European Medicines Agency

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# MANDATE, OBJECTIVES AND RULES OF PROCEDURE FOR THE CHMP GENE THERAPY WORKING PARTY

## I. GENERAL CONSIDERATIONS

Gene therapy/transfer products are used for prevention or treatment of cancer, cardiovascular disease, infectious disease, inherited disorders and other conditions. A number of gene therapy/transfer medicinal products are in clinical development in the EU, some of which will approach the licensing stage in foreseeable time.

The process of gathering knowledge from all relevant communities in gene therapy, both from clinical use and preclinical development, is necessary to support the development of gene therapy/transfer products and accumulation of the necessary expertise to evaluate the properties of these products.

Some gene therapy/transfer products have been designated as orphan drugs, some have entered the scientific advice and other EU procedures. Since the release in April 2001 of the CHMP guideline on gene transfer medicinal products a number of new developments indicate that the rapidly evolving field of gene therapy will necessitate regular updating of the available guidelines. It is therefore important to gather European expertise from member states' regulatory authorities to support the CHMP and its working parties in preparing guidelines and reviewing request of scientific advice or application.

The field of gene therapy is largely developed internationally and issues that are common to all ICH regions and may impact on the existing regional guidelines, therefore, there is a need for contributing to the EU position at international level such as during meetings of the ICH Gene Therapy Discussion Group (ICH GTDG).

In addition, discussion on scientific findings, views and concepts in gene therapy with all relevant parties, including academia and industry, are necessary to supplement the regulatory experience in this field with the current scientific developments.

In case of particular issue published on gene therapy, such as an unexpected serious adverse reaction, ad-hoc expert group meetings may have to be convened to help to establish a common understanding and to facilitate the development of harmonise views within the regulatory communities in the EU. This could also provide an additional scientific basis for the necessary regulatory action in EU member states.

It is crucial that EMEA establishes with academia, industry and other interested parties a dynamic relationship enabling all parties to identify and discuss the issues arising from innovative strategies for the development of medicinal products. In this direction the CHMP has recently implemented a new working mechanism, establishing multidisciplinary working parties supported by an extended specialised experts network including the academia, and as appropriate industry and the public as a whole.

The Gene Therapy Working Party is therefore established as a multidisciplinary forum to provide recommendations to the Committee on all matters relating directly or indirectly to all aspects of gene therapy medicinal products and to perform the task described under section II. The CHMP Gene Therapy Working Group will work in close collaboration with the CHMP Biologicals/Biotechnology Working Party (BWP) on issues related to the quality and quality-related safety aspects in relationship to quality of gene therapy medicinal products, resulting in joint recommendation to CHMP.

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The GTWP is represented on the Bio-coordination group (BCG), which is established to facilitate sharing of information on quality aspects of biotech, biological and emerging technology products (see VI. Rules of procedures, point 3.13).

The present document supersedes the "mandate and composition" (CPMP/31881/02) and rules of procedures (CPMP/33811/03), adopted by the CPMP in January 2003.

## II. MANDATE AND OBJECTIVES

The GTWP is established to provide recommendations to the CHMP on all matters relating directly or indirectly to gene therapy including, but not limited to the tasks defined below:

- Ensure that the CHMP and its working parties are informed on scientific issues specific to gene therapy medicinal products.
- Provide the CHMP with scientific reports on matters of public interest and emergency issues pertaining to gene therapy. They will be made publicly and internally available, marking them as scientific reports summarizing the current state of discussion (or as positions) and as being endorsed by the CHMP.
- Preparation, review and update of guidelines in conjunction with other appropriate working parties.
- Contribute to the scientific advice and protocol assistance provided by the Scientific Advice Working Party of the CHMP, and to CHMP marketing authorisation or post-authorisation procedures on gene therapy medicinal products, upon request.
- Setting up of drafting groups (see VI. Rules of procedure, point 4).
- Advice, through the CHMP, to the European Commission on gene therapy related issues.
- 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 CHMP responses on public health issues to the public in a timely manner.
- Liaise with other Working Parties or Ad-Hoc Expert Groups on gene therapy medicinal products related matters, and on other matters of relevance to the work of GTWP. Co-operation with the BWP, Pharmacovigilance Working Party (PhVWP), Efficacy Working Party (EWP), Safety Working Party (SWP), Paediatric Working Party, Working Party on cell therapy and other CHMP Working Parties on areas of mutual interest.
- International cooperation on gene therapy related matters in conjunction with other appropriate working parties, reinforcing the networking and exchange of experience with FDA, Japan, Canada, WHO, Australia, etc.
- Provide CHMP with scientific contribution on ICH and other international activities.
- Focus and catalyse training for gene therapy product assessment.
- Contribute to and organisation of gene therapy-related workshops and training.
- Liaise with interested parties (e.g. ESGT, EFPIA…)(see VI. Rules of procedure).

