EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Patient involvement in medicines evaluation within the European Medicines Agency

ECPC General Assembly

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- Access to medicines in the EU: the role of the European Medicines Agency (EMA)
- The importance of patient input

What is the European Medicines Agency (EMA)

The EMA is the EU regulatory body responsible for the scientific evaluation and supervision of medicines developed by pharmaceutical companies for use in the European Union (Human and Vet)

What is the role of the EMA?

The Agency is responsible for:

- The evaluation of marketing authorisation applications submitted by pharmaceutical companies (centralised procedure)
- The coordination of European pharmacovigilance (supervision of the medicines on the market)
- The provision of scientific advice on the development of medicines
- The evaluation of applications for orphan designation in EU
- The evaluation of paediatric investigation plans (or waivers)
- The provision of good quality and independent information on the medicines it evaluates to patients and health professionals

The EMA is <u>not</u> responsible for pricing or reimbursement

Marketing Authorisation – key principles

- In order to sell a medicinal product in the EU, a company needs a Marketing Authorisation
- There are a number of ways ('Procedures') for a company to obtain a marketing authorisation
- The main scientific principle used in the evaluation of medicines is the benefit/risk balance, based mainly on quality, efficacy and safety aspects

Marketing approval for medicines - Two European Systems

Centralised Procedure

(via EMA)

Mutual Recognition / Decentralised Procedure (national licences)

Both Systems allow

Better Resource Utilisation
Harmonised Scientific Opinions
Harmonised Information to doctors & patients

Centralised Procedure

- 1 application
- 1 evaluation
- 1 authorisation for all EU
- 1 product information (SPC, Labelling, PL)
- All EU languages

Which medicines are evaluated at the EMA?

- Rare diseases
- HIV, cancer, neurodegenerative disorders, diabetes
- Auto-immune diseases, viral diseases
- All biotech products
- Gene therapy
- Monoclonal antibodies

+ Other innovative products

How does the Agency work?

Scientific Committees:

- Committee for Medicinal Products for Human Use (CHMP)
- Committee for Medicinal Products for Veterinary Use (CVMP)
- Committee for Orphan Medicinal Products (COMP)
- Committee for Herbal Medicinal Products (HMPC)
- Paediatric Committee (PDCO)
- Committee for Advance Therapies (CAT)
- Pharmacovigilance Risk Assessment Committee (PRAC)

Working parties:

- Provide support to the Committees

The EMA network

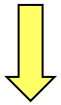
The Agency works with:

- More than 40 national authorities
- Over 4000 EU Experts
- European Parliament
- European Commission
- Establishes relations with non-EU regulatory authorities, international health organisations, industry, academia and the general public

How does the Agency work? (Cont.)

EMA and its committees, working parties & experts:

Formulate scientific opinions



Send to the European Commission

Commission Decision

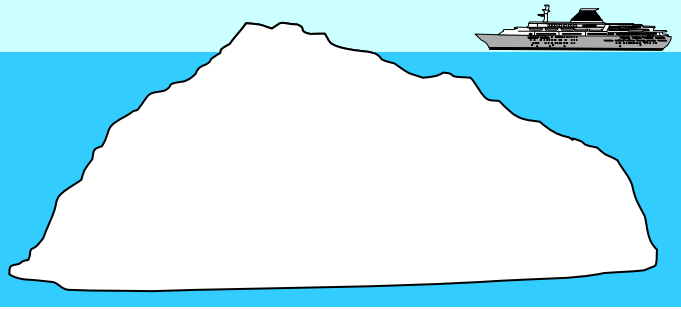
(Pan European Marketing Authorisation)

Pharmacovigilance and Risk Management

What we know at the end of the clinical trial programme…

What we don't know!

. What happens when the medicine is used in normal practice?
. What is its adverse event profile?



