EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Patient involvement in Benefit/Risk Discussions at EMA

CDDF Multi-Stakeholder Workshop on "Involving Patients in Oncology Drug Development", Amsterdam, The Netherlands

Presented by Nathalie Bere on 18 June 2019
Public Engagement Department, Stakeholders and Communication Division

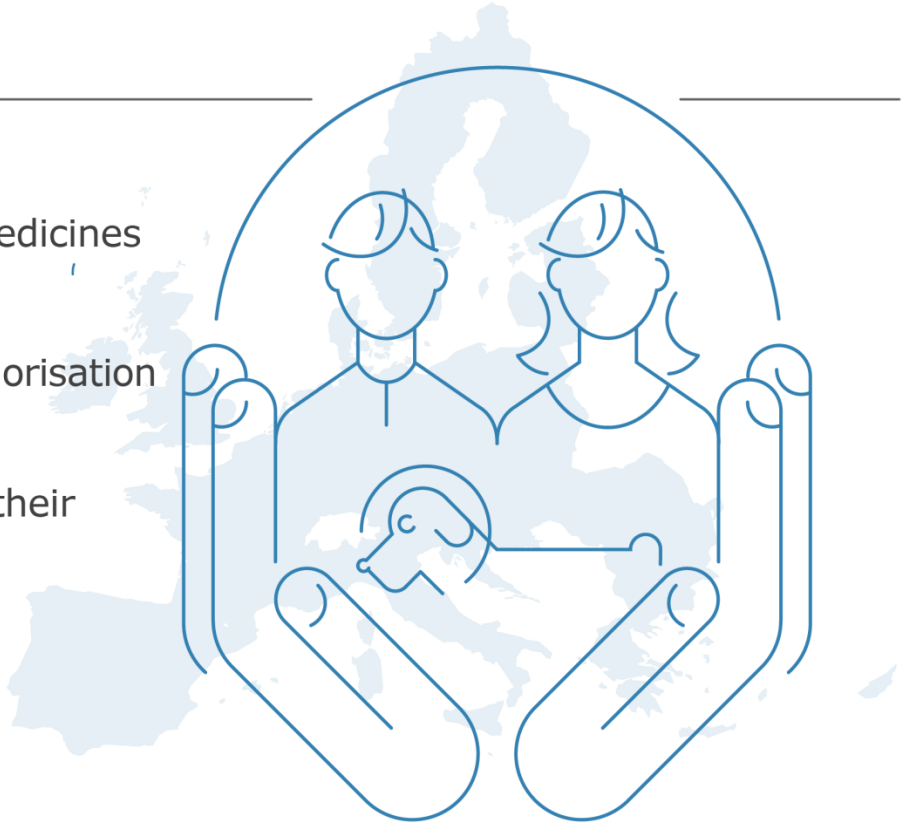
Disclaimer

The presenter does not have any conflict of interests.

What we do

Protect human and animal health

- Facilitate development and access to medicines
- Evaluate applications for marketing authorisation
- Monitor the safety of medicines across their life cycle
- Provide reliable information on human and veterinary medicines to patients and healthcare professionals



