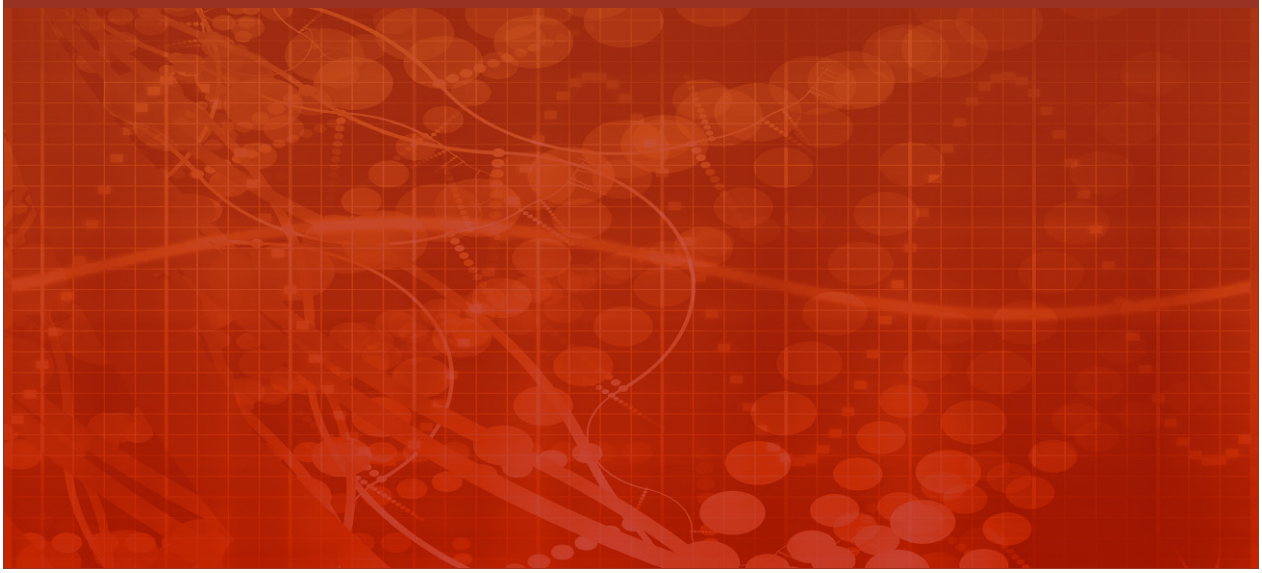
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

9 January 2012
EMA/CAT/27660/2012
Human Medicines Development and Evaluation

Focus groups: a model for fruitful interaction between the CAT and its interested parties

Workshop programme

Thursday, 12 January 2012
European Medicines Agency, 7 Westferry Circus, Canary Wharf, London, United Kingdom



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Introduction

This workshop aims to strengthen dialogue with CAT stakeholders, to facilitate ATMP development and access to the registration procedure.

This one-day workshop is organised by the European Medicines Agency's (EMA) Committee for Advanced Therapies (CAT) as part of its efforts to strengthen the dialogue with all of its stakeholders. The main aim of the workshop is to communicate the outcome of focus group meetings held in 2011 concerning:

- non-clinical development of ATMPs;
- a better system for navigating scientific guidance documents on gene therapy and cell-based medicinal products;
- incentives for academia, hospitals and trusts developing ATMPs.

The workshop will allow stakeholders involved in research and development of medicines based on gene therapy or cells to:

- hear the significant points collected by CAT interested parties on identified bottlenecks in ATMP development;
- learn about CAT focus groups' action plans for 2012;
- participate in the discussions (questions received by participants will form the basis of the panel discussions).

The conference features keynote presentations from the EMA and representatives of CAT, Interested Parties, lively panel debates and case studies.

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Welcome to all participants

On behalf of the Programme Committee and the Committee for Advanced Therapies (CAT), I am pleased to welcome you to the stakeholders workshop 'Focus groups: a model for fruitful interaction between the CAT and its interested parties'.

This one-day workshop is the second event open to all stakeholders, and organised by the CAT as part of the Committee's effort to strengthen the dialogue with them.

In relation with its CAT work programme 2010-2015, the CAT decided in October 2010 to convene focus groups with representatives of the CAT and interested parties (IPs). The CAT-IPs focus groups aim to improve CAT interaction with interested parties and to propose shared solutions on some of the issues previously identified in general hearings with IPs.