The importance of patient input for the work of the EMA

Interaction with patients - background

- Since the beginning the EMA has been engaging with patients & consumers
- Based on a "Framework of interaction", adopted in 2005
- The framework comprises:
  - Scope of the interaction
  - Objectives to be achieved
  - Working methodology
  - Monitoring (including performance indicators)
- Ultimate goal:
  - Involve patients in the Agency's activities
  - Better inform patients

Eligible organisations

- Any organisation representing EU patients or consumers may express an interest to work with the Agency, however they must meet the defined eligibility criteria (application form on EMA website)
- Launched in 2005 and “continuous”
- List of eligible patients’ & consumers’ organisations published on the EMA website

Eligible organisations

Patient involvement in EMA activities (1/3)

- **EMA Working Party with Patients' and Consumers' Organisations (PCWP)**
  - 19 eligible organisations + representatives from Agency's committees
  - To provide recommendations to the Agency and its Committees on all matters related to medicines; a platform/forum for exchange between patients and regulators
- **Members of:**
  - Management Board (MB)
  - Committee for Orphan Medicinal Products (COMP)
  - Paediatric Committee (PDCO)
  - Committee for Advance Therapies (CAT)
  - Pharmacovigilance Risk Assessment Committee (PRAC)

Patient involvement in EMA activities (2/3)

Product development:

- Participation in scientific advice/protocol assistance

Benefit/risk evaluation:

- Participation in scientific advisory groups (SAGs)
- Respond to ad-hoc requests on assessment of medicines from scientific committees and working parties
- Review of information on medicines: Package leaflets, EPAR summaries, safety communications (Q&As) and other Agency documents for the public

Patient involvement in EMA activities (3/3)

General:

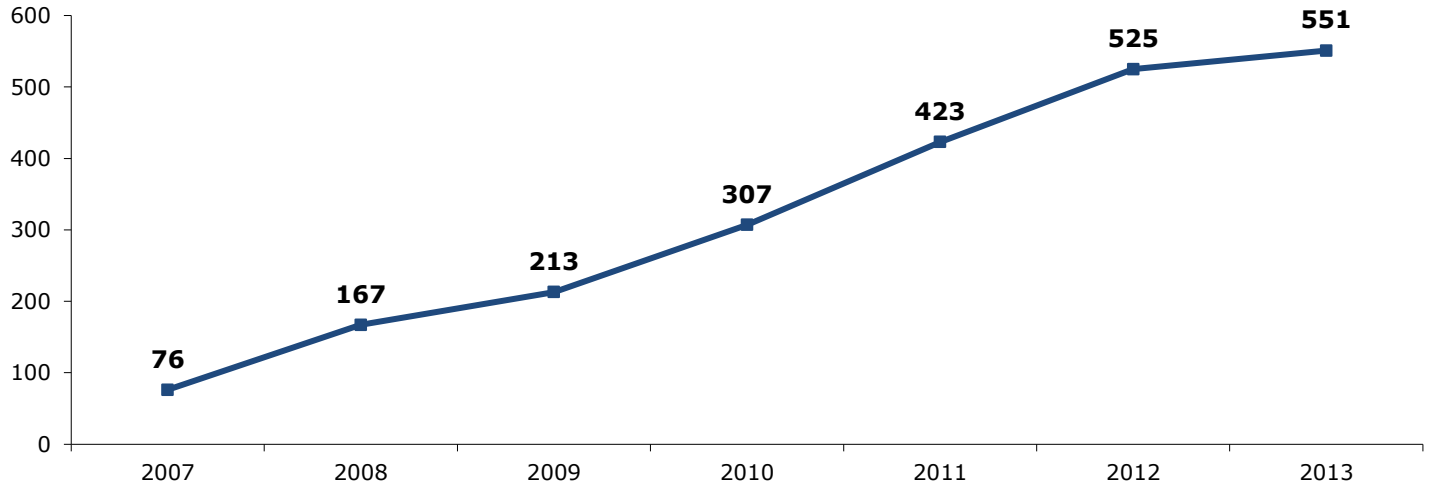
- Input in the preparation of guidelines
- Involvement in several on-going EU-wide initiatives, e.g:
  - EudraCT (EU clinical trials register),ENCEPP (European Network of Centres for Pharmacoepidemiology and Pharmacovigilance), and Enpr-EMA (European Network of Paediatric Research)
- Participate in Agency conferences and workshops

