Regulatory perspective - EMA

ISCTM 2020 Autumn Conference
Patient centricity: Design and conduct of clinical trials in orphan diseases

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Disclaimer

The presenter does not have any conflict of interests.

The views expressed in this presentation are the personal opinion of the author and should not be understood as being made on behalf of or reflecting the position of the EMA or one of its committees or working parties.

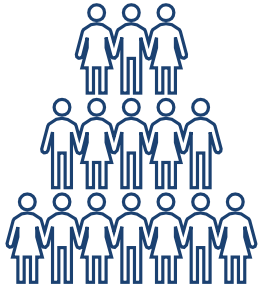
Small populations: different regulatory frameworks built for purpose

Orphan conditions (valid for products that received an orphan designation)	• Orphan designation • Protocol Assistance • PRIME
Paediatric conditions (most are rare, need dedicated research)	• Scientific advice • PIP, potentially PUMA • PRIME
Subsets of common diseases (e.g. biomarker + cancers, agnostic indications)	• ITF • Scientific advice • PRIME
Personalised medicine (innovative trials, out of the box proposals for regulators)	• ITF • Scientific advice • PRIME

Tools to help address potential challenges:

Parallel FDA-EMA scientific advice

Joint HTA-EMA scientific advice



Registries task force

PIP and related advice

Regulatory support for SMEs

Orphan designation

Dedicated guidance on small populations trials

- Small trial considerations (EMA Guideline and commentary publication)
- Significant benefit (contextualisation of effects vs. satisfactory methods of treatment) (COMP Recommendations, and COMP publication)
- Extrapolation (Reflection on the use of extrapolation in the development of medicines for paediatrics)

