15 June 2020
EMA/CAT/326151/2020
Human Medicines Division

Committee for Advanced Therapies (CAT)

Agenda for the meeting on 17-19 June 2020

Chair: Martina Schüßler-Lenz; Vice-Chair: Ilona Reischl

17 June 2020, 14:00 – 18:00, virtual meeting

18 June 2020, 09:00 – 13:00, virtual meeting

19 June 2020, 09:00 – 12:00, virtual meeting

Disclaimers

Some of the information contained in this agenda 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, this agenda is a working document primarily designed for CAT members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the agenda 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).

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

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1. Introduction

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

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the CAT plenary session to be held 17-19 June 2020. See June 2020 CAT minutes (to be published post-July 2020 CAT meeting).

1.2. Adoption of agenda

CAT agenda for 17-19 June 2020 meeting

1.3. Adoption of the minutes

CAT minutes for 18-20 May 2020 meeting

2. Evaluation of ATMPs

2.1. Opinions

No items

2.2. Oral explanations

No items

2.3. Day 180 list of outstanding issues

No items

2.4. Day 120 list of questions

No items

2.5. Day 80 assessment reports

No items

2.6. Update on ongoing initial applications

No items

2.7. New applications

2.7.1. Idecabtagene vicleucel - Orphan - EMEA/H/C/004662

Accelerated assessment

Celgene Europe BV; treatment of multiple myeloma

Scope: timetable for assessment

Action: for adoption

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 - variation of therapeutic indication procedure according to Commission Regulation (EC) No 1234/2008

2.11.1. Kymriah - tisagenlecleucel - Orphan - EMEA/H/C/004090/II/0022/G

Novartis Europharm Limited

Rapporteur: Rune Kjeken

Scope: quality: Opinion

Action: for adoption

2.11.2. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/II/0015

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus

Scope: quality: Opinion.

Action: for adoption

Request for Supplementary Information adopted on 24.04.2020, 21.02.2020.

2.12. Extension applications

No items

2.13. Other Post-Authorisation Activities

2.13.1. Imlygic - talimogene laherparepvec - EMEA/H/C/002771/ANX/001.1

Amgen Europe B.V.

Rapporteur: Olli Tenhunen, CHMP Coordinator: Tuomo Lapveteläinen

Scope: clinical

From initial MAA:
The MAH should submit the preliminary results of Study 20120325 (a phase 2, multicenter, open-label, single-arm trial to evaluate the correlation between objective response rate and baseline intratumoral CD8+T-lymphocyte density in subjects with unresected stage IIIB to IVM1c melanoma treated with talimogene laherparepvec). Interim report

Action: for adoption

2.13.2. Yescarta - axicabtagene ciloleucel - Orphan - EMEA/H/C/004480/REC/009

Kite Pharma EU B.V.

Rapporteur: Jan Mueller-Berghaus, CHMP Coordinator: Jan Mueller-Berghaus

Scope: quality

Action: for adoption

3. Certification of ATMPs

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

3.1. Opinion

No items

3.2. Day 60 Evaluation Reports

No items

3.3. New Applications

No items

4. Scientific Recommendation on Classification of ATMPs

Timetable:

-Start of the procedure:	19.06.2020
-Draft CAT co-ordinator's report:	03.07.2020
-ITF peer-review comments:	08.07.2020
-Revised scientific recommendation:	10.07.2020
-Adoption of scientific recommendation by CAT:	17.07.2020

4.1. New requests – Appointment of CAT Coordinator

4.1.1. Recombinant adeno-associated viral vector (serotype 8) carrying an optimised gene for human cyclic nucleotide gated channel subunit alpha 3 (CNGA3) protein

Intended for the treatment of achromatopsia caused by mutations in the CNGA3 gene

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.2. Irradiated allogeneic induced-pluripotent stem cells expressing pluripotent genes and cancer-specific embryonic neo-antigens

Intended for the treatment malignant solid tumours including all epithelial cancers in sub-group type harbouring a stemness mesenchymal-like signature and haematopoietic malignancies

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.3. Autologous naïve regulatory T cells transduced with a lentiviral vector encoding for a Chimeric Antigen Receptor (CAR) to recognize the HLA-A*02 antigen

Intended for the prevention of immune-mediated graft rejection in HLA-A*02 mismatched renal transplantation

