EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Development and Evaluation

Mandate, objectives and rules of procedure for the CAT Gene Therapy Temporary Working Party (GTWP)

Adopted

Interim proposal of mandate which is aligned with the CHMP draft proposal of a restructuring of working parties and follows the Committee for Advanced Therapies (CAT) need for support from a temporary working party on gene therapy issues. The current version of the mandate is subject to further modification depending of the outcome of the review and restructuring of working parties.

1. General considerations

A number of gene therapy medicinal products are in clinical development in the EU, some of which have already been subject to Marketing Authorisation Application. Since the release of the CHMP note for guidance on gene transfer medicinal products in April 2001 a number of new developments indicate that the rapidly evolving field of gene therapy will necessitate regular updating of the available guidelines. Furthermore, there is a need for contributing to the EU position at international level such as within ICH framework. With implementation of the Regulation on advanced therapies and the establishment of the CAT at the Agency, it is considered important to reorganise the European expertise gathered from member states' regulatory authorities and scientific institutions to better support activities coordinated by the CAT.

According to the rules of procedure of the CAT, the Committee may consult its working parties on any scientific issue related to their specific fields of expertise. The Committee may also delegate certain tasks associated with the scientific evaluation of applications, or drafting of guidelines to the relevant working parties. The tasks identified by the Committee should be included in the work programme of each working party to be adopted by the CAT.

The Gene Therapy Working Party (GTWP) will therefore act as a multidisciplinary working party to provide recommendations to the CAT, on all matters relating directly or indirectly to all aspects of gene therapy medicinal products and to perform the task described under section II. For coordination with the CHMP, other committees and working parties see the EMA Policy on appropriate coordination between the scientific committees of the Agency (Reference No. EMEA/124704/2005 Rev.1).

The GTWP shall be represented at the Working Party Coordination Group.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

2. Mandate and objectives

The GTWP is established to provide recommendations to the CAT on all matters relating directly or indirectly to gene therapy including the tasks defined below:

- Prepare, review and update guidelines and other guidance documents (e.g. reflection papers) in conjunction with other appropriate working parties. Scientific guidelines will be adopted jointly by the CAT and CHMP; other guidance documents will be adopted by the CAT.
- Ensure that the CAT and, through the CAT, scientific committees or working parties are informed on scientific issues specific to gene therapy medicinal products and provide scientific reports on matters of public interest and emergency issues pertaining to gene therapy.

At the request of the CAT:

- Contribute to scientific advice, paediatric investigation plan evaluation, orphan designation, marketing authorisation applications or post-authorisation procedures, certification and other relevant procedures on gene therapy medicinal products.
- Contribute to briefing meetings related to gene therapy with external parties (pharmaceutical companies, academia, public/private partnership or patients' associations) through the participation of experts in close collaboration with the Innovation Task Force.
- Liaise with interested parties (e.g., learned societies, patients' organisations, academia networks, pharmaceutical industry representatives), in close collaboration with the CAT (see VI. Rules of procedure).
- Contribute to international cooperation on gene therapy related matters reinforcing the networking and exchange of experience, including scientific contribution on ICH.
- Focus and catalyse training for gene therapy product assessment and contribution to and/or organisation of gene therapy-related workshops, conferences and other training.
- Constitute a rapid-acting crisis group to take on board specific issues related to gene therapy with the objective of exchanging information on a European level and to co-ordinate the CHMP and/or the CAT responses on public health issues to the public in a timely manner.
- Any other task delegated by CAT for example liaison with the EC, other committees and working parties on matters relevant to gene therapy.

3. Composition and rules of participation

The Gene Therapy Working Party is composed of experts proposed from the European experts list according to their specific expertise.

Up to 10 experts, identified and nominated by the CAT on the basis of their specific scientific expertise and/or regulatory experience on the subjects covered within the scope of the Working Party's mandate, shall constitute the core group, including:

- one person from the BWP,
- one person from the SWP,
- one person from the CAT,

who have a background experience in gene therapy.

From the 10 members, a chair and a vice-chair shall be appointed by the CAT.

Depending on the topic of discussion, the scientific secretariat shall ensure the consultation of the relevant therapeutic temporary working parties and drafting groups.

Depending on the topics included in the agenda, the Agency secretariat and the GTWP Chair may propose additional experts for individual GTWP meetings (see VI.5). The list of additional experts shall be agreed by the CAT in advance of the GTWP meeting.

Members who want to bring additional participants to a GTWP plenary meeting should notify the Agency secretariat in advance of the meeting, subject to the agreement of the Chair.

Membership of a working party implies a commitment to participate actively in the work of that working party and to attend the meeting of the working party regularly. If a member does not participate in 3 consecutive meetings or does not contribute actively, the Chair of the WP will bring this to the attention of the CAT that may decide on a replacement.

