EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

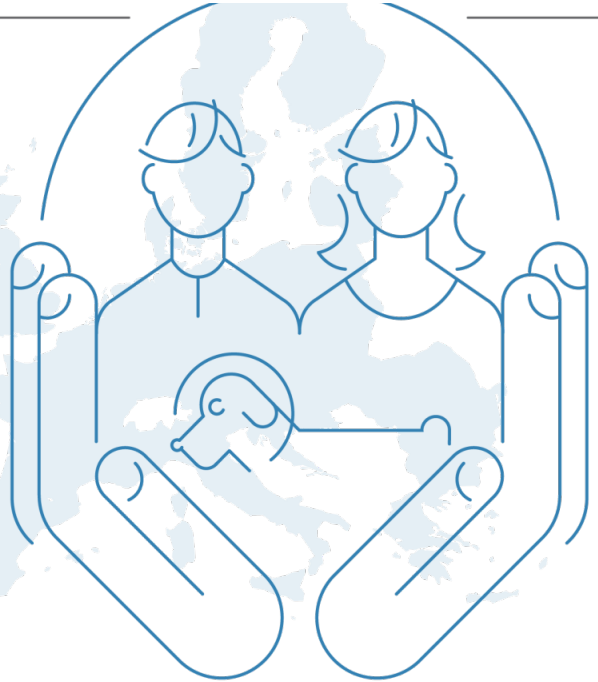
Patient engagement at EMA

Digestive Cancers Europe 8th Annual MasterClass

1 July 2023
Maria Mavris
Public and Stakeholders Engagement Department

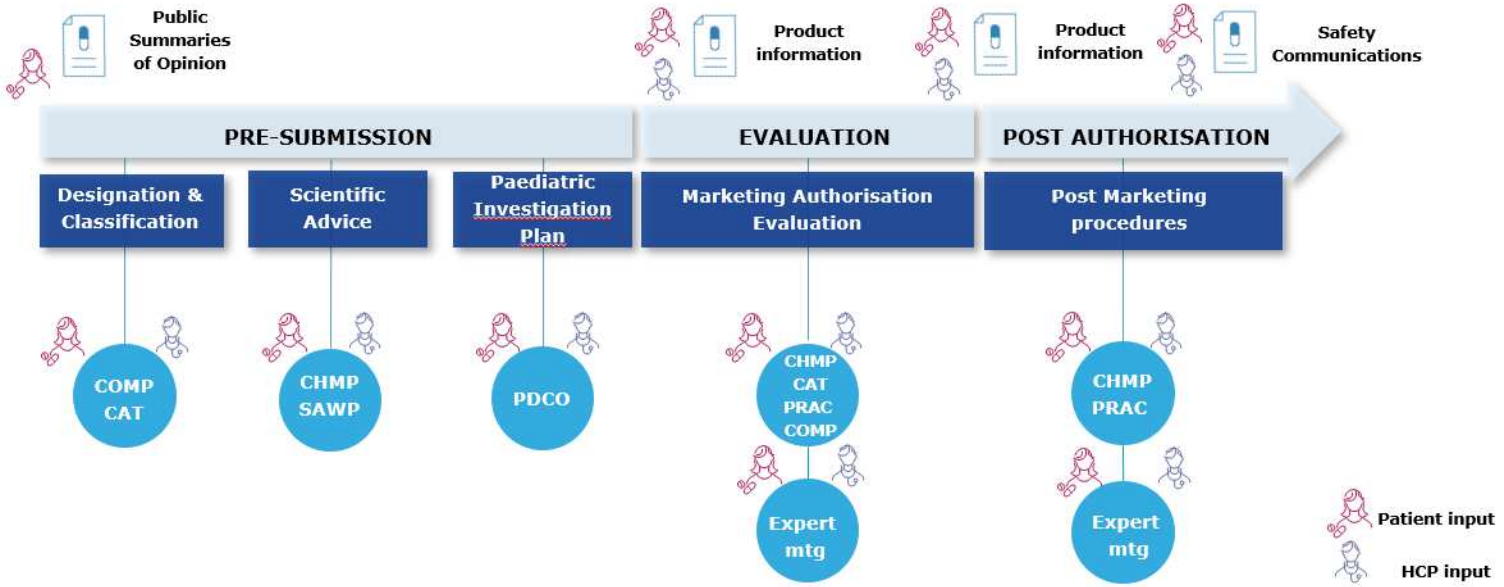
What we do

- 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



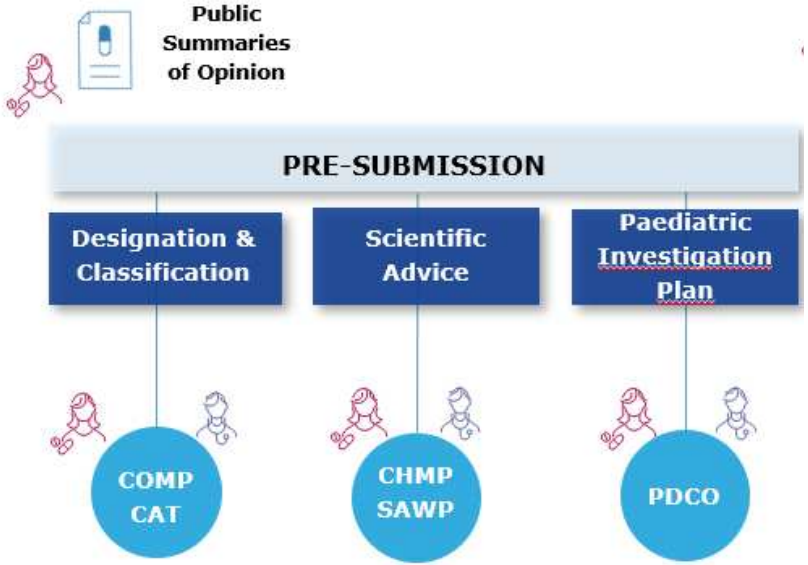
Interaction with patients and consumers: a progressive journey…

