

4 December 2015
EMA/588712/2015 Rev. 2

Demonstrating significant benefit of orphan medicines: concepts, methodology, and impact on access

Workshop agenda

Chair: Bruno Sepodes (COMP Chair)

Monday 7 December 2015, 09:00 – 17:15, Room 3A/3M

Main aims of the workshop

- To explore concepts and demonstration of significant benefit of orphan medicines over existing treatments;
- To discuss existing methodologies for comparative efficacy and effectiveness, and for major contribution to patient care, including patient preferences, and how they could be applied to the demonstration of significant benefit at marketing authorization;
- To discuss the impact of significant benefit on HTA assessment, pricing decisions, and access to orphan medicines;

Target participants: medicine developers, regulators, healthcare professionals, academia, patients, health technology assessment (HTA) bodies and healthcare payers

Time	Topic	Initials	Mins
08:30-09:00	Registration		30
09:00-09:10	Welcome and introduction by EMA Senior Management and Chair	J. Llinares B. Sepodes	10
09:10- 09:15	Objectives of the workshop	B. Sepodes	5

Time	Topic	Initials	Mins
Significant benefit concepts and experience			
09:15-09:30	Significant benefit: origins and experience up to date	K. Westermarck	15
09:30-09:45	Clinically relevant advantage and major contribution to patient care	L. Fregonese	15
09:45-10:05	Questions and discussion	L. Greene	20
The significant benefit of oncology products			
10:05-10:20	Significant benefit of oncology products at marketing authorization: the COMP experience	F. Naumann-Winter	15
10:20-10:35	The ESMO-MCBS Clinical benefit scale of anti-cancer therapies	R. Sullivan	15
10:35-10:50	Questions and discussion	K. Larsson	15
Coffee break 10:50-11:15			
Stakeholders' perspective on significant benefit			
11:15-11:30	HTAs/payers	N. Hedberg	15
11:30-11:45	Industry	A. Heathfield	15
11:45-12:00	Patients	Y. Le Cam	15
12:00-12:20	Questions and discussion	L. Fregonese	20
12:20-12:30	Presentation of the breakout sessions	L. Fregonese B. Sepodes	10
Lunch break 12:30 to 13:45			
Afternoon breakout sessions			
13:45-15:15	Breakout session 1: Methodological tools for indirect comparisons: which use for significant benefit?	A. Thomson P. Motte	
	Breakout session 2: Significant benefit of oncology products: what is significant?	P. Casali P. Evers	
	Breakout session 3: Patient preferences and PROs in significant benefit	A. Beyer D. O'Connor	
Coffee break 15:15-15:45			
Wrap-up of breakout sessions			
15:45-17:00	Presentation of the results of the working groups and general discussion	All J. Llinares	90
17:15	End of workshop	B. Sepodes	

Organising Committee

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