EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

15 January 2015
EMA/CAT/781788/2014
Procedure Management and Business Support Division

Committee for Advanced Therapies (CAT)

Agenda for the meeting on 15–16 January 2015

Chair: Martina Schüßler-Lenz

15 January 2015, 11:00hrs – 18:30hrs, room 3-E
16 January 2015, 09:00hrs – 15:00hrs, room 3-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 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 monthly reports once the procedures are finalised and start of referrals will also be available.

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).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Table of contents

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 15-16 January 2015.

See January 2015 minutes (to be published post February 2015 CAT meeting)

1.2. Adoption of agenda of the meeting of 15-16 January 2015

1.3. Adoption of the minutes of the previous CAT meeting on 11-12 December 2014

1.4. Table of Decisions of the previous CAT meeting on 11-12 December

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 Outstanding Issues

No items on the agenda

2.4. List of Questions

2.4.1. (talimogene laherparepvec) (EMA/H/C/H0002771). Therapeutic indication: treatment of adults with melanoma that is regionally or distantly metastatic

For discussion:

- Proposal to convene a SAG meeting (oncology)
- Proposed questions to SAG
- Request for nomination of expert to attend the SAG
- BWP report

For adoption:

- Draft list of questions
- Response timetable

-The SAWP gave SA in 2008 and 2013
-The CAT issued a classification as a gene therapy medicinal product in July 2012

2.5. Day 80 Assessment Report

No items on the agenda

2.6. Re-Examination Procedure (New Application)+Under Article 9(2) of Regulation No. 726/2004

No items on the agenda

2.7. Withdrawal of Application

No items on the agenda

2.8. Ongoing Evaluation Procedures

No items on the agenda

2.9. New Applications

No items on the agenda

2.10. GMP and GCP Inspections Requests

No items on the agenda

2.11. Post-Authorisation

2.11.1. Type II Variations

To be completed

2.11.2. Other PA Activities

2.11.2.1.	**Glybera** (EMEA/H/C/002145/S/0039), (alipogene tiparvovec), MAH: uniQure biopharma B.V. *Orphan*. Second Annual Reassessment **For adoption:** ▪ Draft Opinion/ RSI	CAT Rapporteur: CHMP Co-ordinator: PRAC Rapporteur:

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.

4. Scientific Recommendation on Classification of ATMPs

4.1.	[solid flexible implant with chondrocytes fixed in biodegradable human origin fibrin based excipient]. Proposed indication: intended for the treatment of focal non-arthrotic cartilage defects of Outerbridge Grade III or IV of the femoral condyle including the trochlea **For information**: ▪ ATMP Classification report	*The European Commission raised no comments*

4.2. [adipose-derived mesenchymal stem cells]. Proposed indication: intended for the treatment of autoimmune diseases. **For adoption**: ▪ ATMP Classification report	
4.3. [Tumour-infiltrating lymphocytes derived from metastatic melanoma]. Proposed indication: intended for the treatment of metastatic melanoma **For adoption**: ▪ ATMP Classification report	
4.4. [human extracellular matrix on a absorbable polymer matrix]. Proposed indication: intended for the surgical/ interventional treatment of congenital heart malformations **For adoption**: ▪ ATMP Classification report	
4.5. [adult human bone-marrow derived, *ex-vivo* expanded, pooled allogeneic mesenchymal stromal cells]. Proposed indication: intended for thromboangiitis obliterans (Buerger's disease) **For information:** ▪ Request received on 22.12.14. **For adoption:** ▪ Appointment of CAT Co-ordinator ▪ Timetable	
4.6. Reflection Paper on Classification of ATMPs **For information:** ▪ Update on the activities of the Drafting groups	Following CAT members will take part in the review of the comments received: - Drafting group on substantial manipulation: - Drafting group on non-homologous use

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.

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 Plan (PIP)

7. ITF Briefing Meetings in the field 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.

8. Organisational Matters

8.1. Regulatory and Procedural Guidance

8.1.1.	Application of ATMP Regulation **For discussion:** ▪ Oral feedback from the joint telecon of the CAT reflection groups on quality-related issues and risk based approach ▪ Next steps	CAT reflection groups: - Quality related issues: - Risk based approach:
8.1.2.	Development of GMP requirements for investigational ATMPs **For discussion** ▪ First draft or oral feedback from DG	CAT drafting group members:
8.1.3.	Draft Guidance on meetings with applicants on responses to questions received from EMA Scientific Committees during the evaluation within the centralised procedure **For re-adoption:** ▪ Revised guidance document	Committees drafting group members:

8.2. CAT Meeting Organisation

8.2.1.	CAT/CHMP/COMP joint informal meeting that took place in Rome in October 2014 under the auspices of the Italian Presidency of the Council of the European Union **For information:** ▪ Minutes	*Postponed to February 2015*
8.2.2.	Revised Declaration of Interest form. Submission before end of January 2015: **for action**	Note: Reference is made to the EMA's Chief Policy Adviser's presentation in June 2014 on the revised policy on Conflict of Interest.
8.2.3.	CAT membership **For information:** ▪ IT – Luca Sangiorgi – new alternate nominated on 30th December 2014	
8.2.4.	Training to CAT members on the electronic voting equipment: for information	AudioVisual Team

8.3. Co-ordination with Committees/WPs/SAGs

8.3.1.	CHMP December 2014 ToD: **for information**	

8.4. CAT's Workplan

8.4.1. Joint CAT/ISCT workshop/satellite meeting to take place in the margins of the European Meeting of the ISCT to be held on 24-26 September 2015, Seville (Spain): '*What should and can we do to make cellular therapies that bring value to patients available to these patients as soon as possible?'*

For information:
- Draft Workplan 2015-2016

For agreement:
- Appointment of CAT participants for the programme committee

For discussion:
- Topics for the agenda

This relates to Topic 4 in the CAT Workplan 2015-2016: 'Provide assistance to ATMP developers via the organisation of a scientific workshop in collaboration with a scientific society'.

