22 January 2016
EMA/CAT/77433/2016
Procedure Management and Committees Support Division

Committee for Advanced Therapies (CAT)

Minutes of the meeting on 10-11 December 2015

Chair: Paula Salmikangas - Vice-chair: Martina Schüßler-Lenz

10 December 2015, 09:00 – 18:30, room 03-E
11 December 2015, 09:00 – 14:00, room 03-E

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

Some of the information contained in these minutes is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the CAT meeting reports once the procedures are finalised.

Of note, these minutes are a working document primarily designed for CAT members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the minutes cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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1. Introduction

1.1. Welcome and declarations of interest of members, alternates and experts

In accordance with the Agency's policy on handling of declarations of interests of scientific committees' members and experts, based on the declarations of interest submitted by the Committee members, alternates and experts and based on the topics in the agenda of the current meeting, the Committee Secretariat announced that no restriction in the involvement of meeting participants in upcoming discussions was identified.

Participants in this meeting were asked to declare any changes, omissions or errors to their declared interests and/or additional restrictions concerning the matters for discussion. No new or additional interests or restrictions were declared.

Discussions, deliberations and voting took place in full respect of the restricted involvement of Committee members and experts in line with the relevant provisions of the Rules of Procedure and as included in the list of participants. All decisions taken at this meeting were made in the presence of a quorum of members (i.e. 22 or more members were present in the room). All decisions, recommendations and advice were agreed by consensus, unless otherwise specified.

Meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the CAT plenary session held on 10-11 December 2015.

1.2. Adoption of agenda

The CAT agenda for 10-11 December 2015 was adopted with one amendment to point 5.2.5 (name of the CAT resource).

1.3. Adoption of the minutes

The CAT minutes of 12-13 November 2015 were adopted with one amendment to point 5.4.1.

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 List of outstanding issues (LoOIs)

No items

2.4. Day 120 Lists of questions (LoQs)

No items

2.5. Day 80 assessment reports

No items

2.6. Ongoing initial full application

No items

2.7. New applications

2.8. Withdrawal of initial marketing authorisation application

No items

2.9. Re-examination of initial application procedures under Article 9(2) of Regulation no. 726/2004

No items

2.10. GMP and GCP inspections requests

No items

2.11. Type II variations

No items

2.12. Other post-authorisation activities

2.12.1. ChondroCelect – Characterised viable autologous cartilage cells expanded *in vivo* expressing specific marker proteins; EMA/H/C/00878/MEA 16.5., 18.5.

TiGenix N.V.

Rapporteur: Egbert Flory; Co-rapporteur: Tiina Palomäki; CHMP Coordinator: Jan Müller-Berghaus

Scope 16.5.: randomised control trial protocol TIG/ACT/04/2009
Scope 18.5.: non-interventional registry of ChondroCelect, study TGX001-2011 & randomised controlled study in small lesions using microfracture as comparator

Action: for adoption

Document:
Preliminary Response Assessment Report

CAT discussed the Rapporteur's Assessment Report. CAT agreed with the conclusions that based on the data provided a confirmatory randomised control trial in small lesions is no longer required; the company will have to continue to provide data generated under real world conditions from the ongoing non interventional study and to provide data from a new clinical trial to confirm the efficacy of the product in large lesions. The MAH is advised to seek

EMA scientific advice on the trial in large lesions. CAT adopted the final response assessment report.

A variation will have to be submitted by the applicant to amend their RMP in line with the outcome of this assessment.

3. Certification of ATMPs

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

3.1. New applications

No items

3.2. Day 60 evaluation reports

No items

3.3. Opinions

No items

3.4. Ongoing initial application

4. Scientific Recommendation on Classification of ATMPs

4.1. New requests – appointment of CAT Co-ordinators

Adeno-associated viral vector serotype 2 containing the human *RPE65* gene Intended for the treatment of inherited retinal degeneration due to autosomal recessive *RPE65* gene mutations

Scope: appointment of CAT Co-ordinator and adoption of timetable

Action: for adoption

Document:
Request received 26th November 2015

Nominations were received. The CAT member was appointed as the CAT coordinator for this procedure.

4.1.1. *Ex vivo* expanded allogeneic human immuno-modulatory progenitor (iMP) cells

Intended for the treatment of incomplete revascularisation as an adjunct to CABG in patients with congenital coronary artery malformations

Scope: appointment of CAT Co-ordinator and adoption of timetable

Action: for adoption

Document:
Request received 26th November 2015

Nominations were received. The CAT member was appointed as the CAT coordinator for this procedure.

