



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

# Engagement Framework: EMA and patients, consumers and their organisations



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# Executive Summary

The European Medicines Agency (EMA) has established interactions with its stakeholders since its creation in 1995. Engagement has evolved and adapted as experience has been gained, taking into consideration new legislations, advances in science and crisis situations.

This framework describes the objectives and methodologies for EMA's engagement with patients and consumers and their organisations in relation to medicines for human and veterinary use. It describes the overarching principles developed from experience gained to date and establishes a roadmap for the future.

EMA is committed to ensuring that the patient voice is included in the different regulatory activities of a medicine's lifecycle, which improves the quality of and trust in the regulatory decisions and in new medicines placed onto the EU market.

The framework establishes the basis for:

- supporting access to individual patients' real-life experiences of living with a condition, its management and the current use of medicines. This complementary perspective provides context and understanding of the value of the scientific evidence provided during the evaluation process;
- promoting the generation, collection and use of evidence-based patient experience data for benefit-risk decision-making;
- enhancing patients and consumers understanding of medicines regulation and their role in the process;
- contributing to efficient and targeted communication to patients and consumers to support their role in the safe and rational use of medicines and to foster trust in the EU Medicines Regulatory Network.
- integrating animal owners (consumers) perspectives, where relevant. While to date the majority of EMA's engagement has focused on human medicines, the principles described in this framework also apply to engagement with veterinary stakeholders.

Relevant legislation calls for the inclusion of patients and consumers as members of the EMA Management Board and certain scientific committees. It also foresees consultation of individual experts and organisations in the evaluation of medicines and other Agency activities<sup>1</sup>.

The framework relies on EMA's broad network of patients and consumers organisations, its Patients and Consumers Working Party (PCWP) and the pool of disease-specific individual patient experts. It also acknowledges the necessity to further develop patients' capacities, including training and other support measures, which facilitate integration in the Agency's work while raising awareness about EMA and the European Regulatory Network.

A Stakeholder Engagement report will be presented biennially to the EMA Management Board.

## 1. Introduction

The Agency has engaged with patients and consumers since it began operating in 1995. These stakeholder relations have evolved over time and the type and degree of engagement varies depending upon the particular EMA activity.

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<sup>1</sup> Regulations (EC) No 726/2004; 141/2000; 1901/2006; 1394/2007; 1235/2010

Regulation (EC) No 726/2004 gives responsibilities to the EMA, its Management Board and its various Scientific Committees to develop contacts with patients and consumers. In addition to direct interaction with patients' and consumers' organisations, the legislation also defines the framework for providing them with clear and useful information. Furthermore, the Pharmaceutical legislation calls for the Agency to implement specific forms of interaction with patients' and consumers' organisations and their representatives. For example, patients are represented in the EMA Management Board, the Committee for Orphan Medicinal Products (COMP), the Paediatric Committee (PDCO), the Committee for Advanced Therapies (CAT) and the Pharmacovigilance and Risk Assessment Committee (PRAC). In addition, the Agency has put in place methods to collect patients' input through direct consultation.

The majority of experts involved in the evaluation of medicines are scientists who are made available to the Agency by the national competent authorities of the EU and EEA. Beyond the scientific assessment, there is an established need to understand the patient perspective and to better inform them about the medicines in order to promote their safe and rational use. To achieve this objective, the Agency considers it essential to engage in close cooperation and partnership with its various stakeholders including patients, consumers, healthcare professionals, and learned societies.

The experience acquired to date demonstrates that the participation of patients in the Agency's activities has resulted in increased transparency and trust in regulatory processes and mutual respect between regulators and the community of patients and consumers. It is also acknowledged that their contribution to the evaluation of medicines enriches the quality of the opinion given by the scientific committees and working parties. This positive experience confirms the importance for the Agency to continue supporting and facilitating patient contribution to its work.

Finally, engagement with patients and consumers is a necessary complement to interactions with healthcare professionals and allows EMA to provide a platform of exchange and dialogue at the European level where the views from all users and prescribers of medicines (patients and healthcare professionals) can be considered.

Four levels of stakeholder involvement have been identified in EMA stakeholder relations management framework and are aligned with the EC Better Regulation Guidelines (SWD (2015) 111 final): Inform, Consult, Consult and involve, Cooperate and participate.

## **2. Objectives**

The framework includes the following objectives:

1. Facilitate participation of patients and consumers in regulatory activities all along the lifecycle of the medicines, from early development, to evaluation of benefit-risk, and post-marketing surveillance to capture patients' perspectives and preferences (e.g. disease burden, unmet needs, meaningful outcomes) and obtain information on the current use of medicines.
2. Foster the development of guidance and methodologies to increase collection and use of relevant patient experience data in the context of regulatory decision making (e.g. ICH guidance on collection and use of patient experience data)
3. Ensure that patients, consumers and their representative organisations are consulted and, where appropriate, involved in the development of EMA policies and plans;
4. Support optimal participation of patients and consumers in EMA activities by establishing a training programme that provides good understanding of the activities, their context and the role of patients and consumers.

