19 April 2018
EMA/COMP/8212/00 Rev. 4
Committee for Orphan Medicinal Products

Committee for Orphan Medicinal Products (COMP)

Rules of Procedure

Article 55 of Parliament and Council Regulation (EC) No 726/2004 of 31 March 2004 establishes the European Medicines Agency with the responsibility for coordinating the existing scientific resources put at its disposal by Member States for the evaluation, supervision and pharmacovigilance of medicinal products.

Parliament and Council Regulation (EC) No 141/2000 of 16 December 1999 lays down a Community procedure for the designation of medicinal products as orphan medicinal products, provides incentives for the research, development and placing on the market of designated orphan medicinal products, and sets up a Committee for Orphan Medicinal Products within the Agency;

Since the Committee is part of the Agency, the Integrated Quality Management System, endorsed by the Agency Management Board on 11 March 2004, applies to the Committee and its working groups.

Each competent national authority shall monitor the level and independence of the evaluation carried out and facilitate the activities of nominated members and experts. Members States shall refrain from giving Committee members and experts any instruction which is incompatible with their own individual tasks or with the tasks and responsibilities of the Agency.

Having regard to the EEA Joint Committee Decision No 140/2002 of 8 November 2002 amending Annex II (Technical regulations, standards, testing and certification) and Protocol 37 to the EEA Agreement;

The Committee adopts the following rules of procedure:

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Composition

Article 1

1. The Committee consists of one member nominated by each of the EU Member States, three members nominated by the Commission to represent patients' organisations, three members nominated by the Commission on the basis of a recommendation from the Agency, and a Chairperson. The members of the Committee shall be appointed for a term of three years, which may be renewed.
2. The Committee shall also include one member appointed by each of the EEA-EFTA states, for a term of three years, which may be renewed.

Responsibilities of Chairperson and Vice-Chairperson

Article 2

1. The Chairperson, and in his/her absence the Vice-Chairperson, is responsible for the efficient conduct of the business of the Committee and shall in particular:

- plan the work of the Committee meetings together with the EMA Secretariat;
- monitor, together with the EMA Secretariat, that the rules of procedures are respected;
- ensure, at the beginning of each meeting, that any potential competing interest is declared regarding any particular item to be discussed by the Committee;
- decide when a vote is necessary when consensus is not possible;
- ensure, together with the Committee and the Secretariat, the regulatory and scientific consistency of opinions and recommendations;
- ensure that scientific grounds are adequately reflected in the Committee opinions;
- co-ordinate, together with the EMA secretariat, the work of this Committee with that of the other Committees of the Agency.

2. The Vice-Chairperson will deputise for the Chairperson when the latter is unable to chair either all or part of the Committee meeting. On such occasions the Chairperson will seek the agreement of the Vice-Chairperson as early as possible, prior to the meeting and the EMA Secretariat shall be informed immediately.
3. If the Vice-Chairperson takes the chair, he/she shall retain his/her deliberating vote.

Election of Chairperson and Vice-Chairperson

Article 3

1. The Chairperson and Vice-Chairperson of the Committee shall be elected by and from amongst its members for a term of three years, which may be renewed once.

2. Nominations for Chairperson and Vice-Chairperson should be submitted in writing to the EMA secretariat no later than at the start of the Committee meeting at which the election is to take place.

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

4. The election of the Chairperson and the Vice-Chairperson shall be by absolute majority of the members (i.e. favourable votes by at least half of the total number of Committee members eligible to vote plus one) and by secret ballot. At each round, the candidate(s) with the lowest number of favourable votes shall withdraw. In the case of a tie in the decisive round, another round is organised with two remaining candidates. If, at the decisive round, the candidate with the highest number of votes does not get an absolute majority, a further voting is organised with this candidate only, where he/she needs favourable votes by at least half of the total number of Committee members eligible to vote plus one, to be elected Chairperson or Vice-Chairperson, as the case may be.

5. After the election of the Chairperson, the authority which appointed him/her will designate a new member to replace the Chairperson as a member of the Committee. From the date of this appointment, the Chairperson shall lose his/her vote.

6. In the event of resignation of the Chairperson, the Vice-Chairperson shall take the chair until a new election is convened.

7. The members appointed by the EEA-EFTA states may not vote nor be elected Chairperson or Vice-Chairperson of the Committee.

Rapporteurs and Assessment team

Article 4

1. For any scientific evaluation in respect of orphan product designation, one rapporteur from amongst the members of the Committee shall be appointed on the basis of objective criteria including expertise and a fair distribution of the work; the rapporteur will work very closely with one EMA scientific officer appointed to the same procedure. For any scientific evaluation in respect to protocol assistance with a significant benefit question, one peer reviewer from amongst the members of the Committee shall be appointed. For the review of orphan designation for orphan medicinal products at the time of initial marketing authorisation, two rapporteurs from amongst the members of the Committee (preferably, the rapporteur involved in the initial orphan designation and, as second rapporteur, the COMP member from a CHMP rapporteur's country, alternatively an expert in the particular scientific field) shall be appointed to work very closely with the EMA scientific officer for the same procedure.

