EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

How medicines are approved in the EU: role of patients, healthcare professionals and academics

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Patient interaction / Stakeholders and communication Division

1995 2015 EMA

An agency of the European Union

What is the European Medicines Agency (EMA)

The EMA is the EU regulatory body responsible for the scientific evaluation and supervision of medicines developed by pharmaceutical companies for use in the European Union (Human and Veterinary).

The European System

Centralised Procedure (EMA)

Mutual Recognition/ Decentralised Procedure

National Procedures

- **Optimised utilisation of resources**
- **Harmonised scientific opinions**
- **Harmonised information to healthcare professionals & patients**

EMA: focal point of the centralised procedure

ONE

Marketing Authorisation application

Evaluation

- Authorisation in all EU MS
- Invented name
- Product information
  (Summary of Product Characteristics (SmPC), Labelling, Package Leaflet (PL))

ALL

EU languages

Medicines that are mandatory for evaluation at EMA

- Rare diseases
- HIV, cancer, neurodegenerative disorders, diabetes
- Auto-immune diseases, viral diseases
- All biotech products
- Gene therapy
- Monoclonal antibodies

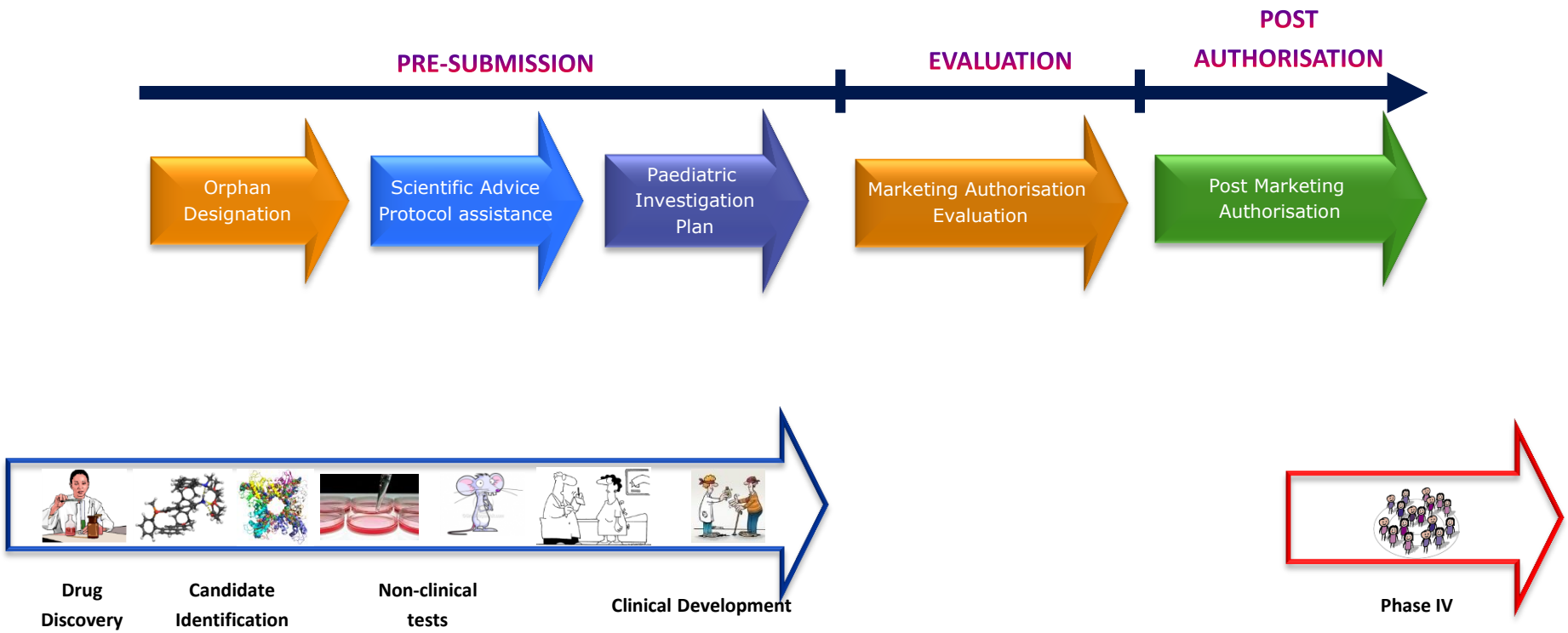
± Other innovative products

The EMA is **not** responsible for pricing or reimbursement

The various roles of the EMA

- **Evaluation of marketing authorisation** for **human** and **veterinary** applications submitted by pharmaceutical companies
- Coordination of European **pharmacovigilance** (supervision of the medicines on the market)
- Provision of **scientific advice** on the development of medicines
- Evaluation of applications for **orphan designation** in EU
- Evaluation of **paediatric investigation** plans (or waivers)
- Evaluation of **arbitration** and **referral** procedures
- Provision of good quality and independent **information** on the medicines it evaluates to patients and healthcare professionals
- Coordination of Member States' **inspections**

Medicines Lifecycle: Development and Regulatory



EMA-EU Network

28 EEA Member States
+ 4,500 European experts

EU institutions:
Commission - Parliament

Management Board

Committee for Human Medicinal Products (CHMP)

Committee for Veterinary Medicinal Products (CVMP)

EMA
Secretariat

Paediatric Committee (PDCO)

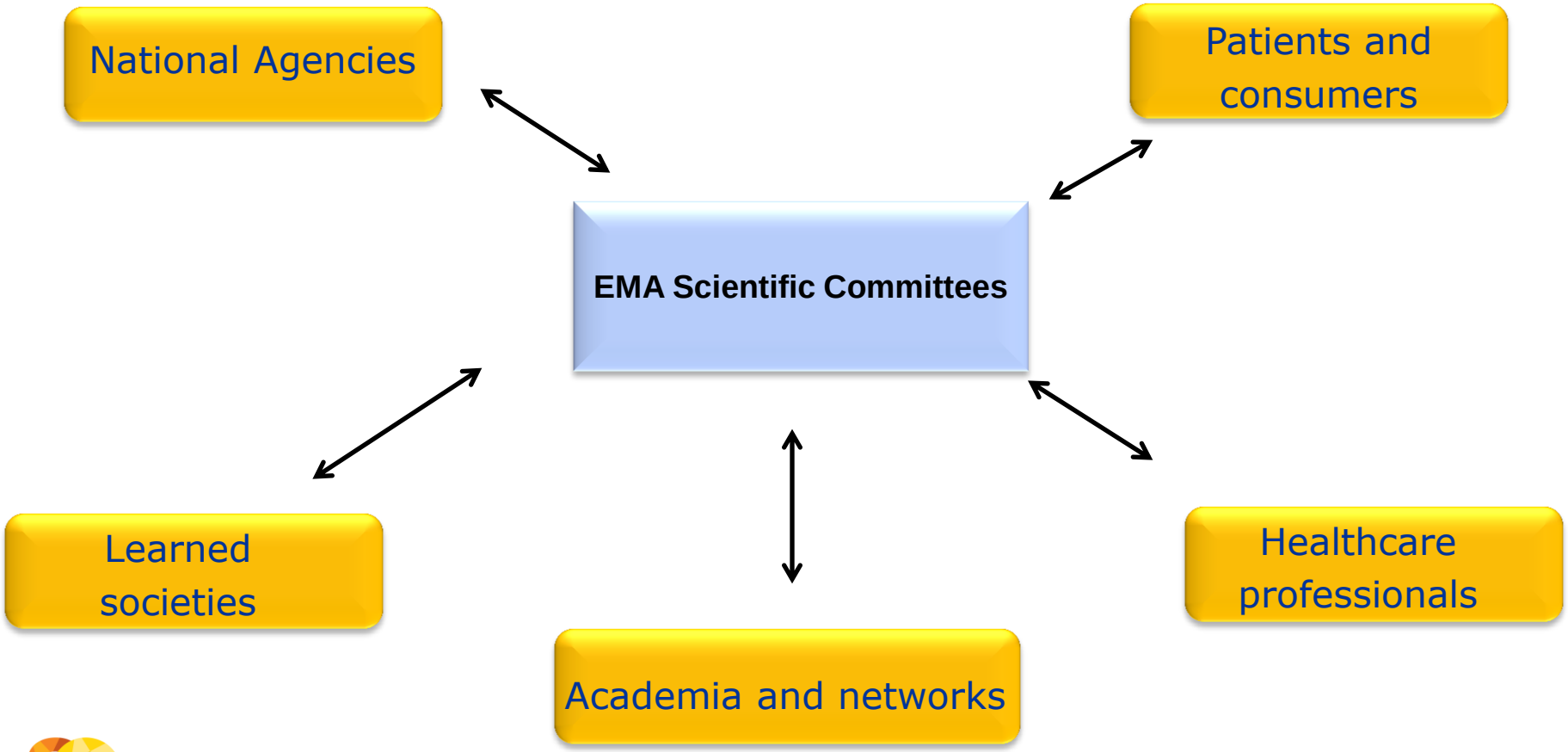
Committee for Orphan Medicinal Products (COMP)