5. Enhance patients and consumers organisations understanding of the mandate and role of the Agency and the EU Regulatory Network within the context of the development, evaluation monitoring and provision of information on medicines;
6. Optimise communication tools (content and delivery) to facilitate and encourage the cascade of information to the patients and consumers' organisations members (*i.e.* to reach out to individual patients and consumers) with the aim of supporting their role in the safe and rational use of medicines.

### 3. Definition of stakeholders

EMA relies on a broad network of European patients and consumers comprising both organisations and individuals:

**Patients organisations** are defined as not-for profit organisations that are patient focused, and where patients and/or carers (the latter when patients are unable to represent themselves) represent a majority of members in governing bodies.

Relevant organisations include general umbrella organisations (*e.g.* representing either European disease-specific organisations and/or national umbrella organisations) and European disease specific organisations representing national organisations or individual patients on acute and/or chronic diseases.

**Consumers organisations** are defined as not-for profit organisations that defend and promote the general interests of European consumers - citizens as purchasers or users of goods and services.

**Individual patients or carers** are people with experience of living with a particular condition who are interested in engaging with EMA.

Contacts will be preferentially established with EU-wide organisations rather than national ones. However, it is expected that the European organisations act as multipliers for information, dialogue and feedback within each organisation's structure (*i.e.* individual members and/or national level).

### 4. Working methodology

Based on legal provisions and experience so far, patients and consumers participate in the Agency's activities in various ways. They are involved as either representing European patients and consumers, or their own organisation or as individual experts depending on the nature of the activity.

Patients representing their community	Patients representing their organisation	Patients as individual experts
<ul style="list-style-type: none"> <li>• <a href="#">Management Board</a></li> <li>• <a href="#">Scientific Committee(s)</a></li> <li>• Emergency Task Force (ETF)</li> </ul>	<ul style="list-style-type: none"> <li>• <a href="#">Patients' and Consumers' Working Party</a> (PCWP)</li> <li>• <a href="#">EMA consultations</a></li> <li>• Workshops</li> </ul>	<ul style="list-style-type: none"> <li>• <a href="#">Scientific advice / protocol assistance procedures</a></li> <li>• <a href="#">Scientific advisory / ad hoc expert groups</a></li> <li>• Scientific committee consultations</li> <li>• Review of documents</li> </ul>

**Patients representing their community:** as members (and alternates) of some of the Agency's scientific committees (COMP, PDCO, CAT and PRAC) and of the Agency's Management Board. These members are appointed by the European Commission and their [role](#) has been defined<sup>2</sup>.

**Patients representing their organisation:** are consulted and participate in Agency discussions to express the views of their organisation on general issues. When organisations participate in EMA activities they must fulfil the eligibility criteria<sup>3</sup> that include a requirement for the organisation to be fully transparent with regard to their activities and funding sources.

**Patients as individual experts:** Patients and consumers contribute to EMA's activities as individual experts in medicines' evaluations. In this case, they are required to complete a declaration of interest and confidentiality undertaking and abide by the Agency's Code of Conduct<sup>4</sup>.

Patients and consumers also participate as **observers** in certain aspects of the Agency's work.

Interactions with patients and consumers are also affected by time constraints, budget and availability for the organisations and EMA. Streamlining the interactions and focusing on areas where mutual benefit can be anticipated are two underlining principles to consider when implementing the framework.

In order to achieve the objectives identified under section 3, the framework will rely on seven critical elements:

1. A network of European patients' and consumers' organisations;
  2. A permanent forum for exchange with patients' and consumers' organisations; EMA Patients' and Consumers' Working Party (PCWP);
  3. A pool of patients, consumers or carers, registered in EMA's stakeholder database for individuals;
  4. A programme of actions for capacity-building, focusing on training to optimise participation, and raising awareness about the European medicine's regulatory system;
  5. An established range of engagement methodologies that enable the patient and consumer voice to be included along the medicine's regulatory lifecycle
  6. Foster the development of guidance on the generation, collection and use of patient experience data;
  7. Interaction with the EU Regulatory Network.
1. Through its **network of European patients' and consumers' organisations** the Agency has built consistent and targeted interactions with a broad group of organisations across Europe with a diverse range of expertise and interests. These organisations represent the various therapeutic areas of the mandatory scope of medicines evaluated by EMA. The Agency has established a set of eligibility criteria for selection of organisations (endorsed by EMA Management Board)<sup>5</sup> which

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<sup>2</sup> [https://www.ema.europa.eu/en/documents/other/role-members-representing-patients-healthcare-professionals-organisations-ema-scientific-committees\\_en.pdf](https://www.ema.europa.eu/en/documents/other/role-members-representing-patients-healthcare-professionals-organisations-ema-scientific-committees_en.pdf)