The objective of this workshop is to communicate the outcome of the discussions held in focus group meetings in 2011, to enrich the scientific discussion on the three identified topics (non-clinical development of ATMPs, guidelines, and incentives for non-commercial ATMP developers), and to share the proposed action plans for 2012. The event is designed to foster direct interaction between regulators and CAT stakeholders by means of round-table debates on questions from participants.

We believe that, within existing regulatory frameworks, a proactive approach to tackling specific bottlenecks on ATMP development and a focussed dialogue between regulators and stakeholders are crucial to foster translation of ATMPs from research labs to the market.

We look forward to meeting you in London!

Yours sincerely,

Christian Schneider

CAT Chair

Programme overview

Scope

The workshop will be of interest to all CAT stakeholders: industry, including SMEs; academics from gene and cell therapy communities; regulatory professionals; healthcare professionals in the field; patient associations; civil-society organisations; corporate decision-makers; and regulators.

Sessions

Session 1 Focus group: non-clinical development of ATMPs.

Session 2 Focus group: a better system to navigate guidelines for ATMPs.

Session 3 Focus group: incentives for academia, hospitals and charities.

Additional information

See important practical information at the end of this programme.

Programme Committee

Christian Schneider	Workshop Chair, CAT Chair
Paula Salmikangas	CAT Vice-chair, CPWP Chair
Pablo de Felipe	GTWP member
Maria Cristina Galli	CAT member, GTWP Chair
Carla Herberts	CPWP member
Romaldas Maciulatis	CAT member, CHMP member
Monica Neagu	CAT member
Sol Ruiz	CAT member, CHMP member
Beatriz Silva Lima	CAT member, CHMP member, SWP Chair
Henrik Tang Vestergaard	CAT member
Jean-Hugues Trouvin	CAT member, BWP Chair
Lucia D'Apote	EMA, CAT Scientific Secretariat

Programme details

Thursday, 12 January 2012

8.00 Registration and welcome coffee

Go to reception on the second floor to register, receive your badge and buy the lunch voucher. Then join delegates in room 2A, where coffee will be served before the conference begins.

9.00 Welcome and opening remarks

Christian Schneider (conference chair)
European Medicines Agency, CAT Chair

9.10 CAT dialogue with its stakeholders

Focus groups: a new model for fruitful interaction between the CAT and its stakeholders
Lucia D'Apote
European Medicines Agency, CAT Scientific secretariat

9.30 Session 1: Focus group: non-clinical development of ATMPs

Objectives of the focus group and outcome discussions in 2011
Christian Schneider
European Medicines Agency, CAT Chair

Overview of scientific advice given on ATMPs - Non-clinical issues
Carla Herberts
Senior Non-clinical Assessor, CBG-MEB
Henrik Tang Vestergaard
Non-clinical Assessor, DKMA

Possible scenarios to address issues in non-clinical studies for ATMPs
Roberto Liddi
EUCOMED

Panel discussion
Beatriz Silva Lima, Dario Pirovano, Decebal Bora (session co-chairs)
CAT experts: Carla Herberts, Romaldas Maciulatis, Jean-Hugues Trouvin, Henrik Tang Vestergaard
Stakeholders: (EBE), Alicia J. El Haj (TERMIS), Len Seymour (ESGCT)

11.25 Coffee break

11.55 Session 2: Focus group: a better system for navigating guidelines for ATMPs

Objectives of the focus group and outcome discussions in 2011

Maria Cristina Galli

CAT member, GTWP Chair

Interactive flow-chart for Gene Therapy guidelines

Pablo de Felipe

GTWP member

The way forward: action plan for 2012

Christian Schneider

European Medicines Agency, CAT Chair

Panel discussion

Maria Cristina Galli, Decebal Bora, Duncan MacKay (session co-chairs)

CAT experts: Pablo de Felipe, Monica Neagu, Sol Ruiz, Paula Salmikangas

Stakeholders: Estelle De Barbeyrac (EUROPABIO), Odile Cohen-Haguenauer (CLINIGENE), Dario Pirovano (EUCOMED)

13.30 Lunch

Buffet lunch will served in the lobby of room 2A on the second floor.