4.1.2. hAMMSCs - Human amniotic membrane mesenchymal stem cells

Intended for the treatment of burns and non-healing wounds

Different product formulations:
-hAMMSCs in suspension
-hAMMSCs as sheet
-hAMMSCs seeded on acellular amniotic matrix
-hAMMSCs seeded on acellular dermal matrix

Scope: appointment of CAT Co-ordinator and adoption of timetable

Action: for adoption

Document:
Request received 2th November 2015

Nominations were received. The CAT member was appointed as the CAT coordinator for this procedure.

4.1.3. hUSCs - Human umbilical cord mesenchymal stem cells

Intended for the treatment of burns and non-healing wounds

Different product formulations:
-hUSCs in suspension
-hUSCs as sheet
-hUSCs seeded on acellular amniotic matrix
-hUSCs seeded on acellular dermal matrix

Scope: appointment of CAT Co-ordinator and adoption of timetable

Action: for adoption

Document:
Request received 2th November 2015

Nominations were received. The CAT member was appointed as the CAT coordinator for this procedure.

4.1.4. Co-culture of keratinocytes and human umbilical cord mesenchymal stem cells

Intended for the treatment of burns and non-healing wounds

Different product formulations:
-seeded on acellular amniotic matrix
-seeded on acellular dermal matrix

Scope: appointment of CAT Co-ordinator and adoption of timetable

Action: for adoption

Document:
Request received 2th November 2015

Nominations were received. The CAT member was appointed as the CAT coordinator for this procedure.

4.1.5. Co-culture of keratinocytes and human amniotic membrane mesenchymal stem cells

Intended for the treatment of burns and non-healing wounds

Different product formulations:
-seeded on acellular amniotic matrix
-seeded on acellular dermal matrix

Scope: appointment of CAT Co-ordinator and adoption of timetable

Action: for adoption

Document:
Request received 2th November 2015

Nominations were received. The CAT member was appointed as the CAT coordinator for this procedure.

4.2. Day 30 Co-ordinators' first reports

4.2.1. Autologous cells of stromal vascular fraction (SVF) and autologous adipose derived stem cells

Intended for the treatment of treatment of (1) diabetic foot ulcer and (2) keloid scars and aging skin

Action: for adoption

Document:
ATMP classification report

CAT discussed the ATMP classification report. CAT adopted by consensus the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.2. Autologous adipose-derived regenerative cells encapsulated in carboxymethylcellulose

Intended for the treatment of cosmetic dermal filling

Action: for adoption

Document:
ATMP classification report

CAT discussed the ATMP classification report and decided to request some additional information from the applicant prior to concluding on this classification request.

4.2.3. Human hepatoblastoma cells (HepG2) encapsulated in alginate, expanded in a fluidised bed bioreactor

Intended for the treatment of acute liver failure

Action: for adoption

Document:

ATMP classification report

CAT discussed the ATMP classification report. CAT adopted by consensus the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.4. Adeno-associated virus serotype 8 vector encoding human ornithine transcarbamylase

Intended for the treatment of ornithine transcarbamylase tl

Action: for adoption

Document:

ATMP classification report

CAT discussed the ATMP classification report. CAT adopted by consensus the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.5. Fibroblasts and keratinocytes co-culture

Intended for the treatment of deep and extensive burns, chronic wounds, skin donor sites

Different product formulations:
-suspension of cell in platelet leukocyte rich gel
-in sheet
-seeded on acellular amniotic matrix
-seeded on acellular dermal matrix
-seeded on transgenic porcine acellular dermal matrix

Action: for adoption

Document:
ATMP classification report

CAT discussed the ATMP classification report for the following products: Fibroblast and keratinocytes in suspension; Fibroblast and keratinocytes in sheet; Fibroblast and keratinocytes seeded on acellular amniotic matrix; Fibroblast and keratinocytes seeded on acellular dermal matrix. CAT adopted by consensus the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

CAT discussed the ATMP classification report for Fibroblast and keratinocytes seeded on transgenic porcine dermal matrix and decided to request some additional information from the applicant prior to concluding on this classification request.

4.2.6. Human acellular amniotic matrix

Intended for the treatment of deep and extensive burns, chronic wounds, skin donor sites

Action: for adoption

Document:
ATMP classification report

CAT discussed the ATMP classification report. CAT adopted by consensus the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.7. Human acellular dermal matrix

Intended for the treatment of deep and extensive burns, chronic wounds, skin donor sites

Action: for adoption

Document:
ATMP classification report

CAT discussed the ATMP classification report. CAT adopted by consensus the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.8. Allogeneic chondrocytes and irradiated genetically modified chondrocytes expressing human TGF-β1

Intended for the treatment of degenerative joint disease

Action: for adoption

Document:
ATMP classification report

CAT discussed the ATMP classification report. CAT adopted by consensus the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.9. Allograft tendon combined with suture ready to use