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.1.4. Live-attenuated, genetically modified Mycobacterium bovis expressing the gene coding for listeriolysin from Listeria monocytogenes

Intended for treatment of non-muscle invasive bladder cancer

Scope: appointment of CAT Coordinator and adoption of timetable

Action: for adoption

4.2. Day 30 ATMP scientific recommendation

4.2.1. Allogeneic CD34+-enhanced cell suspension derived from umbilical cord blood

Intended for the treatment of patients with inherited metabolic disorders [cerebral adrenoleukodystrophy, Hurler syndrome] where haematopoietic stem cell transplant is indicated

Scope: ATMP scientific recommendation

Action: for adoption

4.2.2. Aggregates of defined size of human embryonic stem cell derived insulin secreting pancreatic beta cells, encapsulated within an encapsulation device

Intended for the treatment of type I diabetes mellitus

Scope: ATMP scientific recommendation

Action: for adoption

4.2.3. Homogenate of antlerogenic stem cells

Intended for the treatment of chronic obstructive pulmonary disease, bronchial asthma

Scope: ATMP scientific recommendation

Action: for adoption

4.2.4. Autologous adipose-derived mesenchymal stem cells, cartilage lesions

Intended for the treatment of cartilage lesions

Scope: ATMP scientific recommendation

Action: for adoption

4.2.5. Wharton's jelly derived mesenchymal cells myelitis

Intended for the treatment of myelitis

Scope: ATMP scientific recommendation

Action: for adoption

4.2.6. Wharton's jelly derived mesenchymal cells meningitis

Intended for the treatment of meningitis

Scope: ATMP scientific recommendation

Action: for adoption

4.2.7. Wharton's jelly derived mesenchymal cells, meningomyelocele

Intended for the treatment of meningomyelocele, myelomeningocele, spina bifida

Scope: ATMP scientific recommendation

Action: for adoption

4.2.8. Wharton's jelly derived mesenchymal cells, cerebellum syndrome

Intended for the treatment of cerebellum syndrome

Scope: ATMP scientific recommendation

Action: for adoption

4.2.9. Wharton's jelly derived mesenchymal cells, encephalitis

Intended for the treatment of encephalitis

Scope: ATMP scientific recommendation

Action: for adoption

4.2.10. Wharton's jelly derived mesenchymal cells, Krabbe disease

Intended for the treatment of Globoid cell leukodystrophy (Krabbe disease)

Scope: ATMP scientific recommendation

Action: for adoption

4.2.11. Wharton's jelly derived mesenchymal cells, osteoarthritis

Intended for the treatment of osteoarthritis

Scope: ATMP scientific recommendation

Action: for adoption

4.2.12. Wharton's jelly derived mesenchymal cells, spinal and bulbar muscular atrophy

Intended for the treatment of spinal and bulbar muscular atrophy

Scope: ATMP scientific recommendation

Action: for adoption

4.3. Day 60 revised scientific recommendation (following list of questions)

4.3.1. Autologous adipose-derived mesenchymal stem cell, diabetic foot syndrome

Intended for the treatment of diabetic foot syndrome

Scope: awaiting responses from the applicant to the LoQs. Revised ATMP scientific recommendation

Action: for adoption

4.4. Finalisation of procedure

4.4.1. Recombinant adeno-associated viral vector (serotype 8) carrying an optimised gene for human cyclic nucleotide gated channel subunit beta 3 (CNGB3) protein - H0005013

Intended for the treatment of achromatopsia caused by mutations in the CNGB3 gene

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

4.4.2. Genetically modified Lactococcus lactis strain , engineered to secrete human pro-insulin and human IL-10 - H0005671

Intended for the treatment of clinical recent-onset Type 1 diabetes mellitus

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

4.4.3. Autologous CD34+ cells transduced with a lentiviral vector encoding a modified γ-globin gene - H0005672

Intended for the treatment of sickle cell disease (SCD) and β -thalassemia

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

4.4.4. Human autologous hematopoietic stem cells transduced with a lentiviral vector containing codon-optimized cDNA encoding for functional human alpha galactosidase

Intended for the treatment of Fabry disease

Scope: comments received from the European Commission. Final ATMP scientific recommendation

Action: for discussion

4.4.5. Human autologous hematopoietic stem cells transduced with a lentiviral vector containing codon-optimized cDNA encoding for functional human glucocerebrosidase

