



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH



**Principles on the involvement of young patients/consumers within EMA activities**



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## Principles on the involvement of young patients/consumers within EMA activities

The purpose of this document is to establish the principles for the involvement of young patients, consumers, and their carers, in the activities of the Agency's scientific committees and working parties in a consistent and efficient manner, whenever such involvement is appropriate in the interest of the ongoing scientific assessment within a particular (scientific) committee. This document aims at:

- Identifying in which situations it may be helpful to seek input from young patients/consumers their carers and the organisations they may be members of;
- Defining what is expected from young patients/consumers/carers' involvement when they are consulted, and how best to capture their opinions;
- Establishing an appropriate process to identify, support and consult with them.

### 1. Introduction

Since its creation EMA has engaged in a constant dialogue and interaction with patients, consumers and their carers. The EMA Management Board has endorsed a [framework of interaction](#) which defines the structure and formalisation of the interaction and also tackles the challenges in ensuring the patients' voice is heard throughout the Agency's different activities in a manner which is mutually beneficial. In this respect, although progress reports show that the work achieved so far has established a systematic interaction and involvement of patients and consumers within many different Agency activities, there is still a need to further enhance interaction in some areas; one such area relates to the inclusion of the views and opinions of young patients/consumers and their carers, wherever it may be appropriate.

Historically, children have been underrepresented, and often excluded, from clinical research despite evidence that it can have many advantages; in addition to promoting children's rights, wellbeing, and development of self-confidence, involving young people has been shown to improve health services targeted to children.

Any reference to consulting with "young people" at EMA is typically linked to teenagers/adolescents or even older children able to respond to the type of questions a scientific committee may ask, which would depend on their individual knowledge, experience and maturity. In addition, such consultation



would only be initiated when it is felt that the perspectives of young people would enhance a specific discussion related to the development and assessment of paediatric medicines.

## 2. Legal basis

Article 78(2) of Regulation (EC) No 726/2004<sup>1</sup> allows the EMA scientific committees and their Rapporteurs to establish contacts, on an advisory basis, with parties concerned with the use of medicinal products, in particular patient organisations and healthcare professionals' associations.

Following the entry into force of Regulation (EC) No 1901/2006<sup>2</sup> in 2007, the Agency has an increased responsibility to consult the views and opinions of young people on medicines that are for their use, when such interaction can be of mutual value and benefit.

In accordance with these legal provisions, principles are proposed herein to foster the interaction between EMA's scientific committees and young patients/consumers/carers, whenever appropriate. Extra attention needs to be taken to ensure that the rules and modalities of participation sufficiently take into account and accommodate the specific requirements for these individuals (and their parents/carers) due to their potential vulnerability (e.g. parental consent, child-sensitive environment, etc.).

## 3. Potential contribution from young patients/consumers/carers

Reference is made to the document "The role of patients as members of the EMA Human Scientific Committees" ([EMA/351803/2010](#)). Although this document focuses on the role of (adult) patient members of scientific committees, some of the principles laid down can be extrapolated and applied accordingly to young patients/ consumers and their carers. Young patients/consumers/carers are able to contribute to the predominantly scientific discussions, with their unique perspectives based on real-life experience of living with a disease or condition. This element fills a gap which other (scientific) experts cannot fill, and which has proven valuable in achieving the best possible outcomes for all those concerned.

Involving young patients/consumers/carers at this level will also increase their understanding and trust in healthcare; while literature is scarce on the impact of participation of children in healthcare decisions, there is evidence that it can have a positive effect on the outcome of their treatment. For example, it can lead to increased adherence, adaptation, as well as a sense of competence and understanding of their illness (Angst and Deatrck, 1996). Moreover, the way adolescents are treated by healthcare professionals has been shown to be an important predictor of their satisfaction with healthcare (Beresford and Sloper, 2003). Similarly, research on informed consent indicates that patients who have been involved and have received more information tend to respond better to treatment.

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<sup>1</sup> Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency (OJ L 136, 30.04.2004, p. 1).

<sup>2</sup> Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004 (OJ L 378, 27.12.2006, p. 1).

## 4. Scope

Young patients/consumers and their carers should only be consulted in cases where their involvement can bring added value to the discussion, and it is for EMA/committee members/rapporteurs to decide on a case-by-case basis when it would be beneficial to consult them and the manner in which this consultation should take place.