Stakeholder engagement across medicines lifecycle



CHMP — Committee for Human Medicinal Products
COMP — Committee for Orphan Medicinal Products
PDCO — Paediatric Committee
CAT — Committee for Advanced Therapies
PRAC — Pharmacovigilance and Risk Assessment Committee
SAWP – Scientific Advice Working Party

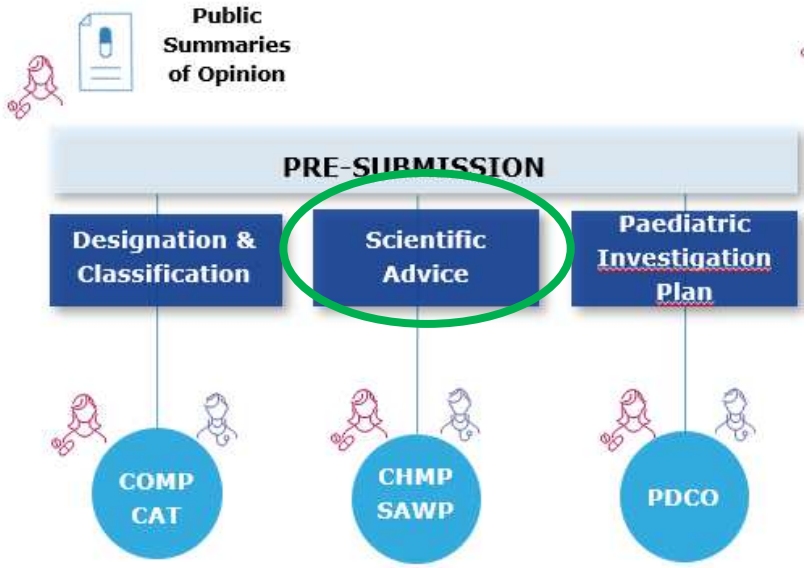
Patient Engagement in pre-submission phase: committees