A member may nominate a replacement to participate in those exceptional cases where he or she is unable to attend a meeting. The member shall inform the Agency secretariat at the latest 1 week in advance of the meeting if she/he will be replaced.

Meeting documentation will be distributed to an agreed list of recipients drawn up by the Agency with the agreement of the Chair.

Observers from the European Commission, EDQM, WHO and other international and EU organisation may attend the meetings of the GTWP, if agreed with the CAT and the Agency secretariat.

Members from other Committees and/or their working parties may participate with the agreement of the Chair and the Agency.

GTWP members also members of other working parties and/or scientific advisory groups will act as contact persons with other working parties to ensure good communication in areas of common interest, and in particular to report on relevant activities to respective working parties.

CAT members are encouraged to take an active role in the activities of the GTWP and to ensure information of the activities of the group is disseminated at national level.

4. Meeting frequency

The frequency of the GTWP meetings shall be agreed by the CAT in accordance with the adopted work programme. The dates of the meetings shall be included in the work programme of the Working Party.

Separate drafting group meetings may be organised by teleconference, or when appropriate, face-to-face upon the CAT agreement. The composition of those drafting groups can be different from the composition of regular plenary GTWP meetings.

5. Duration of activity

The continuity of the GTWP will be confirmed once a year by the CAT.

6. Rules of procedure

6.1. Responsibilities of Chair and Vice-chair

6.1.1 The Chair, in his absence the Vice-chair, is responsible for the efficient conduct of the business of the GWTP, in conjunction with the Agency secretariat, and shall in particular:

- Plan the work of the Working Party together with the Agency.
- Monitor, together with the Agency secretariat that the rules of procedure are respected.
- Ensure that at the beginning of each meeting any potential conflict of interest is declared regarding any particular item to be discussed by the Working Party.
- Aim to achieve consensus on issues discussed by the Working Party.
- Decide in exceptional cases, when a vote is necessary.
- Ensure, together with the Working Party and the Agency secretariat, the regulatory and scientific consistency of the Working Party's recommendations.
- Co-ordinate, together with the Agency secretariat, the work of this Working Party with that of the other relevant working parties of the Agency, in conjunction with the Working Party Coordination Group.
- Report on the activities of the Working Party to the CAT, other working parties as appropriate, and the Agency.

6.1.2 The Vice-chair will deputise for the Chair when the latter is unable to chair either all or part of the Working Party meeting. On such occasions the Chair will seek the agreement of the Vice-chair as early as possible and prior to the meeting. The Agency secretariat shall be informed immediately.

6.2. Election of Chair and Vice chair

The Chair and Vice-chair of the GTWP shall be elected by the members of the CAT for a 3-year term, which may be renewed once.

Nominations should be submitted to the Agency secretariat in writing no later than the start of the Committee meeting at which the election of the working party chair is to take place.

Candidates shall submit a mission statement in support of their candidature at the time of the nomination.

The election of the chair and the vice-chair, where appropriate, shall follow the same procedure as that for the election of the chair of Committee as stated in Article 3, paragraphs 1 to 4, of the Rules of Procedure of the CAT.

6.3. Organisation of meetings and reporting arrangements

6.3.1 The GTWP shall meet regularly at the Agency.

6.3.2 The dates of meetings are decided on an annual basis in consultation with the Working Party, the CAT and the Agency.

6.3.3 The meetings will be held and minuted in English.

6.3.4 The draft agenda for every meeting, as adopted by the CAT in consultation with the Chair, shall be circulated, together with the relating documents, by the Agency secretariat at least 7 calendar days before the meeting.

6.3.5 When a member of the GTWP is unable to participate in a meeting, part of meeting, or discussion topic due to conflict of interest, he/she must inform the secretariat in advance in writing.

6.3.6 The Working Party may identify and propose topics for consideration. Any proposal for a guideline, providing adequate justification, shall be approved by the CAT and endorsed by the Agency and shall be preceded by a concept paper to be endorsed by the CAT and the CHMP. Other guidance documents will be adopted by the CAT.

6.3.7 Any recommendation from the Working Party shall be transmitted to the CAT for adoption.

6.3.8 The Working Party shall prepare an annual work programme for adoption by the CAT which shall include topics identified in accordance with point 6 above and any specific tasks identified by the CAT. The work programme shall be regularly reviewed and updated as necessary with the agreement of the CAT and the Working Party Coordination Group.

6.3.9 Table of conclusions/minutes of the meetings of the Working Party should be circulated to the CAT for information.

6.3.10 Where the chair is a member of the Working Party, he/she will be invited to attend the CAT and where relevant the CHMP meetings to report on the activities on the Working Party and ensure liaison with the work of the CAT.