14.30 Session 3: Focus group: incentives for academia, hospitals and charities

Objectives of the focus group and outcome discussions in 2011

Paula Salmikangas

CAT member, CPWP Chair

Raising awareness on available incentives (EMA)

Patrick Celis

European Medicines Agency, CAT Scientific secretariat

Improve certainty of regulatory outcome: an identified incentive by stakeholders to foster translation of academic research into commercial products

Decebal Bora

EuropaBio (presentation prepared in coordination with EATRIS)

Optimising resources: is it possible to have one set of data for different procedures (e.g. EMA scientific advice/protocol assistance, CTA application, proposal submission for FP7 research funding)? Can the criteria for assessment of data follow the same rationale during the entire product development?

Didier Caizergues

Genethon

Carla Paganin

Telethon

The way forward: action plan for 2012 and panel discussion

Paula Salmikangas, Odile Cohen-Haguenauer (session co-chairs)

CAT experts: Christian Schneider, Maria Cristina Galli, Patrick Celis

Stakeholders: Michele Lipucci Di Paola (EURORDIS), Decebal Bora (EuropaBio), Didier Caizergues (Genethon), Massimo Dominici (ISCT), Carla Paganin (Telethon), Natalie Cartier (ESGCT)

16.25 Coffee break

16.45 Wrap-up

Christian Schneider

European Medicines Agency, CAT Chair

17.15 – 17.30 Closing Remarks

Christian Schneider

European Medicines Agency, CAT Chair

List of speakers

Decebal Bora	EuropaBio
Didier Caizergues	Genethon
Natalie Cartier	ESGCT
Patrick Celis	European Medicines Agency, CAT Scientific secretariat
Odile Cohen-Haguenauer	CLINIGENE
Lucia D'Apote	European Medicines Agency, CAT Scientific secretariat
Estelle De Barbeyrac	EUROPABIO
Pablo de Felipe	GTWP member
Massimo Dominici	ISCT
Alicia J. El Haj	TERMIS
Maria Cristina Galli	CAT member, GTWP Chair
Carla Herberts	Senior Non-clinical Assessor, CBG-MEB
Roberto Liddi	EUCOMED
Michele Lipucci Di Paola	EURORDIS
Romaldas Maciulatis	CAT member, CHMP member
Duncan MacKay	EBE
Monica Neagu	CAT member
Carla Paganin	Telethon
Dario Pirovano	EUCOMED
Sol Ruiz	CAT member, CHMP member
Paula Salmikangas	CAT member, CPWP Chair
Christian Schneider	European Medicines Agency, CAT Chair, Workshop Chair
Len Seymour	ESGCT
Beatriz Silva Lima	CAT member, CHMP member, SWP Chair
Henrik Tang Vestergaard	CAT member
Jean-Hugues Trouvin	CAT member, BWP Chair

Biographies of the workshop chair and session co-chairs

Dr Christian Schneider, MD - Workshop Chair, CAT Chair

Dr med. Christian Schneider joined the Danish Medicines Agency in October 2011 as a Senior Medical Officer. He is currently on leave from the Paul-Ehrlich-Institut (PEI), the German Federal Institute for Vaccines and Biomedicines. At the European Medicines Agency, he is the Chair of the Committee for Advanced Therapies (CAT), since 2009, and is also the Chair of the CHMP Working Party on Similar Biological (Biosimilar) Medicinal Products. Between September 2007 and July 2011, he was a member of the Committee for Medicinal Products for Human Use (CHMP), co-opted for the area of advanced therapies – gene, cell and tissue therapies. Before he joined the Paul-Ehrlich Institut, Dr Schneider worked for more than two years as a postdoctoral researcher at the Max-Planck-Institute for Neurobiology, Department of Neuroimmunology (Martinsried, Germany), where he worked in the field of experimental T cell immunology. During his clinical years, Dr Schneider worked in the field of clinical immunology and hemato-oncology (Department of Internal Medicine III, University Erlangen-Nuremberg, Germany). His current research interest in regulatory science is the optimisation of the development and marketing authorisation of biomedicines, including aspects such as biosimilars of more complex molecules, first-in-human clinical trials, and unwanted immunogenicity.