Intended for the treatment of anterior cruciate ligament reconstruction

Action: for adoption

Document:
ATMP classification report
List of Questions to applicant

CAT discussed the ATMP classification report. CAT adopted by consensus the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.2.10. Transgenic porcine acellular dermal matrix

Intended for the treatment of deep and extensive burns, chronic wounds, skin donor sites

Action: for adoption

Document:
ATMP classification report

CAT discussed the ATMP classification report. CAT adopted by consensus the ATMP classification report. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.3. Day 60 Co-ordinators' revised reports following List of Questions

4.3.1. Autologous bone marrow derived non-haematopoietic stem cells

Intended for the treatments of patients with rheumatoid arthritis; patients after ischemic stroke; patients after myocardial infarction; type I diabetes; type II diabetes

Action: for adoption

Documents:
Revised ATMP classification report
Applicant's response to the LoQs

Further to receipt of the additional information, the revised ATMP classification report was discussed. The classification report was further updated after the discussion. CAT adopted the revised classification report by consensus. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.3.2. Autologous peripheral blood-derived total nucleated cells

Intended for the treatment of critical limb ischemia

Action: for adoption

Documents:
Revised ATMP classification report
Applicant's response to the LoQs

Further to receipt of the additional information, the revised ATMP classification report was discussed. The classification report was further updated after the discussion. CAT adopted the revised classification report by majority (18 members and Norway voted in favour of classifying this product as an ATMP). The Divergent position was signed by 11 CAT members. CAT secretariat to send the draft scientific recommendation to the European Commission for comments
The CAT recommendation will be considered final if no comments are received from the European Commission. The final report will be sent thereafter to the applicant.

4.4. Finalisation of procedures

4.4.1. Autologous adipose derived regenerative cells encapsulated in hyaluronic acid

Intended for the treatment of articular cartilage and bone defects

Action: for information

Document:
ATMP classification report

Note:
The European Commission raised no comments

The final report was noted.

4.4.2. Autologous cells of stromal vascular fraction (SVF) of adipose tissue

Intended for (1) cosmetic lipofiling; (2) treatment for non-healing wounds and scared tissue; (3) treatment of osteoarthritis in the knee

Action: for information

Document:
ATMP classification report

Note:
The European Commission raised no comments

Further to the discussion on the product 'Autologous adipose-derived regenerative cells encapsulated in carboxymethylcellulose' (agenda point 4.2.2), CAT introduced an amendment to the final classification report to ensure the same message was included on the use of human cells for cosmetic filling. The updated classification report was adopted.

4.4.3. Allogeneic pro-inflammatory monocyte-derived dendritic cells

Intended for the treatment of metastatic renal cell carcinoma (mRCC)

Action: for adoption

Document:
ATMP classification report

Note:
The European Commission raised no comments

4.5. The final report was noted. Follow-ups and guidance

No items

5. Scientific Advice

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

5.1. New requests – appointment of CAT Co-ordinators

5.2. CAT Rapporteurs' reports

5.3. Lists of issues

5.4. Finalisation of Scientific Advice procedures

6. Pre-Authorisation Activities

Disclosure of information related to this section cannot be released at the present time as it is deemed to contain commercially confidential information.

6.1. Paediatric investigation plans (PIP)

No items

6.2. ITF briefing meetings in the field of ATMPs

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

No items

7.2. Coordination with EMA Scientific Committees

7.2.1. Committee for Medicinal Products for Human Use (CHMP)

Scope: Summary of Outcomes (SoO) for the November 2015 meeting
Scope: Workplan for 2016

Action: for information
Documents:
-Summary of Outcomes
-Workplan

Note: the CHMP adopted their workplan in January 2016

The information was noted.

7.2.2. Experience with 'Early Background Summaries'

Scope: upcoming survey to gather feedback from rapporteurs and assessors on the experience with the Early Background Summaries that were introduced in 2014

Action: for information

Feedback was provided by EMA. One ATMP was amongst the products chosen for the pilot on Early Background Summaries. An e-mail (with the survey) will be sent to the CAT Rapporteurs and assessors.

7.2.3. Guideline on Good Pharmacovigilance Practices (GVP) Module V: Risk Management Plan (RMP), rev. 1.5.

Scope: First major revision of the guideline

Action: for discussion

Note:
-an e-mail with the module was sent to CAT member on 18.11.15. with deadline for comments 2nd December 2015
-the section on ATMP specific guidance is to be found in rows 529 to 590

A presentation was given on the 8 main changes introduced to the document. It was requested that CAT is mentioned in the document where relevant. It was also noted that this GVP module will only apply to ATMPs until the Guideline of Safety and Efficacy – RMP will be updated (see point 7.2.4).

7.2.4. Guideline on Safety and Efficacy – RMP for ATMPs

Scope: Update and follow up on the guideline

Action: for information

http://www.ema.europa.eu/docs/en_GB/document_library/Regulatory_and_procedural_guideline/2009/10/WC500006326.pdf

Note:
EMA has set up a task force to re-visit the document and are looking for CAT volunteers.

