Regulatory agencies perspectives on involving patients in drug development and decision making

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Collaboration with patients
EMA journey... so far

1995
- EMA created

1996
- Dialogue with HIV patients

2000
- Patients join COMP as full members

2003
- Working group with patients created

2005
- Framework of interaction with patient and consumer organisations

2006
- Patients and Consumers Working Party (PCWP) created

2014
- Systematic inclusion of real life experience EMA regulatory output

2017
- Dedicated Department created & framework updated

2017
- Ongoing...

- Public Hearing

- Systematic inclusion of real life experience EMA regulatory output

- Dedicated Department created & framework updated

- Public Hearing
A framework of interaction based on five critical elements

1. A network of European patients and consumers organisations
2. A forum of exchange: EMA Working Party with Patients and Consumers’ organisations
3. A pool of patients acting as experts in their disease and its management
4. Interaction with the EU Regulatory Network
5. Capacity-building focusing on training and raising awareness about EU regulatory system
Patient involvement along the medicine lifecycle at EMA

PRE-SUBMISSION
- Orphan Designation
- Scientific Advice
- Paediatric Plan

EVALUATION
- Marketing Authorisation Evaluation
- CHMP CAT PRAC COMP
- Expert mtg

POST AUTHORISATION
- Post Marketing procedures
- CHMP PRAC
- Expert mtg

Public Summaries of Opinion
- Package Leaflets (PL) EPAR summaries
- Package Leaflets (PL) (renewal)
- Safety Communications
- Public Summaries
- Public Summaries of Opinion
- Patient input
Increasing involvement in EMA activities

Overall number of patient & consumer involvement in EMA activities  2007–2016
Three ways to participate

1. Patients representing the *patient community*

2. Patients representing *their organisation*

3. Patients as *individual experts*
Patients representing the patient community

Members of:

- EMA Management Board (MB)
- Committee for Orphan medicines (COMP)
- Committee for paediatric medicines (PDCO)
- Committee for advanced therapies (CAT)
- Pharmacovigilance Risk Assessment Committee (PRAC)
Patients representing their organisations

- **PCWP** - Platform for dialogue and exchange between the EMA and patient organisations
- Representation from 20 organisations, EMA committees & Management Board
- Four plenary meetings per year, and consulted as and when needed

**2016:**

10th Anniversary!
Patients representing their organisations

- Discuss EMA policies and initiatives
- Help implement new legislation
- Participate in EMA conferences and workshops
- Respond to EMA consultations
- Comment on draft guidelines

Other EU-wide initiatives:
- EMA activities to implement EC’s recommendation to improve product information
- EnprEMA
- ENCePP
Patients as individual experts

**Medicines’ development:**

- Participation in **scientific advice/protocol assistance** procedures
- **Orphan designation** and **Paediatric investigation plan**

**Medicines’ evaluation:**

- **Expert meetings, SAGs** and **Ad-hoc consultations** during the assessment of medicines conveyed by all Committees
- **Oral explanations** at CHMP
- **Review information on medicines** - **Package leaflets, EPAR summaries, safety communications** (Q&As)
CHMP pilot - patients in oral explanations

- Based on demonstrated added value of patients in benefit-risk evaluation

- Feedback from CHMP/EMA received during pilot is generally **positive**
  - Patients report a very positive experience; increases transparency and trust in the work of the CHMP

- Involvement has been a **learning curve** and has **improved with experience**
  - More relevant questions for the patients; focused on the assessment
  - Everyone involved knows better what to expect

- **NOW** – **Systematic participation** when added value anticipated
Engagement methodologies

- Face to Face
- Conference call
- Committee meetings
- Surveys
- In writing

Patients, Healthcare Professionals and Academia at EMA
Monitoring and measuring

- Feedback collection from patients
- Proposals for improvements included in the next work-plan
- Annual report to EMA Management Board

Feedback

- Meeting minutes
- Thank you acknowledgement
- Comments taken into account

Review of documents

70% of comments led to changes
Who do we work with?

- **EMA Network of patients organisations and patients experts**
- 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 the [EMA website](https://www.ema.europa.eu))
- Launched in 2005 and “continuous”
- List of eligible patients & consumers organisations published on the EMA website
- Since 2016, **individuals** can also register to be involved
Challenges

- Finding suitable patients (e.g. language barrier, availability)
- Training
- Define the role in the different activities / committees to manage expectations from all angles
- Managing potential conflicts of interest
- Representativeness
- Measuring the value / impact of patients
Added value of patient involvement

Engagement with patients:

► Improves the **regulatory outcome** – ALWAYS

► Patient input included in the assessment report -AddValue

► Brings the everyday aspects of living with a disease into the scientific discussions and helps bridge the gap between clinical trial data and real world data

► Improves transparency and trust

► Increases understanding and dissemination of EMA outcomes

ALL perspectives are crucial and have ultimately resulted in more meaningful decisions for all concerned.
Public hearings
1st EMA Public Hearing: Valproate

Held during the October PRAC meeting

Tuesday, 26 September 2017
A public hearing provides

- An opportunity for the **public to be heard** by the PRAC leading to a more rounded understanding by PRAC of the issue
- An opportunity for **stakeholders groups** to listen and to be heard by others
- An opportunity to show a **listening and engaged** regulatory system
Speakers

32 speaker requests
25 contributions selected, grouped in 16 speaker slots

United Kingdom 8/17
Ireland 2
Belgium 2
France 2
Italy 1
Sweden 1

General public (patient representatives, carers, families)
Healthcare professionals and academia
Pharmaceutical companies
Conclusion

➤ A milestone in EU medicines regulation

➤ A major step towards openness and transparency

➤ Need to make it valuable:
  – For the assessment
  – For the public

➤ Measure impact – ‘lessons learned’ exercise in 2017
Thank you for your attention

Further information

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