## III. COMPOSITION AND RULES OF PARTICIPATION

The Gene Therapy Working Party is composed of experts selected from the European experts list according to their specific expertise. Gene Therapy experts are identified by the CHMP 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.

Up to 12 experts, identified by the CHMP, shall constitute the core group, including:

- one representative from the BWP,
- one representative from the SWP,
- one representative from the EWP.

From the 12 core members, a chairperson and a vice-chairperson shall be appointed by the CHMP

The final composition of the core group shall be agreed by the CHMP.

In addition, experts from any of the other CHMP Working Parties and/or Scientific Advisory Groups may be invited as appropriate.

Depending on the topics included in the agenda, the EMEA Secretariat and the GTWP Chairperson may propose up to 5 additional experts for individual GTWP meetings. These experts will be chosen from the European expert list, based upon their specific scientific expertise. The list of additional experts shall be agreed by the CHMP in advance of the GTWP.

CHMP members will also appoint one contact person per member state with specific expertise in gene therapy to ensure that information of the activities of the group is disseminated at national level and to provide input in to the activities of the GTWP. They may attend meetings.

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.

A member may nominate an alternate to participate in those exceptional cases where he or she is unable to attend a meeting. The list of alternate should be made available to the CHMP. The core member shall inform the EMEA secretariat at the latest 1 week in advance of the meeting if she/he will be replaced by the alternate. Alternates are encouraged to attend the GTWP meetings.

Members who want to bring additional experts should notify the EMEA secretariat in advance to the meeting, subject to the agreement of the chairperson.

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

Representatives of the Commission may attend meetings of the GTWP.

Observers from EDQM may attend meetings of the GTWP.

Observers from non-EEA countries may participate with the agreement of the chairperson and the EMEA. Observers from accession countries and MRA partners may have standing invitations to participate at certain working parties. Specific confidentiality rules will apply to observers.

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

Certain GTWP members may be designated as contact persons with other working parties and/or scientific advisory groups to ensure good communication in areas of common interest. The concerned working parties will agree on the responsibilities of the contact person.

## IV. MEETING FREQUENCY

The CHMP GTWP shall meet 3 times per year in accordance with the adopted work programme. The dates of the meetings shall be included in the work programme of the working party.

A drafting group meeting may be convened in preparation of the EU contribution to the ICH process.

To allow rapid exchange of information in case of new safety information, one or more extraordinary meetings may be convened with CHMP agreement.

## V. DURATION OF ACTIVITY (IN THE CASE OF TEMPORARY WORKING PARTIES)

Not applicable.

## VI. RULES OF PROCEDURE

### 1. Responsibilities of Chairperson and Vice-Chairperson(s) *(where appointed)*

1.1 The chairperson, and if appointed, in his absence the vice-chairperson, is responsible for the efficient conduct of the business of the working party and shall in particular:

- Plan the work of the working party together with the EMEA Secretariat.
- Monitor, together with the EMEA 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, EMEA Secretariat, the regulatory and scientific consistency of the Working Party's recommendations.
- Co-ordinate together with the EMEA Secretariat the work of this Working Party with that of the other relevant Working Parties of the Agency.
- Report on the activities of the Working Party to the CHMP or to other working parties as appropriate.