Increasing involvement

- The interaction between the Agency and patients/consumers has been increasing steadily since 2005; both in numbers and range of activities
- Eligible organisations increased from 19 in 2007 to 37 today
- PCWP membership was enlarged to 19 organisations in 2013
- Involvement is more formal and systematic

Number of patients involved in recent years

Overall number of patient & consumer involvement in EMA activities
2007–2013



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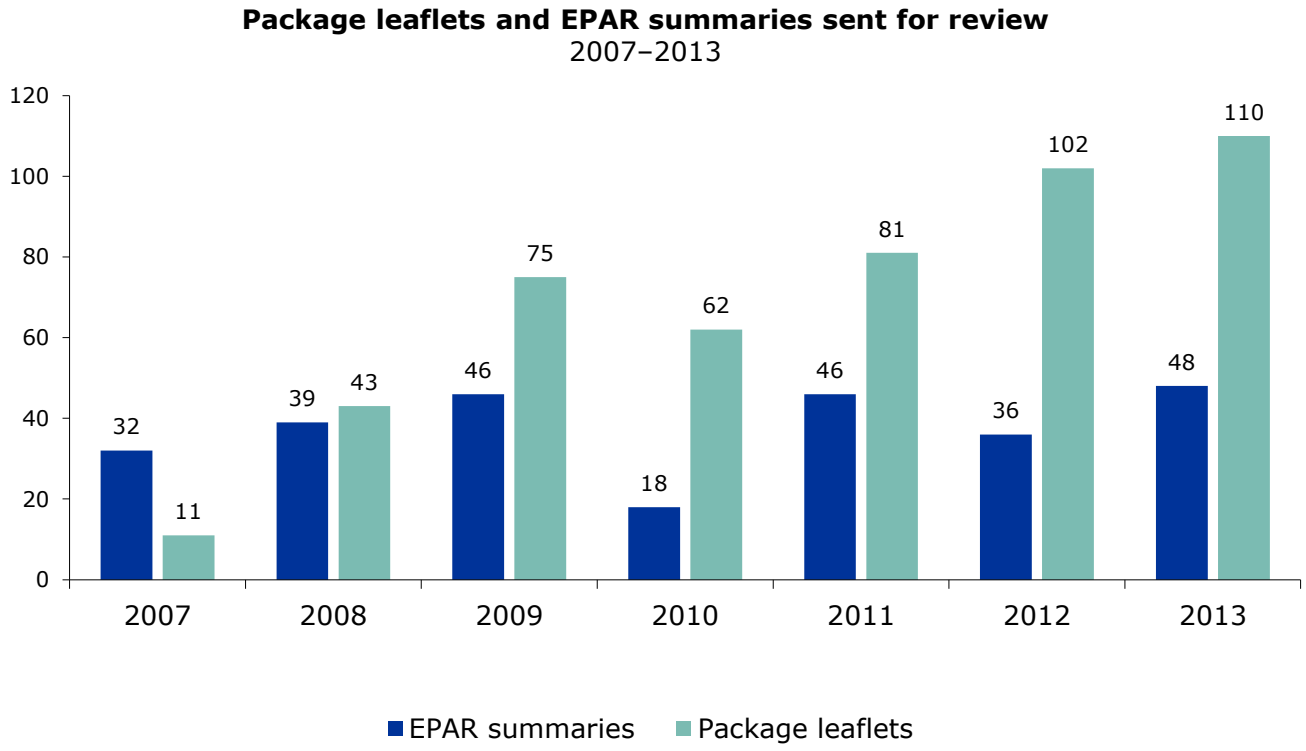
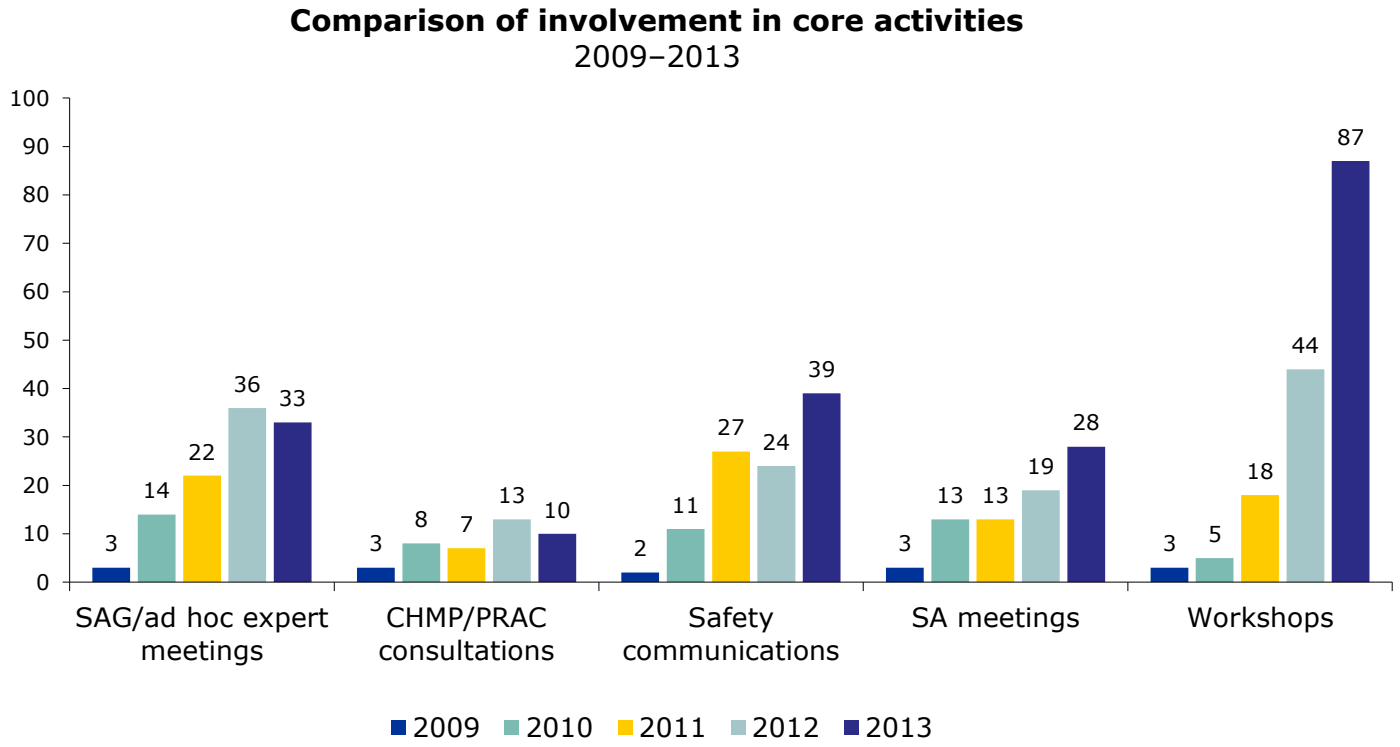


Increasing range of EMA activities



Added value of involving patients in EMA

- They represent patients interests and bring unique real-life experiences and "patient perspectives", which complements the scientific data
- They help ensure quality of patient information and communication on medicines
- Help disseminate committees' outcomes when they become public; passing on information to other patients and patients' organisations
- The added value that 'lay experts' bring to the Agency's activities is well acknowledged

Challenges

- Resources within the organisations
- Finding suitable experts (e.g. language barrier, availability)
- Need for tailored training on regulatory environment to facilitate participation
- Definition of patient role in the different activities / committees

The impact of interaction

- Improves the outcome of regulatory decisions
- Increases transparency and builds confidence in the regulatory system
- Involvement at operational level leads to tangible impact on outcomes e.g.
  - Patients' views on benefit-risk deliberations contributes to final recommendations from the committees
  - Review of product information and safety communications - comments are taken into account.

Conclusion

- This collaborative interaction allows patients to share their experiences and provide meaningful feedback on the real-life implications of regulatory outcomes, which ultimately contributes to the quality of the medicines assessment and outcome.
- We continue to endeavour to address the challenges in order to open further our activities to more medicine users throughout Europe

Any questions?