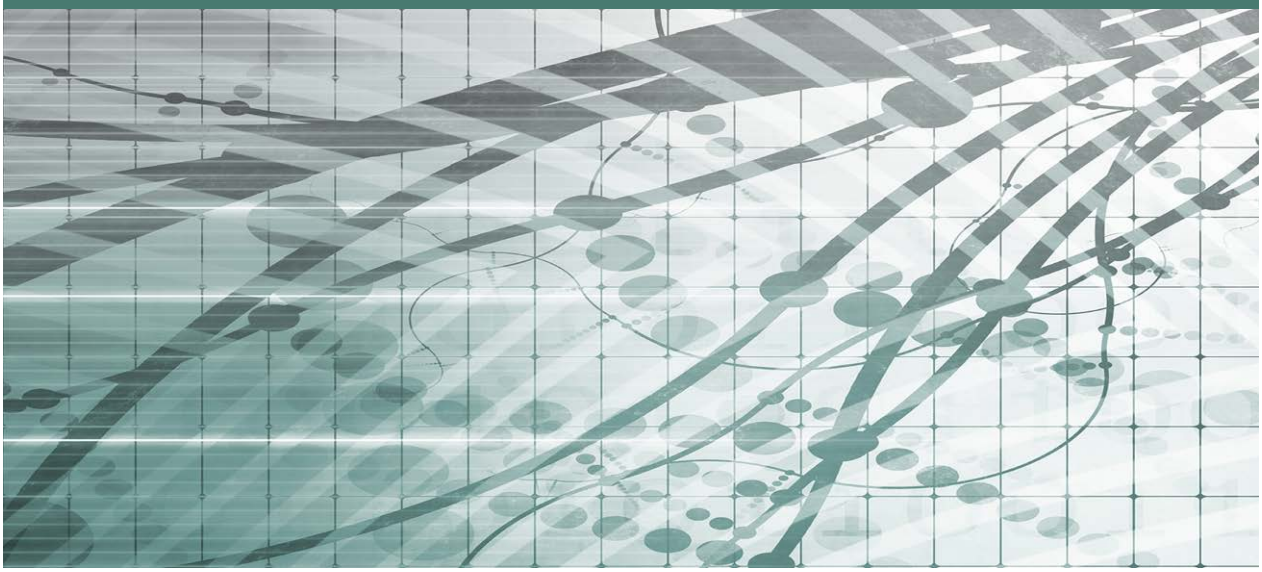
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

18 September 2012
EMA/895687/2011
Human Medicines Development and Evaluation

Workshop on endpoints for cystic fibrosis clinical trials

Agenda

27-28 September 2012
European Medicines Agency, London, United Kingdom



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An agency of the European Union

Programme overview

Scope

Clinical trials in cystic fibrosis (CF) investigate a widening array of novel therapeutics addressing the basic underlying defect in CF. Advances in symptomatic treatment options and patient care have resulted in great improvement of survival with the majority of young children with CF having spirometry within the normal range and rarely experiencing pulmonary exacerbations.

This success has created challenges to the development of future CF therapies: statistically significant changes in pulmonary function and exacerbation rate become more difficult to demonstrate as baseline population values improve. Disease-modifying drugs should ideally be administered prior to development of lung damage, adding to the challenges of designing and conducting clinical trials to evaluate the efficacy of such agents.

A systematic approach to outcome-measures development is needed to provide regulators with the tools necessary for evaluating new therapies.

Workshop chair, panellists and other experts

Chairperson	Irmgard Eichler (EMA)
Panellists, speakers:	
Christiane De Boeck	European Cystic Fibrosis Society
Stuart Elborn	European Cystic Fibrosis Society
Elmer Schabel	CHMP Gastroenterology guideline drafting group
Steffen Thirstrup	CHMP Respiratory drafting group
Paolo Siviero	Italian Medicines Agency (AIFA)
Mair Powell	Infectious Disease Working Party / Scientific Advice Working Party (IDWP / SAWP)
Birgit Dembski	Patient representative, CF Europe
Emma Lake	Patient representative, CF Trust
Johannes Taminiau	Paediatric Committee (PDCO)
Laura Fregonese	EMA
Michael Berntgen	EMA
Marco Cavaleri	EMA
Efthymios Manolis	EMA
Concepcion Prieto Yerro	Committee for Medicinal Products for Human Use (CHMP)

Other experts:

Patrick Salmon	CHMP
Jane Davies	Expert - European Cystic Fibrosis Society
Harm Tiddens	Expert - European Cystic Fibrosis Society
Matthias Griese	Expert - European Cystic Fibrosis Society
Isabelle Fajac	Expert - European Cystic Fibrosis Society
Janice Abbott	Expert - European Cystic Fibrosis Society
Juliet E. Foweraker	Expert - European Cystic Fibrosis Society
Felix Ratjen	Expert - European Cystic Fibrosis Society
Maria Jesús Fernández Cortizo	PDCO / IDWP
Irja Lutsar	PDCO / IDWP
Johan W. Mouton	Radboud University Nijmegen Medical Centre
Caroline Auriche	SAWP
Robert James Hemmings	SAWP
Frank Bodewes	University Medical Center Groningen

Programme details

Thursday, 27 September 2012

08:00-08:50 **Registration**

09:00 **Start of the Workshop**

Room 2A **Welcome, health & safety and declarations of interests**

Participants will be welcomed, instructed about health and safety issues and asked to confirm that they have no conflicts of interests in relation to any of the issues, products or applications to be discussed during the meeting.

Introduction by workshop chairperson (10')

Brief introduction of the objectives, topics and panel/participants of the workshop.

Presentations to set the scene for the break-out group discussions

Presentation 1 ***CF – update: changing demographics of patient population and evolving standards of care – implications for clinical-trial design and endpoints (15')***
Speaker: Stuart Elborn

Presentation 2 ***Difficulties/challenges encountered – look into the future: academia perspective (15')***
Speaker: Christiane De Boeck

Presentation 3 ***Difficulties/challenges encountered – look into the future: industry perspective (15')***
Speaker: David Waltz (Vertex Pharmaceuticals Incorporated)

Presentation 4 ***Difficulties/challenges encountered – look into the future: patient perspective (15')***
Speaker: Emma Lake

General discussion (20')

10:30-10:45 **Intermission**

Presentation 5 ***Difficulties encountered – regulatory perspective: focus on endpoints used in clinical trials***

- Orphan designation, speaker: Laura Fregonese (10')
- Scientific advice, speaker: Efthymios Manolis (10')
- Paediatric investigation plans, speaker: Irmgard Eichler (10')
- Marketing authorisation, speaker: Concha Prieto Yerro (15')
- Endpoints for added clinical benefit in view of HTA, speaker: Paolo Siviero (15')

General discussion (30')

12:15-13:30 **Lunch break**

Break-out sessions (180' + 15' intermission)

Participants will be pre-assigned to the following groups.

Room 2A **Session 1:**

Pulmonary disease (Chairs: Christiane De Boeck – Steffen Thirstrup)

Topic 1.1 *Clinical efficacy endpoints*

Topic 1.2 *Biomarkers*

Topic 1.3 *Surrogate endpoints*

Topic 1.4 *PRO*

Room 2G **Session 2:**

Bronchopulmonary infection (Chairs: Mair Powel – Stuart Elborn)

Topic 2.1 *General microbiological issues*

Topic 2.2 *PK/PD studies – particular focus on inhaled antibiotics*

Topic 2.3 *Microbiological endpoints*

- Antibiotic eradication therapy
- Chronic suppressive(pseudomonas) antibiotic treatment

Topic 2.4 *Methodological issues*

- Study duration
- Choice of comparator
- Study design – inhaled antibiotics: on/off regime, alternating antibiotics

Room 3C **Session 3:**

Exocrine pancreatic insufficiency (Chairs: Johannes Taminiau– Elmer Schabel)

Topic 3.1 *(Novel) outcome measures*

- New pancreatic enzyme products
- CFTR modifiers

15:00-15:15 **Intermission**

16:45 **Close of day**

Friday, 28 September 2012

09:00	**Start of day**
	Break-out sessions continue (90')
	Participants continue working in pre-assigned groups.
Room 2A	Session 1: Pulmonary disease
Room 4C	Session 2: Bronchopulmonary infection
Room 3C	Session 3: Exocrine pancreatic insufficiency
10:30-10:45	**Intermission**
	Break-out sessions continue (90')
	Participants continue working in pre-assigned groups.
12:15-13:30	**Lunch break**
Room 2A	**Presentation of outcomes to plenary from each group and discussion**
Session 1:	**Pulmonary disease,** Christiane De Boeck – Steffen Thirstrup
Session 2:	**Bronchopulmonary infection,** Mair Powel – Stuart Elborn
Session 3:	**Exocrine pancreatic insufficiency,** Johannes Taminiau – Elmer Schabel
	General discussion
	Summary and conclusions
15:30	**Close of workshop**

Conference venue and secretariat

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