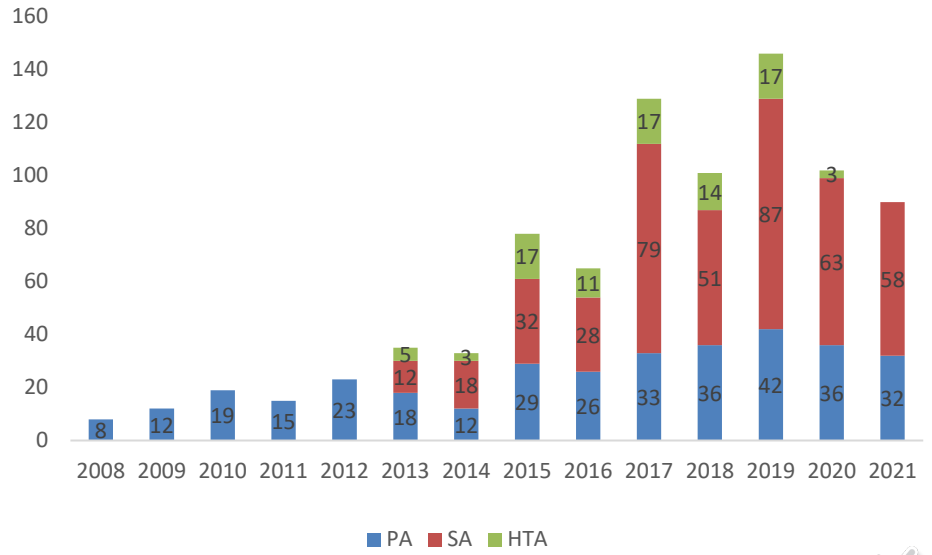


- Membership in COMP, PDCO and CAT
- Consultations on disease specific issues by committees
- Review of documents destined for public
- Experts invited to scientific advice

COMP — Committee for Orphan Medicinal Products
PDCO — Paediatric Committee
CAT — Committee for Advanced Therapies
SAWP – Scientific Advice Working Party

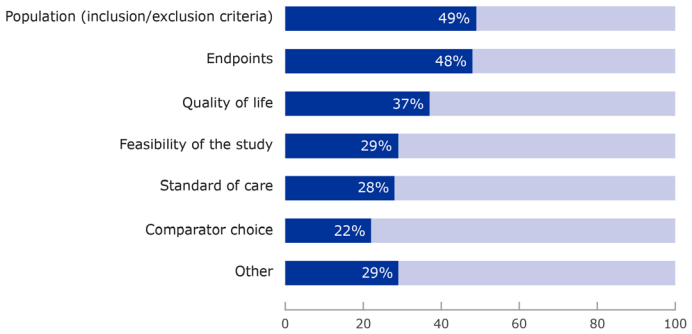
Patient Engagement in pre-submission phase: Scientific Advice



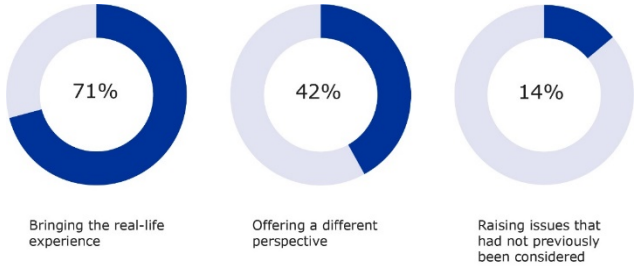


Published in Frontiers in Medicine

Where patients gave input



Added value of patient input and involvement



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

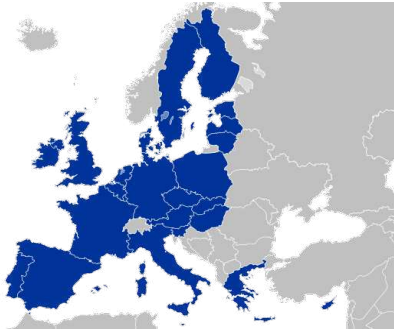
20% of cases - recommendations made to the developer were modified based on patient contributions.