History of patient involvement at EMA

1995
EMA created

1996
Dialogue with patients

2000
Patients become Committee members

2003
Working group with patients

2005
Framework of interaction with patient & consumer organisations

2006
Patient & Consumer Working Party (PCWP)

2014
Public Engagement Department

2017
Public Hearings

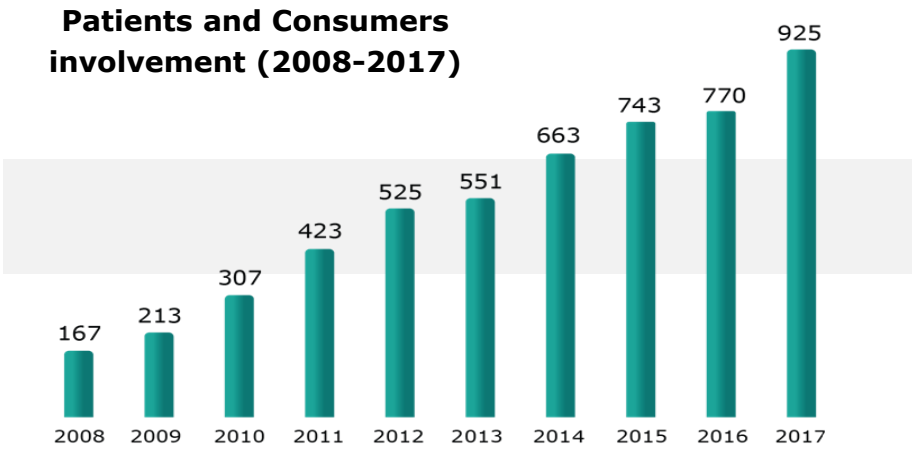
Involving young people

Today…
Systematic inclusion of patient input along medicine lifecycle

All along the medicine lifecycle

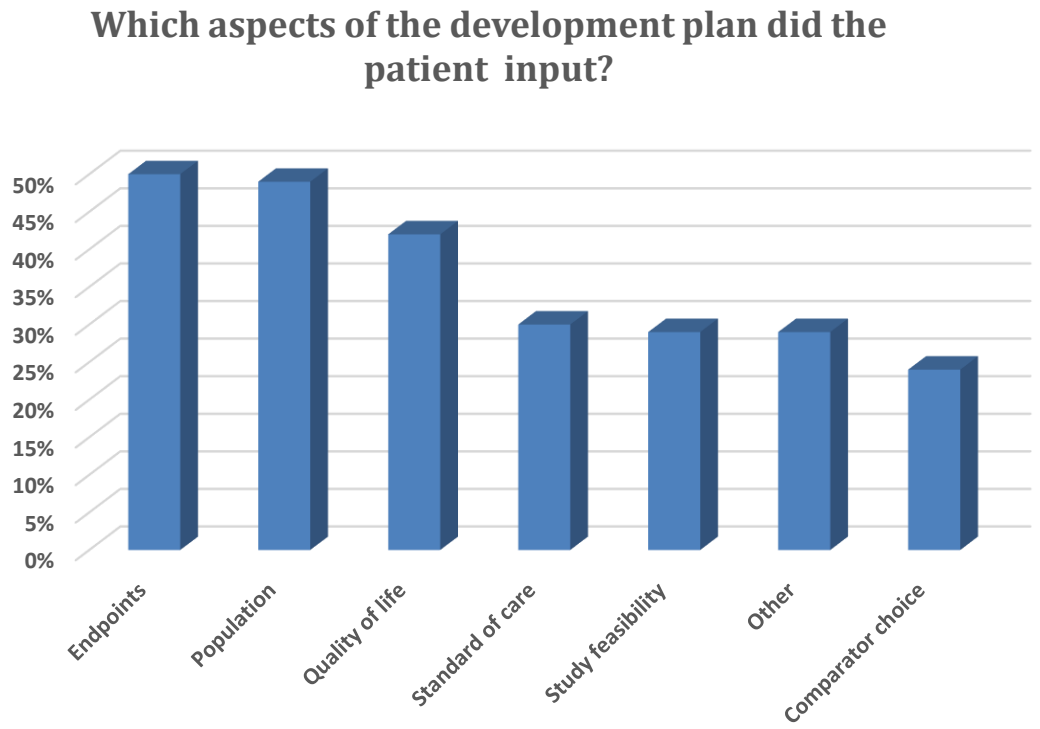
Patient input

Increasing and varied involvement throughout EMA activities



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

Evaluating the impact / value



3 year survey; ≈ 300 Scientific Advice procedures:

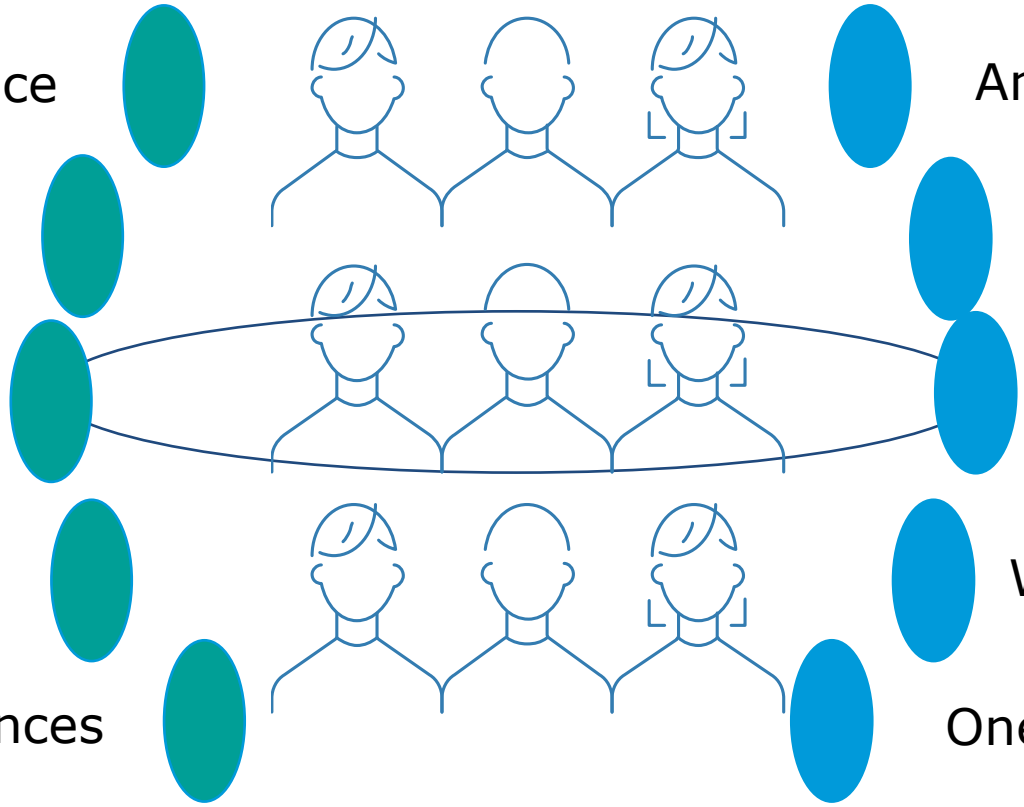
- 79% patients agreed with the proposed development plans
- 53% of the patient`s comments resulted in further discussion
- 23% of the patient`s input resulted in a modification of the final advice

Vital elements

One size does not fit all!

Flexible engagement methodologies

- Face to face
- In writing
- Committee meetings
- Surveys
- Patient preferences



Appropriate support and training

- Annual training day
- Info-sheets
- Videos
- Webpages
- One-to-one personalised support

Challenges / success factors

- Finding suitable patients (e.g. language barrier, availability)
- Ensuring tailored support to facilitate and enhance participation
- Providing a clear definition of patients role within the different activities / committees to manage expectations from all angles
- Managing potential conflicts of interest
- Representativeness (multi-stakeholder communication is key!)
- Measuring the value / impact of patients

Conclusions:

- Selection of an appropriate regulatory framework to tackle the problem helps!
- Make sure to take advantage of the variety in the EMA toolbox and ask questions whenever in doubt
- EMA strives to involve patients systematically throughout medicine life cycle. Learnings and success factors are shared with stakeholders to further our collaboration in this area
- Perspectives of all stakeholders are important and are considered in EMA consultations procedures

Thank you for your attention

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