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Annex II: EMA activities where patients* and consumers are involved

Patients and consumers are involved in a diverse array of Agency activities[1] either as **individual experts** (as indicated in Figure 1) where they provide input on product-specific issues or where they represent a **patient organisation**.

These activities include:

- participation in governance of the Agency via the Management Board;
- inclusion in scientific committees as members;
- contributions on disease and product-specific questions via Scientific Advice Working Party (SAWP) and Scientific Advisory Groups (SAG); and consultation by the EMA scientific committees;
- consultation on guidelines and policies;
- review of documents destined for the public;
- dissemination of information;
- participation in workshops, networks and research projects.

1. Non product-specific involvement in EMA activities

Where patients/consumers are invited on non-product specific matters such as those described below, they are considered to be representing their patient organisation or disease-specific community.

1.1. EMA Management Board (MB)

The Management Board has a supervisory role with general responsibility for budgetary and planning matters, the appointment of the Executive Director and the monitoring of the Agency's performance (Rules of Procedure)[2].

Two patients sit on the Management Board[3].

* The term 'patient' is used throughout this document to refer to any individual who represents the views and interests of patients in EMA activities.

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1.2. European Medicines Agency Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP)

While the EMA has engaged in dialogue with patients and consumers since its creation in 1995, a formal working party, European Medicines Agency Human Scientific Committees' Working Party with Patients' and Consumers' Organisations (PCWP), has existed since 2006. The Agency engages with patients and consumers via a network of over 30 European **patients' and consumers' organisations** (called eligible organisations). The PCWP is a core group of representatives from these eligible organisations that provide recommendations to the EMA and its Human Scientific Committees on **all** matters of direct or indirect interest to patients in relation to medicinal products (Mandate)[4].

Particular attention has been paid to include patients' groups representing the medicinal products that fall within the mandatory scope of the centralised procedure (i.e. HIV, cancer, neurodegenerative disorders, diabetes, auto-immune diseases, viral diseases, biotech products, monoclonal antibodies, innovative products and products for rare diseases).

In addition, groups representing the interests of special populations such as paediatric, older people, women or certain disease-specific organisations are also represented.

Patients are predominantly identified via the eligible organisation for the following activities:

1.3. Involvement in workshops, conferences, webinars etc..

Patients/consumers are invited to most EMA workshops and increasingly are involved as speakers within the sessions.

Some examples of workshops include Medication errors, Patient-support programmes and market-research programmes and the Pharmacovigilance Stakeholders Forum.

2. Product-specific involvement in EMA activities

Where patients/consumers are consulted on product-specific matters, they are involved as individual experts and are required to complete all the same documents as all other experts prior to attending the meeting at EMA. These documents include the Confidentiality Agreement, Declaration of Interests and curriculum vitae.

2.1. EMA scientific committees

Patients have been included as full voting members of EMA scientific committees since 2000. The Committees that include patients are listed below as well as the year of their creation. Activities that are covered by these committees and hence patients include orphan designation of medicinal products, assessment of paediatric investigation plans, classification of advanced therapies and assessment and monitoring of safety issues of medicines.

- COMP[5] - Committee for Orphan Medicinal Products (since 2000)
- PDCO[6] - Paediatric Committee (since 2007)
- CAT[7] - Committee for Advanced Therapies (since 2009)
- PRAC[8] - Pharmacovigilance and Risk Assessment Committee (since 2012)

A detailed document describing the role of patients as members of the EMA Human scientific committees[9] was elaborated in collaboration with the EMA patient representatives.

Committees can also consult patients on an ad-hoc basis on product-specific issues where their input on the medicine under evaluation, based on their real-life experience with the disease and/or the product is of added value.

2.2. Scientific Advice Working Party

Pharmaceutical companies can request scientific advice from the European Medicines Agency (EMA) regarding the development of a medicine. This advice is aimed at ensuring the company carries out the most appropriate studies, thus avoiding major objections related to the study design during the marketing authorisation evaluation, which can significantly delay the authorisation, or even result in its refusal.

The Agency also offers parallel scientific advice with health-technology-assessment (HTA) bodies. The aim of this is to allow medicine developers to gain feedback from regulators and HTA bodies at the same time, early in the development of a medicine.

