EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

17 September 2024
EMA/188551/2023 rev. 1
Stakeholders & Communication Division

CHMP early dialogue with patient and healthcare professional organisations: process and FAQs

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1. Background

Patients and healthcare professionals are involved in a wide range of European Medicines Agency (EMA) activities, either as representatives of their organisations or as individual experts, depending on the nature of the activity.

In activities related to the evaluation of specific medicines, patients and healthcare professionals (HCP) are systematically engaged as individual experts.

A methodology of engaging with patient organisations at the start of evaluation of new marketing authorisation applications (MAA) for orphan medicines by the Committee for Human Medicinal Products (CHMP) was piloted in 2021-22 with a successful outcome. This was extended to include i) non-orphan medicines and ii) healthcare professional organisations. This early dialogue is additional and complementary to the other engagement methodologies and primarily targets organisations representing patients or HCP that fulfil the EMA's eligibility criteria.

2. Consultation process

2.1. Identification of procedures for consultation

The evaluation phase of the regulatory lifecycle of a medicine provides various opportunities for patient and HCP engagement. In addition to the involvement of individual experts in Scientific Advisory Groups/Ad hoc Expert Groups and oral explanations during the CHMP plenary meetings, organisations can contribute at the start of the evaluation process (Day 1 of MAA) (Figure 1).

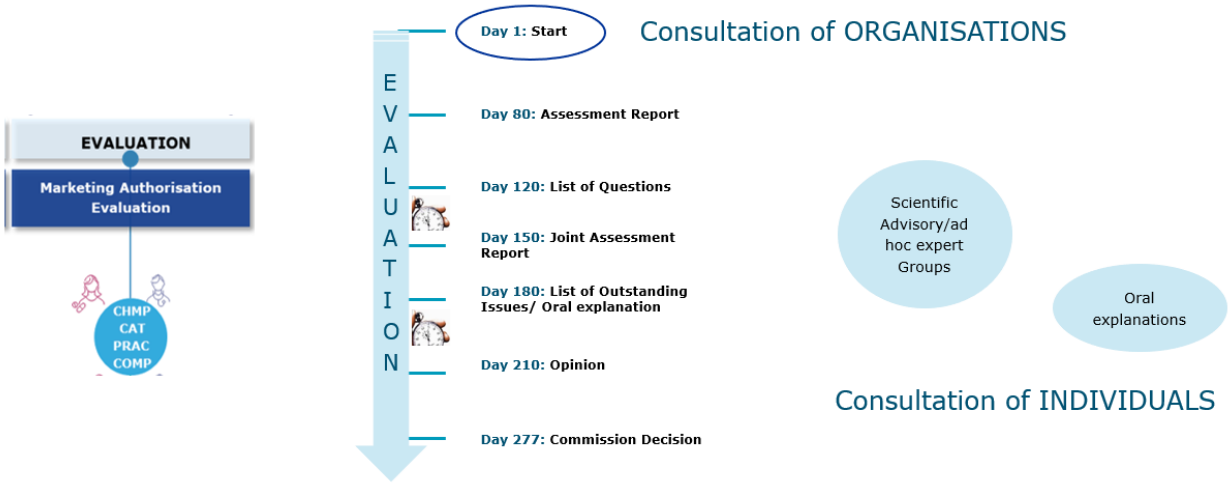

Figure 1: Evaluation phase of the regulatory lifecycle of a medicine and opportunities for patient and HCP engagement.

The process for identifying and selecting products for consultation is shown in Figure 2 (below): EMA extracts a list of initial marketing authorisation applications whose evaluation began in the month in question, consults with the core group at CHMP, and follows up on the selected products (those containing a new active substance) with the relevant patient organisations (PCO) and healthcare professional organisations (HCPO) for general input on their condition, in particular, standard treatments and how acceptable they are, therapeutic/unmet medical needs, quality of life, what benefits would be hoped for in new medicines and what level of side effects would be considered acceptable.

The eligible organisations have 4-5 weeks from receiving the request to return their comments using a template (see Annex). They can reach out to their members on the questions asked and return the input along with relevant information already held by the organisation if applicable, which is then shared with the CHMP rapporteurs, EMA product lead and the applicant.

