EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16th January 2014
EMA/CAT/27142/2014 rev. 3
Patient Health Protection

Committee for Advanced Therapies (CAT)

Draft Agenda of the 16th – 17th January 2014 meeting

Chair: vacant, Vice-chair: Paula Salmikangas

16th January 2014, 09:00hrs – 13:00hrs, Virtual
17th January 2014, 09:00hrs – 13:00hrs, Virtual

Declaration on conflict of interest

In accordance with the Agency's revised policy and procedure on the handling of conflicts of interests, participants in this meeting are asked to declare any conflict of interests on the matters for discussion (in particular any changes, omissions or errors to the already declared interests). Discussions, deliberations and voting will take place in full respect of the restricted involvement of Scientific Committee members and, where relevant, experts attending the plenary meeting, as announced by the Scientific Committee Secretariat at the start of meeting.

Health & 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 this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regards to therapeutic indications listed against products, it must be noted that these may not reflect the full wording proposed by the applicant and may also vary during the course of the review. The procedures discussed at CAT are on-going and therefore certain aspects are considered confidential. Additional details on some of the procedures (for example the ATMP classification procedure) will be published in the CAT monthly report. For orphan medicinal products the product name and the applicant are published to be consistent with already publicly available information. Documents mentioned in the agenda cannot be released at present as they are currently in draft format or are classified as confidential. They will become public when adopted in their final form or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006). Of note, this agenda is a working document primarily designed for CAT members and the work the Committee undertakes. Further information with relevant explanatory notes can be found at the end of this document.

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1. PLENARY RELATED DOCUMENTS

1.1. AGENDA (EMA/CAT/737884/2013) and **TIMESCHEDULE** (EMA/CAT/13248/2014) for the CAT plenary to be held on 16th and 17th January 2014: **for adoption**

1.2. TABLE OF DECISIONS CAT plenary held on 12th and 13th December 2013 (EMA/CAT/792076/2013): **for information**

1.3. MINUTES of the CAT plenary held on 12th and 13th December 2013 (EMA/CAT/ 800897/2013): **for adoption**

1.4. 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 of 16th – 17th January 2014: **for information**

See January 2014 minutes (to be published post February 2014 CAT meeting)

2. EVALUATION OF ATMPS

2.1. OPINION

No items on the agenda

2.2. ORAL EXPLANATION

No items on the agenda

2.3. LIST OF QUESTIONS

No items on the agenda

2.4. DAY 80 ASSESSMENT REPORT

No items on the agenda

2.5. RE-EXAMINATION PROCEDURE (NEW APPLICATIONS)+UNDER ARTICLE 9(2) OF REGULATION No 726/2004

No items on the agenda

2.6. WITHDRAWAL OF APPLICATION

No items on the agenda

2.7. NEW APPLICATIONS

2.7.1. (allogeneic human heterologous liver cells) (EMA/H/C/003750). Therapeutic indication: Treatment of urea cycle disorders.
For adoption:
- Timetable

2.8. PRE-SUBMISSION ISSUES

No items on the agenda

2.9. ONGOING EVALUATION PROCEDURES

No items on the agenda

2.10. PAEDIATRIC INVESTIGATION PLAN

No items on the agenda

2.11. GMP and GCP INSPECTIONS REQUESTS

No items on the agenda

2.12. VARIATIONS

2.12.1. Type II Variations

2.12.1.1. Glybera (EMEA/H/C/002145) MAH: UniQure Biopharma B.V. Orphan

II/05 (quality)
Scope:
For adoption:
- Extension of timetable

II/29 (quality)
Scope:
For adoption:
- Opinion

II/30 (clinical)
Scope: Update of Protocol for the CM efficacy and safety study requested in the Annex II
For adoption:
- Timetable