6.3.11 The mandate of the Working Party shall be agreed by the CAT. It shall be reviewed, at least every 3 years.

6.3.12 The GTWP Chair or its delegate and the Agency secretariat will participate, on a regular basis, in the meetings of the Working Party Coordination Group.

6.4. Drafting Groups

When further consideration is required in order to prepare proposals on specific topics the GTWP may convene drafting groups constituted of members of the Working Party or experts, as appropriate.

The drafting group will report to its working party in direct line.

6.5. Participation of Experts in meetings

6.5.1 When necessary, the Working Party may avail itself of the services of experts in specific scientific or technical fields. Such experts shall have proven experience in the assessment of medicinal products or in their field of expertise and be included in the European Experts list. Where appropriate, members from patient organisations or other health care professionals may act as experts.

6.5.2 The names of these experts shall be notified to the Agency secretariat before the meeting, they are due to attend.

6.6. Guarantees of independence

6.6.1 The members of the Working Party and experts referred to above shall not have any direct interests in the pharmaceutical industry, which could affect their impartiality. They shall undertake to act in the public interest and in an independent manner, and shall make an annual declaration of their financial interests. All indirect interests which could relate to the pharmaceutical industry shall be

entered in a register held by the Agency, which is accessible to the public, on request at the Agency's office.

6.6.2 Members of the Working Party and experts attending these meetings shall declare at the beginning of each meeting any specific interests, which could be considered to be prejudicial to their independence with respect to the points of the agenda. These declarations shall be made available to the public.

6.6.3 The specific provisions for handling declaration of interests and confidentiality undertakings as defined in the EMA Policy on the Handling of Conflicts of Interests for Committee Members and Experts, adopted by the Management Board (EMEA/H/31653/03), are applicable to members of the Working Party and experts participating in the activities of the Working Party.

6.7. Code of conduct

Members of the Working Party and experts participating in the Agency's activities shall abide by the principles set out in the Agency Code of Conduct.

6.8. Agency secretariat

6.8.1 Under the authority of the Executive Director, the Agency secretariat shall provide technical, scientific and administrative support to the Working Party. This includes the following:

- Provide technical and scientific support to rapporteurs/co-ordinators and other members of the Working Party.
- Provide legal, regulatory and scientific support to the Working Party.
- Prepare and co-ordinate the work of the Working Party in consultation with their chairs.
- Ensure, if appropriate, that the periods laid down by Community legislation for the adoption of the opinions are complied with.
- Organise meetings of the Working Party ensuring timely circulation of meeting documents.
- Facilitate the necessary contacts between the Working Party, the Committees and other concerned working parties and/or scientific advisory groups.
- Ensure adequate co-ordination of the work carried out within the Working Party, the scientific Committee(s) and other concerned working parties and/or scientific advisory groups.
- Contribute to the overall quality assurance and assurance of scientific and regulatory consistency of the documents/recommendations of the Working Party in co-operation with the Chair or Vice-chair, as appropriate.
- Prepare the minutes of the meetings of the Working Party in consultation with the chairs.
- Communicate when necessary any CAT recommendations relevant to the Working Party to interested parties.
- Contribute to the identification of experts.

6.8.2 The Executive Director of the Agency and members of the Agency secretariat may attend all meetings of the Working Party.

6.9. Contacts with interested parties

6.9.1 As agreed by the CAT and where relevant, the Working Party will establish contacts, on an advisory basis, with parties concerned with the use of medicinal products, in particular patient organisations and health-care professionals' associations.

6.9.2 Pharmaceutical industry, health care professionals, patients/consumers or other interested parties have the opportunity to comment in writing on draft guidelines and general regulatory developments during the public consultation of the documents.

6.9.3 When considered appropriate by the Working Party and the CAT, oral presentations by interested parties can be made during Working Party meetings in earlier stages of development of guidelines. The working parties may also meet with interested parties to discuss general matters or specific scientific issues with the agreement of the CAT and under specific conditions to be agreed by the CAT.

6.9.4 In any case, the Working Party shall neither conduct any deliberations nor reach any formal decisions in the presence of members of interested parties.

6.9.5 Before any consultation session, interested party representatives and Working Party members will communicate to the Agency secretariat the points they would like to be discussed, so that an agenda of the session can be prepared for agreement by the Working Party Chair and circulation by the Agency secretariat.

6.10. General provisions

The members of the Working Party as well as observers and all experts shall be bound, even after the cessation of their duties, not to disclose any information, which, by its nature, must be covered by individual professional secrecy.

When participating in international or other fora on behalf of the committee(s), members shall ensure that the views expressed are those of the committee(s).

When participating in international or other fora not specifically on behalf of the committee(s), members shall make clear that the views expressed are their own views and not those of the committee(s).