CAT topic leader: Paula Salmikangas

Other Committee participants: Dariusz Sladowski, Tiina Palomäki, Martina Schüßler-Lenz

First telecon of the programme Committee:
- Monday 26.01 at 17.00 or 18.00 CET (16.00 or 17.00 UK time)
- Wednesday 28.01 at 16.00 CET (15.00 UK time)

8.5. Interested Parties to CAT

8.5.1. CAT meeting with Interested Parties

For adoption:
- Report of the meeting of 11th December 2014

9. CAT's DGs / PCWP and HCPWP

9.1. DG on GTMP Guidelines

9.1.1. Guideline on the quality, non-clinical and clinical aspects of gene therapy medicinal products

For discussion:
- Comments by the Guideline Consistency Group (GCG)

9.2. DG on CTMP and TEP Guidelines

No items on the agenda

9.3. PCWP and HCPWP

10. Other Scientific Topics

10.1. Council of Europe – Guide to the Quality and Safety of Tissues and Cells for Human Application, second edition

For information:
- Letter from EDQM to CAT chair dated 11th December 2014
- Draft Guide, published for external consultation
- Form to provide comments to the Tissue and Cell Guide, 2nd edition

CAT has provided comments to EDQM/Council of Europe on 18 November 2014.

The Draft Guide has now been published for external consultation until 31 January 2015.

10.2. Draft INN naming scheme for cell therapy products **For discussion** : ▪ INN scheme ▪ Comments by CAT and BWP	Note: the draft has been developed by the WHO INN secretariat in collaboration with the INN expert group.
10.3. European Directorate for the Quality of Medicines & HealthCare (EDQM). Meeting of the Advisory Group of the Official Control Authority Batch Release (OCABR) Network for Human Biologicals which took place in October 2014, Strasbourg. **For information:** ▪ Letter to the CAT from the OCABR dated 19th November 2014 on the outcome of their discussion on *'Batch release requirements for human blood and plasma derived excipients used in ATMPs'* ▪ Annex IIf	
10.4. Public Consultation on the preliminary opinion on Synthetic Biology II. Risk assessment methodologies and safety aspects: **for comments**	http://ec.europa.eu/health/scientific_committees/consultations/public_consultations/scenihr_consultation_26_en.htm Please provide comments to CAT secretariat by 2 February 2015.
10.5. International Pharmaceutical Regulators Forum (IPRF) – Gene Therapy Working Group. In-person meeting in the margins of the ASGCT Annual Meeting (New Orleans, Louisiana, U.S.A., 13-16 May 2015). **For discussion:** ▪ CAT participation	
10.6. Joint meeting between Competent Authorities for tissues and cells / medicines and CAT, to take place in first half of 2015 **For discussion:** ▪ Draft agenda	

11. Any Other Business

Date of next CAT meeting:
Thursday 19th – Friday 20th February 2015

Explanatory notes

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

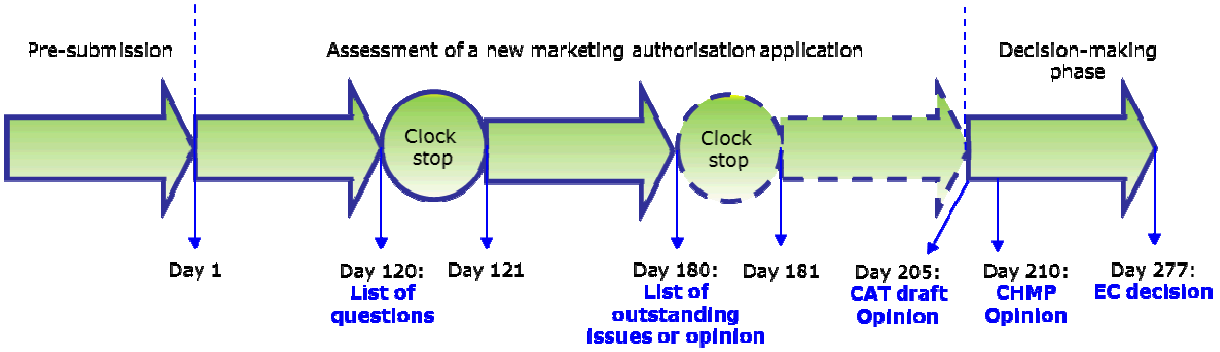
Evaluation of ATMPs (section 2)

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 2.9*)

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 2.7 (**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.

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 2.11.*)

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.

Pre-Authorisation (section 6)

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 (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 8)

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's DGs / PCWP and HCPWP (section 9)

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 Topics (section 10)

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.

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