2. The role of the rapporteurs is to perform the scientific evaluation and to present it to the Committee according to the timetable agreed for the evaluation procedure, taking into account the timeframe laid down in the relevant legislation. More specifically, the role of the COMP rapporteur is to provide a critical independent review and to contribute to the elaboration of the summary report and the opinion prepared by EMA. The rapporteurs are responsible of identifying issues and propose them for discussion by the Committee.

3. COMP rapporteurs and other COMP members may propose experts in specific scientific or technical fields (including patients) included in the European experts list available at the EMA to help with the assessment. This is notified to EMA Secretariat as soon as possible when recognising that further expertise will be needed.

4. The format and quality of the opinion should be determined and judged by the Committee.

Scientific opinions, decisions and recommendations

Article 5

1. The quorum required for the adoption of scientific opinions or recommendations by the Committee shall be reached when two thirds of the total members of the Committee eligible to vote are present. The votes should be positive or negative (unless the provision concerning conflicts of interest is applied).

2. Whenever possible, scientific opinions, decisions or recommendations of the Committee shall be taken by consensus. If such a consensus cannot be reached, the scientific opinion will be adopted if supported by at least two-thirds of the total number of Committee members eligible to vote.

3. The divergent positions of and the names of the members expressing the divergent positions shall be mentioned in the opinion of the Committee, and where relevant, in the minutes of the Committee. Members having divergent positions shall provide them in writing, stating clearly the reasons on which they are based. They will be appended to the opinion.

4. The members from the EEA-EFTA states may not vote but their positions shall be stated separately in the opinion, where relevant, in the minutes of the Committee and in case of divergent opinions appended to the Committee's opinion. Their position is not counted in reaching the Committee's opinion.

5. In the absence of a two-third-majority position in favour of the designation of a medicinal product as an orphan medicinal product, the Committee's opinion is deemed to be negative.

Written Procedure

Article 6

1. Draft opinions and recommendations can, after approval of the Chairperson, be submitted by the EMA Secretariat to the Committee for adoption by written procedure. However, such written procedures should be restricted to measures required to be taken between scheduled meetings.

2. Draft opinions or recommendations are addressed to members of the Committee, who may raise objections within a specified time period, to be established in agreement with the Chairperson. The Secretariat shall present a full report on the outcome of the written procedure at the following meeting of the Committee.

3. In case of serious objections, the Chairperson decides whether the written procedure should be suspended and the adoption of the draft opinion or recommendation postponed to the next meeting of the Committee.

Re–examination of opinions

Article 7

1. For the implementation of the procedures for the re-examination of opinions mentioned in Article 5(7) of Regulation (EC) No 141/2000, different rapporteurs will be appointed to assess the grounds for the re-examination of opinions. This re-examination shall be made by using the best endeavours to ensure a new examination, independent from the first opinion.

2. The re-examination may deal only with the points of the opinion initially identified by the applicant and is based only on the scientific data available when the Committee adopted the initial opinion. The applicant may request that the Committee consult a scientific advisory group (if and when established) in connection with the re-examination. In this case, the Committee shall request the advice of additional available expertise.

Oral Explanations

Article 8

1. The Committee may invite a sponsor to provide oral explanations in connection with a designation procedure on its own initiative or where requested by the sponsor.
2. The Committee may also invite on its own initiative or may consider a request of any other relevant third party for an oral explanation in connection with a designation procedure and review of orphan designation.
3. Oral explanations shall be indicated clearly in the draft agenda of the meeting.
4. The Committee shall not make any conclusions during these presentations or in the presence of the sponsor's representatives or the third parties.
5. In all cases the sponsor is informed of the trend at COMP level at the end of the scientific discussion ahead of any formal vote to conclude the evaluation process.

Organisation of Meetings

Article 9

1. The Committee shall meet monthly at the Agency with the exception of the month of August during which no meeting is convened unless explicitly required. In this case, the meeting shall be convened by Executive Director or his/her representative after consultation with the Chairperson.
2. The dates of meetings are decided on an annual basis in consultation with the Committee. In exceptional circumstances and on motivated grounds agreed with the Chairperson an extraordinary meeting may be convened at short notice.
3. The meetings will be held and minuted in English.
4. The draft agenda for every meeting shall be circulated together with the relating documents by the EMA Secretariat, in consultation with the Chairperson, at least 14 calendar days before the

meeting. This draft agenda shall enable the Committee to perform its duties as defined in Article 4, Regulation (EC) No 141/2000.

5. The Minutes of the meeting shall be provided no later than the week in advance of the following Committee meeting.

6. When a Member of the Committee is unable to participate to a meeting, part of meeting, or discussion topic due to conflict of interest, he/she must inform the Secretariat. Such declarations will be recorded in the minutes of the respective meeting.

7. Where members are absent for 3 consecutive meetings, the Chairperson, on behalf of the Committee, may bring this to the attention of the Commission who may raise the issue with the appointing authority.