The aim and scope of the revision of this guideline was presented. Sponsor from all concerned Committees (CAT, PRAC, CHMP) are sought to support the work from the EMA team. N Ferry and M. O'Donovan were appointed as CAT sponsors.

7.3. Coordination with EMA Working Parties / Working Groups / Drafting Groups

7.3.1. Good Laboratory Practice (GLP) requirements of non-clinical studies for ATMPs

CAT drafting group: Una Riekstina, Tiina Palomäki, Egbert Flory, Ilona Reischl, Carla Herberts (NL), Isabel Vieira (PT)

Scope: Application of GLP principles on ATMPs

Action: for adoption

Document:
CAT's position

Note:
June 2015: presentation by the EMA GLP Inspections Working Party (IWP) on GLP requirements for ATMPs
July 2015: CAT agreed on the composition of a drafting group to draft a document summarising experiences and expectation in relation to the GLP requirements of non-clinical studies of ATMP
26 October 2015: teleconference of the drafting group to develop a draft CAT position
13 November 2015: discussion at CAT with one-month for comments to 8th December 2015

Further to the November CAT discussion, comments were received from A Grey (GLP inspector) and EMA. A drafting group telecon was organised on 8 December, and the revised CAT position was tabled in MMD. This version was further discussed at the CAT in the presence of A Grey (via telecon). There was general agreement on the proposed flexibility for GLP for ATMPs. It was agreed that a small drafting group would gather and amend the document in line with the CAT discussion. The documents will be adopted via a written procedure.
The European Commission representative mentioned that after adoption, the CAT position will be sent to the member state competent authorities responsible for clinical trials. It is expected that the document will be published on their website.

Post-meeting note: the CAT position on the Application of GLP principles to ATMPs was adopted via a written procedure on Tuesday 15 December 2015. The adopted document is included in CAT-MMD.

7.3.2. Questions and Answers on minimally manipulated ATMPs

CAT drafting group: M. Lipnik Stangelj (Rapp), P. Salmikangas (Rapp), T. Palomäki, E. Flory, M. Menezes Ferreira, P. Doevendans, M. Hrubiško

Scope: to create a Q&A document following the discussion that took place at the CAT-CHMP joint Strategic Review & Learning meeting in May 2015

Action: feedback from drafting group meeting

Note:
-16 September 2015: CAT break-out meeting of the drafting group

An oral feedback was given from the CAT drafting group of 9 December 2015. A breakout session will be organised in the margins of the January 2016 meeting to progress the drafting of this Questions and Answers document.

7.3.3. Interaction between SAWP and CAT

Scope: further areas for improvement in the CAT-SAWP workflow

Action: for discussion

Topic postponed until the January 2016 CAT meeting

7.3.4. Scientific Co-ordination Board (SciCoBo) - meeting 3rd December 2015

CAT resources: Paula Salmikangas

Action: for information

Document:
Agenda

The CAT chair provided oral feedback from the topics discussed at the SciCoBo meeting of 3 December.

Linked to one of the topics in the agenda, H Ovelgönne informed the CAT on the Strategic Review and Learning meeting (Presidency meeting) that will be organised in the Netherlands on 2-3 June 2016. CAT will meet jointly with PDCO and the Clinical Trial Facilitation Group.

7.3.5. EMA/Cancer Drug Development Forum (CDDF) workshop in 2016 on cancer immunotherapy: 'Challenges for the approval of anti-cancer immunotherapeutic drugs'

Scope: to discuss regulatory issues and the design of pivotal trials for the new immunotherapies together with a multi-stakeholder audience of regulators, industry representatives and academics.

Action: for information

Topic postponed until the January 2016 CAT meeting

7.4. Co-operation within the EU regulatory network

7.4.1. Good manufacturing practice (GMP) requirements for ATMPs

CAT drafting group members: I. Haunerova, M. Menezes-Ferreira, G. Panté, I. Reischl, P. Salmikangas, B. Sekkali, M. Timón, Jürgen Scherer, Marcel Hoefnagel, C Niederlaender

Scope: Comments received during the external consultation and next steps

Action: for information

Documents:
Minutes of the meeting that took place on 16.11.15.
Minutes of the meeting that took place on 30.11.15.

External comments
http://ec.europa.eu/health/files/advtherapies/2015_pc/publ_cons_doc_2015.pdf

Feedback was provided from the discussions in the telecons of 16 and 30 November 2015 and the plan of action to review the comments received during the external consultation. This will be a joint activity by the GMP inspectors and CAT drafting group members. Work will start in February 2016, and two teleconferences will be organised per month to progress the work. CAT will be informed of the progress at regular timepoints.