Intended for the treatment of Gaucher disease

Scope: comments received by the European Commission. Final ATMP scientific recommendation

Action: for discussion

4.4.6. Wharton's jelly derived mesenchymal cells – H0005673

Intended for the treatment of patients with COVID-19 infections

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

4.4.7. Wharton's jelly derived mesenchymal stem cell, COVID-19 - H0005674

Intended for the treatment of patients with COVID-19 infections

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

4.4.8. Wharton's jelly derived mesenchymal stem cell, optic atrophy

Intended for the treatment of optic atrophy

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

4.4.9. Wharton's jelly derived mesenchymal stem cell, IFAP syndrome

Intended for the treatment of patients with Ichthyosis follicularis with alopecia and photophobia (IFAP) syndrome

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

4.4.10. Wharton's jelly derived mesenchymal stem cell, bone marrow transplant rejection

Intended for the treatment of bone marrow transplant rejection

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

4.4.11. Wharton's jelly derived mesenchymal stem cell, secondary graft failure

Intended for the treatment of secondary bone marrow transplant failure/secondary graft failure

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

4.4.12. Wharton's jelly derived mesenchymal stem cell, progressive supranuclear palsy

Intended for the treatment of progressive supranuclear palsy

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

4.4.13. Wharton's jelly derived mesenchymal stem cell, multiple system atrophy

Intended for the treatment of multiple system atrophy

Scope: the European Commission raised no comments. ATMP scientific recommendation

Action: for information

5. Scientific Advice

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 Rapporteurs

Timetable:
-Final Briefing Package: 28.08.2020
-Start of the procedure at SAWP: 03.07.2020
-CAT report due by: 08.07.2020
-CAT recommendation: 17.07.2020

5.2. CAT reports

5.3. List of Issues

5.4. Finalisation of SA procedures

6. Pre-Authorisation Activities

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

No items

6.2. ITF briefing meetings in the field of ATMPs

6.3. Priority Medicines (PRIME) – Eligibility requests

6.3.1. Month 0 - Start of the procedure

Timetable for assessment:

Procedure start:	11.06.2020
SAWP recommendation:	09.07.2020
CAT recommendation:	17.07.2020
CHMP adoption of report and final recommendation:	23.07.2020

6.3.2. Month 1 – Discussion of eligibility

6.3.3. Month 2 – Recommendation of eligibility

6.3.4. Ongoing support

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the CAT

7.1.1. CAT membership

Sweden: Maria Lüttgen – membership mandate to end on 15 June 2020

Sweden: Björn Carlsson – membership mandate to end on 14 June 2020

Action: for information

7.2. Coordination with EMA Scientific Committees

No items

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

7.3.1. Guideline on registry-based studies – consultation of committees

Scope: introduction of the guideline to committees' members in advance of the written consultation planned in June-July 2020 (deadline: 31 July 2020)

Action: For information

7.3.2. Scientific advice for ATMPs

Scope: new procedure for providing CAT input to SAWP on scientific advices for ATMPs

Action: for discussion

Note: CAT members can provide comments on the proposed new procedure by 12 June 2020 (comments to: CATsecretariat@ema.europa.eu). A formal discussion will be scheduled for the June CAT meeting.

7.3.3. Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)

Scope: meeting Summary from the PCWP/HCPWP joint meeting, held on 3-4 March 2020

Action: for information

7.4. Cooperation within the EU regulatory network

7.4.1. European Union Network-Pharmacovigilance Oversight Group (EU-POG)

Scope: call for nomination of a new CAT member to join the EU-POG. Mandate of the EU-POG

CAT: Martina Schüssler-Lenz

Action: for nomination of a CAT member

Note: former CAT member was Corina Spreitzer who resigned in March 2020

7.4.2. Commission initiative related to GMO for medicinal products

Scope: oral feedback from the European Commission

Action: for information

7.5. Cooperation with international regulators

7.5.1. ATMP cluster teleconference with FDA-USA, Health Canada and PMDA-Japan

CAT: Martina Schüssler-Lenz, Carla Herberts, Ilona Reischl, Pille Säälik

Scope: feedback on the teleconference that took place on 30 April 2020

Action: for information

7.5.2. ATMP cluster teleconference with FDA-USA, Health Canada and PMDA-Japan

CAT: Martina Schüssler-Lenz

Scope: agenda for the teleconference to take place on 25 June 2020

Action: for discussion

7.6. CAT work plan

None

7.7. Planning and reporting

7.7.1. Planning estimates of forthcoming ATMP MAAs

Scope: Q2/2020 update of the business pipeline report for the human scientific committees