Committee for Herbal Medicinal Products (HMPC)

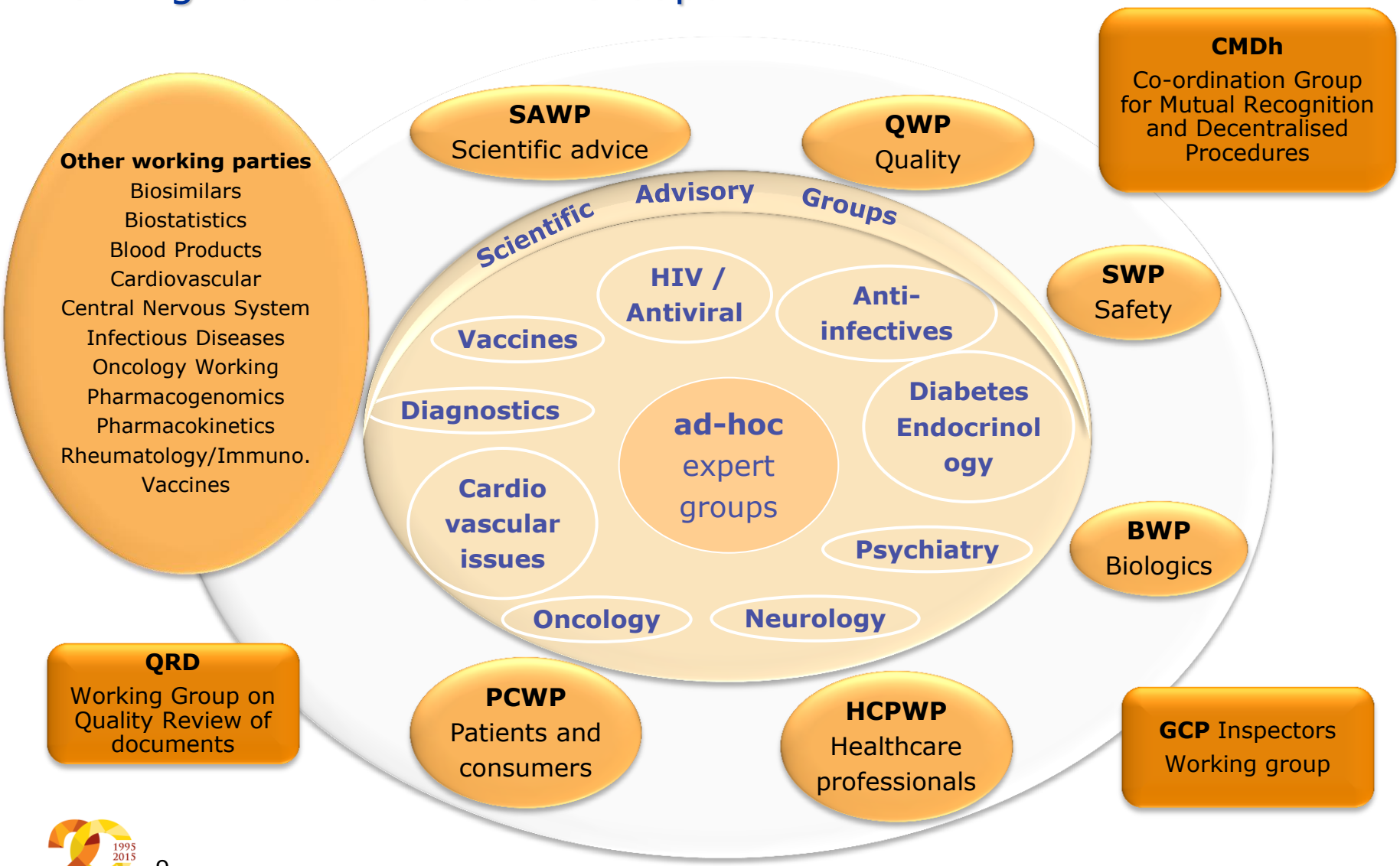
Pharmacovigilance Risk Assessment Committee (PRAC)

Committee for Advanced Therapies (CAT)

Sources of expertise helping the scientific committees



Working Parties and other Groups



Type of Approvals

Normal:
Comprehensive data

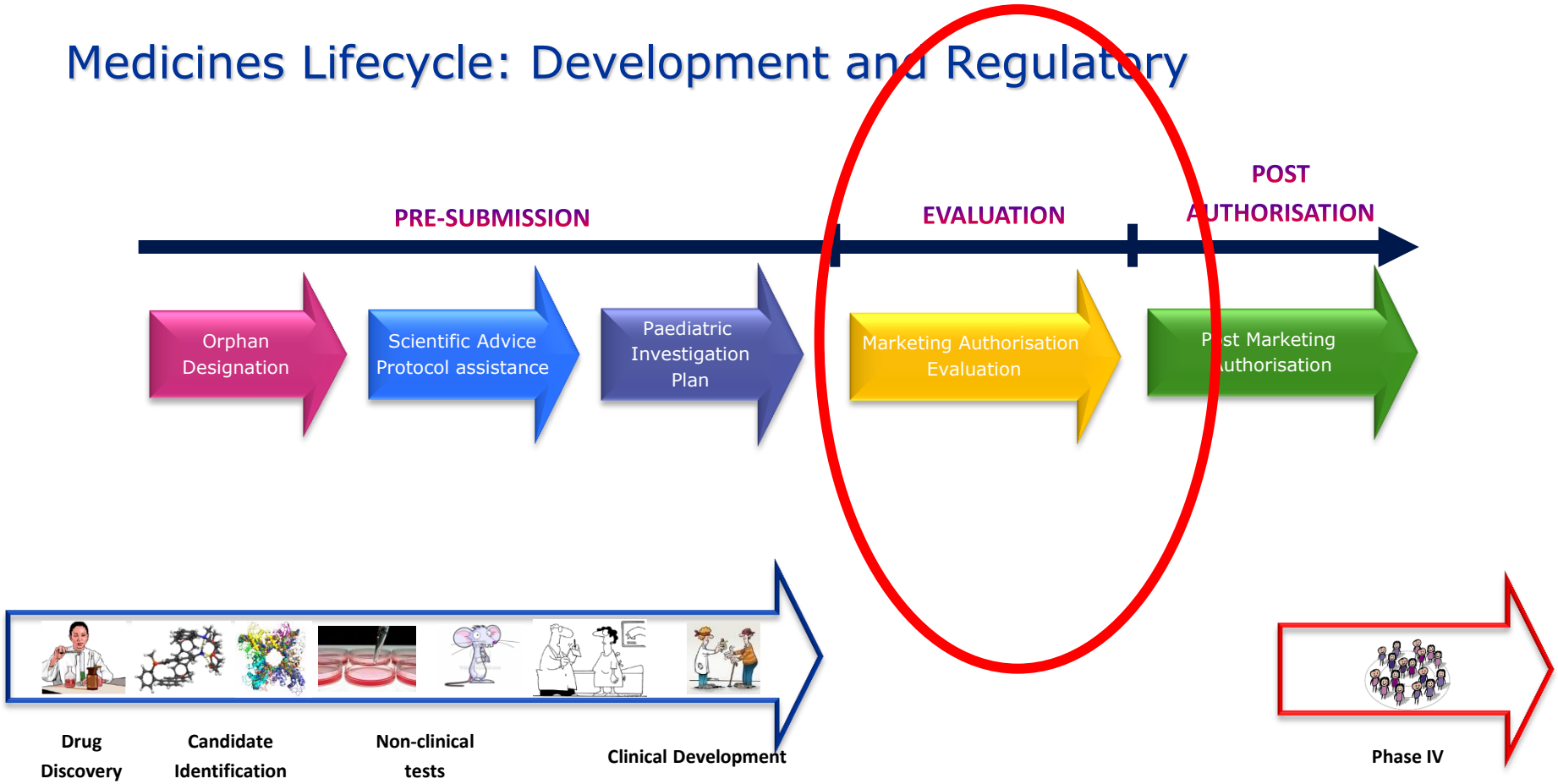
Exceptional Circumstances:

- Comprehensive data not available and cannot be provided
- Must meet criteria (rarity, medical ethics, state of scientific knowledge)