<sup>3</sup> [https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/criteria-be-fulfilled-patient-consumer-healthcare-professional-organisations-involved-european\\_en.pdf](https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/criteria-be-fulfilled-patient-consumer-healthcare-professional-organisations-involved-european_en.pdf)

<sup>4</sup> [https://www.ema.europa.eu/en/documents/other/european-medicines-agency-code-conduct\\_en.pdf](https://www.ema.europa.eu/en/documents/other/european-medicines-agency-code-conduct_en.pdf)

<sup>5</sup> [https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/criteria-be-fulfilled-patient-consumer-healthcare-professional-organisations-involved-european\\_en.pdf](https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/criteria-be-fulfilled-patient-consumer-healthcare-professional-organisations-involved-european_en.pdf)

ensure that the Agency establishes contact with the most suitable organisations representing European patients and consumers in a transparent manner.

The evaluation process relies on:

- An open call for expressions of interest via the EMA website; new applicants, may apply at any time. Guidance on how eligibility is evaluated is published on the EMA website, addressing in particular the transparency of funding sources and the relationship between organisations and the pharmaceutical industry.
- Assessment by an evaluation committee within the Agency to determine whether applicants meet the eligibility criteria. Eligibility of the organisations is re-assessed annually by the Agency.
- Publication of the list, logos and description of eligible patients' and consumers' organisations on the Agency website with a link to the organisation webpages.

EMA reaches out first to eligible organisations for involvement in EMA activities.

There will be occasions when the Agency may need to engage with non-eligible organisations when looking for individual patients in specific therapeutic areas (i.e. not covered by an eligible organisation).

2. The **EMA Patients and Consumers Working Party (PCWP)**, is a platform for dialogue and exchange with patients and consumers organisations on relevant issues concerning medicines. Through the PCWP, the Agency provides information and obtains feedback and contributions from patients and consumers on various Agency initiatives. The PCWP also provides a forum to further identify gaps and priorities.

It includes a balanced representation of i) general organisations representing patients and consumers, ii) organisations representing diseases within the mandatory scope of the centralised procedure and iii) organisations representing special populations such as older people and women. Representatives of EMA human scientific committees are also members of the working party. Management Board observers and the European Commission are also invited to participate.

Members of the PCWP are selected from the EMA's eligible patients and consumers organisations.

3. A pool of registered **individual patients** created from different sources:

- Direct requests to the eligible organisations;
- Open call for expressions of interest via the EMA website<sup>6</sup>;
- Active promotion via patient conferences and training;
- Disseminating the link via social media

The pool of experts enables the Agency to quickly and efficiently identify patients that can be involved in medicine-related activities, review of product information and communication material. Registered individuals may also receive information targeted to their area of interest.

Guidance related to registration is published on the website. All personal information on experts included in the database is processed in accordance with [Regulation \(EU\) 2018/1725](#) on the protection of natural persons with regard to the processing of personal data by the Union institutions, bodies,

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<sup>6</sup> <https://fmapps.emea.europa.eu/stakeholders/signup.php>

offices and agencies and on the free movement of such data. There is a specific [privacy policy](#) published on EMA's website.

4. **Capacity-building:** To optimise their contribution, EMA provides patients with a training programme to help them understand the Agency's mandate as well as their expected role in the medicine's regulatory process. The training provided is tailored and based on the particular activity where the patient will participate and is complemented by personalised and one-to-one support. Some patients' organisations or collaborative projects have also developed training to empower patients to play a recognised advocacy role at European level.
5. A range of **engagement methodologies** have been tested and established at EMA and implemented according to the specific activity and timeline. EMA continually looks to incorporate additional methods.
6. To address the increasing need for **patient experience data**, EMA has various options to gather this data, such as early dialogue with patient organisations at start of marketing authorisation application, conducting disease-specific focus groups and contributing to and use of patient preference studies. EMA also contributes to the development of global guidance (e.g. CIOMS, ICH patient focused drug development (PFDD)) and regularly discusses and exchanges with other decision makers and medicines developers.
7. Interaction with the **EU Regulatory Network** in the fields of engagement and communication will provide a valuable contribution to support the existing structures for information dissemination in the Member States. Furthermore, collaboration between these networks will promote the provision of validated and up-to-date information to patients and consumers on the benefits and risks of medicines and contribute to the preparation and dissemination of clear messages on the safe and rational use of medicines intended to reach the public across the EU.

## 5. Monitoring and reporting

A [report on stakeholder engagement](#) including an analysis of participation, feedback received from patients and consumers and their representative organisations through targeted surveys, work of the PCWP and an overview of joint activities of patients, consumers and healthcare professionals, will be presented to the EMA Management Board on a regular basis.

Every two years an overall satisfaction survey is carried out with patients and consumers who contributed to the Agency activities.

The work to be undertaken in the context of the Agency's engagement with patients, consumers and their organisations will be reflected in the joint PCWP/HCPWP work plan, and the Engagement Strategic Plan with a dedicated Action plan for patients and consumers.