Patient representatives[10] were invited to participate in EMA scientific advice procedures in the context of orphan medicines (called Protocol Assistance) from 2005. This was then extended to medicines for non-rare indications in January 2013. Patients are invited to share their 'real-life' perspective and experience (in a face-face meeting or via written comments) with the Scientific Advice Working Party (SAWP)[11] and the pharmaceutical company, in relation to a particular medicine in their disease area.

2.3. Scientific Advisory/ad hoc Expert Group meetings

Scientific Advisory Groups (SAGs) are convened at the request of the Committee for Medicinal Products for Human Use (CHMP) or PRAC to deliver answers, on a consultative basis, to specific questions addressed to them (mandate)[12]. Currently eight SAGs exist for specific areas.

When the issues refer to a therapeutic area for which no specific SAG has been constituted, an ***ad-hoc* expert group** is organised that follows the SAG mandate.

The outcome of the 2011 pilot phase, resulting in the systematic inclusion of patients in Scientific Advisory Group (SAG) meetings, is published[13].

2.4. Patients at CHMP

A pilot[14] (beginning Sept 2014) to include patients directly in the benefit-risk evaluation of medicines with the Committee for Medicinal Products for Human Use (CHMP) will explore how patients can be involved effectively in oral explanations at the CHMP.

Inviting patients to participate in the CHMP will also increase patient awareness of the committee's deliberations and makes the assessment process of medicines more transparent.

2.5. Public hearings

Public hearings are a new tool for the EMA to engage European Union (EU) citizens in the regulatory process of the supervision of medicines. The meetings will be held by the Pharmacovigilance and Risk Assessment Committee (PRAC) and are expected to give EU citizens a voice in the evaluation of the safety of medicines and empower them to express their views on issues related to the safety of certain medicines and the management of risks.

2.6. Future involvement of children (and/or their parents/carers/legal representatives) and plans for strengthening the participation of members representing patients' organisations at the PDCO

Further to the recommendations outlined in the published concept paper on the involvement of children and young people at the PDCO, the possibility of establishing a framework of interactions between EMA, PDCO and children (and/or their parents/carers/legal representatives) is currently being discussed at the PDCO. Primary discussions focus on defining the scope of the involvement of children (and/or their parents/carers/legal representatives), their role, expectations and the clear criteria on which situations, a consultation and/or dialogue between the PDCO and children (and/or their parents/carers/legal representatives) may occur.Also, in order to strengthen the participation from members representing patients' organisations at the PDCO plenary meetings, it is planned to define the clear criteria, on which situations, a systematic feedback and input from these members may be required in the PDCO day-to-day activities.

3. Communication:

3.1. Review of documents destined for the public:

The EMA invites patient and consumer representatives to review information on medicines published by the Agency, such as European Public Assessment Report (EPAR) summaries for the public and Package Leaflets as well as information on the safety of medicines that are addressed to the public (Training manual)[15]. The Agency also invites patients to review Public Summaries of Opinion (PSO) on orphan designation.

3.2. Consultations

Patients/consumers are consulted on guidance and policy documents via the EMA website as well as targeted emailing to ensure to capture the input of this group. In addition, patients can be consulted on an ad-hoc basis on product-specific topics linked to the prevention of medication errors, such as educational and packaging materials.

Patients are consulted along with healthcare professionals and other experts, in closed workshops, on the guidelines for the clinical investigation of disease-specific medicinal products (e.g. Duchenne Muscular Dystrophy, Cystic fibrosis…)

3.3. Meetings, conferences and workshops

Four meetings for the PCWP are organised on average annually, several of these are combined with the HealthCare Professionals Working Party (HCPWP) and one meeting with all eligible organisations is also held annually.

A number of conferences and workshops are also held where patients are invited to participate as presenters and attendees.

3.4. Human Medicines Highlights (HMH) newsletter

A monthly newsletter addressed primarily to organisations representing patients, consumers and healthcare professionals provides a summary of key information relating to medicines for human use published during the previous month by the EMA.

3.5. EMA website

The homepage of the EMA website contains a feed of all of the Latest Information and Press Releases. Under the News and Events tab a scrolling feed of 'What's New' also provides links to monthly information and Press Releases.