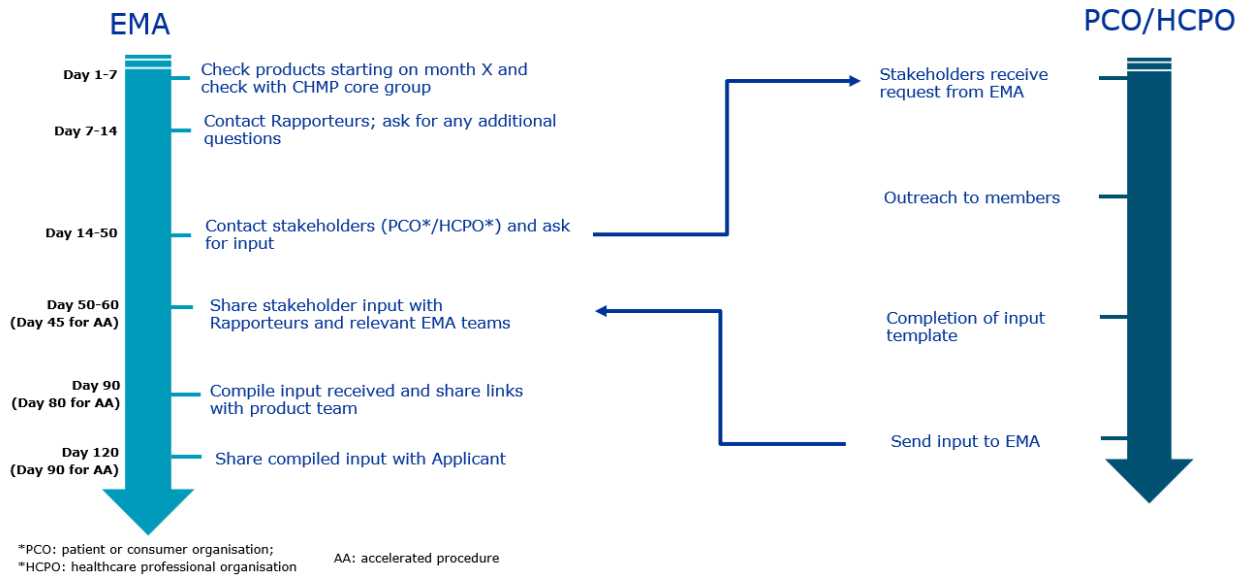

Figure 2: Process and interactions between EMA and patient (PCO)/ healthcare professional (HCPO) organisations

2.2. Purpose of early consultation

It is important to note that at this very early stage of consultation only the name of the active substance and proposed indication are shared[1] with the organisations. No information submitted by the applicant is shared. The aim of early consultation is to gain insights into aspects of a condition and its currently available treatments (if any) that regulators may not be aware of, so that they may take them into consideration at the beginning of the assessment process.

Patients and healthcare professionals can provide important contributions on the impact of the condition on patients, most important symptoms, how patients are treated, unmet needs, expectations for future treatments to name a few examples.

The early consultation does not aim to collect positions on the medicine under assessment from the consulted organisations.

[1] https://www.ema.europa.eu/en/documents/other/principles-publication-agendas-minutes-ema-scientific-committees_en.pdf

3. Feedback and frequently asked questions (FAQs)

3.1. When receiving a request from EMA

- **What kind of input is useful to the CHMP?**

The CHMP is seeking the input of:

Patients and carers with experience of living with the disease in order to fully appreciate their perspective and concerns about the condition targeted by the medicine under assessment. This will help the CHMP understand what is important for patients, such as quality of life, current treatment options, unmet medical needs, considerations for pregnant people/people of child-bearing potential and what benefits they would hope for in new treatments such as efficacy and acceptability of potential side effects. This early dialogue can also guide CHMP on when to seek more in-depth interactions with patients or carers.

Healthcare professionals specialised in the disease area of the medicine under assessment since they are most informed on the pathognomonic signs of the disease, its diagnosis and prognosis, available treatments, treatment duration, and whether the duration needs optimisation; any possible therapeutic/unmet medical needs due to lack of specific approaches or due to severe or unmanageable adverse events and what benefits they would hope for in new medicines and what level of side effects would be considered manageable, as well as considerations for pregnant people/people of child-bearing potential.