7.4.2. European Commission request for definition of: 'Principal Molecular Structural Features'

Scope: revision of the European Commission Regulation (EC) No 847/2000 of April 2000 laying down the provisions for implementation of the criteria for designation of a medicinal product as an orphan medicinal product and definitions of the concept 'similar medicinal product' and 'clinical superiority'

Action: for information

The request for the European Commission was presented. CAT will contribute for gene and cell-based ATMPs. BWP will discuss this topic at their January meeting; N. Ferry agreed to join the BWP discussion. The BWP input will be brought to the January CAT meeting.

7.4.3. Analysis of European Clinical Trials Database (EudraCT)

CAT resources: Tomáš Boráň, Margarida Menezes-Ferreira, Ilona Reischl, Paula Salmikangas, Nicolas Ferry, Romaldas Mačiulaitis, Dariusz Śladowski, Michele Lipucci di Paola, Bernd Gänsbacher

Scope: feedback from the breakout meeting of 9 December 2015

Action: for information

Note:
-16 September 2015: CAT's first break-out meeting of the drafting group
-16 October CAT: presentation and discussion of the first results of the EudraCT analysis

A brief update was given by T Boráň. The drafting group is now preparing a manuscript with the results. T Boráň will present the results of the analysis at the January CAT meeting.

7.5. Co-operation with international regulators

7.5.1. ATMP cluster teleconference with FDA and Health Canada

The teleconference will take place during the plenary meeting on Thursday 10th December from 14.00hrs – 15.00hrs

CAT resources: Paula Salmikangas

Action: for adoption

Document table:
Agenda

During the ATMP cluster teleconference, following FDA guidance documents were discussed: Draft guidance on homologous use of human cells, tissues and cellular and tissue-based product; Draft Guidance – recommendations for microbial vectors used for gene therapy.

7.5.2. International Pharmaceutical Regulators Forum (IPRF) Cell therapy group

CAT resource: Paula Salmikangas

Scope: Oral feedback from the teleconference of 7 December 2015 and request for CAT involvement in the topic 6: 'Nature and duration of patient follow-up after receiving a cell therapy product'.

Action: for discussion

Document:
Agenda

Note: Documents circulated for the IPRF meetings are available in MMD (General / International Activities / Regulators Forum cell therapy group)

Feedback was provided from the discussion at the recent IPRF cell therapy group teleconference. CAT members were asked to investigate if their NCA was aware / involved in the ongoing preparation of the ISO TC276 Biotechnology standard: some of the new proposals and preliminary work items relate to methods and requirements for gene and cell-based products.
N Ferry and P Doevendans agreed to review the document 'Nature and duration of patient follow-up after receiving a cell therapy product' and provide feedback to the CAT secretariat.

7.5.3. International Pharmaceutical Regulators Forum (IPRF) Gene therapy group

CAT resource: Paula Salmikangas

Scope: Next international teleconference will take place on 7 January 2016

Action: for information

Documents:
Draft agenda

The draft agenda for the upcoming IPRF gene therapy group telecon was presented. Feedback from the discussions will be provided at the January CAT meeting.

7.6. CAT Work Plan

7.6.1. CAT- International Society for Cellular Therapy (ISCT) Joint Workshop: 'Challenges and Opportunities for the Successful Development and Approval of Advanced Therapy Medicinal Products', Seville (Spain), 25th September 2015

CAT resources: Paula Salmikangas

http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2015/06/news_detail_002357.jsp&mid=WC0b01ac058004d5c1

Action: for information

Documents:
Presentations

Topic postponed until the January CAT meeting.

7.6.2. ATMP assessor training 2016

CAT organising committee: Margarida Menezes-Ferreira, Paula Salmikangas, Martina Schüßler-Lenz, Simona Badoi

Scope: outline of the programme and appointment of CAT moderators and presenters

Action: for discussion

Note: The ATMP assessor training is included in the CAT work plans of 2015 and 2016.

Feedback was provided to the CAT from the discussion at a recent telecon with the CAT organising Committee. It is proposed to hold a 1.5 day assessor training on 23-24 June 2016. The training will be on quality, non-clinical and clinical aspects of ATMPs, including combined ATMP; sessions on ERA review and RMP will be included.
Following moderators were appointed: for Quality session: M Menezes-Ferreira and I Reischl; Clinical session: M Schüßler-Lenz and S Badoi. CAT members interested to act as moderators for the non-clinical session should inform the CAT Secretariat.

7.6.3. Webinar on ATMP classification

Date: 11 December 2015, 13.00-14.00
Presenters: Nicolas Ferry, Belaïd Sekkali, Paula Salmikangas, Patrick Celis

Scope: this Webinar is addressed to the national authorities (NCAs) who are conducting ATMP classifications in their member state

Action: for information

CAT was reminded that they can join the webinar, which will be attended by over 90 assessors from the EU NCAs.

