EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

13th February 2014
EMA/CAT/89478/2014 rev. 3
Procedure Management and Business Support Division

Committee for Advanced Therapies (CAT)

Agenda of the 13th – 14th February 2014 meeting

Chair: vacant, Vice-chair: Paula Salmikangas

13th February 2014, 13:00hrs – 18:30hrs, Room 3A
14th February 2014, 09:00hrs – 15:00hrs, Room 3A

Election of CAT Chairperson. Call for nomination

For information:
Procedure for the election of CAT Chair

For discussion:
Nominations received:

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

Timetable:
-Deadline for receipt of candidatures: 12.02.14.
-Election: first agenda item at the CAT February meeting: 13.02.14.

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

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7051
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

publicly available information. 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.

Note on access to documents

Some documents mentioned in the agenda/minutes cannot be released at present following a request for access to documents under 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).

1. PLENARY RELATED DOCUMENTS

1.1. AGENDA (EMA/CAT/58386/2014) and **TIMESCHEDULE** (EMA/CAT/58385/2014) for the CAT plenary to be held on 13th and 14th February 2014: **for adoption**

1.2. TABLE OF DECISIONS CAT plenary held on 13th and 14th January 2014 (EMA/CAT/792076/2013): **for information**

1.3. MINUTES of the CAT plenary held on 13th and 14th January 2014 (EMA/CAT/51170/2013): **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 13th – 14th February 2014: **for information**

See February 2014 minutes (to be published post March 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. (talimogene laherparepvec) (EMA/H/C/H0002771).
Therapeutic indication: treatment of adults with unresectable or metastatic melanoma.

For information:

- Rapporteur appointment to take place in March 2014

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) CAT Rapporteur: Elaine French
MAH: UniQure Biopharma B.V. CHMP Co-ordinator: Greg Markey
Orphan

II/30 (clinical)

Scope: Update of Protocol for the CM efficacy and safety study requested in the Annex II

For discussion:

- RSI

For adoption:

- Revised timetable

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	CAT Rapporteur: Elaine French CHMP Co-ordinator: Greg Markey
2.12.2.2. ChondroCelect (characterised viable autologous cartilage cells expanded *ex vivo* expressing specific marker proteins) (EMA/H/C/00878). MAH: TiGenix N.V. **Scope:** Five-year renewal **For information:** ▪ Timetable	CAT Rapporteur: E. Flory (DE) CHMP Co-ordinator: J. Müller-Berghaus (DE)
2.12.2.3. ChondroCelect (characterised viable autologous cartilage cells expanded *ex vivo* expressing specific marker proteins) MAH: TiGenix N.V. (EMA/H/C/00878/016) **Scope:** Randomised control trial protocol TIG/ACT/04/2009 **For information:** ▪ Timetable	CAT Rapporteur: E. Flory (DE) CHMP Co-ordinator: J. Müller-Berghaus (DE)
2.12.2.4. ChondroCelect (characterised viable autologous cartilage cells expanded *ex vivo* expressing specific marker proteins) MAH: TiGenix N.V. (EMA/H/C/00878/018) **Scope:** Non-interventional registry on the use of ChondroCelect to document the clinical effectiveness and safety outcome of treatment with ChondroCelect in real life in a patient population within the authorised indication **For information:** ▪ Timetable	CAT Rapporteur: E. Flory (DE) CHMP Co-ordinator: J. Müller-Berghaus (DE)

3. CERTIFICATION

Disclosure of information related to ATMP certification cannot be released at the present time as these are deemed to contain commercially confidential information.

4. SCIENTIFIC RECOMMENDATION ON CLASSIFICATION OF ATMPs

4.1. [Nuclear fraction separated from autologous bone marrow aspirate]. Proposed indication: stage I-III of osteoarthrosis and osteochondral lesion**For information:**

- Response to the second list of issues received 10th February 2014

For adoption:

- Revised Timetable

4.2. [autologous *ex vivo* expanded leukocytes treated with *European* 5-aza-2'-deoxycytidine]. Proposed indication: solid tumours.

For information:

- Comments received from EC dated 3rd February 2014

For adoption:

- Revised ATMP Classification report

Comments received from the European Commission

4.3. [allogeneic genetically engineered TCR/CD52/RQR8+/CD19 CAR+ T cells]. Proposed indication: CD19+ B-cell lymphomas

For information:

- Request received on 17th January 2014

For adoption:

- Appointment of CAT Co-ordinator
- Timetable

4.4. [characterised viable autologous stemcells expanded in vitro]. Proposed indication: treatment of degenerative arthritis, osteoarthritis (OA), articular cartilage defects in the knee, ankle or hip joints.

For information:

- Request received on 30th January 2014

For adoption:

- Appointment of CAT Co-ordinator
- Timetable

4.5. Reflection paper on classification of ATMPs. For information:

- Oral feedback from Drafting Group meeting

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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.

