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Committee for Advanced Therapies (CAT)

Procedural advice on the provision of scientific recommendation on classification of advanced therapy medicinal products in accordance with article 17 of regulation (EC) no 1394/2007

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1. Introduction

The scientific recommendation on classification of Advanced Therapy Medicinal Products (ATMPs) (thereinafter called 'ATMP classification') is an optional procedure for applicants, which involves the Committee for Advanced Therapies (CAT).

The purpose of this procedure is to allow applicants to clarify, in case of doubt, whether a given product based on genes, cells or tissues meets the scientific criteria which define ATMPs, in order to address, as early as possible, questions of borderline with other areas such as cosmetics or medical devices, which may arise as science develops. It is recommended that this is done before submission of request for scientific advice/protocol assistance, Paediatric Investigation Plan (PIP) evaluation, certification of quality and non-clinical data for SMEs developing ATMPs, orphan drug designation and Marketing Authorisation Application (MAA).

Within 60 calendar days following receipt of a valid request, the CAT shall deliver its 'scientific recommendation on ATMP classification'¹ after consultation with the European Commission (EC). The EMA shall also publish summaries of this recommendation, after deletion of all information of commercial confidential nature.

This document describes the procedure and gives guidance for the steps to be followed by the applicant and EMA for the ATMP classification. The content of the summaries of recommendations for publication on the EMA website is also presented.

2. Legal basis

- According to Recital 24 of Regulation (EC) No 1394/2007:
"The Agency should be empowered to give scientific recommendations on whether a given product based on genes, cells or tissues meets the scientific criteria which define advanced therapy medicinal products, in order to address, as early as possible, questions of borderline with other areas such as cosmetics or medical devices, which may arise as science develops. The Committee for Advanced Therapies, with its unique expertise, should have a prominent role in the provision of such advice."
- According to Article 17 of Regulation (EC) No 1394/2007:
"1. Any applicant developing a product based on genes, cells or tissues may request a scientific recommendation of the Agency with a view to determining whether the referred product falls, on scientific grounds, within the definition of an advanced therapy medicinal product. The Agency shall deliver this recommendation after consultation with the Commission and within 60 days after receipt of the request.
2. The Agency shall publish summaries of the recommendations delivered in accordance with paragraph 1, after deletion of all information of commercial confidential nature."

3. Scope

The request for ATMP classification is available only for products based on genes, cells or tissues, as starting material, active substance or finished product including when combined with medical devices, bio-materials, scaffolds or matrices, and for which there are doubts as to whether or not they fall within the definition of an ATMP².

In the case that the products fulfil such definition, the CAT in its scientific recommendation concludes as to whether it fulfils the definition of gene therapy medicinal product, somatic cell therapy medicinal product or tissue engineered product.

¹ Thereinafter referred also as 'scientific recommendation'

² See Regulation (EC) No 1394/2007 – Article 2:
http://eur-lex.europa.eu/LexUriServ/site/en/oj/2007/l_324/l_32420071210en01210137.pdf

4. Roles and Responsibilities of all parties involved in the ATMP classification

4.1. CAT

The CAT is the committee responsible for the adoption of scientific recommendations on ATMP classification taking into account the legal provisions in force, the scientific state of the art and the input from the European Commission.

The CAT:

- Appoints the CAT coordinator (on basis of a rota system);
- Discusses and adopts the draft classification reports.

The CAT coordinator:

- Reviews the draft classification report prepared by the EMA coordinator;
- Presents the classification report at the CAT meeting
- Identifies whether a consultation of another Scientific Committee or Working Parties is needed. Those consultations are agreed at the CAT meeting (Day 30 of the procedure);
- Amends, as appropriate, the scientific recommendation report for CAT adoption

CAT members provide comments on the draft scientific recommendation that is included in the CAT pre-mail package.

4.2. EMA Secretariat

The CAT Secretariat is responsible for coordinating the ATMP classification within the Committee.

The Advanced Therapies Office:

- Validates the application for ATMP classification;
- Appoints the EMA coordinators;
- Identifies the CAT coordinator (on basis of a rota system);
- Organises the internal review meeting;
- Maintains the regulatory and scientific memory of the classifications carried out;
- Is responsible for the transmission of the classification report to the European Commission and the applicants;
- Publishes the summaries of the ATMP classifications.

The EMA coordinator:

- Prepares the draft classification report;
- Supports the CAT coordinator;
- Acts as the contact person for the applicant.