7.7. Planning and reporting

No items

7.8. Others

7.8.1. Talk on: 'The value of involving patients and healthcare professionals in medicines regulation', Promenade lounge (-1 floor), 10th December 2015, 13:00-14:00hrs.

Speakers invited: David Haerry - Co-Chair of the PCWP; Gonzalo Calvo - Co-Chair of the HCPWP; Bruno Sepodes - chair of the COMP and Isabelle Moulon head of EMA's Patients and Healthcare Professionals Department

Scope: panel discussion and open floor debate

Action: for information

Note:
The debate is part of a series of events that have been planned to celebrate this year's 20th anniversary of the European Medicines Agency. The talk will be video recorded and later published on the EMA website to reach a wider audience.

CAT members are encouraged to attend this panel discussion in the margins of the December 2015 CAT meeting.

The information was noted.

7.8.2. Biomedical Excellence for Safer Transfusion Collaborative (BEST), UK. – letter addressed to the CAT Chair on interference of drugs with blood group testing before transfusion

CAT resources: Paula Salmikangas

Action: for information

Document:
Letter from BEST dated 11 November 2015

The letter was included in MMD, for information.

7.8.3. Pharmaceuticals and Medical Devices Agency (PMDA) and The Japanese Society for Regenerative Medicine (JSRM): International Regulatory Forum of Human Cell Therapy and Gene therapy Products, 16th March 2016, Osaka, Japan

CAT resources: Paula Salmikangas

Action: for information

Document:
Programme

The information was noted.

8. Any other business

Date of next CAT meeting:
Thursday 21st – Friday 22nd January 2016

9. Explanatory notes

The Notes give a brief explanation of relevant agenda items and should be read in conjunction with the agenda.

Abbreviations / Acronyms

AR: Assessment report

ATMP: Advanced Therapy Medicinal Product

BWP: Biologics Working Party

CAT: Committee for Advanced Therapies

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

DG: Drafting Group

EC: European Commission

GCP: Good Clinical Practice

GLP: Good Laboratory Practice

GMP: Good Manufacturing Practice

GVP: Good Pharmacovigilance Practice

ITF: Innovative Task Force

LoOI: List of Outstanding Issues

LoQ: List of Questions

MAA: Marketing Authorisation Application

PDCO: Paediatric Committee

PIP: Paediatric Investigation Plan

PL: Package leaflet

PRAC: Pharmacovigilance and Risk Assessment Committee

RSI: Request for Supplementary Information

SA: Scientific Advice

SAG-O: Scientific Advisory Group-Oncology

SAWP: Scientific Advice Working Party

SMEs: Small and Medium-size Enterprises

SmPC: Summary of Products Characteristics

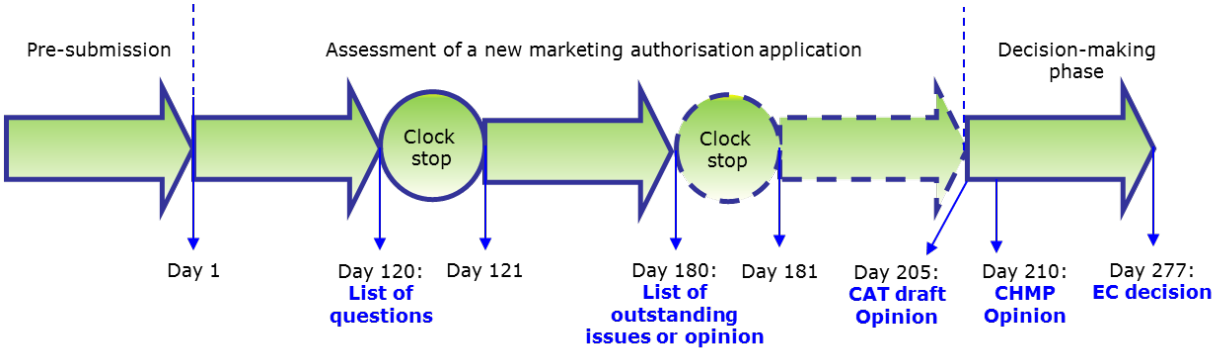
Evaluation of ATMPs (section 2)

This section lists applications for marketing authorisations of new Advanced Therapy Medicinal Products (ATMPs) that are to be discussed by the Committee. It also lists any ATMP related inspection requests (*section 2.9*) and Post-authorisation activities (*section 2.10*).

New applications (sections 2.1. to 2.12.)

Section 2.1 is for ATMPs nearing the end of the evaluation and for which the CAT is expected to adopt a draft **opinion** at this meeting on whether marketing authorisation should be granted. Once adopted, the CAT opinion is transmitted to the CHMP for final adoption. The CHMP opinion will be forwarded to the European Commission for a final legally binding decision valid throughout the EU. More information on the evaluation of ATMPs can be found here.