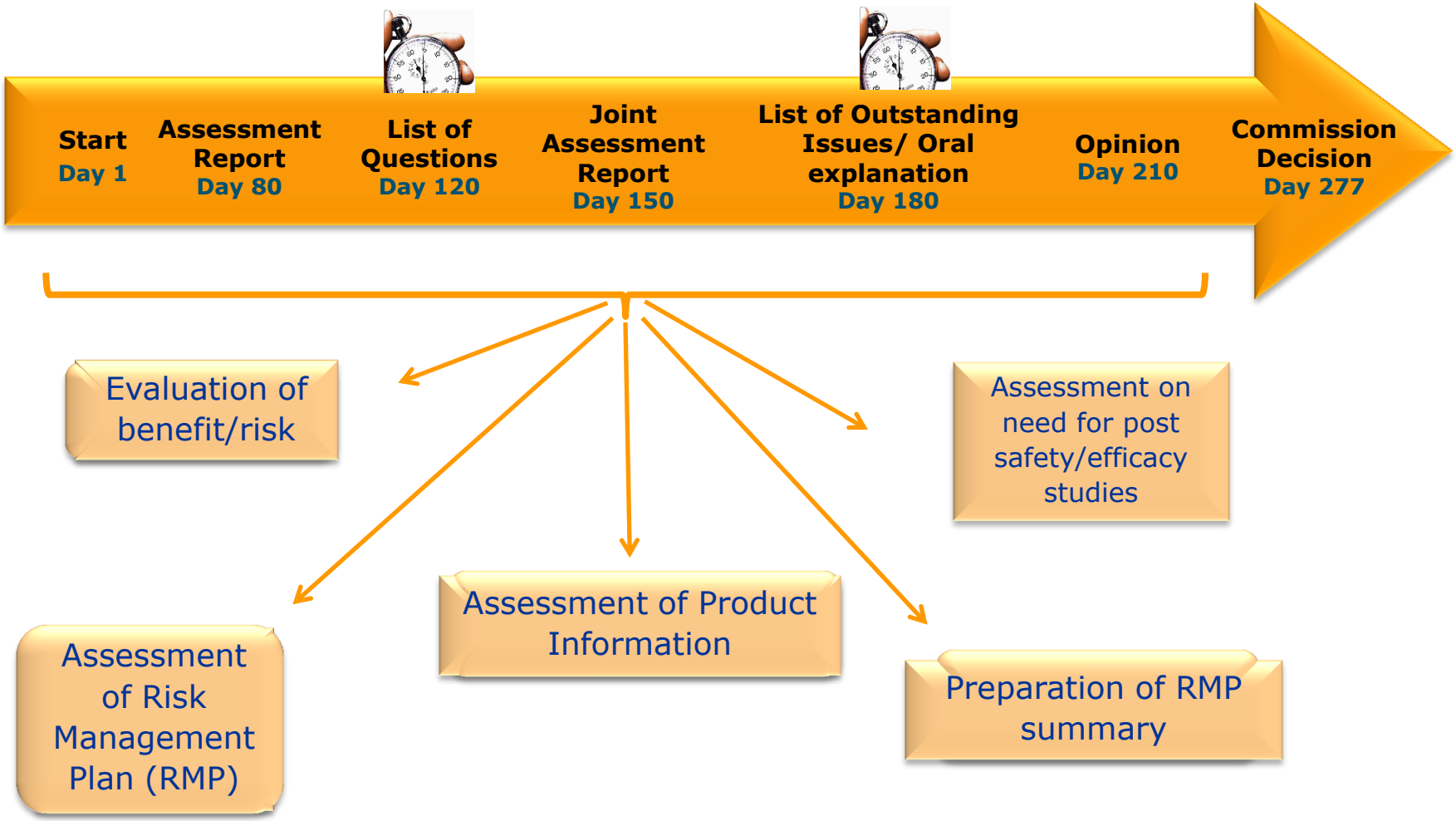
Conditional Approval:

- Comprehensive data not available; to be provided after approval
- Must fulfil scope (orphan drugs, emergency threats, serious and life-threatening diseases) Approval valid for 1 year, renewable

Medicines Lifecycle: Development and Regulatory



EVALUATION OVERVIEW - CHMP



Authorised!

What now?

Pharmacovigilance and Risk Management

What we know at the end of the clinical trial programme…

Is the tip of the iceberg

compared to what we don't know! … which is the rest of the iceberg..

Pharmacovigilance; Data Collection & Management

Patient with Adverse Drug Reaction (ADR)

