EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

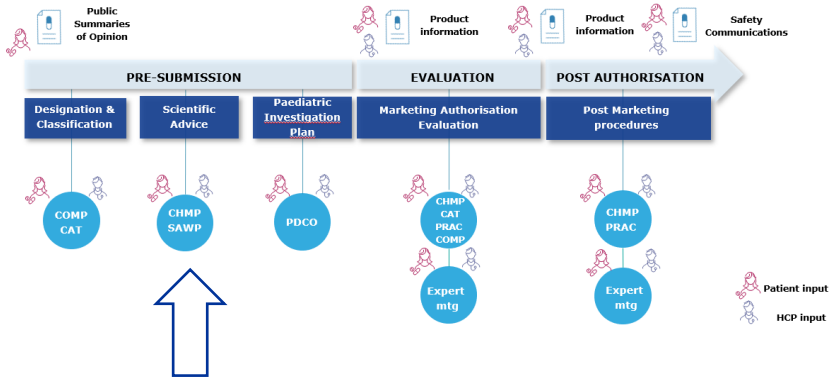
The Added Value of Patient Engagement in Early Dialogue at EMA

Scientific Advice as a Case Study

Cancer Drug Development Forum

Presented by Maria Mavris on 19 September, 2022
Patients and Consumers Liaison, EMA

Patient Engagement in Scientific Advice

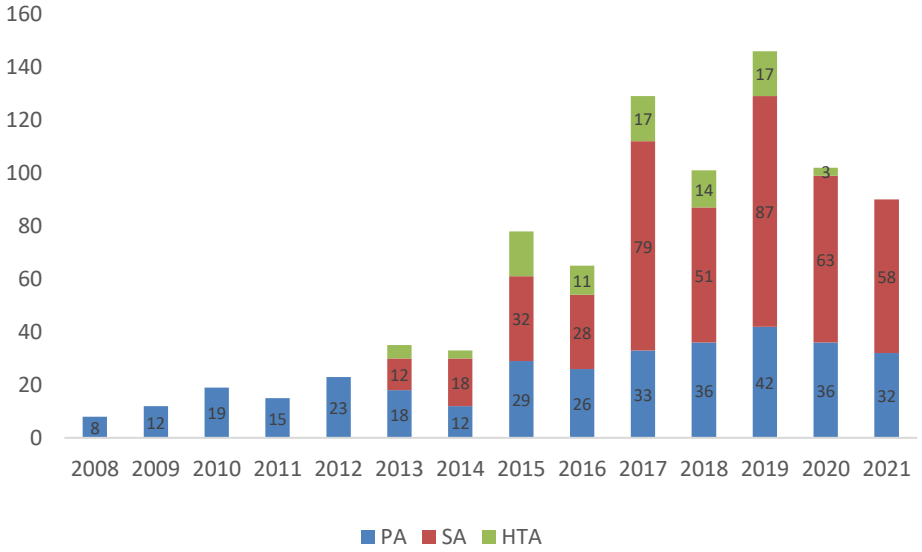


Scientific advice

- Analysis of patient input in scientific advice procedures
- Survey of EMA scientific officers
- Four years of data
- Published in Frontiers in Medicine

Patient involvement in scientific advice

- Data for patient involvement since 2008
- First patients in protocol assistance
- Extended to non-orphans in 2013
- Involvement of patients in HTA in 2013



Survey analysis

- 2017–2020
- 371 survey responses received for 478 patients (78%)

Year	N responses/ patients	Response Rate
2017	90/129	70%
2018	75/101	74%
2019	110/139	79%
2020	96/102	94%

- 20 different scientific officers responded to the survey:
  10 consistent scientific officers over 4 years

Patients:

Identified via:

- EMA eligible organisations
- Individual database
- Contacts via training

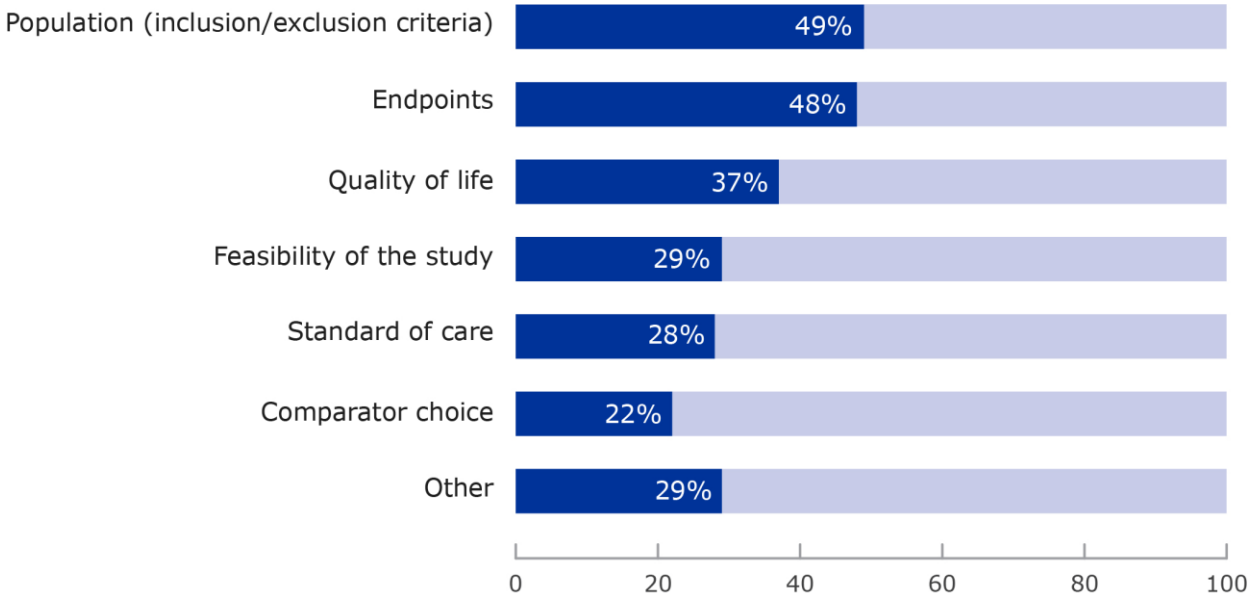
Criteria:

- English is language at EMA
- Level of experience can vary
- Training is helpful but not essential
- Free of competing interests

Results

- Involved in 1 in 5 procedures with clinical questions (20%)
- Reasons for requesting patients included:
  - study population (77%)
  - endpoints (74%)
  - study feasibility (52%)
  - quality of life (48%)
  - other aspects such as PROs, biomarkers and safety issues

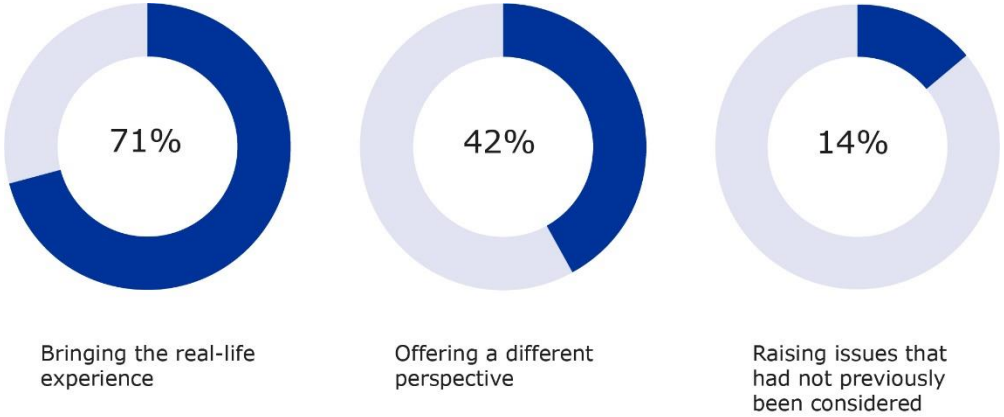
Where patients gave input



Patient input resulted in further reflection in 52% of cases.

"Other" areas included general insights into the condition, its daily impact and treatment options.

Added value of patient input and involvement



Patient input:

- **complements** the scientific and medical contributions to the specific questions.
- contributes to **future recommendations** in the same therapeutic area.

Impact of patient input and involvement

20% of cases - recommendations made to the developer were modified based on patient contributions.

>85% cases where patient input did not change the final advice correlated to patient **agreement** with the proposed development plan.

Surveys to patients

125 responses from patients for same period

- **>80%** patients understood what was expected of them
- **>80%** felt that they were able to provide input to the issues
- **75%** of patients felt their comments were taken into account
- **80%** felt positive about their overall experience
- main **barriers** were complex information and short deadlines

Support and documents

Early identification of patients by participation in validation meetings with EMA team

- Supporting material: info sheet and scientific advice video
- Phone call by engagement team for administrative aspects and background
- Phone calls with coordinators: **91%** contacted patients prior to their involvement to explain the process of scientific advice and where their input would be helpful.
- Training patients in EMA training day by scientific advice team

Conclusion

- The **added value** of patient input is not exclusive to scientific advice
- they are involved in other regulatory procedures such as scientific advisory groups and in consultations by EMA committees.
- Demonstrated value of patient inclusion in scientific advice not only supports EMA's continued inclusion of the patient voice throughout the medicine's lifecycle and the diversification of activities where patients participate, but also provides **evidence of impactful importance** of engaging with patients.

Patient input examples

Protocol assistance for rare epilepsy,

the company claimed that a particular authorised product was not widely used in Europe and therefore would not make a good comparator for their product, the patient representative was able to gather information from the patient community and confirmed the use of this product in 40% of cases.

This resulted in the SAWP recommending the use of this product as a comparator in their studies.

Patient input examples

SAG for multiple myeloma,

Discussion on whether the observed benefit of the medicine, in the absence of a significant effect on overall survival, was sufficient to balance the adverse event profile.