EMA stakeholder engagement

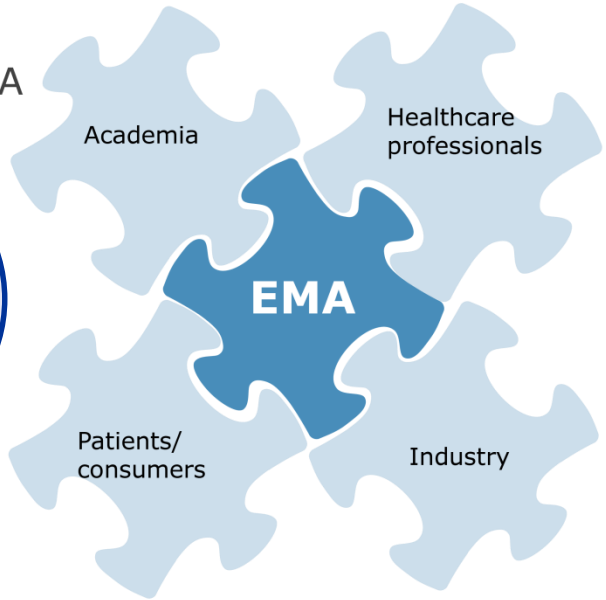
Promoting multi-stakeholder discussions

- Engage and involve stakeholders in EMA activities
- Enable stakeholders to share relevant issues with EMA

- Provide reliable, targe
- Enhance understandi
  network
- Increase transparency a

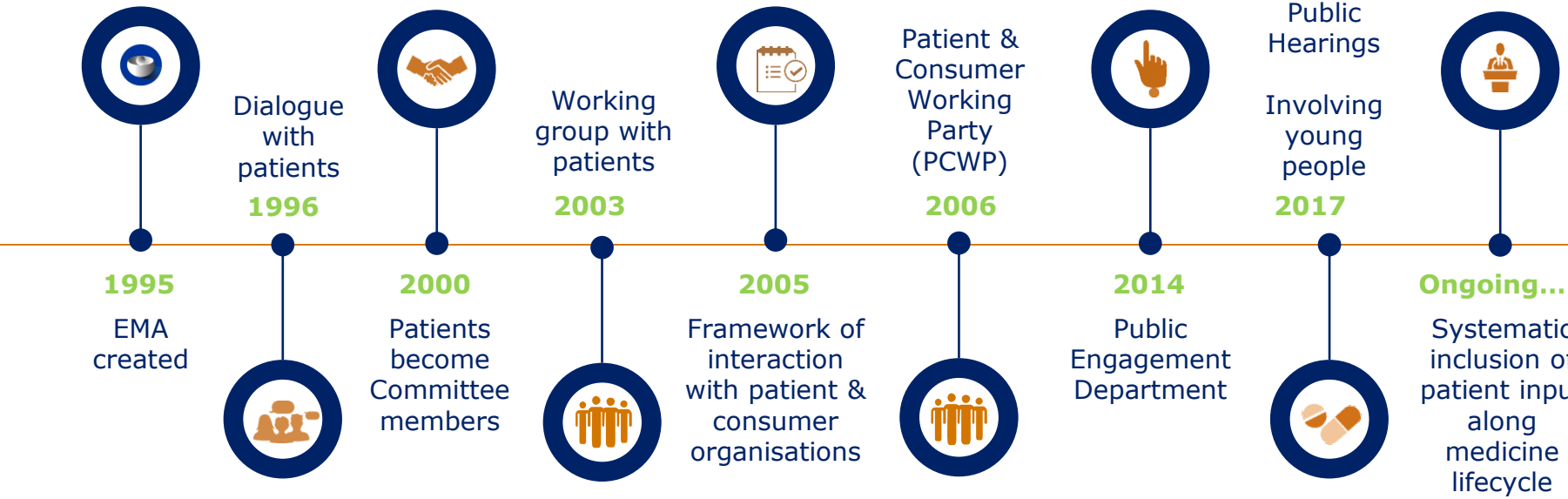
- Use stakeholder relations to further support EMA's strategic priorities

Together foster scientific excellence in the evaluation and supervision of medicines, for the benefit of EU public health

