9 December 2019
EMA/668786/2019

EMA EORTC workshop on novel PRO and QoL approaches in cancer clinical research

Event

- 12 March 2020, 13:00 - 13 March 2020, 13:10 (Thursday - Friday)
- EMA, Domenico Scarlattilaan 6, 1083 HS Amsterdam, The Netherlands
- Room 1D

Background and objectives

Patient-reported outcomes (PRO) and quality of life (QoL) assessments are an integral part of cancer clinical research and cancer drug development. Novel PRO / QoL instruments and new approaches to tailor and use these instruments are being developed, to improve patient-relevant assessments in trials.

For example, the EORTC Item Library has been developed so that researchers and clinicians can select parts of existing, validated instruments that best fit a trial's research questions and can add provisions to capture symptoms and adverse events not covered by existing instruments.

Approaches that use such composite instruments or instruments that adapt to a patient's response in a cancer trial lead to questions such as how they can be validated and how they can be used for regulatory purposes, and what is the potential impact on drug development and availability.

Overall aims of the workshop:

1. To discuss novel approaches to define and use PRO / QoL instruments (in particular, the item library) for cancer clinical research and regulatory submissions, in the context of evolving regulatory science strategies, guidelines and policies for trials and assessments by regulatory and HTA bodies
2. To understand benefits and limitations of response-adaptive PRO / QoL instruments, their use and interpretation
3. To explore qualification of new types of instruments (e.g. qualification procedure in EU)
4. To explore avenues towards PRO and QoL being an integral part of the regulatory patient centred authorization and access processes for new treatments

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

Meeting chairs

- Jan Bogaerts (Scientific director, EORTC)
- Peter Mol (Scientific advice working party, EMA)
- Pierre Demolis (Scientific advice working party, EMA)

Agenda

Day 1

Evolution of PRO and QoL: perspectives and ambitions

Session chairs: Andrew Bottomley and Pierre Demolis

Item	Topic	Duration	Time
1.	Welcome, objectives and organisation of workshop • Pierre Demolis • Jan Bogaerts	15	13:00
2.	EORTC views • Denis Lacombe, Anne-Sophie Darlington	20	
3.	Regulatory views, practice and challenges in assessment of PRO – what does the regulator need? • Pierre Demolis	20	
4.	Application and importance of PRO from HTA perspective • Beate Wieseler	20	
5.	Which research is going on for using PRO and QoL tools, what are further aspirations, what do stakeholders wish to have? • Panel discussion	45	
6.	Coffee break		15:00

Item	Topic	Duration	Time
7.	Patient's expectations for embedding PRO in access to new treatments • Kathy Oliver	25	15:30
8.	Health Canada's vision on PRO in oncology • Maxime Sasseville	25	
9.	FDA's patient-focused drug development in oncology, in particular PRO and (briefly) validation • Belinda King-Kallimanis	25	
10.	Opportunities towards a cancer patient-centred drug development process – gaps, needs, bottlenecks, actors, first steps • Panel and plenary discussion	45	
11.	End of day 1		17:30

Day 2

Developing new standards for PRO in trials

Session chairs: Anne-Sophie Darlington and Peter Mol

Item	Topic	Duration	Time
12.	Evidence-based guidelines for inclusion of PRO in trial protocols and for PRO outcome reporting • Melanie Calvert	15	09:00
13.	International guidelines for standards of analysis of QoL data from trials: SISAQOL and other developments • Madeline Pe	15	
14.	Discussant • Filip Josephson	10	
15.	Discussant • Beate Wieseler	10	
16.	Discussant • Belinda King-Kallimanis	10	
17.	New standards in PRO trials – presented suggestions and beyond • Panel and plenary	45	
18.	Coffee break		10:45

Qualification of QoL instruments in oncology product development

Session chairs: Jan Bogaerts and Ralf Herold

Item	Topic	Duration	Time
19.	EORTC Item Library in cancer clinical trials • Dagmara Kulis	10	11:15
20.	EORTC computer adaptive tests: item response theory and experience • Morten Aagaard Petersen, Mogens Groenvold	10	
21.	Methodological challenges integrating PRO in clinical trial designs • Corneel Coens	10	
22.	Commentary and EMA qualification • Maria Tome	15	
23.	Collaborations and contributions for novel method qualification • Panel and plenary discussion, questions:	60	
24.	Wrap-up and conclusions	10	
25.	End of meeting		13:10

Participants

Kathy Oliver (IBTA)

Denis Lacombe (EORTC)

Jan Bogaerts (EORTC)

Vassilis Golfinopoulos (EORTC)

Anne-Sophie Darlington (EORTC)

Mogens Groenvold (EORTC)

Andrew Bottomley (EORTC)

Corneel Coens (EORTC)

Madeline Pe (EORTC)

Dagmara Kulis (EORTC)

Morten Aagaard Petersen (EORTC)

Pierre Demolis (EMA)

Peter Mol (EMA)

Filip Josephson (EMA)

Maria Tome (EMA)

Ralf Herold (EMA)

Francesco Pignatti (EMA)

Maxime Sasseville (Health Canada)

Paul Kluetz (FDA)

Belinda King-Kallimanis (FDA)

Vishal Bhatnagar (FDA)

Giovanni Tafuri (EUnetHTA)

Beate Wieseler (IQWiG)

Krystyna Hviding (NOMA)

Joanna Wójtowicz (AOTMIT)