4.3. Other Scientific Committees and Working Parties

Other Scientific Committees and Working Parties (WPs) can be consulted by the CAT in case specific scientific issues need to be addressed in order to conclude on the classification of the product. When possible, this consultation takes place during the procedure and will not lead to clock stop as a rule.

4.4. European Commission

In line with Article 17.1 of Regulation (EC) No 1394/2007, the European Commission is consulted on all ATMP classifications. This consultation will take place in the 60-day procedural time (see section 6.2)

The Commission can also be consulted by the CAT in case specific legal and/or regulatory issues need to be addressed in order to conclude on the classification of the product. When possible, this consultation takes place during the procedure and will not lead to clock stop as a rule.

5. Documentation required

The applicant submits two documents according to the templates published on the ATMP classification webpage³:

- Administrative information
- Classification request form and briefing information, which will have to include the following information:
 - Information on the product (e.g. on active substance, finished product, mechanism of action and proposed use);
 - Information on the development of the product (including element of the manufacturing, quality aspects and outline of the non-clinical and clinical development) relevant for the ATMP classification;
 - Applicants should also substantiate their positions on the classification of their product in light of legal definitions in force.

6. Procedure

A general outline of the procedure for ATMP classification is provided in Figure 1.

6.1. Activities prior to the start of the procedure

- The request for ATMP classification (see section 5) shall be received by EMA, at the latest 15 days before the start of the procedure. Submission and start dates are published on the ATMP classification webpage.
- The Advanced Therapies Office checks if in the request sufficient information and justifications are provided by the applicant to substantiate the claim that their product is an ATMP.
- If major additional information is needed that cannot be provided within 5 working days, the procedure is initiated at the next starting date, provided that the required information is made available.
- The EMA coordinator is appointed.

6.2. CAT procedure for ATMP Classification

The procedure starts at the CAT meeting according to the timetable published on the ATMP classification webpage.

Day 1:

- The CAT appoints the CAT coordinator, following a rota system.
- The timetable for the ATMP classification is adopted by the CAT.

³ See: <https://www.ema.europa.eu/en/human-regulatory/marketing-authorisation/advanced-therapies/advanced-therapy-classification>

By Day 13:

- The EMA coordinator prepares the draft scientific recommendation.
- The EMA coordinator presents the draft recommendation in the internal review meeting and updates the report if needed.

Day 14:

- The EMA coordinator sends the draft scientific recommendation to the CAT coordinator for comments by Day 19.
- The EMA coordinator also identifies the need for additional information from the applicant (to be provided either by written responses or via an oral explanation) and prepares the draft list of questions.

Day 21:

- The EMA coordinator addresses the comments and input from the CAT coordinator and amends the report accordingly. The draft report is subsequently tabled in the European Medicines Agency's Managing Meeting Documents system (MMD).
- CAT members provide comments on the draft classification reports.

Day 27:

- The EMA coordinator, supported by the CAT coordinator, addresses the comments received from CAT members and, if needed, amends the draft scientific recommendation for discussion at the next CAT meeting.

Day 30: CAT meeting

- The CAT coordinator presents the draft scientific recommendation at the CAT meeting.
- CAT discusses and decides if additional information is required from the applicant before finalisation of the ATMP classification:

1. If there is no need for additional information:

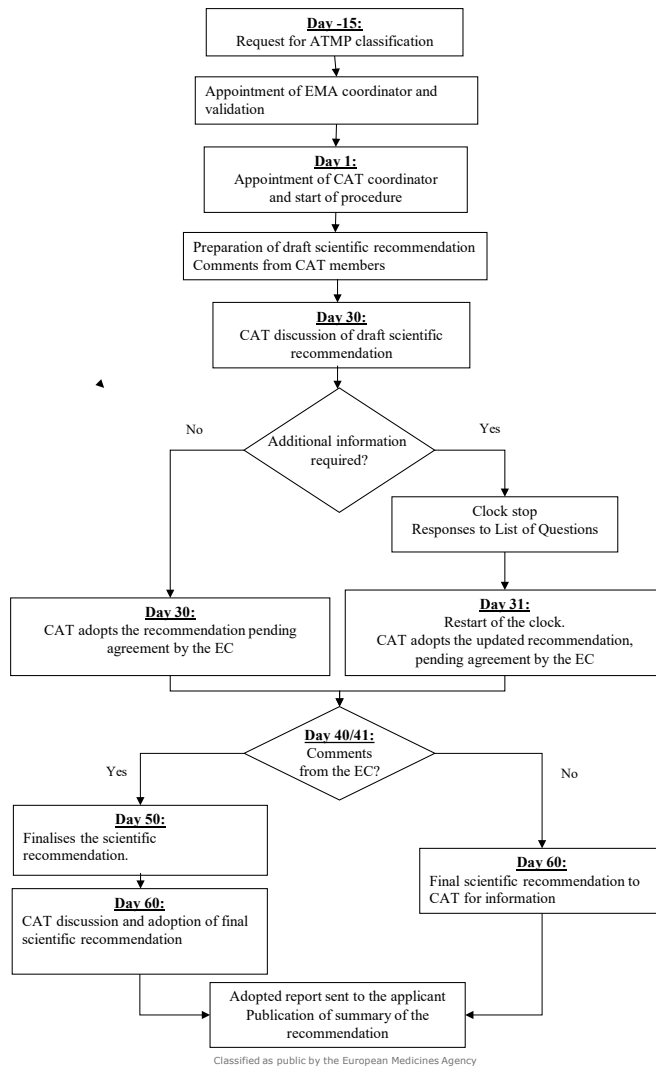
- CAT adopts the draft scientific recommendation, pending consultation of the EC
- The Advanced Therapies Office sends the scientific recommendation to EC for comments within 10 days.
- When no comments have been received from the EC:
 - The final CAT scientific recommendation is tabled at the next CAT meeting for information.
 - **Day 60:** the final CAT scientific recommendation is sent to the applicant and represents the final position of the CAT
 - A summary of the scientific recommendation is published (see section 7)
- In case comments have been received from the EC:
 - The EMA coordinator, supported by CAT coordinator, amends the draft classification report.
 - Adoption by CAT of final scientific recommendation at the next CAT meeting.
 - **Day 60:** The final CAT scientific recommendation is sent to the applicant and represents the final position of the CAT.
 - A summary of the scientific recommendation is published (see section 7)

2. There is a need for additional information

- **Day 30:** The EMA coordinator sends the list of questions to the applicant. CAT can invite the applicant for an oral explanation.

- A clock stop of 1 month will be proposed. The clock will restart taking into account that the discussion and final adoption at the CAT must take place within 60 days active review time. The applicant can request for a longer clock stop.
- In the case CAT decides to consult another Committee, a Working Party or the European Commission prior to finalisation of the scientific recommendation on classification, this will take place, as far as possible, within the remaining 30 days. If more time would be required for this consultation, the EMA coordinator will agree a clock stop with the applicant.
- The applicant will submit responses to the list of questions 10 working days before the next CAT meeting
 - The EMA coordinator, supported by the CAT coordinator, updates the draft scientific recommendation.
- **Day 31: clock restarts** at the next CAT meeting.
 - CAT adopts the draft updated scientific recommendation, pending consultation of the EC.
 - The Advanced Therapies Office sends the scientific recommendation to the EC for comments within 10 days.
- When no comments have been received from the EC:
 - The final CAT scientific recommendation is tabled at the next CAT meeting for information.
 - **Day 60:** the final CAT scientific recommendation is sent to the applicant and represents the final position of the CAT
 - A summary of the scientific recommendation is published (see section 7)
- In case comments have been received from the EC,
 - The EMA coordinator, supported by CAT coordinator, amends the draft classification report.
 - Adoption by CAT of final scientific recommendation at the next CAT meeting.
 - **Day 60:** the final CAT scientific recommendation is sent to the applicant and represents the final position of the CAT.
 - A summary of the scientific recommendation is published (see section 7).

Figure 1: Overview of timelines and steps for adoption of scientific recommendation by CAT



7. Summaries for publication

According to Article 17(2), "(...) the Agency shall publish summaries of the recommendations delivered in accordance with paragraph 1, after deletion of all information of commercial confidential nature".

In the adopted report a section with the proposed summary for publication is included. This section is based on the public information proposed by the applicant and revised during the procedure by the EMA and CAT coordinator.

This section will consist of the following information:

- Product description
- Indication
- Conclusion of the scientific recommendation
- Outcome of classification
- Date

Within 2 weeks the applicant can comment on this section of the report taking into account the principles of confidential information, as described in the EMA document "Principles to be applied for

the Deletion of Commercially Confidential Information for the Disclosure of EMEA Documents (Doc. Ref. EMEA/45422/2006)".

This section will then be published, if needed after consideration by the EMA and CAT coordinators of the applicant's comments.

Abbreviations

ATMPs: Advanced Therapy Medicinal Products

CAT: Committee for Advanced Therapies

EC: European Commission

EMA: European Medicines Agency

WP: Working Party