EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Stakeholders & Communication Division

CHMP early contact 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 relating 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.

As part of the continued implementation of this methodology, it was agreed to extend the selection of medicines from orphans to include non-orphans and to reach out to healthcare professional organisations in addition to patient organisations. This early engagement is additional and complementary to the other engagement methodologies and targets organisations representing patients or healthcare professionals that fulfil the EMA's eligibility criteria. The best way to reflect patient and HCP organisations input in the assessment report has been explored.

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

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, organisations can contribute at the start of the evaluation process (Day 1 of MAA) (Figure 1).

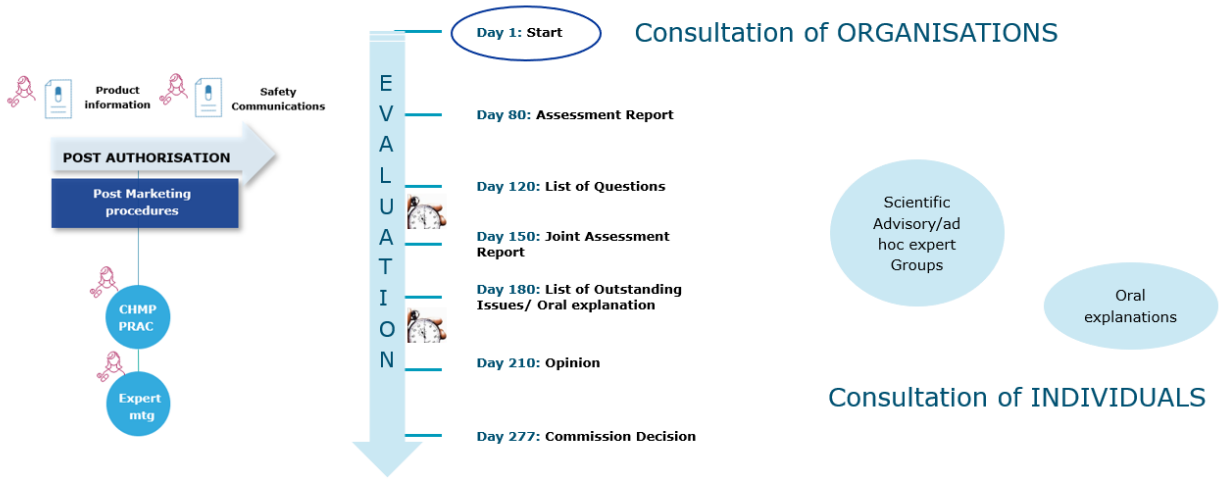

Figure 1: Evaluation phase of the regulatory lifecycle of a medicine and opportunities for patient and HCP engagement.

The process for identifying and selecting products for consultation is shown in Figure 2 (below): EMA extracts a list of submissions for the month in question, consults with the core group at CHMP, and follows up on the selected products with the eligible patient organisations (PO) and healthcare professional organisations (HCPO) for general input on the disease and the main concerns related to available treatment(s). The products selected are those that contain a new active substance.

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 feedback along with relevant information already held by the organisation if applicable, which is then shared with the CHMP rapporteurs, EMA product lead and the sponsor/applicant.

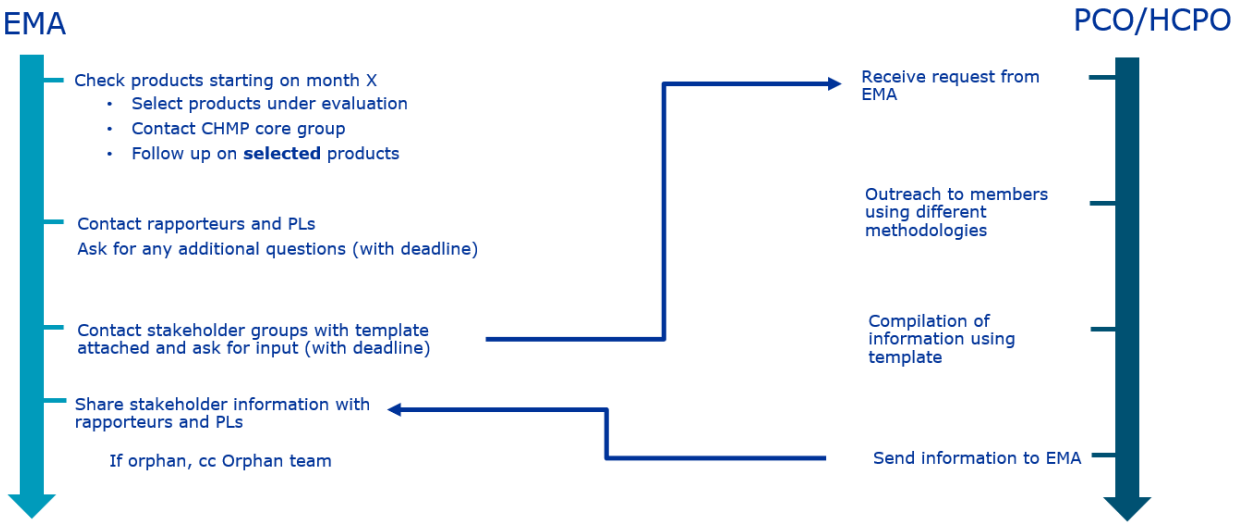

Figure 2: Process for contacting patient organisations (PO) and healthcare professional organisations (HCPO)

2.2. Purpose of early consultation

It is important to note that at this very early stage of consultation, the name of the active ingredient and proposed indication are shared[1]; however, no information submitted by the applicant is shared. The aim of early consultation is to gain insight on aspects of a condition and its currently available treatments (if any) that regulators may not be aware of, so that they may consider those aspects at the beginning of the assessment process.