1.2 The Vice-Chairperson (if appointed) will deputise for the Chairperson when the latter is unable to chair either all or part of the Working Party meeting. On such occasions the Chairperson will seek the agreement of the Vice-Chairperson as early as possible and prior to the meeting. The EMEA Secretariat shall be informed immediately.

### 2. Election of Chairperson and Vice Chairperson(s)

The chairperson of a working party shall be elected by the members of the appropriate CHMP (Committee) for a term of the Committee, which may be renewed. A Committee member, an alternate or a member of the working party may be elected by the Committee to fulfil this responsibility. Regardless of the time of election of the Chairperson he/she shall be appointed for the term of the Committee.

A vice-chairperson(s) may be elected by the Committee if the working party and Committee considers it appropriate.

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

Candidates shall submit a brief résumé in support of their candidature at the time of the nomination.

The election of the Chairperson and the Vice-Chairperson(s), where appropriate, shall follow the same procedure as that for the election of the chairperson of Committee as stated in Article 3, paragraphs 1 to 4, of the Rules of Procedure of the CXMP.

### 3. Organisation of meetings *and reporting arrangements*

3.1 The Working Party shall meet regularly at the Agency.

3.2 The dates of meetings are decided on an annual basis in consultation with the Working Party and the Committee.

3.3 The meetings will be held and minuted in English.

3.4 The draft agenda for every meeting shall be circulated, together with the relating documents, by the EMEA Secretariat, in consultation with the chairperson, at least 7 calendar days before the meeting.

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

3.6 The Working Party may identify and propose topics for consideration by the Working Party. Any proposal for a guideline, providing adequate justification, shall be transmitted to the Committee(s) for endorsement and shall be preceded by a concept paper to be endorsed by the Committee(s).

3.7 Any recommendation from the Working Party shall be transmitted to the Committee for adoption.

3.8 When considered appropriate by the Working Party, oral presentations by companies can be made during Working Party meetings on matters directly related to the activities of the working Party, following agreement of the Committee.

3.9 The working party shall prepare an annual work programme for adoption by the Committee(s) which shall include topics identified in accordance with point 6 above and any specific tasks identified by the Committee(s). The work programme shall be regularly reviewed and updated as necessary with the agreement of the Committee(s).

3.10 Agenda, table of conclusions and minutes of the meetings of the working party should be circulated to the Committee(s).

3.11 Where the Chairperson is an alternate or a member of the working party, he/she will be invited to attend plenary Committee meetings to report on the activities on the working party and ensure liaison with the work of the Committee.

3.12 The mandate of the Working Party shall be agreed by the Committee(s). It shall be reviewed, at least at the start of each new term of the committee(s).

3.13 The GTWP Chairperson or its delegate and the EMEA Secretary will participate, on a regular basis, in the meetings of the BCG.

**4. Drafting Groups**

When further consideration is required in order to prepare proposals on specific topics the working party 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.

**5. Participation of Experts in meetings**

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.

5.2 The names of these experts shall be notified to the EMEA Secretariat before the meeting, which they are due to attend.

**6. Guarantees of independence**

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.

6.2 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.

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

**7. Code of conduct**

Members of the working party and experts participating in the EMEA's activities shall abide by the principles set out in the EMEA Code of Conduct.

**8. EMEA Secretariat**

8.1 Under the authority of the Executive Director, the EMEA secretariat shall provide technical, scientific and administrative support to the working party. This includes the following:

- Provide technical and scientific support to rapporteurs 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 chairpersons.
- 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 CHMP 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 Chairperson or Vice-Chairperson, as appropriate.
- Prepare the minutes of the meetings of working party in consultation with the Chairpersons.
- Communicate when necessary any Committee recommendations relevant to the Working Party to interested parties.
- Contribute to the identification of experts.

8.2 The Executive Director of the Agency and members of the EMEA secretariat may attend all meetings of the working party.

**9. Contacts with Interested Parties**

9.1 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.

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.

9.3 When considered appropriate by the Working Party, 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 Committee and under specific conditions to be agreed by the Committee.

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.

9.5 Before any consultation session, interested party representatives and working party members will communicate to the EMEA 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 Chairperson and circulation by the EMEA secretariat.

**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, 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).