Working groups

Article 11

1. The Committee may establish any standing or temporary working groups when work of a temporary or ad-hoc nature is required such as preparation of proposals on a specific scientific topic, preparation of responses to specific questions raised by the Committee, drafting of new guidelines or revision of existing ones in relation to specific scientific fields.

2. Working groups consist of members of the Committee.

Participation of Experts in meetings

Article 12

1. When necessary, the Committee, and its working groups may avail themselves of the services of experts in specific scientific or technical fields (including patients). Such experts shall be included in the European Experts list.

2. In addition members of the Committee may be accompanied by the experts mentioned in paragraph 1 (at their own expense). The names of these experts shall be notified to the EMA Secretariat before the meeting, which they are due to attend.

Guarantees of independence

Article 13

1. The membership of the Committee shall be made public. When each appointment is published, the professional qualifications of each member shall be specified.

2. The members of the Committee, members of working groups, and experts mentioned in various articles of the present Rules of Procedure, 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. In addition, the Declarations of Interests of the members of the Committee shall be made available on the Agency's website.

3. Members of the Committee and working groups (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.

4. The specific provisions for handling declaration of interests and confidentiality undertaking as defined in the EMA Policy on the handling of competing interests for scientific committees' members and experts, adopted by the Managements Board, are applicable to members of the Committee, working groups and experts participating in the scientific activities of the Agency

Code of conduct

Article 14

Members of the Committee, working groups, and experts participating in the EMA's activities shall abide by the principles set out in the EMA Code of Conduct.

EMA Secretariat

Article 15

1. Under the authority of the Executive Director, the EMA Secretariat shall provide technical, scientific and administrative support to the Committee and its working groups with a view to the performance of its duties as set out in Article 4 and 5, Regulation (EC) No. 141/2000. This includes the following:

- Organise meetings of the Committee and its working groups ensuring timely circulation of meeting documents;
- Provide technical and scientific support to members of the Committee and its working groups;
- Provide legal and regulatory support to the Committee and its working groups;
- Prepare and co-ordinate the work of the Committee and its working groups in consultation with the Chairperson;
- Ensure compliance with the periods and procedures laid down by Community legislation for the adoption of the opinions;
- Carry out the validation of the applications and prepare summary reports of the particulars submitted to the Agency;
- Facilitate the necessary contacts between the Committee and the sponsors or person responsible for placing the product on the market;
- Ensure scientific and regulatory consistency of the opinions/recommendations of the Committee in co-operation with the Chairperson or Vice-Chairperson, as appropriate;
- Ensure adequate co-ordination of the work carried out within this Committee and its working groups;

- Ensure that all relevant information is shared between COMP and CHMP;
- Organise when necessary joint meetings with the CHMP;
- Prepare the minutes of the meetings of the Committee and its working groups in consultation with the Chairpersons;
- Communicate to sponsors the relevant opinions or recommendations of the Committee;
- Prepare and communicate relevant public information related to the activities of the Committee such as press releases, public statements, Q & A documents and summaries of opinions after consultation of the Committee, where appropriate;
- Communicate to interested parties relevant recommendations of the Committee;
- Communicate the views of the Committee to the European Commission;
- Communicate the views of the Committee in international *fora*.

2. The Executive Director of the Agency, members of the EMA secretariat, and representatives of the Commission, may attend all meetings of the Committee and its working groups.

Contacts with Interested Parties

Article 16

1. The Committee and its working groups 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. The Committee may agree to invite representatives of such interested parties to address a plenary meeting.
2. Concept papers, draft guidelines and general regulatory developments will be subject to public consultation of all interested parties (industry, health care professionals, patients/consumers or other).
3. When considered appropriate by the Committee, oral presentations by interested parties can be made during working group meetings in earlier stages of development of guidelines. The working groups 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.
4. In any case, the Committee, working groups shall neither conduct any deliberations nor reach any formal decisions in the presence of members of interested parties.
5. Before any consultation session, interested party representatives and Committee members will communicate to the EMA Secretariat the points they would like to be discussed, so that an agenda can be prepared for agreement by the Committee Chairperson and circulation by the EMA secretariat.

Observers

Article 17

1. At the initiative of the European Commission and in agreement with the Management Board, the Committee may admit representatives of international organisations as observers at the Committee and working groups' meetings or meetings arranged for this purpose to discuss topics of common interest. The conditions for participation shall be determined beforehand by the European Commission.
2. For the purposes of regulatory co-operation visiting experts or other representatives from non-EEA regulatory authorities may also participate as observers to the Committee and its working groups. Participation shall be agreed with the respective Chairperson in advance of the meeting.
3. The observers shall be bound by the rules of confidentiality mentioned in Article 13.

General Provisions

Article 18

The Committee may if, they consider it appropriate, seek guidance on important questions of a general scientific or ethical nature.

Article 19

The Members of the Committee and working groups, 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 professional secrecy.

Article 20

The decision to adopt or to amend these rules of procedure shall be taken by an absolute majority of the Members of the Committee (i.e. favourable votes by at least half of the total number of Committee members eligible to vote plus one).

Adopted by the Committee on 19 April 2018

Date of entry into force on 19 June 2018