Paula Salmikangas PhD, Ass. Prof. - CAT Vice-chair, CPWP Chair

Member and Vice-chair of the Committee for Advanced Therapies at the European Medicines Agency. Dr Salmikangas is a biochemist by training, with a PhD in muscle cell biology. Her main research career has been in cell and molecular biology of various inherited diseases. Since 2006, she has been an Associate Professor of Biochemistry at the University of Helsinki. Dr Salmikangas has worked as a senior researcher at the Finnish Medicines Agency since 2003. Her main areas of expertise are biological medicinal products, especially cell-based medicinal products and combination products. Dr Salmikangas has been a member of Cell Products Working Party (CPWP) at the European Medicines Agency since 2005, and the Chair of the CPWP since 2007. She is also a member and Vice-chair of the Committee for Advanced Therapies.

Maria Cristina Galli, PhD - CAT member, GTWP Chair, Istituto Superiore di Sanità, Roma, Italy

Degree in Biological Sciences, PhD in Molecular Medicine; currently senior researcher, Cell Biology and Neurosciences Department, Istituto Superiore di Sanità, Roma, Italy. Dr Galli was active for more than 20 years as researcher in experimental oncology, cellular biology, molecular immunology; she has been active for the past 15 years as quality assessor for biotechnology and gene therapy in national as well as European procedures; she has been active for the past 10 years as a GMP and GLP inspector. Dr Galli is currently Chair of the Gene Therapy Working Party, and a CAT member at the EMA.

Prof. Beatriz Silva Lima - CAT member, CHMP member

Prof. Beatriz Silva Lima works for INFARMED, the national competent authority for medicines in Portugal. Her extensive activities and responsibilities include: national expert on pharmacotoxicology, member of the National Board of Medicines, European expert at the EMA since January 1995, where she has been a member of the Safety Working Party, Chair of the Safety Working Party (since 2001), a member of the CPMP and the CHMP since 2001 (re-elected in 2011), a member of the Scientific Advice Working Group since 2001 and subsequently of the SAWP, a member of the CAT (CHMP representative) since 2009, a member of the non-clinical working group supporting the PDCO since its implementation, and EU Deputy topic leader of ICH - M3 guideline and ICH S6 R1 guideline (addendum to ICH S6). In her role as Professor at the iMed.UL, University of Lisbon, she is in charge of the coordination of the

independent research group at the university. In particular, her role entails coordination of research on inflammation and cellular mechanisms of contractility, and she is Research Leader on Unit research granted by the Ministry of Research (FCT).

Dr Decebal Bora - EUROPABIO

Dr Decebal Bora has held the position of Director Regulatory Affairs at ActoGeniX N.V. (Ghent, Belgium) since 2007. Within this function, he strategises and participates in the implementation of global development of GMO/gene therapy medicinal products.

Decebal earned his Pharm. D. in 1993 and Masters in Public Health (MPH) in 1995 at the Université Catholique de Louvain (Belgium) and at the Faculté de Droit - Jean Monnet (France) respectively. Further to completion of his military duty at the French army's blood transfusion centre (CTSA) (Clamart, France) and of the MPH's trainee program at Afssaps, he held regulatory affairs positions at the headquarters of various biopharmaceutical companies (i.e. ActoGeniX N.V., Baxter Healthcare Europe, Biogen Inc., F. Hoffmann-La Roche Ltd, Solvay Pharmaceuticals S.A.).

Odile Cohen-Haguenauer, MD, PhD - Clinigene

Director of the Gene Therapy Programme at L.B.P.A., FRANCE; Associate-Professor, Oncogenetics, Department of Medical Oncology, Hospital St-Louis and Univ Paris7, Paris. Coordinator of the EC CliniGene-NoE, European Network of excellence for the advancement of clinical gene transfer and therapy. Founder of the European Society of Gene Therapy, launched in 1992 (with Prof. Michel Boiron); and current Chair of the ESGCT Committee on Ethics & Regulatory affairs. She is a member of the Gene Doping Advisory Group and of the World Anti-Doping Agency.

Education and training: I. medical qualifications: Oncology (Oncogenetics)/ Haematology/ Genetics/ Pediatrics & Nuclear Medicine: From paediatric genetics to cancer genetics. 1981-1989 Residency in Onco-Haematology and Pediatrics; 1989-1992: Fellow in Haematology and Oncology; 1989-1991: Qualification Med. Use of Radiopharmaceutics (Nuclear Medicine), Paris 6, 11 & CEA. II. Basic science qualifications: from immuno-genetics to molecular therapy; 1982-1985: PhD in Immunology, Laboratory of Jean Dausset (1980 Nobel Prize in Medicine); 1985-1988: Doctorat d'Etat ès Sciences in Genetics (= PhD + accreditation to supervise research): Human genetic linkage map; first mapping of the CFTR-gene (Nature); 1988-89: Post Doc in Jean-Michel Heard's group, Laboratory of retrovirology & Immunology.