The patient representatives highlighted that the availability of a new treatment, even if associated with modest benefits and significant toxicity, is of value for patients. However they emphasised that the likelihood of experiencing unfavourable effects and the likelihood of benefit should be clearly described to allow informed treatment choice by physicians and patients, considering the available therapeutic options.

EMA annual report 2016

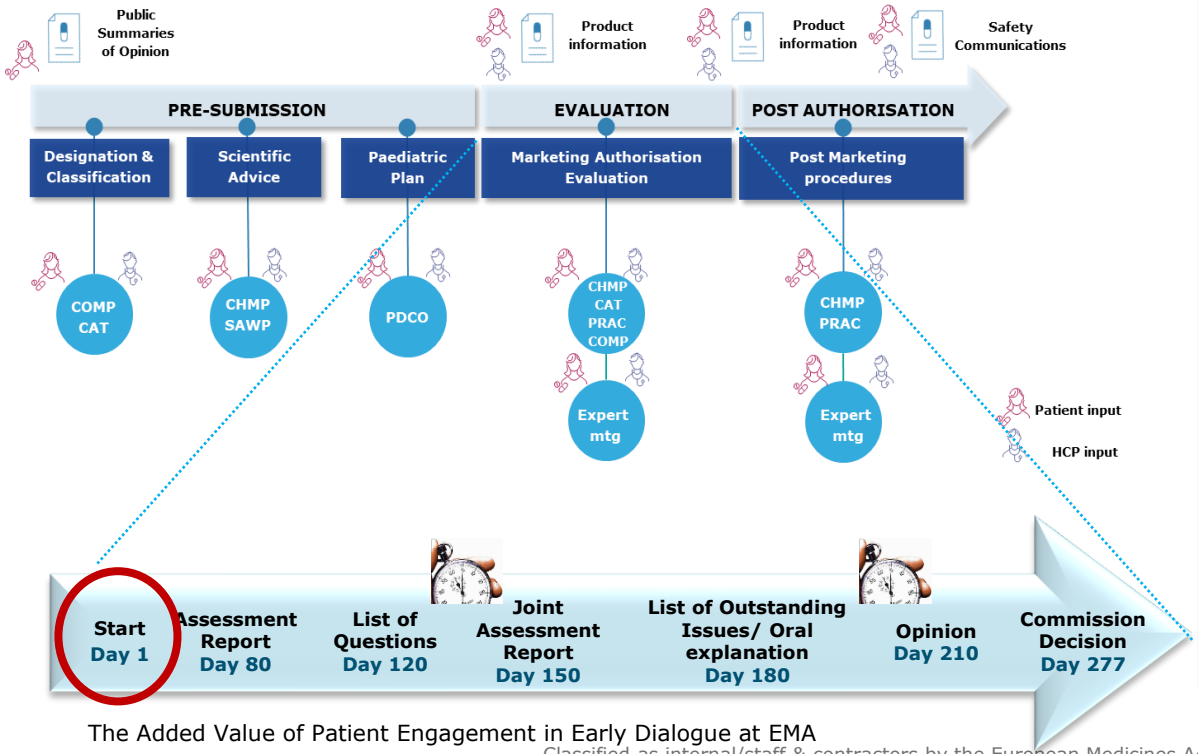
Challenges

- Finding suitable experts (e.g. language barrier, availability)
- Ensuring comprehensive, tailored training to facilitate and enhance participation
- Provide a clear definition of role of experts in the different activities / committees to manage expectations from all angles
- Managing potential conflicts of interest
- Representativeness
- Measuring the value / impact of patients

EMA activities with patients supported by Engagement Framework

1. A **network** of European patients' and consumers' **organisations**;
2. Patients' and Consumers' Working Party (**PCWP**);
3. A pool of **individual** patients, consumers or carers,
4. **Capacity-building** and training
5. Range of **engagement methodologies** enabling patients and consumers to be included along the medicine's regulatory lifecycle
6. Development of guidance on the generation, collection and use of **patient experience data**;
7. Interaction with the **EU Regulatory Network**.

CHMP early contact with patient organisations



- Relevant organisations contacted at start of orphan MAA's
- Patient organisations invited to share key aspects from their perspectives of living with the condition (3-4 weeks to respond) (in advance of first AR).
- Information shared with (Co-) Rapporteurs (and company for transparency) - Rapps decide if information provides added value, is useful for assessing the dossier, and if merits being included in AR.
- Value of patient input received during pilot assessed by short questionnaire

Multi-stakeholder workshop:
Patient experience data in medicines development and regulatory decision-making

21 September 2022, 10:00 – 16:30 (CEST), EMA, Amsterdam

Any questions?

Further information

maria.mavris@ema.europa.eu

Official address Domenico Scarlattilaan 6 • 1083 HS Amsterdam • The Netherlands
Telephone +31 (0)88 781 7821
Send us a question Go to www.ema.europa.eu/contact

Follow us on **@EMA_News**