3.6. Patient and Consumer pages

Patients and Consumers have a dedicated area in the Partners and Networks section of the website providing information on Agency activities where patients and consumers are involved, how they can get involved, which organisations are currently involved with the EMA as well as training and supporting key documents for these activities.

3.7. Send a Question and Social media

Patients and the public in general can request information directly to the EMA from the home page using the Send a Question form.

RSS feeds, Twitter and YouTube are available to subscribers. The EMA uses these means to communicate up to the minute information on authorisations, consultations (Twitter), newsletters on a range of topics (RSS feeds) as well as to transmit information and training (YouTube).

3.8. Leaflet on patient activities at EMA

A leaflet describing the different ways that patients can be and are involved with the EMA has been elaborated and is updated regularly as activities evolve.

3.9. Information exchange

The eligible organisations working with EMA serve as a vital platform for exchange of information.

The EMA consults and informs these groups on relevant Agency activities via targeted emailing. An identified contact person is the recipient of relevant information that is then shared with the members of that organisation.

The eligible organisations support the Agency in providing targeted information in appropriate language to stakeholders and enhance understanding of the regulatory processes. A survey showed that patients' and consumers' organisations disseminated EMA information via links or publication of press releases and Q&A on their website or via social media channels.

4. Monitoring / Training

4.1. Monitoring – surveys and feedback on satisfaction

In order to monitor the satisfaction of patients and consumers who have been involved in any EMA activity, the EMA organises satisfaction surveys every two years. Based on the outcome of these surveys, actions plans are then determined. The results are included within the annual report (REF).

Surveys on the provision of EMA training to patients and consumers involved in its activities are also conducted.

4.2. Training

Patients and consumers are invited to be involved in a variety of activities at the EMA. In order to support and optimise their involvement, the Agency has prepared a training programme to describe the specific training activities and material that will be made available to patients and consumers when taking part in EMA activities and events.

These include documents describing the procedures and the role of the patients; support on how to complete the forms prior to attending a meeting; a video for those attending a meeting at the EMA and in-house training on various patients' activities at EMA.

A number of external training initiatives for patients already exist or are being developed in the EU, some of which are supported by the EMA.

4.3. Involvement in EU-wide projects/Research

The EMA is involved in several research projects in varying capacities. Where possible and increasingly so, patients are invited to participate as partners, in steering groups etc..

ENCePP: The European Network of Centres for Pharmacoepidemiology and Pharmacovigilance is a network of over 170 research centres, existing networks and providers of healthcare data, which is coordinated by the European Medicines Agency. Patients' representatives form part of the Steering Group and the Interested Parties and Stakeholder group.

Enpr-EMA: The European Network of Paediatric Research at the European Medicines Agency is a network of research networks, investigators and centres with recognised expertise in performing clinical studies in children. Patients are involved in the Coordinating Group. Also, a task of a new Enpr-EMA working group, established following the 6th annual Enpr-EMA workshop in June 2014, is to develop a virtual European network of young people to input into the design and delivery of clinical research in children.

EUCTR: The European Union Clinical Trials Register enables searches for protocol and results information on interventional clinical trials that are conducted in the European Union (EU) and the European Economic Area (EEA) and clinical trials conducted outside the EU / EEA that are linked to European -medicine development. Patients were and are still consulted all along the development of this portal regarding aspects from design to information to be included.

IMI-PROTECT: The Pharmacoepidemiological Research on Outcomes of Therapeutics by a European Consortium is a collaborative project that addresses limitations of current methods in the field of pharmacoepidemiology and pharmacovigilance. The EMA is the coordinator of PROTECT and GSK is the deputy co-ordinator of PROTECT. The partners list includes IAPO the International Alliance for Patients' Organisations.

WebRADR: (Recognising Adverse Drug Reactions) is a consortium of leading experts in pharmacovigilance from regulatory agencies, research and academia. The aims will be to set policy and guidance and deliver robust information technology tools to address the potential for the reporting of adverse drug reactions (ADRs) through mobile applications and the recognition of drug safety signals from user comments in social media and the internet. The consortium includes EURORDIS. Additional patients' organisations will be involved in the project through participation in project-related workshops and follow-up activities (e.g. surveys).