>85% cases: patient **agreement** with the proposed development plan.

Evolution of EU clinical trials regulation

Pre 2004: No harmonisation

National rules, different processes in each Member State.
Resulted in **delays** and **complications**



Clinical Trials Directive
(EU 2001/20/EC)

Some harmonisation, but **national systems & processes varied**

Entered into application 1 May 2004

Clinical Trials Regulation
(No.536/2014)

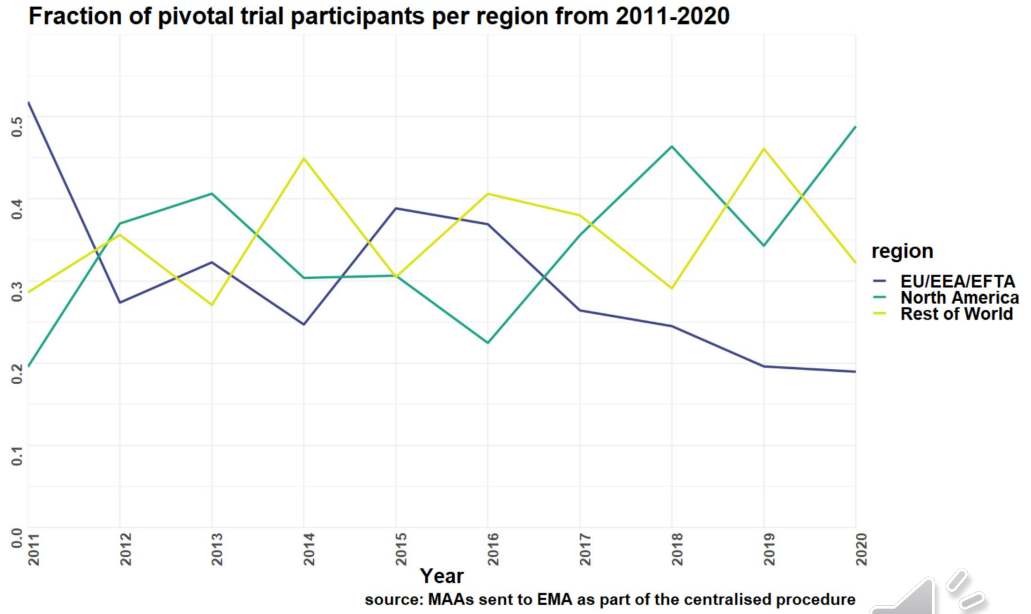
Full harmonisation, collaborative assessment of **multinational trials**, single EU portal & database

Applied from **31 January 2022**

The fraction of EEA trial participants is trending downwards

European trial participants in centralised procedure MAAs:

- Constituted only **19%** in 2020
- Has been **trending downwards** since 2015



- *Vaccines & therapeutics*
- *UK included as Rest of World*

Three pillars of the Clinical Trials Regulation

Harmonisation

Harmonised clinical trial processes

- Collaborative assessment
- Reduced administrative burden
- Enabling multinational trials

Transparency

Increased transparency of clinical trials data

- Empowering patients & HCPs to find recruiting trials
- Enabling research

Safety

Enhanced safety procedures

- Single submission process for sponsors
- Increased cooperation for MS

Accelerating Clinical Trials in the EU (ACT EU)

ACT EU is an initiative to **transform the EU clinical research environment** in support of medical innovation and better patient outcomes.

- **Builds on the momentum** of the Clinical Trials Regulation and CTIS
- **Driven by** the European Regulatory Network Strategy to 2025
- Launched 13 January 2022
- Read the press release and paper

CTIS – Clinical Trial Information System
supports the flow of information between clinical trial sponsors, European Union (EU) Member States, European Economic Area (EEA) countries and the European Commission.

ACT EU – multi-stakeholder platform

- Amongst other priorities, ACT EU recognises that for the clinical trial environment to evolve with advances in regulation, methodologies, technology and science, there is a need for multi-stakeholder discussions to drive and support change.
- Priority Action 3: establish a multi-stakeholder platform.