Healthcare professional

ADR report

OR

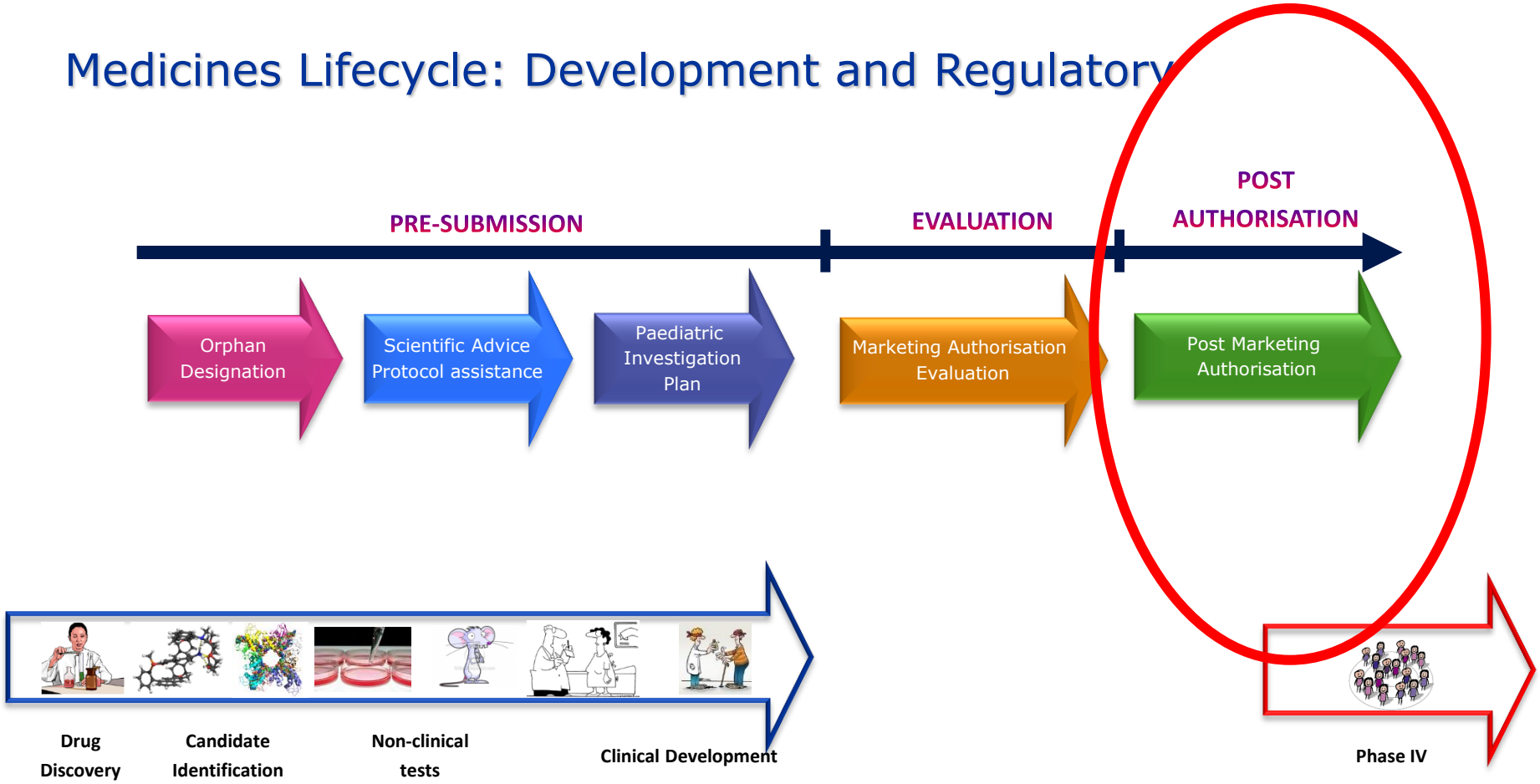
Marketing Authorisation Holder

National Competent Authority

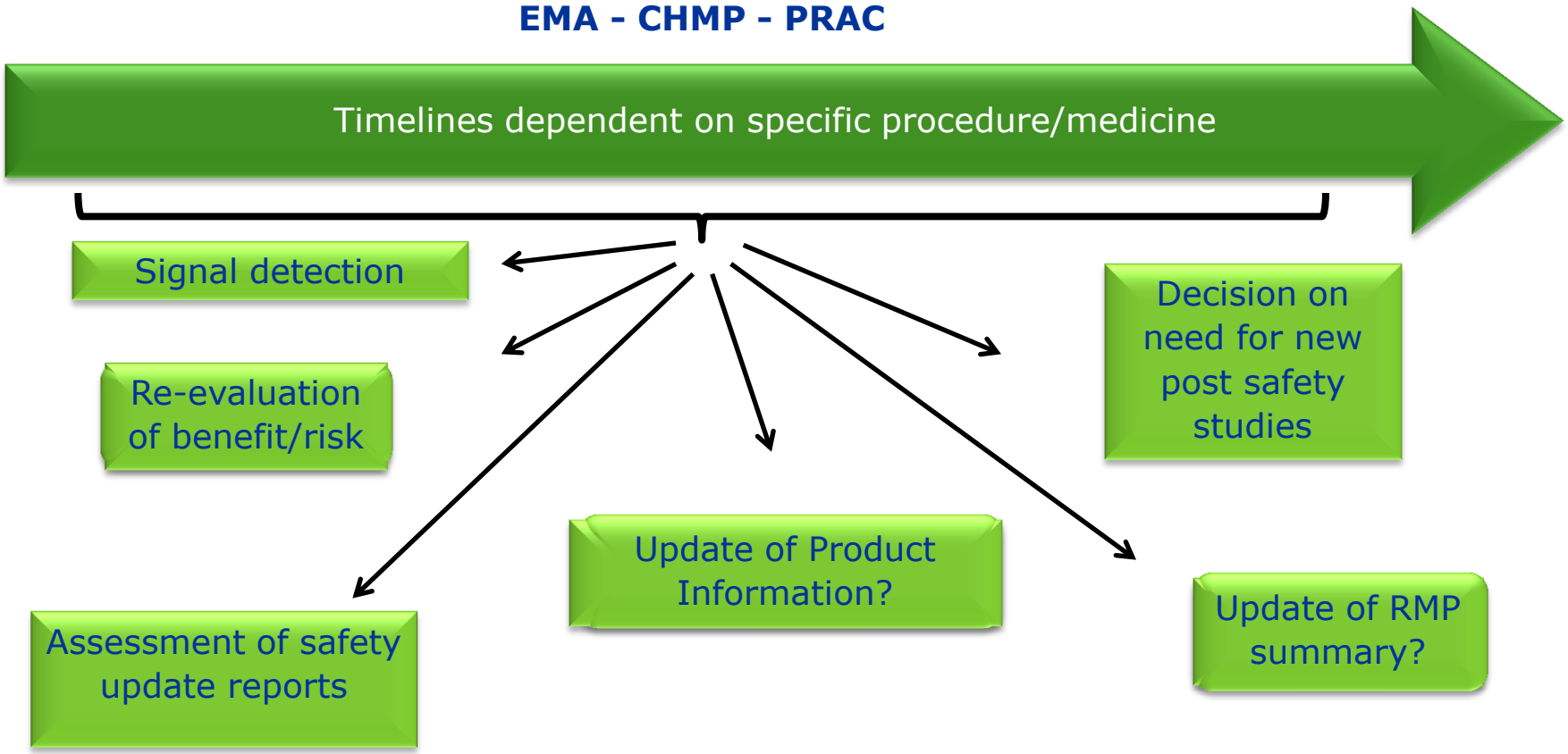
EudraVigilance Human

Safety monitoring

Medicines Lifecycle: Development and Regulatory

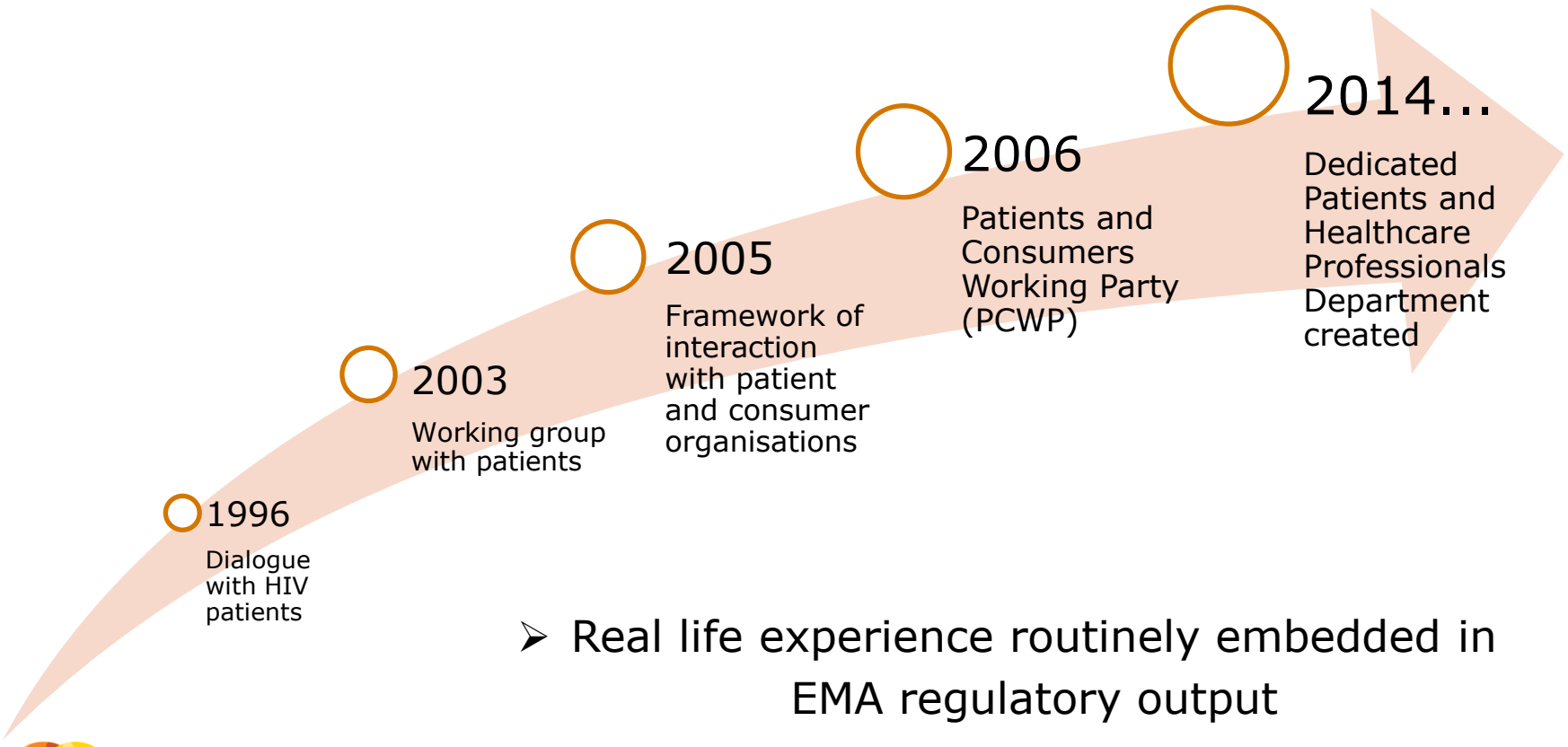


POST-AUTHORISATION OVERVIEW



Patient/consumer involvement in the EMA

Interaction with patients: the EMA journey… so far



- Real life experience routinely embedded in EMA regulatory output

S-PH will lead the EMA in its engagement with patients, consumers, healthcare professionals, academics and their organisations