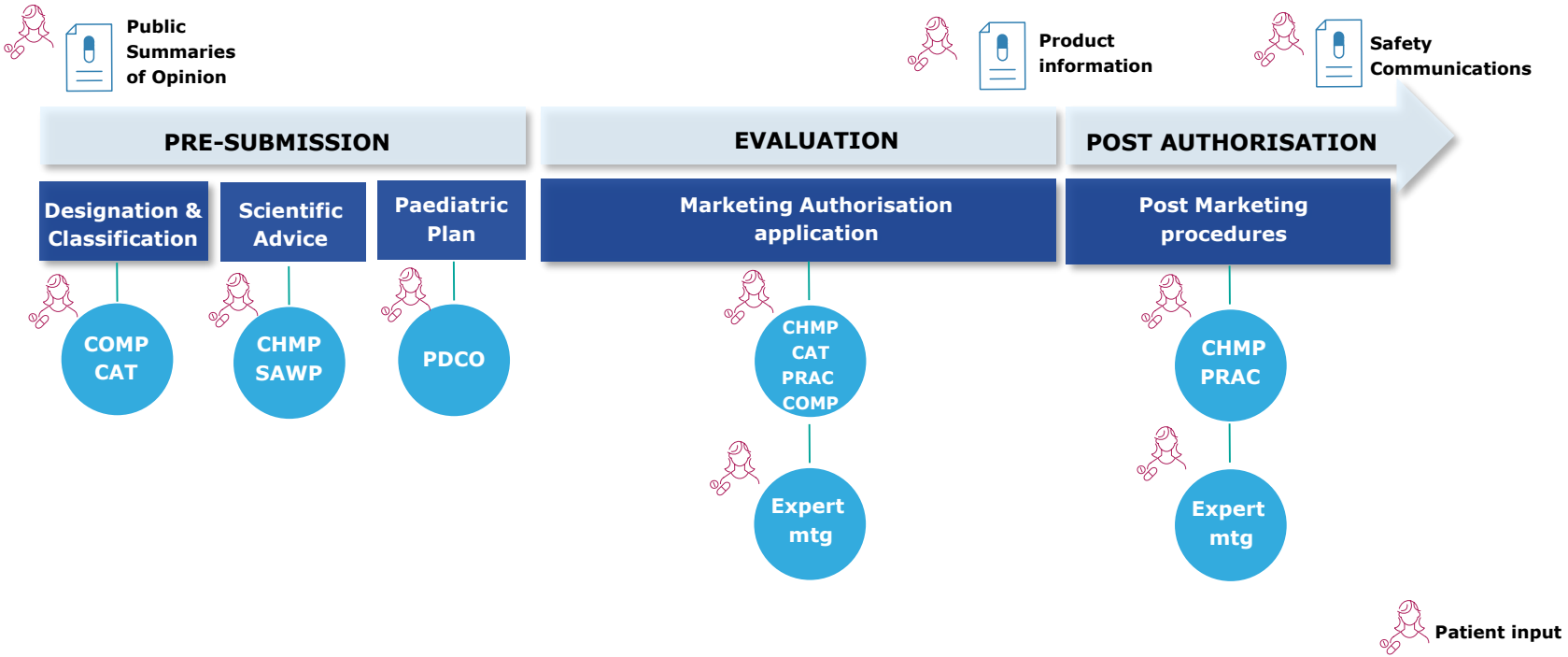


Patient engagement at EMA; our experience

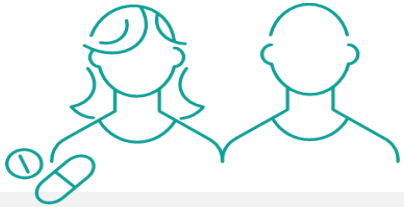
Interaction with patients: a progressive journey…