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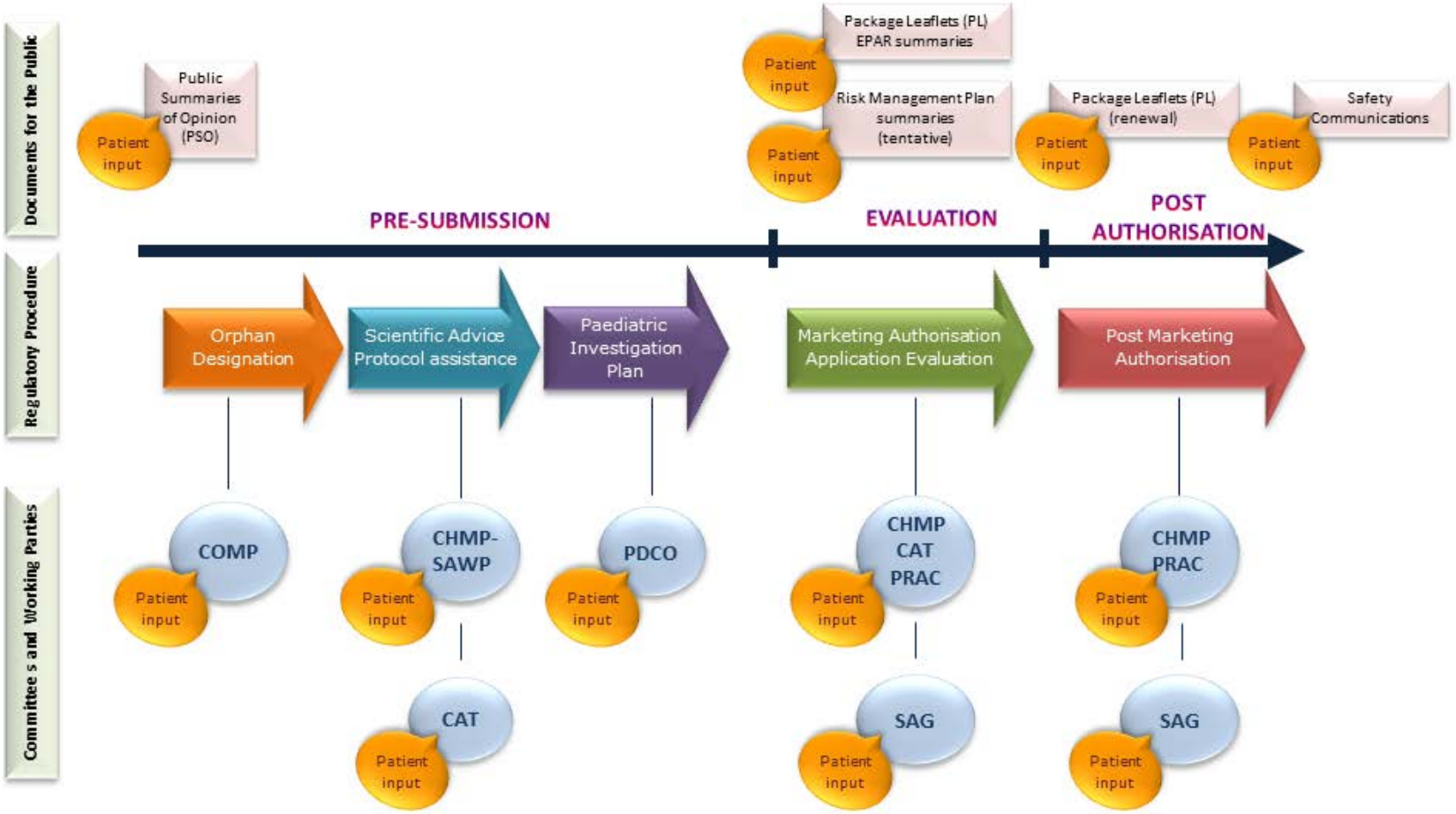
Figure 1: Product-specific patient contributions in the product life cycle



* COMP – Committee for Orphan Medicinal Products; CHMP – Committee for Human Medicinal Products; CAT – Committee for Advanced Therapies; PDCO – Paediatric Committee; SAWP – Scientific Advice Working Party; SAG – Scientific Advisory Group; PRAC – Pharmacovigilance and Risk Assessment Committee; EPAR – European Public Assessment Report