Regulatory expertise: 1994-2002: French Medicines Agency: Expert Group in charge of the review of Clinical protocols & establishment of Gene therapy guidelines; 1998-2002 European Medicines Agency: Expert; GT Note For Guidance; Since 2002: Registered EMA and CAT stakeholder through EC-funded research programmes; 1994-2006: Permanent delegate of the International Bioethics Committee (I.B.C.), UNESCO.

Duncan Mackay - EBE

Graduated in Molecular Biology from Glasgow University and worked for six years in research in the NHS and at Glaxo. Transferred into the International Regulatory Affairs Department in Glaxo in 1989 where he worked for 5 years. Since then he has worked at Bristol-Myers Squibb (ConvaTec), Boston Scientific and as an independent consultant before joining Genzyme Biosurgery in 2004. He has a broad range of regulatory experience in terms of products and markets, having dealt with medicinal products, biologics, human tissues and medical devices. He is particularly interested in the regulation of combination products, advanced therapy and cell therapy products, and in the integration and added value of regulatory affairs with corporate business partners. Experience in cell-based products and tissue repair since 2004. He currently leads the regulatory team for registration of autologous combination tissue-engineered products in Europe and globally.

Dario Pirovano, P.E., PhD - EUCOMED

Dario Pirovano has been a consultant for regulatory affairs with Eucomed since 2002. He worked at the European Commission for four years, where he contributed to the drafting and negotiating of the 90/385/EEC and 93/42/EEC directives.

Dario has over 30 years' experience in medical technology as a designer and regulatory affairs expert. In 1995, he founded Pirovano Management SPRL, a consulting firm advising manufacturers, notified bodies and authorities on regulatory matters relating to medical technology.

Dario can be considered the historical memory of the development of medical devices regulation in Europe. He holds a Doctorate in Engineering from the Politecnico di Milano. An Italian national, Dario is fluent in French and English.

Practical information

Venue

The European Medicines Agency can be reached:

- By Docklands Light Railway (DLR)
  The Agency is a short walk from either Westferry station or Canary Wharf station on the DLR. Services run from Bank, Tower Gateway, Lewisham, Stratford, King George V and Beckton.
- By Underground
  The nearest stop for Westferry Circus is Canary Wharf station on the Jubilee Line.
- By bus
  Canary Wharf is serviced by local bus numbers D3, D7, D8, 135 and 277.
- By boat
  River services run between Embankment, London Bridge and Canary Wharf throughout the day.
- From London City Airport
  Take a taxi to Westferry Circus or alternatively catch the Docklands Light Railway which goes to Westferry station.

Map

Entering the building

The Agency operates a stringent security policy. Upon arrival at ground-floor reception, you will be given an ID badge (with a red lanyard) that will allow you to make your way to the meeting room on the second floor.

Physical disability

Let us know if you would like any specific help or information that would make your stay more comfortable. We will be very happy to help.

Registration

We strongly advise you to arrive up to one hour before the start of the workshop (i.e. at 8.00), to allow you time for registration and settling down. You will see the registration table positioned on the 2nd floor as soon as you step out of the lift.

Meeting room

This workshop will benefit from a full house. You will be able to sit wherever you wish; note that the only reserved seats are for the speakers, CAT experts and the organisers of the workshop.

Presentations

We will not circulate paper printouts of speakers' PowerPoint presentations. However, you will be able to download the presentations from the Agency's website two weeks after the end of the workshop.

Catering

At the time of your online registration, most of you indicated your wish to benefit from a set-price buffet lunch organised by our catering service, to be served in the lobby outside the meeting room.

The lunch will include drinks and coffee.

You need to purchase a lunch voucher for the fixed price of £15.00 as part of the registration process on the morning of the workshop. Note that only cash in pounds sterling will be accepted by the caterers.

Video broadcast

The workshop will be video recorded.

Laptop computers

For those of you travelling from the continent and wishing to use your laptop, may we remind you to bring with you a European-to-UK power adapter.

Workshop secretariat

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Workshop venue

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