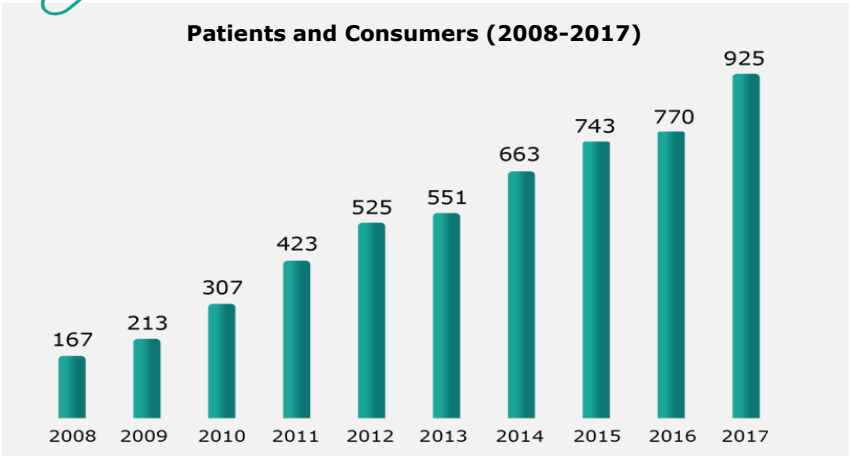


The patient voice along the medicine lifecycle;



Increasing involvement





Different levels of representation:

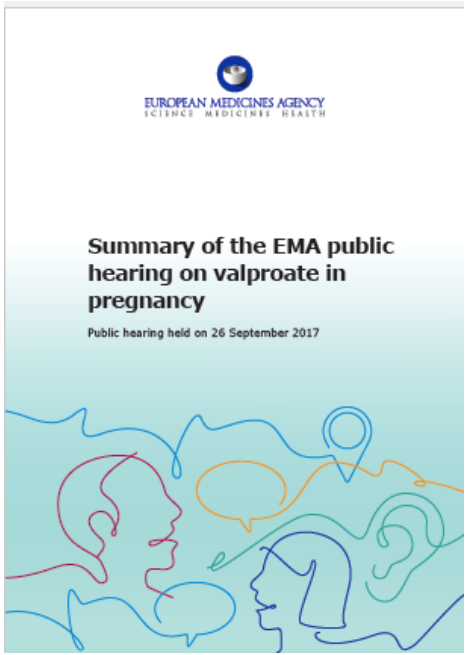
Representing their *community*	*Management Board* *EMA Scientific Committee Members*
Representing their *organisations*	*Working Party (PCWP or HCPWP)* *EMA consultations* *Workshops*
Individual experts	*Scientific Advice / Protocol Assistance Procedures* *Scientific Advisory/ad hoc expert Groups* *Scientific Committee consultations* *Review of documents*

Opportunities for patients to contribute to benefit/risk discussions

- Committee (full) membership
- Scientific Advisory Groups (SAGs) and Ad-hoc expert group meetings
- Committees/Working Parties direct consultations (f2f /writing/surveys)
- Public hearings
- EMA workshops & public consultations on development /update of regulatory guidance
- Review of labelling, risk minimisation measures and safety communications (appropriate language / increase awareness)

Public hearings; listening to all stakeholders..

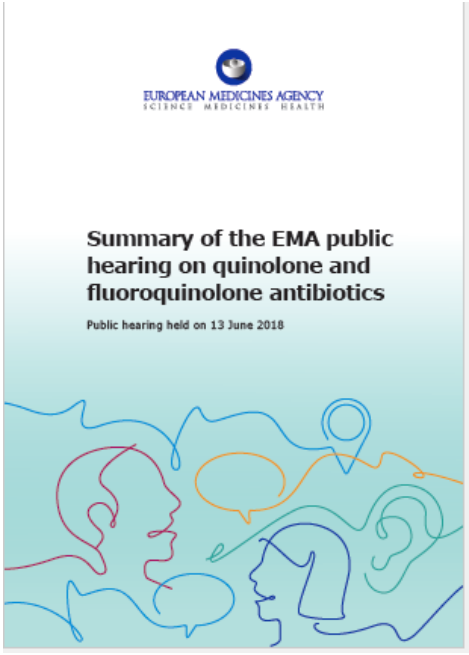
Aim: how to minimise the risk of harm from valproate to unborn babies



Proposals taken into account

Restricted use
Education materials
Communication campaign
Labelling/packaging
Encourage further research

Aim: how to raise awareness on extent of side effects



Patient networks

- **Any organisation** representing EU patients or consumers may express an interest to work with EMA, (eligibility criteria & application form: EMA website)
- **Any individual** patient or carer can register to work with the EMA (application form on EMA website)

Vital elements: flexible engagement methodologies and appropriate support and training

One size does not fit all!