CAT Rapporteur: Elaine French
CHMP Co-ordinator: Greg Markey

2.12.2. Other Post-Authorisation Activities

2.12.2.1. Glybera (alipogene tiparvovec) (EMEA/H/C/2145) MAH: UniQure Biopharma B.V. Orphan. Annual Reassessment
For adoption:
- Draft opinion or RSI

CAT Rapporteur: Elaine French
CHMP Co-ordinator: Greg Markey

2.12.2.2. PROVENGE (autologous peripheral blood mononuclear cells activated with pap-gm-csf) (EMA/H/C/2513). MAH: Dendreon UK Ltd.
Scope MEA 009: Re-evaluate the CD54 up-regulation acceptance criterion, based on quality and clinical data from patient batches manufactured in Europe, when sufficient data is available.
For adoption:
- Timetable

CAT Rapporteur: Egbert Flory
CHMP Co-ordinator: Jan Müller-Berghaus

3. CERTIFICATION

No items on the agenda

4. SCIENTIFIC RECOMMENDATION ON CLASSIFICATION OF ATMPs

4.1. [a suspension of allogeneic unrelated, buffy coat derived activated viable leukocytes]. Proposed indication: treatment of chronic lower extremity ulcers in adult diabetic patients
For adoption:
- Revised ATMP Classification report

Comments received from the European Commission

4.2. [Cultured autologous skin substitute using acellular human donor dermis as matrix]. . Proposed indication: wound healing **For information:** ▪ ATMP Classification report	*The European Commission raised no comments*
4.3. [a cell suspension of autologous skeletal myoblast]. . Proposed indication: oculo-pharyngeal muscular dystrophy **For information:** ▪ ATMP Classification report	*The European Commission raised no comments*
4.4. [Nuclear fraction separated from autologous bone marrow aspirate]. Proposed indication: stage I-III of osteoarthrosis and osteochondral lesion **For discussion:** ▪ Response to the list of issues **For adoption:** ▪ Revised ATMP Classification report	
4.5. [autologous *ex vivo* expanded leukocytes treated with 5-aza-2'-deoxycytidine]. Proposed indication: solid tumours. **For adoption:** ▪ ATMP Classification report	

5. SCIENTIFIC ADVICE

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

6. ORPHAN DRUG DESIGNATION

6.1. Committee for Orphan Medicinal Products (COMP) **For information:** ▪ Agenda for the meeting 7th–8th January 2014	COMP Secretariat

7. OTHER TASKS OF THE CAT

7.1. ITF Briefing Meetings in the field of ATMPs

No items on the agenda

7.2. Other ITF Briefing Meetings of interest to CAT

No items on the agenda

7.3. International Co-operation

No items on the agenda

8. ELIGIBILITY AS ATMP AND RAPPORTEURSHIP

No items on the agenda

9. ORGANISATIONAL MATTERS

9.1. Regulatory and Procedural Guidance

9.2. CAT Meeting Organisation

9.2.1. CAT/PDCO joint informal meeting hosted by the Heads of the Italian and Slovenian agencies, held on 25th-26th November 2013: **for information**

9.2.2. Election of Chairperson to CAT. Call for nomination

For information:

- Timelines

Note: the nomination letter shall include a mission statement in support of the candidature

Timetable:

-Call for election with supporting documents to be sent out by CAT Secretariat: mid-Jan. 2014

-Deadline for receipt of candidatures: 12.02.14.

-Election: first agenda item at the CAT February meeting: 13.02.14.

9.2.3. CAT Membership

For information:

- UK: Elaine French – new member nominated on 3rd January 2014

9.2.4. Principles for publication of agendas and minutes of EMA scientific committees: **for information**

9.3. Co-ordination with Committees/WPs/SAGs/other groups

9.3.1. CHMP December 2013 ToD: for information

9.4. CAT interaction with Interested Parties

9.5. CAT Work Programme

9.5.1. Satellite CAT scientific workshop in the margins of the World Conference on Regenerative Medicine held in Leipzig (Germany) on 23-25 October 2013

For information:

- Feedback on the workshop

10. CAT DGs/OTHER GROUPS

10.1. GTMP Guidelines

10.2. Guidelines for CTMP and TEP

10.2.1. Reflection paper on clinical aspects related to TEPs: **for discussion**

Timetable:

One month reflection: 12.02.14.

