



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Stakeholders & Communication Division

Pilot phase for CHMP early contact with patient / consumer organisations

Background and rationale

Patients and their representatives are involved in many activities at EMA and the added value of including their perspectives within committee evaluations has been well demonstrated.

They are currently involved at various timepoints during the medicines' lifecycle, including CHMP evaluations. However, requests for patient input generally come at a later stage of the evaluation, often once major objections have been identified (e.g. expert meeting, oral explanation). Experience shows that late input may lead to missed opportunities to properly incorporate patient perspectives into the assessment process. Therefore in order to make current engagement practices more efficient and enhance timely participation, it is proposed to establish contact with relevant patient / consumer organisations at the start of new medicines assessment. This will enable patients to share aspects such as quality of life, treatment options and unmet medical needs so that the CHMP is well-aware of all aspects from the beginning. This is also expected to facilitate further interactions with patients as the procedure progresses.

This proposed action and process improvement is in line with both the CHMP work plan objective to: 'Incorporate additional and regular processes to capture and include patients' views and preferences in the benefit/risk evaluations', and EMA's Regulatory Science Strategy recommendations which highlight the need to enhance methods to systematically incorporate patient data in regulatory decision-making.

Legal basis

Article 78 of Regulation (EC) N° 726/2004 allows EMA scientific committees to establish contacts on an advisory basis with patients relevant to the indication of a medicine under evaluation.

Proposal

To enhance the way CHMP currently interacts with patient groups during the assessment of new medicines, it is proposed to reach out to relevant patient/consumer organisations at the start of the evaluation of new Marketing Authorisation Applications (MAAs) so that patients can share their experience and concerns about their condition(s) and key aspects that are important for them in order for this to be taken into account in a timely manner during the assessment process.

A pilot is proposed to define how this could occur to maximal effect, as detailed below.

Proposed process

Which Marketing Authorisation Applications (MAAs) will be included in the pilot?

CHMP and EMA secretariat will identify all new active substance MAAs with orphan status, for which start of procedure is imminent and will contact relevant patient organisations once this has been published on EMA's website.

Which patient organisations will be contacted?

Patient organisation(s) covering the therapeutic area of the MAA will be contacted. Generally these will be part of EMA's established network of [eligible](#) EU patient/consumer organisations.

What will be the timeline for input from the patient organisations?

Organisations will be given a period of 3-4 weeks to respond.

Will any confidentiality information be shared?

No documents or confidential information will be shared at this stage with the patient organisations; all information relating to the new MAA will be available on EMA's website.

How will engagement continue once the patient organisations have submitted information?

The patient organisations will be kept up to date on progress of the procedure (based on published information) and contacted should there be further opportunities for engagement (e.g. expert meeting, Oral Explanation, or written questions).

What is the duration of the pilot phase?

It is proposed that the pilot phase should last around one year to fully assess the practical feasibility and the value of the input, with an interim analysis at 6 months.

How and what will the pilot phase evaluate

- A short evaluation questionnaire will be sent to the rapporteurs, and to the CHMP project leaders for their feedback to assess the contribution and value of the patient information.
- A short questionnaire will also be sent to the patient organisations to gather their feedback in terms of how they felt about collecting and sharing relevant information with the CHMP (timelines, expectations, etc)
- The pilot should also consider how this interaction will be recorded as part of further actions to ensure that patient input throughout the entire evaluation is captured.
- At the end of the pilot, an outcome report will be presented to the CHMP and Patients and Consumers Working Party (PCWP), including:
 - Organisational aspects
 - Feedback from CHMP
 - Lessons learnt and areas for improvement
 - Proposal for full implementation, if outcome of pilot supports it

ANNEX 1

(template for contacting patient organisations)

<Date>

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Stakeholders and Communication Division

CHMP early contact with patient and consumer organisations.

EMA engages with patients and their representatives at multiple stages of its activities and the added value of including their perspectives in the evaluation of medicines has been well demonstrated. They are currently involved at various timepoints during the medicines' lifecycle and we believe this can be further enhanced by establishing contact with relevant patient organisations at the start of the assessment of new medicines.

The [CHMP](#) provides recommendations on the approval and use of medicines in Europe. Its key task is to assess all the scientific data when a company applies for a marketing authorisation. It decides whether a proposed medicine is made to the proper standards, works well and has benefits in treating the illness that outweigh the risks of side effects ('positive benefit-risk'). The CHMP then issues a positive or negative recommendation and the final decision is issued by the European Commission.

Reaching out to patient organisations when medicines are in active development will enable CHMP members to fully appreciate patients' experience and concerns about their conditions. This will help the CHMP understand aspects that are important for patients, such as quality of life, treatment options, unmet medical needs and what benefits they would hope for in new treatments. Early contact can also guide CHMP on when to seek more in-depth interactions with patients or carers.

The CHMP has started its review of <medicine name/INN> intended to treat <indication> and is inviting <organisation name> to share patients' perspectives on behalf of its patient/carer members. See below for details.

We would appreciate your feedback by <date>.

Your views will be shared with the CHMP and anonymously with the pharmaceutical company who has submitted the marketing authorisation application (personal and organisation names will be removed).

For any questions, please don't hesitate to contact Nathalie Bere (nathalie.bere@ema.europa.eu).

PATIENT / CARER EXPERIENCE OF <disease area>

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 with third parties, as applicable.