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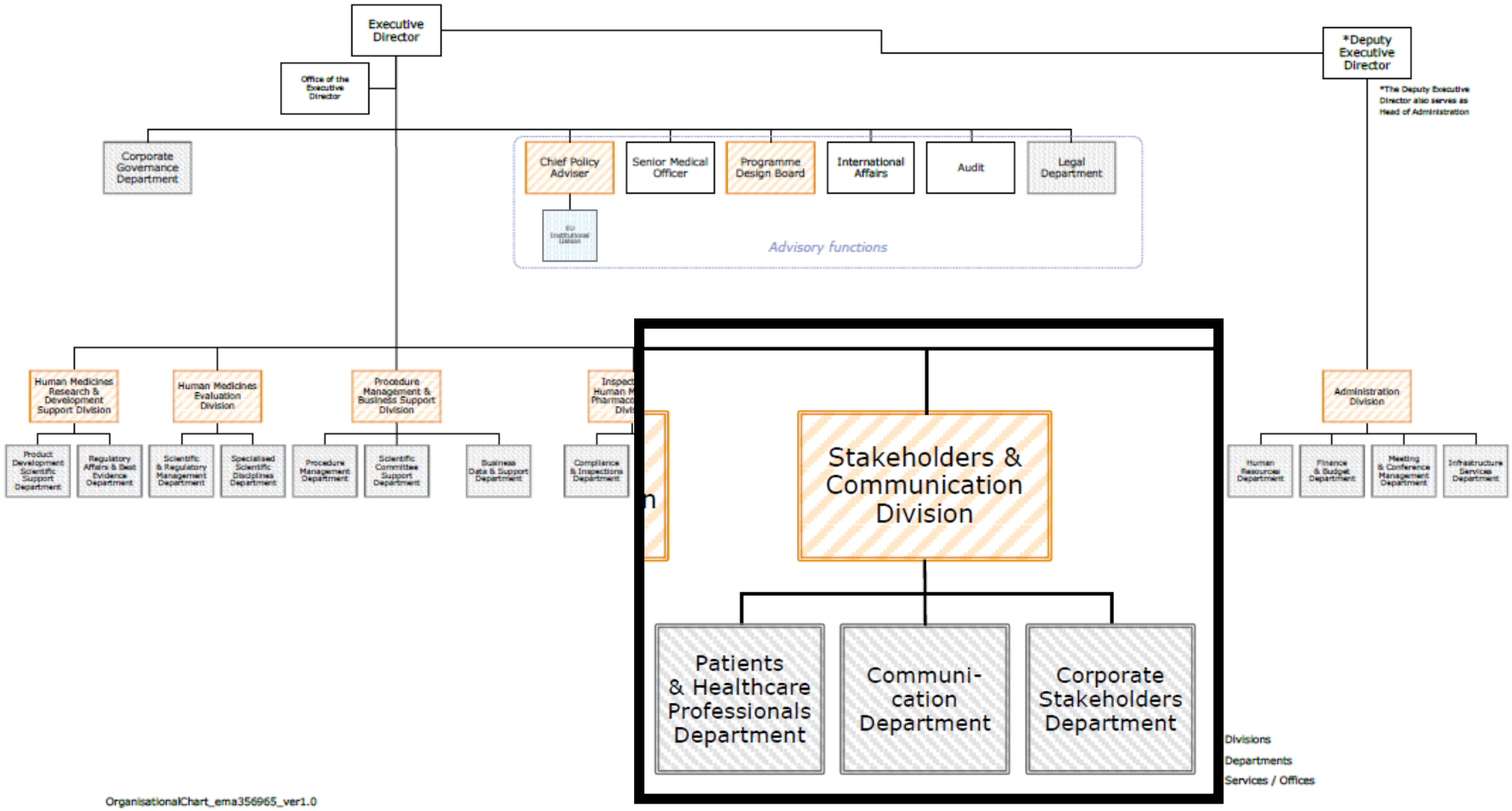
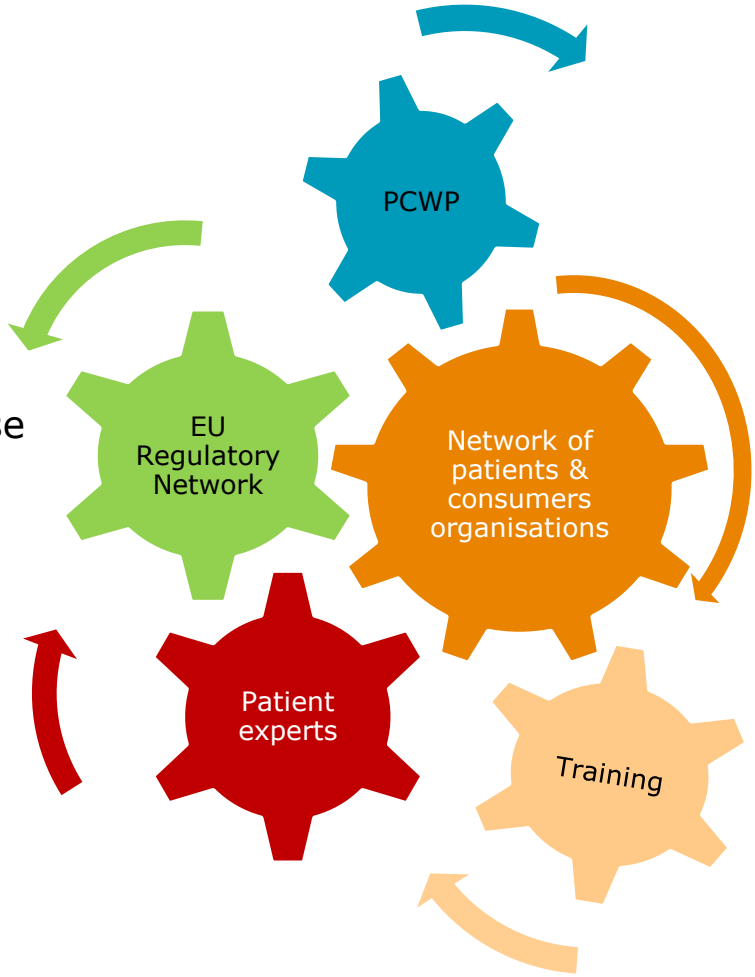


The framework relies on five critical elements

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



Which patient organisations?

- 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)

- Launched in 2005 and "continuous"

- List of eligible patients & consumers organisations published on the EMA website

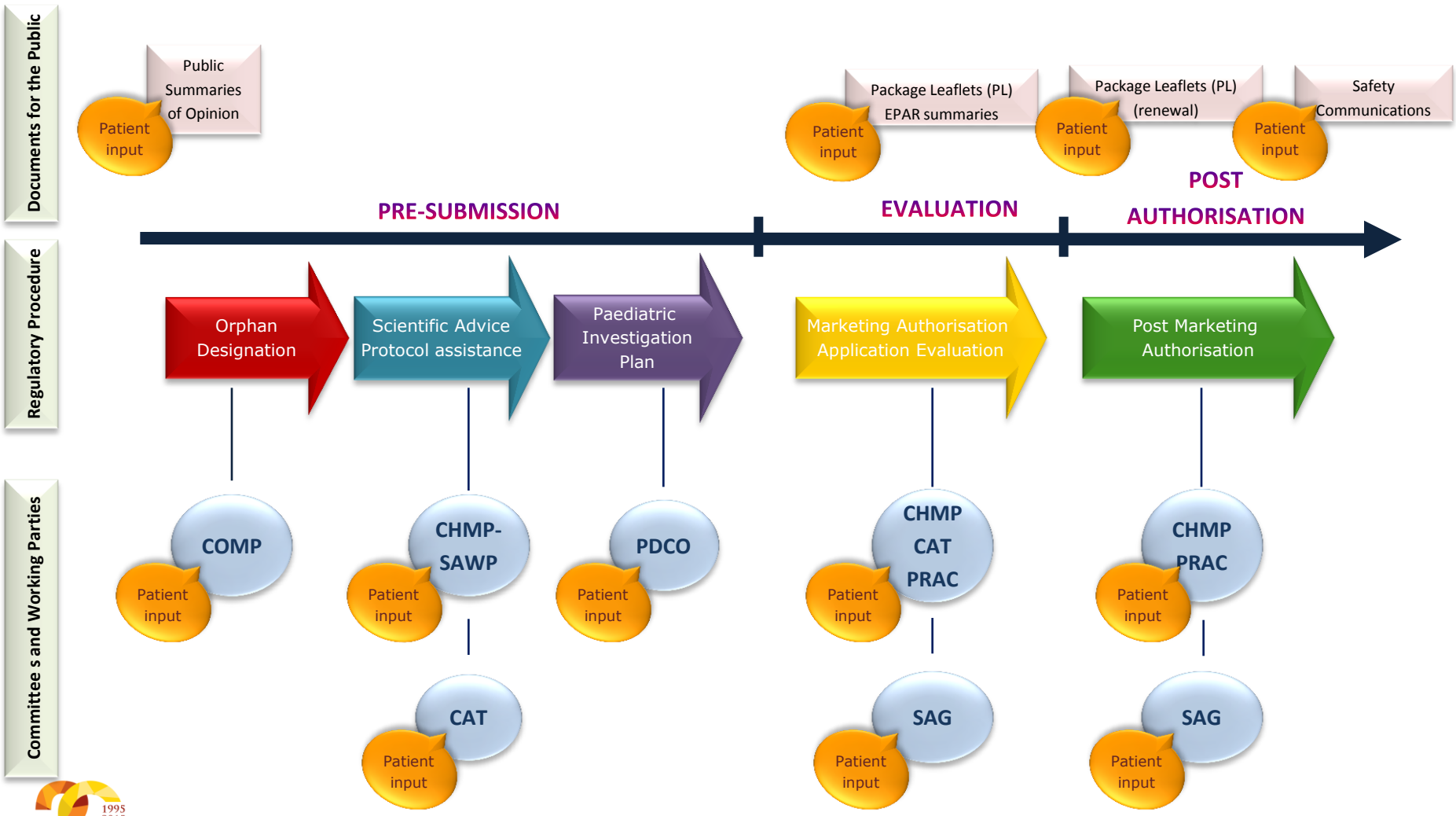
- Now have large network of EU patient organisations and individuals interested and registered to work with the EMA

Criteria to be fulfilled by organisations involved in EMA activities

- **Definition:** not-for-profit patient focused organisation ;
- **Legitimacy:** statutes registered in one of the Member States of the EU/EEA;
- **Mission/objectives:** clearly defined and published on the EMA website;
- **Activities:** specific interest in medicinal products;
- **Representation:** representative throughout the EU/EEA;
- **Structure:** governing bodies shall be patients or carers);
- **Accountability and Consultation Modalities:** statements/opinions of the organisation should reflect the views/opinions of its members;
- **Transparency:** all sources of funding should be public; relationships with corporate sponsorship should be clear and transparent.