5.1. Update on CAT and SAWP interaction: **for information**

6. ORPHAN DRUG DESIGNATION

6.1. Committee for Orphan Medicinal Products (COMP)

For information:

- Agenda for the meeting 4th–5th February 2014

7. ITF BRIEFING MEETINGS IN THE FIELD OF ATMPs

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

8. ELIGIBILITY AS ATMP AND RAPPORTEURSHIP

No items on the agenda

9. ORGANISATIONAL MATTERS

9.1. Regulatory and Procedural Guidance

9.1.1. CAT's Rules of Procedure

For adoption:

- Revised Rules of Procedure
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9.2. CAT Meeting Organisation

9.2.1. Election of Chairperson to CAT. Call for nomination

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

Timetable:

-Deadline for receipt of candidatures: 12.02.14.

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

9.2.2. CAT Membership
For information:

- Bulgaria: Rozalina Kulaksazova – new member nominated on 29th January 2014
- Bulgaria: Evelina Shumkova – new alternate nominated on 29th January 2014
- Bulgaria: Lyubina R. Todorova - termination of mandate for member 28th January 2014
- Bulgaria: Velislava Todorova - termination of mandate for alternate 28th January 2014
- Germany: switch of roles of member and alternate. Martina Schüßler-Lenz becomes the member and Egbert Flory becomes the alternate as of 30th January 2014
- Italy: Giulio Cossu- termination of mandate for alternate 28th January 2014
- Patients' representative – EURORDIS: Monica Ensini - termination of mandate 30th January 2014

9.2.3. CAT/CHMP/COMP joint informal meeting to be held in Rome on 28th – 30th October 2014 under the auspices of the Italian Presidency of the Council of the European Union:
for information

9.2.4. CAT/PDCO joint informal meeting hosted by the Heads of the Italian and Slovenian NCAs in November 2013
For information:

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

9.3.1. CHMP January 2013 ToD: for information

9.4. CAT interaction with Interested Parties

9.5. CAT Work Programme

9.5.1. Objectives 2014-2015. Update: **for discussion**

9.5.2. Satellite CAT scientific workshop in the margins of the World Conference on Regenerative Medicine held in Leipzig (Germany) in 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 adoption** Postponed to March 2014

10.3. EMA/CAT-NB Collaboration Group

10.4. Patients and Consumers WP

10.4.1. Meeting of the EMA Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP) to take place on 25th February 2014
For information:
- Agenda

10.4.2. Joint meeting of the EMA Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals Working Party (HCPWP) to take place on 25th February 2014
For information:
- Agenda

10.4.3. EMA Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP) and Healthcare Professionals Working Party (HCPWP). Workshop on regulatory and methodological standards to improve benefit/risk evaluation of medicines to take place on 26th February 2014
For information:
- Agenda

10.5. Healthcare Professionals WP

11.OTHER SCIENTIFIC GUIDELINES/ISSUES

11.1. CAT international interactions – overview.
For action:
- Call for candidatures for CAT members to take part in Regulators Forum Gene Therapy Discussion Group (RFGTDG) and Regulators Forum Cell Therapy Discussion Group (RFCTDG)

Deadline: 7th March 2014
Please e-mail your interest to CATSecretariat@ema.europa.eu

11.2. EMA/CAT/FDA/Health Canada bimonthly teleconference on ATMP cluster
For adoption:
- Agenda

12.PHARMACOVIGILANCE

13.A.O.B.

13.1. Project 2014: move to 30, Churchill Place, Canary Wharf
For information:
- Update report

13.2. MMDs. All committees' members have been granted access to all documents from the other committees' MMDs:
for information

Date of next CAT meeting:
Thursday 13th – Friday 14th March 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.

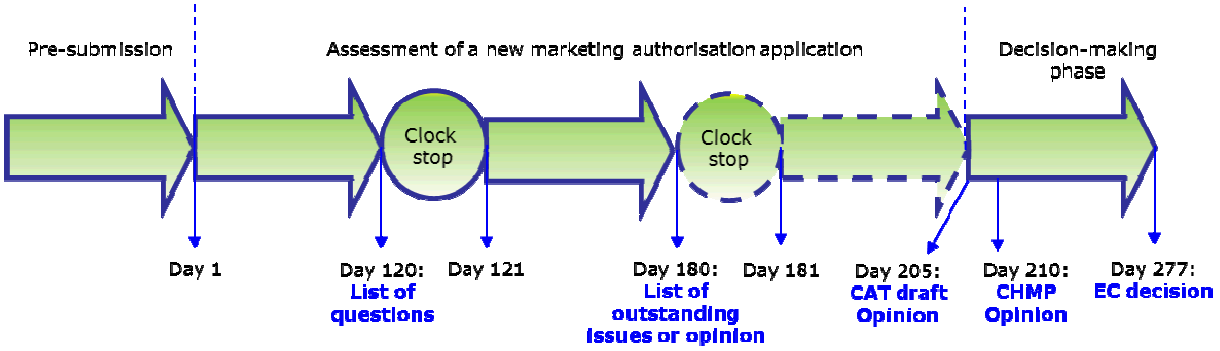
Evaluation of ATMPs (section 2)

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.