Prior to the involvement of young patients/consumers/carers, the format of contact and dialogue for consultation needs to be agreed upon (for example, in person, via teleconference, in writing), and in addition necessary measures need to be in place to ensure that those participating do so in an appropriate manner. In this regard the following points need to be considered:

- Obtain parental consent when required (participation of minors under 18 years old);
- Check if young person is able to express him/herself in English;
- The young patient/consumer/carer being consulted should be made fully aware, in advance, of the proposed nature and scope of the engagement, including:
  - how and where it will take place (according to their preferences, taking into account feasibility of implementation);
  - the registration/conflict of interest/Confidentiality documents required to be filled in by the legal guardian (with EMA assistance / personal guidance throughout);
  - what is their expected contribution (i.e. there is no wrong answer/feedback, it is their personal experience that is of value);
  - what kind of general and personalised support and information will be provided to them, including if possible a “mentor” speaking patient’s own language to explain everything;
  - what will be the follow up after the interaction and the way the contribution will be recorded in the minutes.
- The committee/working party members and EMA staff involved in the engagement with young patients and their carers should be prepared to accommodate them in an appropriate manner and environment (e.g. small groups, appropriate language, support etc.)
- Due consideration shall be given to the protection of personal data and the privacy of the young patients

It is anticipated that the key forum where engagement with young patients/consumers/carers would occur is within the Paediatric Committee (PDCO), but could also be within the Committee for Human Medicinal Products (CHMP) and the Pharmacovigilance Risk Assessment Committee (PRAC), and potentially the Scientific Advice Working Party (SAWP) when these committees/working parties review paediatric medicines.

Examples where consultation/involvement could be of benefit are:

- During PDCO evaluation of Paediatric Investigation Plans;
  - Proposed clinical trial design features (e.g. endpoints, randomisation, placebo, visit frequency, study duration, number of tests);
  - Acceptability of route of administration, formulation, palatability, frequency of dosing, container systems and other packaging issues.

- Evaluations within COMP/PRAC/SAWP on specific paediatric medicines (pre & post-authorisation);
- Definition of therapeutic needs (not product specific), e.g. the development of guidelines.

Young patients/consumers/carers will be selected via the Agency's network of 'patient and consumer organisations', specifically those who have experience working with young people. The Patients and Consumers Working Party (PCWP), together with EnprEMA and the other EMA eligible patient/consumer organisations, are working on establishing appropriate contacts with youth groups across Europe which will be the primary source of contact with young persons.

In order for this collaboration to occur in the most optimal manner, it is also proposed that appropriate training materials be developed, e.g. videos, voice-over slides, short info-sheets.

## 5. Format of the interaction

Once a decision has been made to consult with young patients/consumers/carers (following agreement of guardian/patient) the most appropriate mode of interaction should be decided, taking due consideration of resources as well as minimising interference in committee operations; and taking into account the age range of the young people and their preferred mode of involvement.

Proposals on the manner in which young patients/consumers/carers could participate include:

### In writing

- The Committee secretariat together with (Co-) Rapporteur will agree on the questions to be put to the patient/consumer/carer(s). The question(s) will be circulated to all members for endorsement;
- Background information will be provided to the patient/consumer/carer(s) to allow for an adequate understanding of the issues under review and the questions being asked, in an appropriate format;
- The relevant patient/consumer/carer(s), as identified by the EMA secretariat, is/are consulted and given a deadline for his/their response(s).

### Via teleconference

- The same procedure as described above applies, however, upon request of the (Co) Rapporteur and/or the scientific committee, the patient/consumer/carer may join the committee discussion via teleconference;
- When necessary, written responses may be received in advance of the discussion;
- Alternatively, a teleconference may be held with a selected group of persons, e.g. (Co-) Rapporteurs, assessors and EMA secretariat, without involving the whole committee.

### In Person

- Young patient/consumer/carer(s) could be invited to participate during a committee meeting if this is felt the most useful manner;
- They would attend only the part of the meeting, or a break out session, related to the specific issue under discussion.

It is also important to highlight that the recent introduction of public hearings at EMA (within the framework of the pharmacovigilance legislation) is also an opportunity for young people to share their views and experience with EMA, within safety referral procedures in the Pharmacovigilance Risk Assessment Committee (PRAC). The published guidance for participants of public hearings already

foresees the possibility for young people, aged 12 and above, to attend as observers or speakers, accompanied by their parents. The overall principles set out in this document support and complement the participation of young people in public hearings.

## 6. Conclusion

The above-mentioned principles establish the framework under which young patients/consumers and/or carers could be consulted on a case-by-case basis by the EMA scientific committees in relation to discussions on medicines for paediatric use, if:

- their involvement would be beneficial in order to gain relevant information for the elaboration of a scientific opinion;
- the proposed interaction is carried out in a manner adapted to the particular age range involved, and that the “interviewers” involved are sufficiently prepared in order to respect the sensitive nature of the interaction.

These principles should be regularly reviewed and revised when needed to take into account experience gained.



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