Patients and healthcare professionals can provide important contributions on, for example, the impact of the condition on patients, most important symptoms, how patients are treated, unmet needs, expectations for future treatments, and outcomes or trial endpoints that they would find important for a medicine to demonstrate clinical benefit. The early consultation does not aim to collect positions on the medicinal product under assessment from the consulted organisations.

3. Feedback and frequently asked questions (FAQs)

Patient organisations have been consulted by the CHMP in this early contact methodology since 2021 and have experience and expertise that was shared during the webinar, where HCP organisations also had an opportunity to raise questions. Questions and answers concerning different aspects are addressed below.

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

3.1. When receiving a request from EMA

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

The CHMP is seeking the opinion of:

Patients and carers with experience of living with the disease in order to fully appreciate patients' experience and concerns about the condition targeted by the medicinal product under assessment. This will help the CHMP understand aspects that are important for patients, such as quality of life, current treatment options, unmet medical needs, and what benefits they would hope for in new treatments such as efficacy and acceptability of potential side effects. This early contact 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 medicinal product under assessment since they are most informed on the pathognomonic signs of the disease, its diagnosis, prognosis, available treatments, eventual unmet medical needs due to lack of specific approaches or due to severe or unmanageable adverse events, as well as expectations from new treatments, including efficacy and safety.

Both groups may also reflect on what potential trial endpoints (outcomes) would be important to show a meaningful benefit of a new treatment.

This early insight from people living with the condition, 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. 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.

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

Early engagement with organisations is not related to the product under assessment but aims more broadly to understand patients, carers and HCP perspectives on the condition and its treatment. Product-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 collect, 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 consultation is targeted only to eligible organisations of patients and HCPs, which have been evaluated and confirmed to fulfil the eligibility criteria for working with EMA. If individual patients or HCPs are invited to attend a Scientific Advisory/ad hoc expert group (SAG/AHEG) or oral explanations at CHMP, their individual interests will be assessed at that point.

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 contact 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.

You may already be aware of new medicines that are being developed in your disease area that may soon be submitted for a marketing authorisation process, therefore it could be helpful to think about methods that you can use to seek the opinion of your members quickly. This will help your organisation plan for upcoming CHMP consultations and cope with the tight deadlines.

- **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 HCPs' organisations separately is to preserve their specific viewpoints, which may sometimes be different in some aspects. 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.

3.3. When compiling the information using the EMA template

The CHMP would prefer that the information gathered is compiled by whatever category 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. When sending the information to EMA

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

No identifying information (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 response contains identifying information, this will be removed.

As mentioned above, positive or negative opinions on the medicinal product under assessment should not be included. If the response contains such positions, these will be removed.

You should also remember to tick the box in the template to confirm your consent for EMA to share your views with third parties as applicable.

It should be stated in your response if your organisation has consulted with other organisations besides your membership.

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

No, information collected by EMA in the context of an evaluation of a marketing authorisation cannot be shared further.

3.4. 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 systematically reflected in the (D80) assessment report. 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 report of the rapporteur as well as in European public assessment report (EPAR) to make it transparent and highlight the contribution of users and prescribers of medicines to the decision-making process. The input is already often reflected in the reports, but the templates for clinical assessment reports are being reviewed to add dedicated sections for patient and HCP perspectives.

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.

Annex: Templates for patient and HCP organisations

PATIENT / CARER EXPERIENCE OF: <condition>
Please include below any aspects that are of particular importance to patients/carers, such as quality of life, standard treatments and how acceptable they are, therapeutic/unmet medical needs, what benefits they would hope for in new medicines as well as what level of side effects they would consider acceptable. • Highlight if there are large differences between groups of patients/carers about these aspects or if these views are generally similar across the condition. • Please also mention any aspects about the condition or its treatments that you feel are not well-understood or not sufficiently considered. • Please include anything else you feel is important for EMA to know. Try to keep your main points to 1-2 pages, if necessary, include more details in an appendix. Please do not include any individual patients contact details or health data. ☐ Tick here to confirm you give consent for EMA to share your views anonymously with third parties, as applicable.

RESPONSE:

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HEALTHCARE PROFESSIONAL EXPERIENCE OF: <condition>
Please include below any aspects that are of particular importance to healthcare professionals, such as standard of care or available treatments and to what extent they cover the intended indication, the treatment duration and if in your view it needs to be optimised, therapeutic/unmet medical needs, what benefits you would hope for in new medicines as well as what level of side effects would consider manageable for patients. • Please also mention any aspects about the condition or its treatments 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. ☐ Tick here to confirm you give consent for EMA to share your views anonymously with third parties, as applicable.

RESPONSE:

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