Eligible organisations: patients/consumers

Opportunities for Patient involvement along the medicine lifecycle at EMA



Three categories of patient participation:

1. Member, alternate or observer
2. Individual patient expert
3. Representative of an organisation

Patient involvement in EMA activities (members)

Members of:

- Management Board (**MB**)
- Committee for Orphan Medicinal Products (**COMP**)
- Paediatric Committee (**PDCO**)
- Committee for Advance Therapies (**CAT**)
- Pharmacovigilance Risk Assessment Committee (**PRAC**)

EMA Patients' and Consumers' Working Party (PCWP)

Patients and Consumers Working Party (PCWP)

The PCWP plays a key role in the interaction between the EMA and patient organisations. Platform for dialogue and exchange on relevant issues concerning medicines; mandated to help monitor the interaction and identify gaps and priorities in the overall interaction;

- 19 members and 16 alternates representing Patients and Consumer Organisations;
- 6 members from the EMA Scientific Committees;
- 1 member from the EMA secretariat;
- Observers from the Management Board.

Patient involvement in EMA activities (individuals)

Product development:

- Participation in scientific advice/protocol assistance procedures

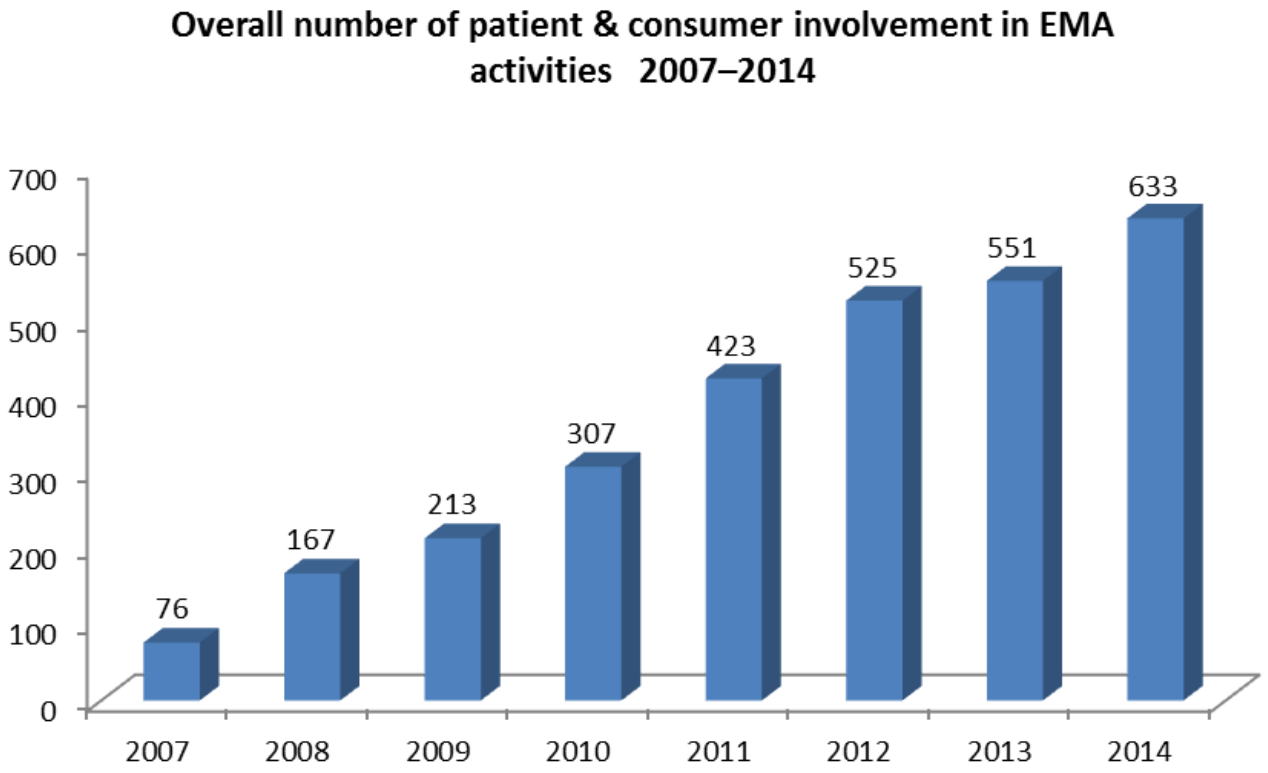
Benefit/risk evaluations:

- Participation in scientific advisory / ad-hoc expert group meetings (SAGs)
- Respond to ad-hoc consultations on assessment of medicines from scientific committees and working parties
- Review information on medicines: Package leaflets, EPAR summaries, safety communications (Q&As) and other Agency documents for the public

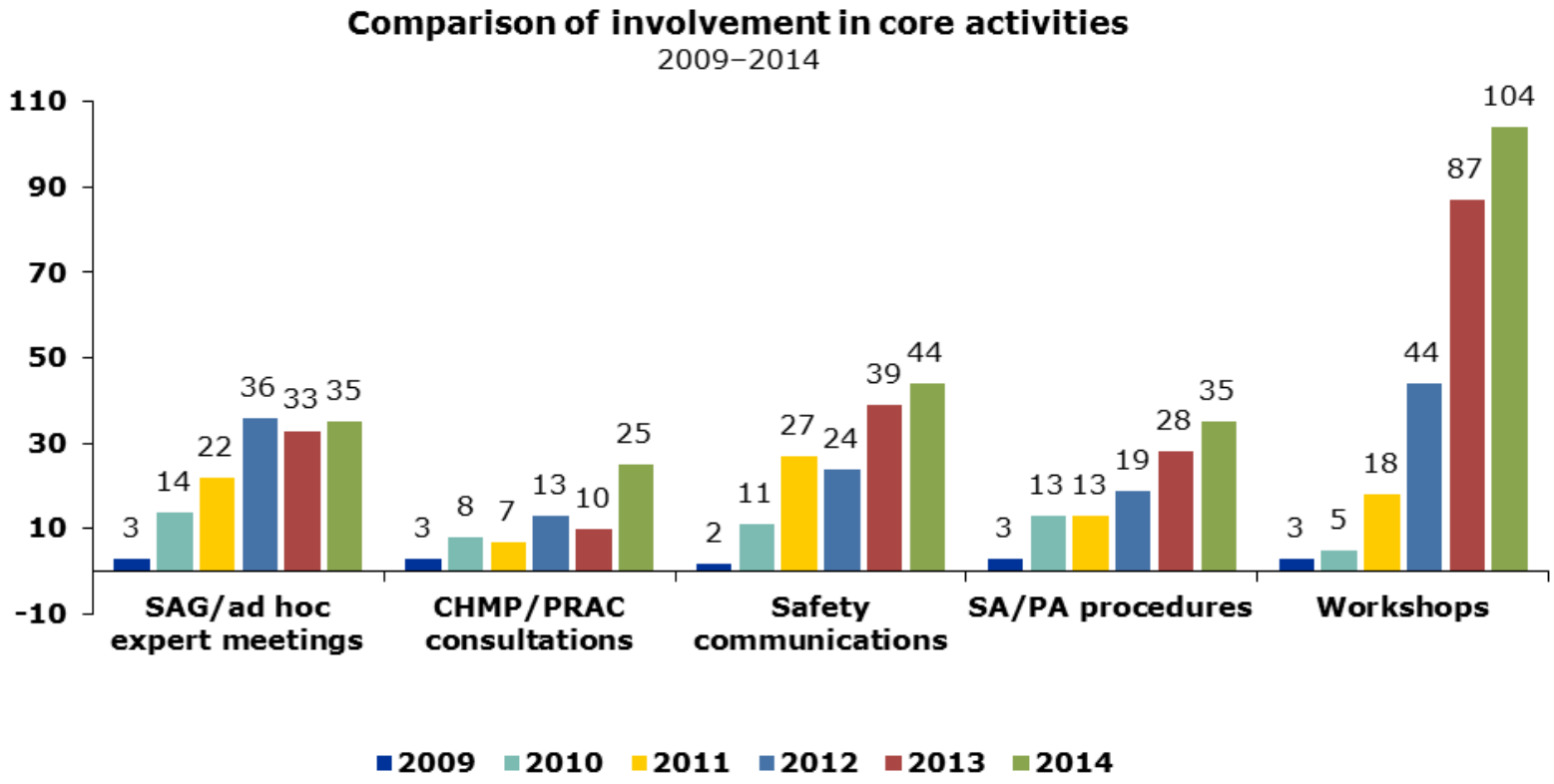
Other activities (representative of organisation)