This early insight from people living with the condition, patients and carers, and healthcare professionals who are treating patients with the currently available options and who will, in case of approval, prescribe, dispense or treat patients with this product, will help CHMP rapporteurs assess the application.

Organisations should **not** include any personal patient data or make any reference to commercially confidential information even if they are aware of the medicine under development.

- **EMA does not provide any information on the medicine under assessment. How can we comment?**

Early dialogue with organisations is not related to the medicine under assessment but aims more broadly to understand patients, carers and HCP perspectives on the condition and its treatment. Medicine-specific discussions take place a later stage of the assessment process, and at that point individual patient or HCP experts can be invited to participate in meetings where relevant.

If it is not clear what information you need to provide, you can contact EMA at public-engagement@ema.europa.eu.

- **Is there a conflict of interest if an organisation receives industry funding?**

The CHMP early dialogue is targeted primarily to eligible organisations of patients and HCPs, which have been evaluated and confirmed to fulfil the eligibility criteria for working with EMA.

Additional relevant organisations such as disease-specific patient organisations that are not in EMA's list of eligible organisations or European Reference Networks (for rare diseases) may be consulted.

If individual members of the responding organisation (patients or HCPs) have consulted or had recent contact with the applicant or are involved in the product development, this should be stated in the

response for transparency. For example, the following statement could be included: '*Please note that 'organisation' has obtained input from experts on 'condition' who had competing interests*.'

3.2. When reaching out to members

- **Is the information provided confidential? Can we share it with our members?**

No confidential information is provided in the context of CHMP early dialogue consultations, so it can be shared internally with your members.

- **What methods should we use to consult our membership?**

Organisations can choose different methods to gather input from their members: some use written consultation while others may conduct focus groups or individual interviews.

If you need to reach out to specific experts, it is expected that i) you send them the email with instructions and template directly and ii) you compile the answers received to provide a consolidated input to EMA. EMA would appreciate that you reach out the experts in your network and not to send a list of experts to be contacted.

- **How representative does our input need to be?**

Organisations may not be able to fully capture the views of all their members, given factors such as differences in standards of care across countries, or the numbers of patients/HCP colleagues who can be consulted in a short time in English. When preparing for member consultations, it is a good idea to think about the best method to gain input that is as representative as possible. However, all input, even if not fully robust, is useful to bring in patient and HCP perspectives. If the EMA needs more input on specific questions, individual patient or HCP experts can be invited to contribute to meetings at a later stage.

- **May we consult or coordinate input with other stakeholders?**

The reason CHMP consults with patients and HCP organisations separately is to preserve their specific viewpoints, which may sometimes be different in some respects. Organisations may reach out to others if they wish, since the information shared by EMA is not confidential, but it should be mentioned in your input.

- **Can the timelines be extended?**

EMA aims to provide on average 4-5 weeks for you to provide input for regular assessment timetables; this may be less for products that are reviewed under 'accelerated assessment'. These timelines are necessary because the input needs to be considered and included in the first assessment report (D80 for a regular assessment). Short extensions can sometimes be accepted in agreement with the Rapporteurs.

3.3. When compiling the information using the EMA template

The CHMP would prefer that the information gathered is compiled by the category that is the most relevant (e.g., per country) whilst highlighting any relevant differences between respondents. Additional individual information (e.g., per patient or HCP) may be provided in an annex.

- **What should we consider when submitting the information template?**

No identifying information such as personal data (names) should be provided, given that the input is submitted on behalf of the organisation and will be shared with the company applying for marketing authorisation. If the input contains commercially confidential information and/or personal data, this will be removed.

As mentioned above, positive or negative opinions on the medicinal product under assessment should not be included.

It should be stated in your input if your organisation has consulted with other organisations besides your membership, as well as any conflict of interest (see section 3.1).

- **May we send our input also to other stakeholders?**

The information collected in the input template is not confidential and the input can therefore be shared with other stakeholders.

3.4. How is my input used?

- **Who receives the input?**

The input received and organisation name will be shared with the CHMP (and other relevant EMA committees, working parties and expert groups) and may be reflected in the assessment report that will be made public at the end of the procedure.