Adoption by CAT: 14.02.14.

10.3. EMA/CAT-NB Collaboration Group
10.4. PCWP guidelines
10.5. Healthcare Professionals WP

11. OTHER SCIENTIFIC GUIDELINES/ISSUES

11.1. European Clinical Trials Framework. Regulation of the EP and the Council on clinical trials on medicinal products for human use and transparency initiatives

For information:

- Published agreed text

12. PHARMACOVIGILANCE

13. A.O.B.

Date of next CAT meeting:
Thursday 13th – Friday 14th February 2014

Explanatory notes

The notes below give a brief explanation of the main sections and headings in the CAT agenda and should be read in conjunction with the agenda or the minutes.

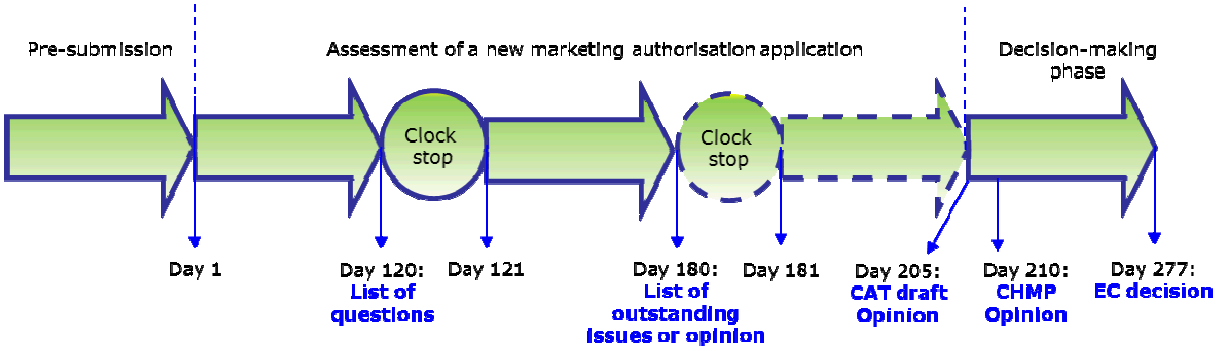
Evaluation of ATMPs (section 2)

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 the Paediatric Investigation Plans for ATMPs discussed by the Committee (*section 2.10*), any ATMP related inspection requests (*section 2.11*) and Post-authorisation activities (*section 2.12*)

New applications (*sections 2.1 to 2.9*)

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 2.9 (**Ongoing evaluation procedures**). Section 2.9 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 2.5*)

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 2.6*)

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 2.7*)

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.

Pre-submission (*section 2.8*)

In some cases the CAT may discuss an ATMPs 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.

Paediatric investigation Plans (*section 2.10*)

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.

Inspections Issues (*section 2.11*)

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.

ATMP Certification (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.

Orphan Drug Designation (section 6)

This section refers to the report from the Committee for Orphan Medicinal Products (COMP).

Other Tasks of the CAT (Section 7)

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 matters (section 9)

This section includes topics related to Regulatory and Procedural Guidance, CAT meeting organisation (including CAT membership) and Co-ordination with other Committees, Working Parties, Scientific Advisory Groups and other groups.

CAT Drafting groups / Other Groups (section 10)

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, the EMA/CAT-Notified Body Collaboration Group, the Patient and Consumer Working Party (PCWP) and the Healthcare Professionals Working Party (HCPWP).

Other Scientific Guidelines/issues (section 11)

This section 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.

Pharmacovigilance (section 12)

Any non-product related Pharmacovigilance issue coming from the discussion of the PRAC will be listed here. PRAC issues related to ATMPs are included in section 2.12.