Multi-stakeholder platform proposal

Key players identified (final composition subject to public consultation feedback)

Patients, healthcare professionals

regulators/ inspectorates

academia/ researchers

ethicists

health technology assessment bodies

payers/policy makers

clinical trial investigators/sponsors/ clinical research organisations

Why a multistakeholder platform?

- The success of clinical trials relies on all stakeholders involved
- To deliver ACT EU, change is needed from every stakeholder
- A platform would enable coordinated change amongst all stakeholders in a neutral forum

How can the platform drive change?

1. Build common understanding -> Build relationships and trust
2. Co-develop change -> Build ownership of change
3. Use and develop resources and influence of the members -> pilot and implement change
4. Measure and publicise implementation -> Incentivise change

What this means for patients and healthcare professionals

- For patients and HCP, the Clinical Trials Regulation, CTIS and ACT EU will:
  - support bigger and **better CTs**;
  - drive **innovation** in CT methods;
  - generate data about clinical trials to better understand and **address health needs**; and
  - provide an **opportunity to engage** through the multi-stakeholder platform

Kick-off meeting

- Kick off meeting for MSP
- Participation – hybrid
- Broadcast live
- Recorded and published on EMA website
- ACT EU website

Conclusions

- Engaging with patients and their organisations:
  - 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: TRUST**
- 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!

Any questions?

Further information

[Insert relevant information sources or contact details as applicable.]

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Telephone +31 (0)88 781 6000
Send us a question Go to www.ema.europa.eu/contact

Follow us on **@EMA_News**

ACT EU objectives

1	Optimise the EU environment for clinical research in Europe by: **a) Strengthening leadership and coordination** on clinical trial authorisation and execution. **b) Optimising ethical oversight** and further integrate ethics committees into the clinical trial and medicines regulatory lifecycle. c) Supporting the **conduct of large-scale multinational clinical trials** **d) Reducing administrative burden and increasing efficiency**.
2	**Strengthening clinical trials** for unmet medical needs, rare diseases, and vaccines and therapeutics for public health crises, ensuring support for HTA bodies as well as for academic and SME sponsors.
3	**Heighten the impact of European clinical trials** through excellent and coordinated **scientific advice** as a complement to trial authorisation and to support marketing authorisation.
4	**Engage all stakeholders** to proactively deliver inclusive patient-oriented medicines development and delivery across populations.
5	**Ensure a clear and unified European position** on clinical trials in strategic matters at the international level.
6	**Build capacity** in all aspects of drug development and regulatory science through, amongst others, research collaboration and training with academia.

Priority actions

PA 1	Map existing initiatives and develop a governance rationalisation strategy (aligning different expert groups and working parties in the EMRN and ethics infrastructure).
PA 2	Successful and timely implementation of the CTR and its implementing acts: • develop KPIs and dashboard to track performance of the European clinical trials environment; • including the promotion of larger, multinational trials specifically in the academic setting.
PA 3	Establish a multi-stakeholder platform, including patients, after stakeholder analysis
PA 4	Implementing the GCP modernisation informed by the development of guidance at ICH
PA 5	Analyse data about clinical trials leveraging academic, non-profit, European, and international initiatives, improving the impact of policymaking and funding on research outputs to support evidence-based decision making
PA 6	Plan and launch a targeted communication campaign to engage all enablers (including data protection experts, academia, SMEs, funders, HTA bodies, healthcare professionals)
PA 7	Reinforce the coordination between scientific advice on CT approval and CT design and link to the methodologies working party domain
PA 8	Develop and publish key methodologies guidance e.g. on AI/ML impacted CTs, complex trials, decentralised CTs and IVDR/CTR interface (to strengthen links between innovation and scientific advice fora)
PA 9	Successfully establish CT safety monitoring and bridge to the EU4Health Joint Action and start its integration into a pre- and post-marketing safety monitoring framework
PA 10	Deliver a clinical trials training curriculum including modules on drug development and regulatory science with links to universities and SMEs (serving as an educational 'ecosystem')