphan designation applications.

COMP topic leader: Daniel O' Connor

Other Committee participants:

Member/Alternate	Name	Member State
Member	Frauke Naumann-Winter	DE
Member	Ingeborg Barišić	HR
Member	Violeta Stoyanova-Beninska	NL
Member	Kerstin Westermark	Nominated by EC
Expert	Virginie Hivert	EURORDIS*

*Affiliation

- Analysis of non-clinical models
 - Publish the outcome of the analysis on non-clinical models initiated in 2016;
 - Decide on a new therapeutic area and initiate analysis of data from non-clinical models.

COMP topic leader: Bruno Sepodes

Other Committee participants:

Member/Alternate	Name	Member State
Member	Fernando Méndez Hermida	ES
Member	Melinda Sobor	HU
Member	Armando Magrelli	IT
Member	Michel Hoffmann	LU
Member	Robert Nistico	MT
Member	Violeta Stoyanova-Beninska	NL
Member	Dinah Duarte	PT
Member	Eva Malíková	SK
Member	Mario Ricciardi	Patients' organisations representative
Member	Giuseppe Capovilla	Nominated by EC
Expert	Julian Isla	EURORDIS*

*Affiliation

- Establish a working group on prevalence to:
 - Update the document "Points to Consider on the calculation and reporting of the prevalence of a condition for orphan designation" (COMP/436/01) and issue a publication on the subject;
 - Finalise a publication based on cases discussed by COMP;
 - Organise a workshop on calculation of prevalence for orphan designation and review of criteria for orphan designation at time of marketing authorisation.

COMP topic leader: Frauke Naumann-Winter

Other Committee participants:

Member/Alternate	Name	Member State
Member	Vallo Tillmann	EE
Member	Geraldine O'Dea	IE
Member	Irena Rogovska	LV
Member	Giuseppe Capovilla	Nominated by EC

- Discuss each protocol assistance question in the Protocol Assistance Working Group to prepare for in-depth discussions within the COMP plenary, with the aim of improving recommendations to sponsors for the demonstration of significant benefit taking into account the Commission's Notice (2016/C 424/03) and principles identified by the working group on significant benefit when assessing criteria for orphan designation.

COMP topic leader: Armando Magrelli

Other Committee participants:

Member/Alternate	Name	Member State
Chair	Bruno Sepodes	
Member	Brigitte Blöchl-Daum	AT
Member	Kateřina Kopečková	CZ
Member	Frauke Naumann-Winter	DE
Member	Karri Penttila	FI
Member	Dinko Vitezic	HR
Member	Violeta Stoyanova-Beninska	NL
Member	Daniel O'Connor	UK
Member (Vice-chair)	Lesley Greene	Patients' organisations representative
Member	Kerstin Westermark	Nominated by EC
Expert	Virginie Hivert	EURORDIS*
Expert	Julian Isla	EURORDIS*

*Affiliation

2. Horizontal activities and other areas

2.1. Committees and Working Parties

2.1.1. Additional objectives and activities

Key objectives

- Through PDCO-COMP interaction meetings, to further elaborate and develop the interaction process between the PDCO and COMP in view of appropriate consistency of opinions, exchange of expertise and effective use of incentives.
- Improve the naming convention of ATMPs across Committees.
- Make sure there is a common understanding across Committees on significant benefit when used for orphan designation and at maintenance.

Activities in 2017

- Discuss in COMP-PDCO working group how to increase transparency on incentives from orphan and paediatric legislation and how to make a better use of data generated in PIPs to obtain incentives.

COMP topic leader: Frauke Naumann-Winter

Other Committee participants:

Member/Alternate	Name	Member State
Member	Irena Bradinova	BG
Member	Armando Magrelli	IT
Member	Aušra Matulevičienė	LT
Member	Bożenna Dembowska-Bagińska	PL
Member	Mario Ricciardi	Patients' organisations representative
Member	Kerstin Westermark	Nominated by EC
Member	Giuseppe Capovilla	Nominated by EC

Member/Alternate	Name	Member State
Member	Ingeborg Barišić	Nominated by EC

- Explore the possibility to set up a COMP-CAT working group to improve the nomenclature classification system for orphan advance therapy medicinal products across Committees.

COMP topic leader: Karri Penttila

Other Committee participants:

Member/Alternate	Name	Member State
Member	Frauke Naumann-Winter	DE
Member	Fernando Méndez Hermida	ES
Member	Armando Magrelli	IT
Member	Ingrid Wang	NO

- Contribute to the development of a regulatory analysis on the various provisions on significant benefit in the context of +1 year, paediatrics and orphans led by CHMP.

COMP topic leader: Bruno Sepodes

Other Committee participants:

Member/Alternate	Name	Member State
Member	Frauke Naumann-Winter	DE
Member	Violeta Stoyanova-Beninska	NL
Member	Kerstin Westermark	Nominated by EC
Member	Mario Ricciardi	Patients' organisations representative

2.2. *International activities*

2.2.1. Exchange of information

Key objectives

- To further develop interactions with the Office of New Drugs, Centre for Drug Evaluation and Research at FDA to discuss post designation issues

Activities in 2017

- Strengthen dialogue with FDA topics such as major contribution to patients' care, prevalence, orphan similarity, incentives, and accessibility to patients.

COMP topic leader: Bruno Sepodes

Other Committee participants:

Member/Alternate	Name	Member State
Member	Frauke Naumann-Winter	DE
Member	Dinah Duarte	PT
Member (Vice-chair)	Lesley Greene	Patients' organisations representative