Annual training day

Info-sheets

Videos

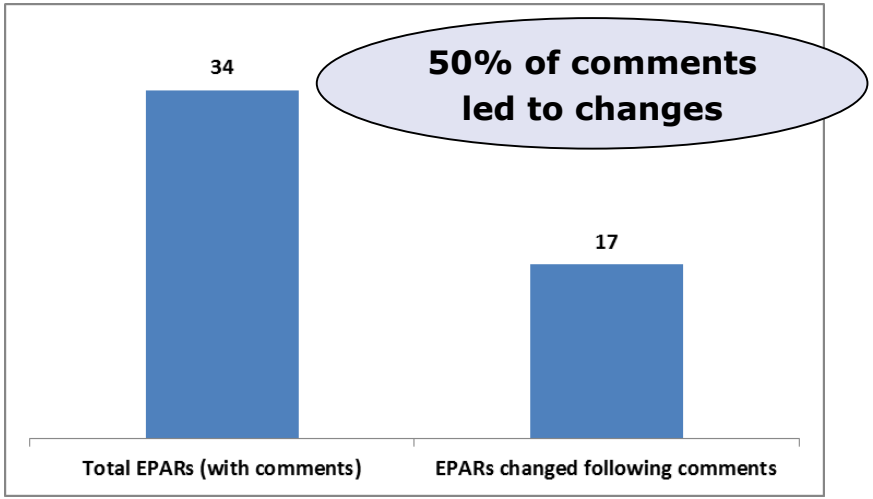
Webpages

One-to-one personalised support

Monitoring and measuring

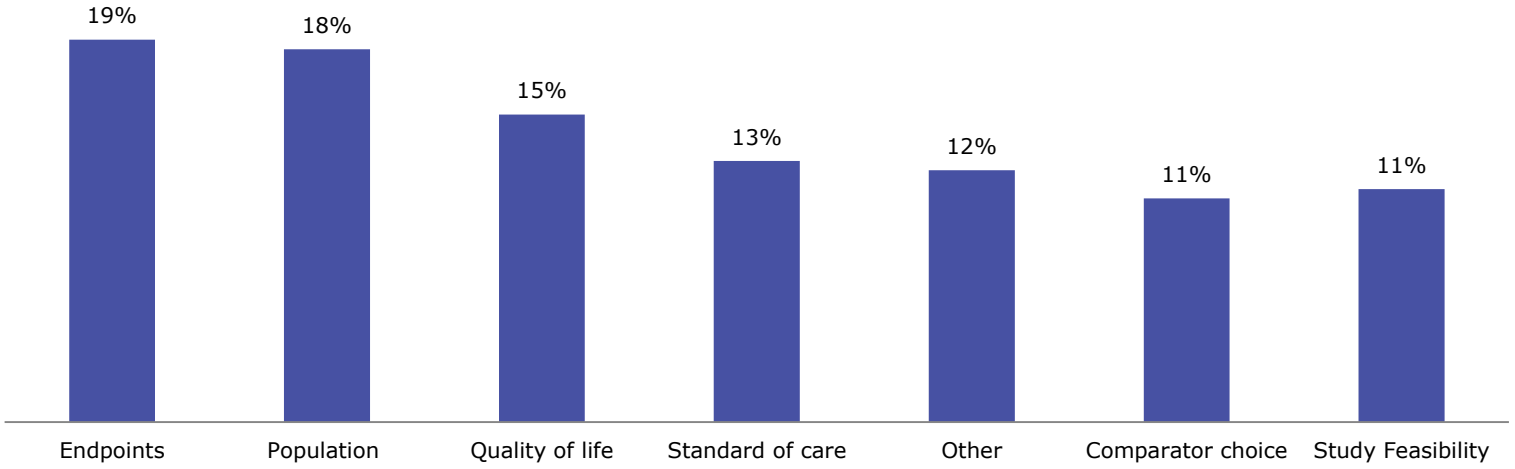
- Continuous monitoring
- Questionnaires sent to patients and assessors who participate
- Demonstrate **value**