Action: for information

7.8. Others

None

8. Any other business

8.1. Participation of CAT members/alternates as speakers or panellist to international conferences

Scope: criteria for participation to international conferences

Action: for discussion

8.2. American Society of Gene & Cell Therapy (ASGCT)'s annual meeting, 11th May 2020, Boston MS (USA)

CAT: Jan Mueller-Berghaus

Scope: feedback on the presentation at the ASGCT pre-meeting workshop: 'Commercialization I Workshop'

Action: for information

Date of next CAT meeting:

15-17/07/2020

9. Explanatory notes

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

Abbreviations / Acronyms

AAV: Adeno-Associated Virus

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

CTFG: Clinical Trial Facilitation Group

DG: Drafting Group

EC: European Commission

EU NTC: European Union Network Training Centre

ERA: Environmental Risk Assessment

FDA: Food and Drug Administration

FL: Final Letter

GCG: Guideline Consistency Group

GCP: Good Clinical Practice

GLP: Good Laboratory Practice

GMO: Genetically modified organism

GMP: Good Manufacturing Practice

GTMP: Gene Therapy Medicinal Product

HTA: Health Technology Assessment Bodies

HSPC: Hematopoietic Stem and Progenitor Cells

ITF: Innovative Task Force

JR: Joint Report

LoOI: List of outstanding issues

LoQ: List of questions

MA: Marketing Authorisation

MAA: Marketing Authorisation Application

MAH: Marketing Authorisation Holder

MSC: Mesenchymal stem cells

PDCO: Paediatric Committee

PMDA: Pharmaceuticals and Medical Devices Agency (Japan)

PIP: Paediatric Investigation Plan

PL: Package leaflet

PRAC: Pharmacovigilance and Risk Assessment Committee #

PRIME: Priority Medicines

RMP: Risk Management Plan

RP: Reflection paper

RSI: Request for supplementary information

SAs: Scientific Advices

SAG-O: Scientific Advisory Group Oncology

SAWP: Scientific Advice Working Party

SR: Summary Report

SWP: Scientific Working Party

SME: Small and medium size enterprises

SmPC: Summary of Products Characteristics

TT: Timetable

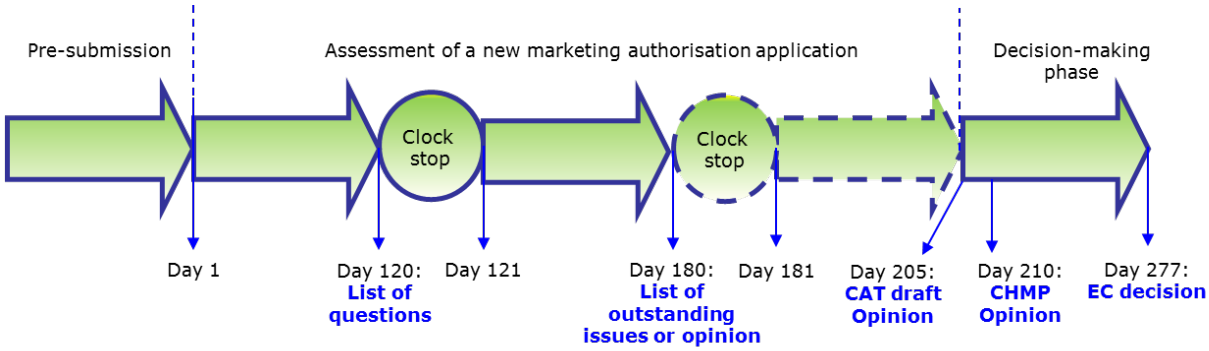
Evaluation of ATMPs (section 2)

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.

Priority Medicines (PRIME)

This section includes the new requests for eligibility to PRIME for ATMPs under development, the discussions in CAT of these eligibility requests and the final recommendations for eligibility of ATMPs adopted by CHMP.

CAT will appoint one of its members as the CAT sponsor for each new ATMP eligibility request who will lead the CAT discussion based on the recommendation from the SAWP.

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/