- Input in the preparation of **guidelines**
- Involvement in several on-going **EU-wide initiatives**:
  - **EudraCT** (EU clinical trials register), **Eudravigilance** (adverse reaction data), **ENCEPP** (European Network of Centres for Pharmacoepidemiology and Pharmacovigilance), and **Enpr-EMA** (European Network of Paediatric Research)
- Participate in Agency **conferences** and **workshops**

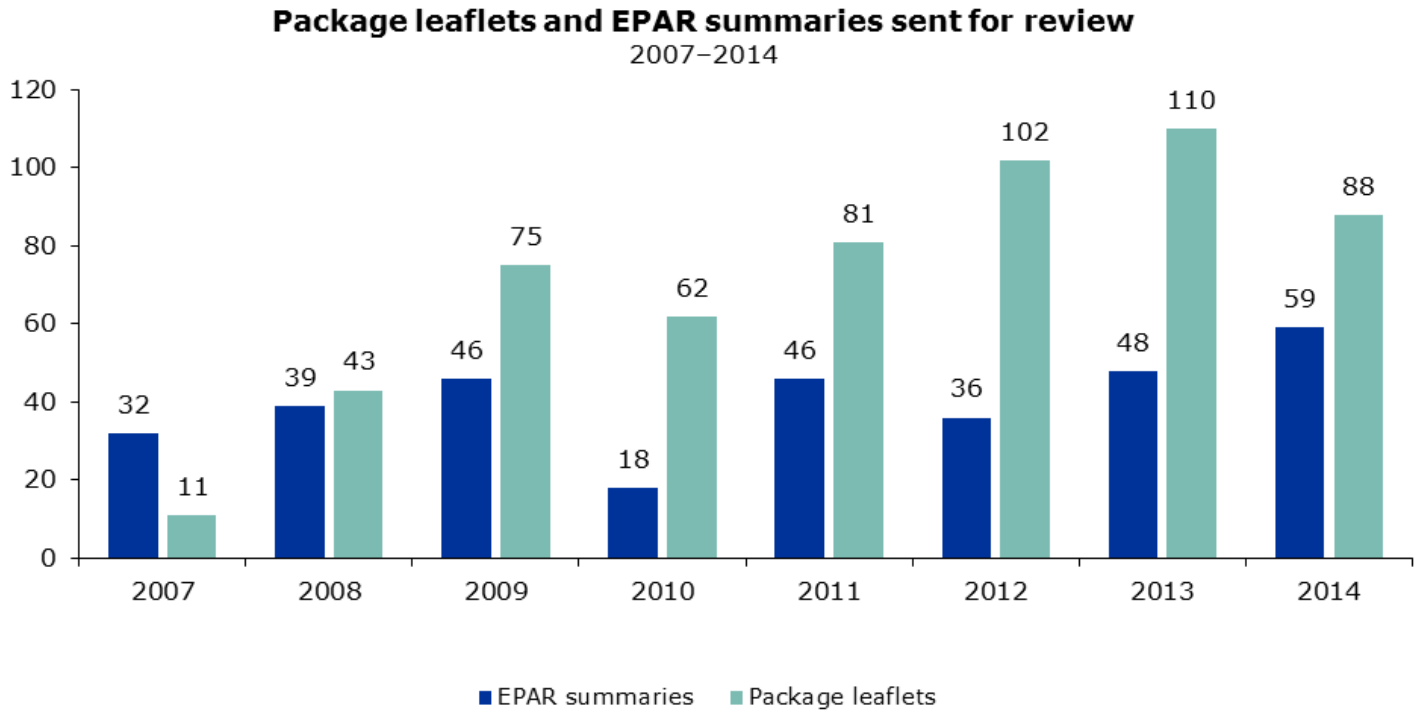
Overall increase of involvement in EMA activities



Increasing across range of EMA activities



Increasing involvement; review of documents



Increasing involvement; safety communications

Monitoring and reporting

- Annual report to EMA Management Board - overview of all activities in which patients and their organisations have been involved – *quantitative* and *qualitative* aspects
- Every 2 years a satisfaction survey is sent to all patients who have participated that year
- Proposals for improvements discussed and included in the next PCWP work-plan

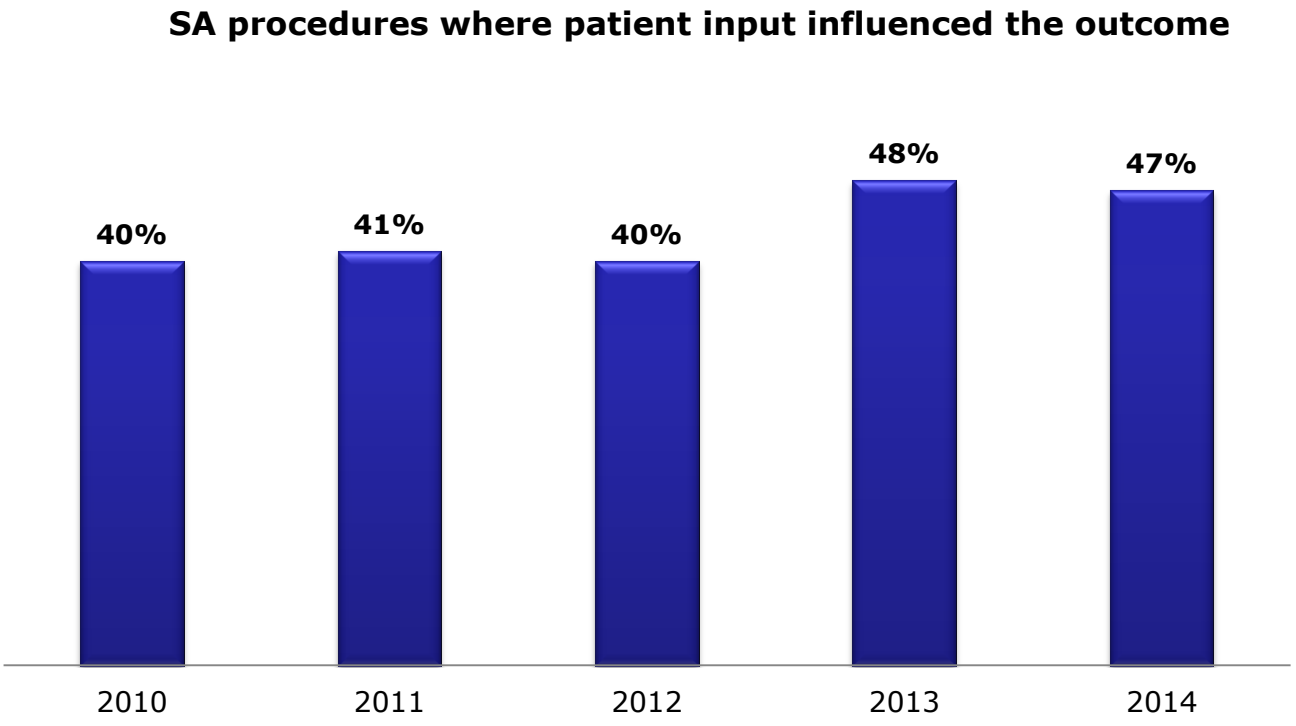
Value and impact of involving patients in EMA

- The involvement of patients and consumers has;
  - Brought the everyday aspects of living with a disease into the scientific discussions
  - Improved the quality of patient information and communication on medicines
  - Increased the dissemination of public EMA outcomes
- Patients are a recognised and integral part of the Agency's work
- Participation has not only increased and diversified but has also been refined to ensure optimal involvement.

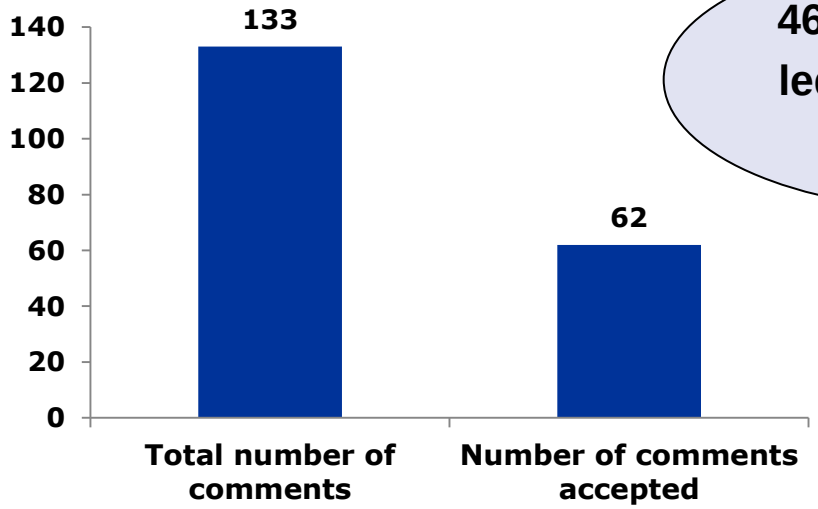
Although this collaborative journey with many stakeholders, cultures and languages has its challenges, different perspectives are crucial and have **ultimately resulted in more meaningful decisions for all concerned.**