Review of documents

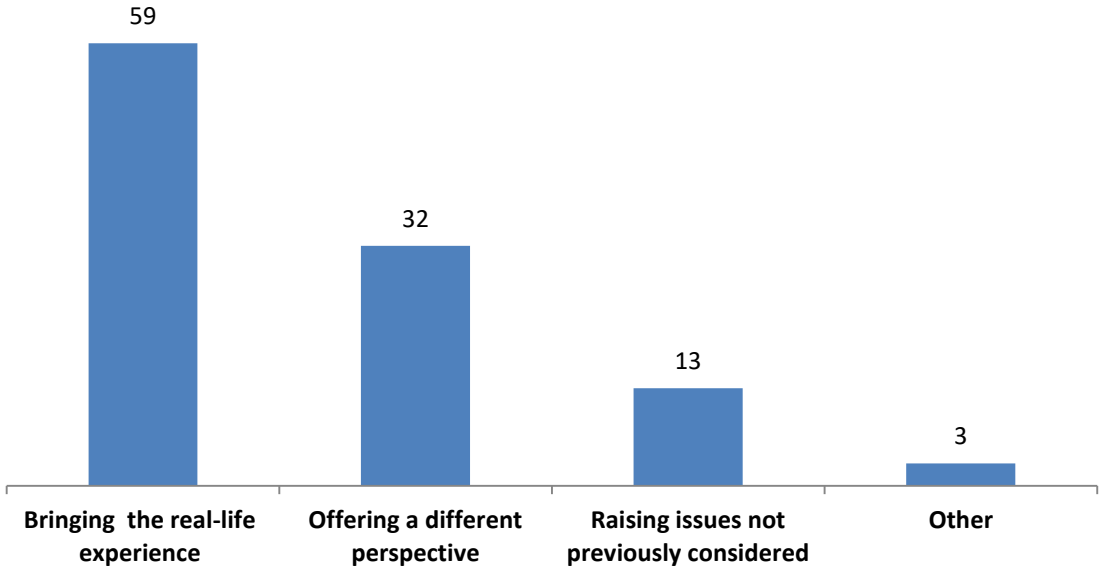


What kind of input?

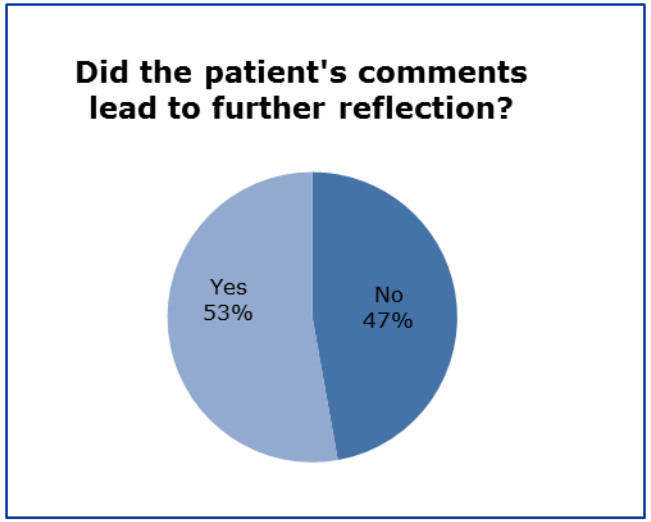
Scientific Advice; aspects of development plans where patients gave input:

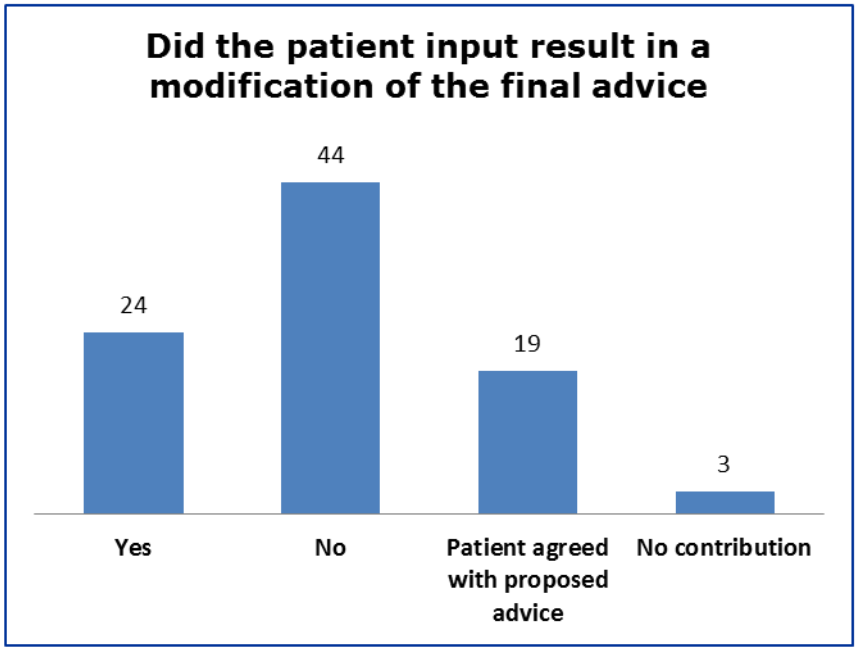


What was the added value of the patient's input?



Did it make a difference?





ICH E8 R1 General Considerations on Clinical Studies (https://www.ema.europa.eu/en/news-events/open-consultations); **2.3 Patient Input into Study Design**

Consulting with patients and/or patient organisations in the design, planning and conduct of clinical studies helps to ensure that all perspectives are captured. Patients' views can be requested on all phases of drug development. Involving patients at the early stage of study design is likely to increase trust in the study, facilitate recruitment, and promote adherence, which should continue throughout the duration of the study. Patients also provide their perspective of living with a condition, which contributes to the determination of endpoints that are meaningful to patients, selection of the right population, duration of the study, and use of the right comparators. This ultimately supports the development of medicines that are better tailored to patients' needs.

Clinical Trial Regulation:

…….Member States should ensure the involvement of laypersons, in particular patients or patients' organisations.

….The protocol shall at least include: (e) where patients were involved in the design of the clinical trial, a description of their involvement;

Summary messages

- Engaging with patients;
  - brings **everyday aspects** of living with a disease **into scientific discussions**
  - helps **bridge the gap** between clinical trial data and real world data
  - increases **transparency**, **awareness** and **understanding**
- Engage in a **stepwise approach**; **learn together** what format works best;
  - **Define roles** - manage expectations
  - Ensure engagement is **mutually beneficial**
- **Everyone** has a role to play to ensure engagement happens
- Engaging with patients leads to **more meaningful outcomes** for everyone!

Patient engagement; where next?

Today:

- Added value of patient input into regulatory decision making has been demonstrated

Challenges: **Resources**, **Independence** and **Representability**

Tomorrow (as highlighted in RSS to 2025):

- Systematic input throughout the product life-span; provide financial compensation
- Robust assessment of independence; a) 'eligibility' criteria; b) conflict of interests policy
- Increased patient data generation; support/conduct patient data collection (e.g. preference studies, quality of life and patient-reported outcomes, use of big-data technologies)
- A strengthened regulatory system that can efficiently integrate evidence from RWD into its assessments

Questions?

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