The other items in the section are listed depending on the stage of the evaluation, which is shown graphically below:



The assessment of an application for a new medicine takes up to 210 'active' days. This active evaluation time is interrupted by at least one 'clock-stop' during which time the applicant prepares the answers to questions from the CAT. The clock stop happens after day 120 and may also happen after day 180, when the CAT has adopted respectively a **Day 120 list of questions** (section 2.3) or a List of outstanding issues to be addressed by the company, which is listed in the agenda under sections 2.7 (**Ongoing evaluation procedures**). Section 2.7 also includes the CAT discussions at any other timepoint of the evaluation procedure of new applications.

Oral explanation (section 2.2.)

Prior to adoption of the CAT opinion, marketing authorisation applicants are normally invited to the CAT plenary meeting to address questions raised by the Committee.

Oral explanations normally relate to ongoing applications, but they can also relate to any other issue for which the CAT would like to discuss with company representatives in person.

Re-examination procedures (new applications) under article 9(2) of regulation no 726/2004 (section 2.6.)

This section lists applications for new marketing authorisation for ATMPs for which the applicant has requested a re-examination of the opinion previously issued by the CHMP. Similar to the initial evaluation of a marketing authorisation of an ATMP, CAT will adopt a draft re-examination opinion, which is transmitted to the CHMP for final adoption.

Withdrawal of applications (section 2.7.)

This section includes information on marketing authorisation applications that are withdrawn by the applicant. Applicants may decide to withdraw applications at any stage during the assessment and a CAT opinion will therefore not be issued. Withdrawals are included in the agenda for information or discussion, as necessary.

New applications (section 2.9.)

In this section, information is included on upcoming marketing authorisation applications for ATMPs, as well as information on appointment of Rapporteurs for new ATMP applications.

GMP and GCP Inspections Issues (section 2.10.)

This section lists inspections that are undertaken for ATMPs. Inspections are carried out by regulatory agencies to ensure that marketing authorisation holders comply with their obligations. Inspection can relate to good manufacturing practice (GMP), good clinical practice (GCP), good laboratory practice (GLP) or good pharmacovigilance practice (GVP).

Post-authorisation activities (section 2.12.)

This section lists type II variations, extension application according to Annex I of Reg. 1234/2008, re-examination procedures for type II variations (including extension of indication applications) for which the applicant has requested re-examination of the opinion previously issued by the CHMP and other issues concerning authorised medicines that are not covered elsewhere in the agenda such as annual reassessments, 5-year renewals, supply shortages, qualify defects. Issues that have been discussed at the previous meeting of the PRAC, the EMA's committee responsible for evaluating and monitoring safety issues for medicines, will also be included here.

Certification of ATMPs (section 3)

This section includes the scientific evaluation by the CAT of quality and non-clinical data that small and medium-sized enterprises have generated at any stage of the ATMP development process. More information on the ATMP certification procedure can be found here.

Scientific Recommendation on Classification of ATMPs (Section 4)

This section includes the scientific recommendation by the CAT on whether medicines based on genes, cells or tissues meet the scientific criteria that define ATMPs. More information on the ATMP classification procedure, including the outcomes of finalised classifications, can be found here.

Scientific Advice (section 5)

This section includes all scientific advice given to companies during the development of an ATMP. Information related to the number of ATMP related scientific advices discussed by CAT can be found in the CAT Monthly reports. Further information on SAWP can be found here.

Pre-Authorisation (section 6)

Paediatric Investigation Plan (PIP)

This section includes the discussion of an ATMP before a formal application for marketing authorisation is submitted. These cases refer for example to requests for an accelerated assessment for medicines that are of major interest for public health or can be considered a therapeutic innovation: in case of an accelerated assessment the assessment timetable is reduced from 210 to 150 days.

CAT contributes to the evaluation of a Paediatric Investigation Plan (PIPs) for ATMPs by the Paediatric Committee. These PIPs are included in this section of the Agenda.

ITF Briefing meeting in the field of ATMPs

This section refers to briefing meetings of the Innovation Task Force and International co-operations activities of the CAT

The Innovation Task Force (ITF) is a body set up to encourage early dialogue with applicants developing innovative medicines. Minutes of meetings with applicants developing ATMPs and of other ITF meetings of interest to the CAT are included in this section of the agenda. Further information on the ITF can be found here.

Organisational, regulatory and methodological matters (section 7)

This section includes topics related to regulatory and procedural guidance, CAT workplan, CAT meeting organisation (including CAT membership), planning and reporting, co-ordination with other committees, working parties and scientific advisory groups.