EMA will also share the input and organisation name with the applicant that has submitted the marketing authorisation application.

If another pharmaceutical company submits an application for the same indication, your input may be shared with the CHMP rapporteurs and the applicant submitting the new procedure after EMA obtains your confirmation (see further details in the next question).

Please note that any document held by EMA may be subject to disclosure in accordance with Regulation (EC) No 1049/2001 of the European Parliament and of the Council of 30 May 2001 regarding public access to European Parliament, Council and Commission documents.

By submitting input, organisations acknowledge and agree with the rules detailed above.

- **Can my input be re-used?**

If you have previously contributed, and another application is received for a similar indication, you will be contacted to confirm whether your previous input is relevant and can be used for the new application. Your previous input will not be shared with the CHMP Rapporteurs until your confirmation is obtained. It is also possible to update, amend, or refuse your previous input.

3.5. Follow-up

- **How can we know if the input provided is useful?**

During the pilot phase of the early contact methodology an analysis showed that 41% of (Co-) Rapporteurs acknowledged that the patient information contributed to the development of the (D80) assessment report. As a consequence, input from stakeholders is now more systematically reflected in the (D80) assessment report, as evidenced by the revised assessment report templates (see below). Unfortunately, the CHMP is not in a position to provide feedback on contributions received.

- **How is the input reflected in EMA's decision-making?**

EMA aims to include the patient and HCP input from the first assessment reports of the Rapporteur(s) and in the European public assessment report (EPAR) to make it transparent and highlight the contribution of users and prescribers of medicines to the decision-making process. Please note that the name of the organisation providing input may be included in the EPAR.

The Day 80 Clinical assessment report template has been updated with dedicated sections to reflect patient and HCP organisations engagement input in the assessment report in a uniform manner, if relevant to the evaluation process.

Input can also be useful in other ways: for example, some medicines, especially orphan products, will be authorised under a conditional marketing authorisation, where the marketing authorisation holder will have to collect further data. Input from patients and HCPs can inform the discussions on further studies required by EMA.

- **Are there other opportunities for input?**

As show in Figure 1, later in the assessment procedure, other opportunities for further input may arise, such as participation in Scientific Advisory/Ad hoc Expert Groups or in an oral explanation for the product in question. Individual patients or HCPs invited to attend such meetings will have to submit a declaration of interest that will be assessed prior to their participation.

Annexes:

Annex 1: Resources

A webinar was held on 19 April 2023 to present the methodology to the HCP organisations. Patient organisations with experience in responding to consultations by CHMP were invited to present and participate.

- 1-year report from HCP (when finalised and published)
- Pilot on early dialogue with patient organisations for orphan marketing authorisation applications: Outcome Report
- Pilot phase for CHMP early contact with patient / consumer organisations

Annex 2: Templates for patient and HCP organisations

PATIENT/CARER EXPERIENCE OF: **indication**
Please include below any aspects that are of particular importance to patients/carers, such as information on: • standard treatments and how acceptable they are, • therapeutic/unmet medical needs, • quality of life, • what benefits would be hoped for in new medicines as well as what level of side effects would be considered acceptable, • considerations for pregnant people/people of child-bearing potential, where applicable. Also mention any aspects about the condition or its treatment that you feel are not well-understood or not sufficiently considered. You may include anything else you feel is important for EMA to know. Please try to keep your main points to 1-2 pages; if necessary, include more details in an appendix.

HEALTHCARE PROFESSIONAL EXPERIENCE OF: **indication**
Please include below any aspects that are of particular importance to healthcare professionals, such as information on: • the standard of care or available treatments and to what extent they cover the intended indication; • the treatment duration; and, if in your view, the duration needs to be optimised; • any possible therapeutic/unmet medical needs; • what benefits you would hope for in new medicines; as well as what level of side-effects would be considered manageable; • considerations for pregnant people/people of child-bearing potential, where applicable. Please also mention any aspects about the condition or its treatment that you feel are not well-understood or not sufficiently considered. Please include anything else you feel is important for EMA to know. Please try to keep your main points to 1-2 pages; if necessary, include more details in an appendix.