Impact of involving patients; scientific advice

Scientific advice on the feasibility of planned investigations (e.g. Clinical Trial designs)

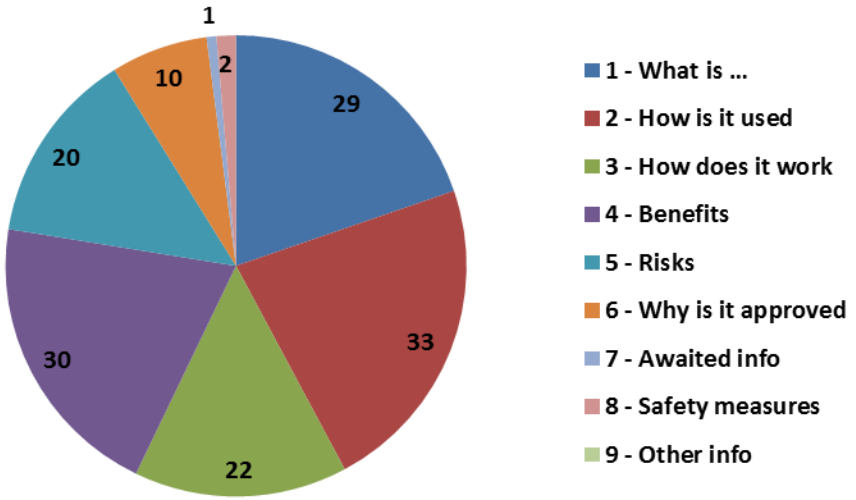


Impact of involving patients; review of documents



46% of comments led to a change in the final text

No. of comments by area



Challenges

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

Healthcare Professional Working Party (HCPWP) – est. Jun 2013)

Membership includes general practitioners, specialist doctors, nurses, pharmacists and representatives of learned societies.

•Gain experience and perspective of healthcare professionals on the current use of medicines in real clinical practice

•Contribute to a more efficient and targeted communication to healthcare professionals

•Encourage and help professional organisations cascade information

•Enhance healthcare professionals understanding of the role of the EU medicines Regulatory Network

- Input in SAG/Ad-hoc expert group meetings, Review of safety communications and DHPCs, Committee consultations & participation in EMA workshops
- **Joint meetings with the PCWP!**

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Working with healthcare professionals

Initiatives at the EMA to enhance collaboration with Academia

Current areas of dialogue

•EMA Committees and working parties, scientific advice (SA), Joint SA-HTA, Scientific Advisory Groups (SAGs), Guideline preparation, etc…

Policy / regulatory initiatives that can impact on clinical research

•Policy on Publication of clinical trial data

•New Pharmacovigilance and Clinical trial regulations

Framework of collaboration with academia

•Under construction – for adoption by management board in December 2015

Incentives for micro, small and medium-sized enterprises (SMEs)

- The EMA provides incentives for SMEs that are developing medicines
- Aims to promote innovation & development of new medicines by SMEs
- Incentives include:
  - administrative and procedural assistance;
  - fee exemptions or reductions for some procedures and administrative services;
  - deferral of the fees payable for an application for marketing authorisation;
  - assistance with translations of the product information documents;

European Network of Paediatric Research at the EMA (Enpr-EMA)

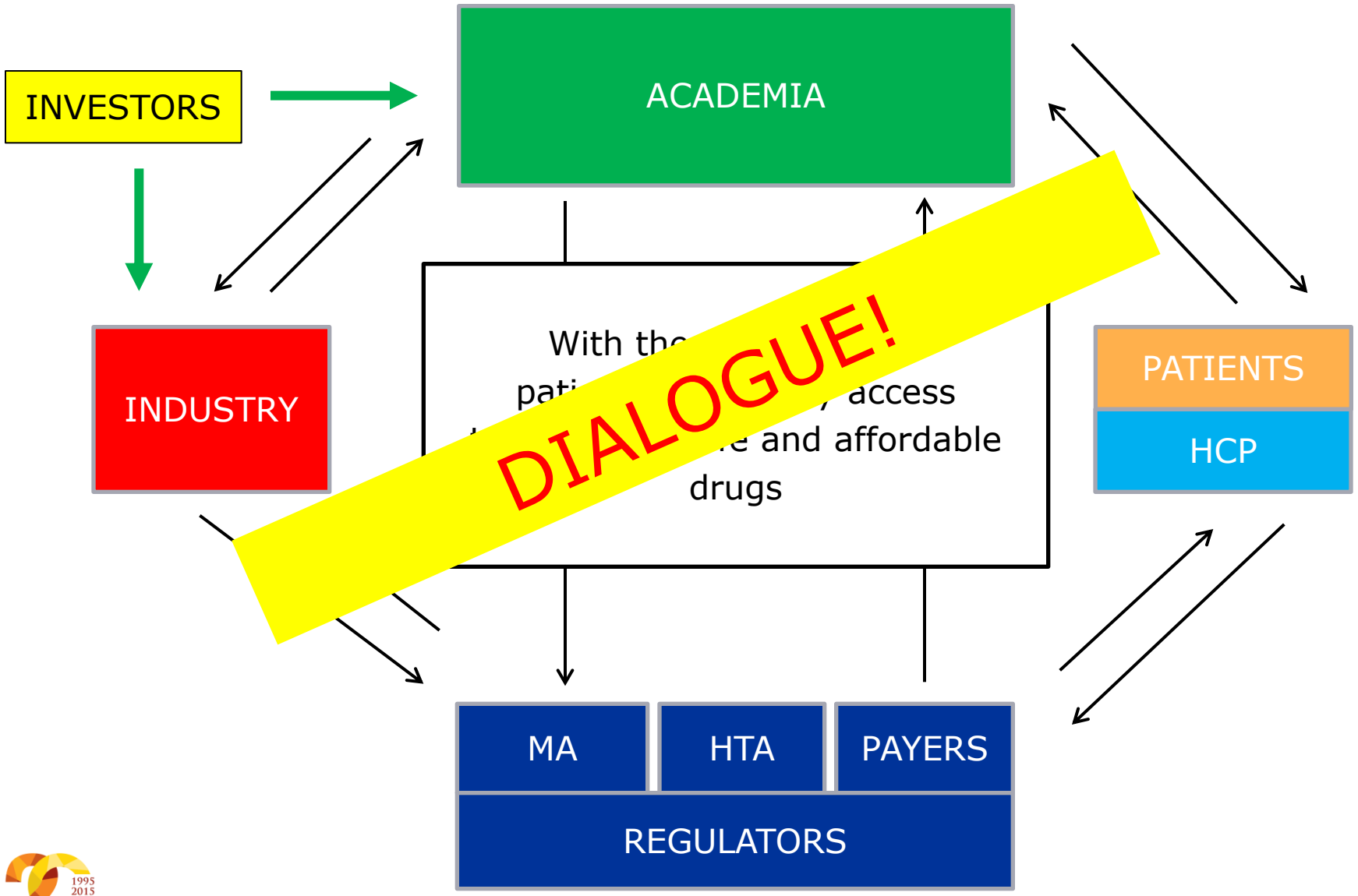
Network of research networks; investigators and centres with recognised expertise in performing clinical studies in children

Main objective: facilitate studies to increase availability of medicines for use in children, by:

- fostering high-quality, ethical research on medicines for use in children;
- enabling collaboration between networks and stakeholders;
- avoiding unnecessary duplication of studies;
- building up scientific and administrative competence at a European level;
- promoting European Commission framework programme applications.

European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP)

- ENCePP is a network of over 170 research centres, existing networks and providers of healthcare data, coordinated by the EMA.
- Its goal is to strengthen post-authorisation monitoring of medicines by facilitating the conduct of multicentre, independent studies focusing on safety and on the balance of benefits and risks, using available European research expertise.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ACADEMIA
INDUSTRY
With the
pati
access
e and affordable
drugs
DIALOGUE!
PATIENTS
HCP
MA
HTA
PAYERS
REGULATORS

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Acronyms

- ADR = Adverse Reaction
- AR = Assessment Report
- CHMP = Committee for Medicinal Products for Human Use
- CD = Commission Decision
- GCP – Good Clinical Practice
- GLP = Good Laboratory Practice
- GMP = Good Manufacturing Practice
- LoQ = List of Questions
- LoOIs = List of Outstanding Issues
- MAH = Marketing Authorisation Holder
- MS = Member State
- OE = Oral explanation
- PASS = Post Authorisation Safety Study
- PI = product information
- PRAC = Pharmacovigilance Risk Assessment Committee
- PSUR = Periodic Safety Update Report
- RMP = Risk Management Plan
- SAG = Scientific Advisory Group
- SmPC = Summary of Product Characteristics