Furthermore, this section refers to the activities of the CAT drafting groups developing scientific guidelines for gene therapy medicinal products and for cell-based medicinal products, cooperation within the EU regulatory network and international regulators as well as direct interaction with interested parties. It also includes topics of scientific interest for the Committee that are not directly related to the work of the CAT drafting groups or CAT associated working parties.

Any other business (section 8)

This section is populated with miscellaneous topics not suitable under the previous headings.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/

List of participants

including any restrictions with respect to involvement of members / alternates / experts following evaluation of declared interests for the 10–11 December 2015 meeting.

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Paula Salmikangas	Chair	Finland	No interests declared	Full involvement
Ilona Reischl	Member	Austria	No interests declared	Full involvement
Claire Beuneu	Member	Belgium	No interests declared	Full involvement
Belaïd Sekkali	Alternate	Belgium	No interests declared	Full involvement
Rozalina Kulaksazova	Member	Bulgaria	No interests declared	Full involvement
Ivica Malnar	Alternate – replacing member	Croatia	No restrictions applicable to this meeting	Full involvement
Ivana Haunerova	Member – via TC	Czech Republic	No interests declared	Full involvement
Tomáš Boráň	Member	Czech Republic	No interests declared	Full involvement
Toivo Maimets	Member	Estonia	No interests declared	Full involvement
Tiina Palomäki	Member	Finland	No interests declared	Full involvement
Olli Tenhunen	Alternate – via TC	Finland	No restrictions applicable to this meeting	Full involvement
Nicolas Ferry	Member	France	No interests declared	Full involvement
Violaine Closson	Alternate	France	No interests declared	Full involvement
Martina Schüssler-Lenz	Member (Vice-Chair)	Germany	No interests declared	Full involvement
Asterios Tsiftsoglou	Member	Greece	No interests declared	Full involvement
Krisztian Fodor	Member	Hungary	No interests declared	Full involvement
Maura O'Donovan	Member	Ireland	No interests declared	Full involvement
Paolo Gasparini	Member	Italy	No interests declared	Full involvement
Una	Member	Latvia	No interests declared	Full involvement

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Riekstina				
Romaldas Mačiulaitis	Member (CHMP member)	Lithuania	No restrictions applicable to this meeting	Full involvement
Guy Berchem	Alternate (to CHMP representative)	Luxembourg	No restrictions applicable to this meeting	Full involvement
Anthony Samuel	Alternate (to CHMP representative)	Malta	No interests declared	Full involvement
Johannes Hendrikus Ovelgönne	Member	Netherlands	No interests declared	Full involvement
Marit Hystad	Member	Norway	No interests declared	Full involvement
Rune Kjeken	Alternate	Norway	No restrictions applicable to this meeting	Full involvement
Dariusz Śladowski	Member	Poland	No restrictions applicable to this meeting	Full involvement
Margarida Menezes-Ferreira	Alternate (to CHMP representative)	Portugal	No interests declared	Full involvement
Simona Badoi	Member	Romania	No interests declared	Full involvement
Mikuláš Hrubiško	Member	Slovakia	No restrictions applicable to this meeting	Full involvement
Metoda Lipnik-Stangelj	Member	Slovenia	No interests declared	Full involvement
Marcos Timón	Alternate (replacing CHMP co-opted member)	Spain	No interests declared	Full involvement
Lennart Åkerblom	Member	Sweden	No interests declared	Full involvement
Christiane Niederlaender	Member	United Kingdom	No interests declared	Full involvement
Pieter Doevendans	Member	Healthcare Professionals' Representative	No interests declared	Full involvement
Esteve Trias-	Alternate	Healthcare	No interests declared	Full involvement

Name	Role	Member state or affiliation	Outcome restriction following evaluation of e-DoI	Topics on agenda for which restrictions apply
Adroher		Professionals' Representative		
Bernd Gänsbacher	Member	Healthcare Professionals' Representative	No interests declared	Full involvement
Michelino Lipucci di Paola	Member	Patients' Representative	No restrictions applicable to this meeting	Full involvement
Kieran Breen	Member	Patients' Representative	No restrictions applicable to this meeting	Full involvement
Guido Panté	Expert - in person*	Italy	No interests declared	Full involvement
Christos Sotirelis	Expert - in person*	UK	No interests declared	Full involvement
Wiebke Hop pensack	Expert - via telephone*	Germany	No restrictions applicable to this meeting	Full involvement
Taina Methuen	Expert - via telephone*	Finland	No interests declared	Full involvement
Outi Maki-Ikola	Expert - via telephone*	Finland	No interests declared	Full involvement
Andrew Gray	Expert - via telephone*	UK	No interests declared	Full involvement
A representative from the European Commission attended the meeting				
Meeting run with support from relevant EMA staff				

* Experts were only evaluated against the agenda topics or activities they participated in.