EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The European Medicines Agency (EMA)

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EMA Training Day

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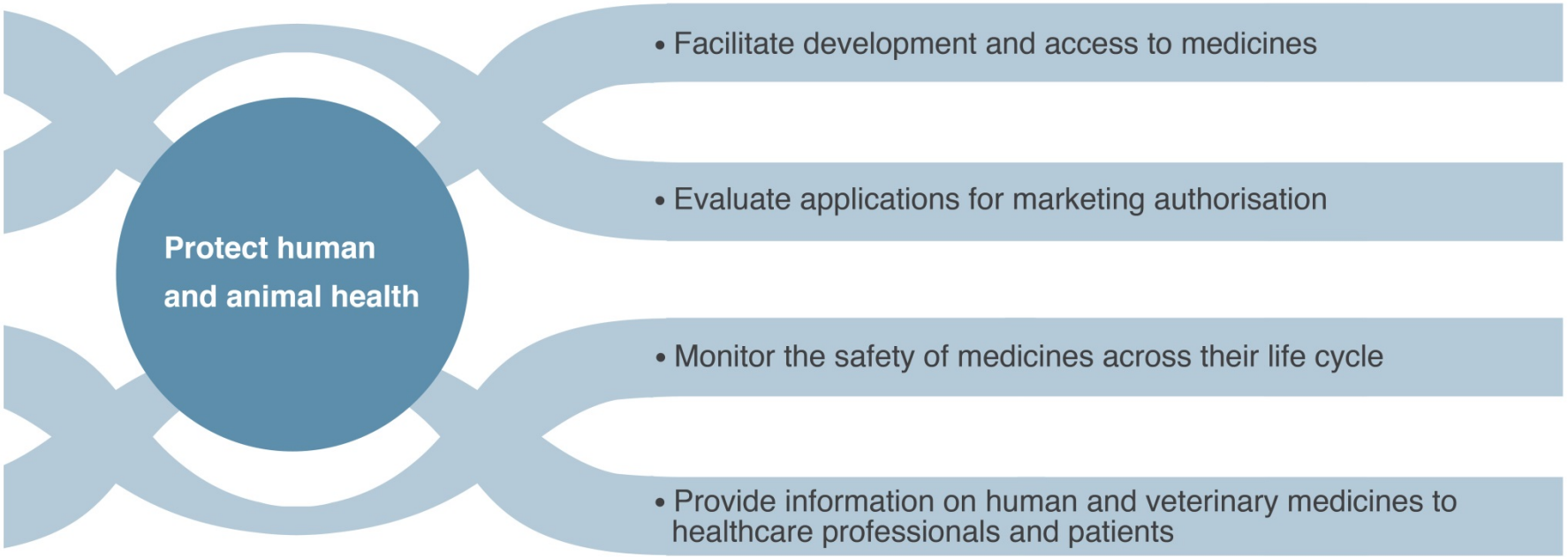
An agency of the European Union

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)

What do 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 information on human and veterinary medicines to healthcare professionals and patients

The key roles of the EMA

- 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 marketing authorisation applications** for **human** and **veterinary** medicines
- Coordination of European **pharmacovigilance** (supervision of medicines)
- Provision of **information** on medicines to patients and healthcare profes
- Evaluation of **arbitration** and **referral** procedures
- Coordination of Member States' **inspections**

The European medicines regulatory network

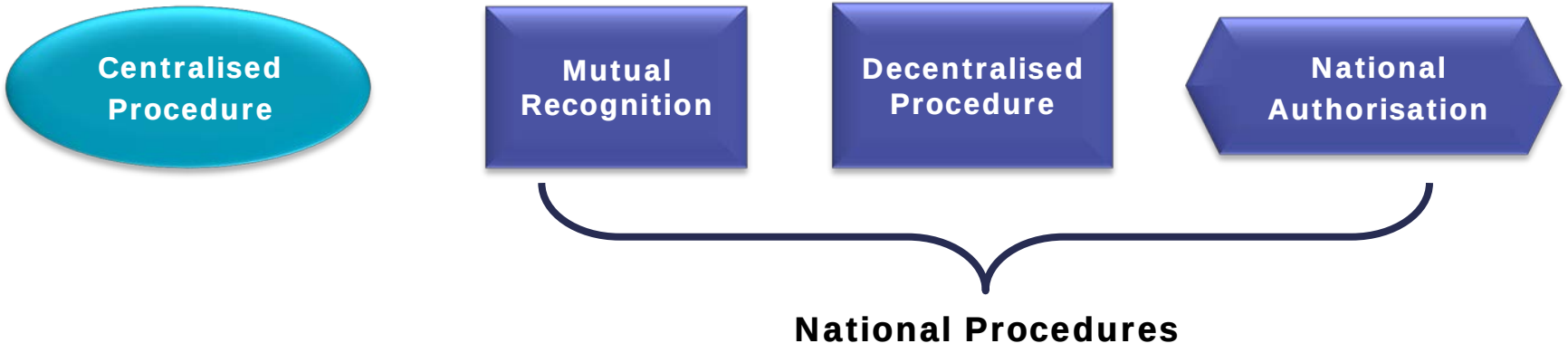
~ 50 national regulatory authorities European Commission European Medicines Agency

The European System

All medicines must have a marketing authorisation before they can be put on the market

Two ways of obtaining authorisation:

1) The centralised procedure or 2) National marketing authorisation procedures



EMA: focal point of the centralised procedure

ONE

Marketing Authorisation application

Evaluation

Authorisation valid in all EU Member States

What is the benefit of the centralised procedure for EU citizens?

- Medicines are authorised for all EU citizens at the same time
- Centralised safety monitoring
- Product information available in all EU languages at the same time

Which medicines are approved through the centralised procedure?

- Human medicines for the treatment of HIV/AIDS, cancer, diabetes, neurodegenerative diseases, auto-immune, and other immune dysfunctions, and viral diseases
- Medicines derived from biotechnology processes, such as genetic engineering
- Advanced-therapy medicines, such as gene-therapy, somatic cell-therapy or tissue-engineered medicines
- Officially designated 'orphan medicines' (medicines used for rare human diseases)

Type of Approvals

Standard:

Comprehensive data

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

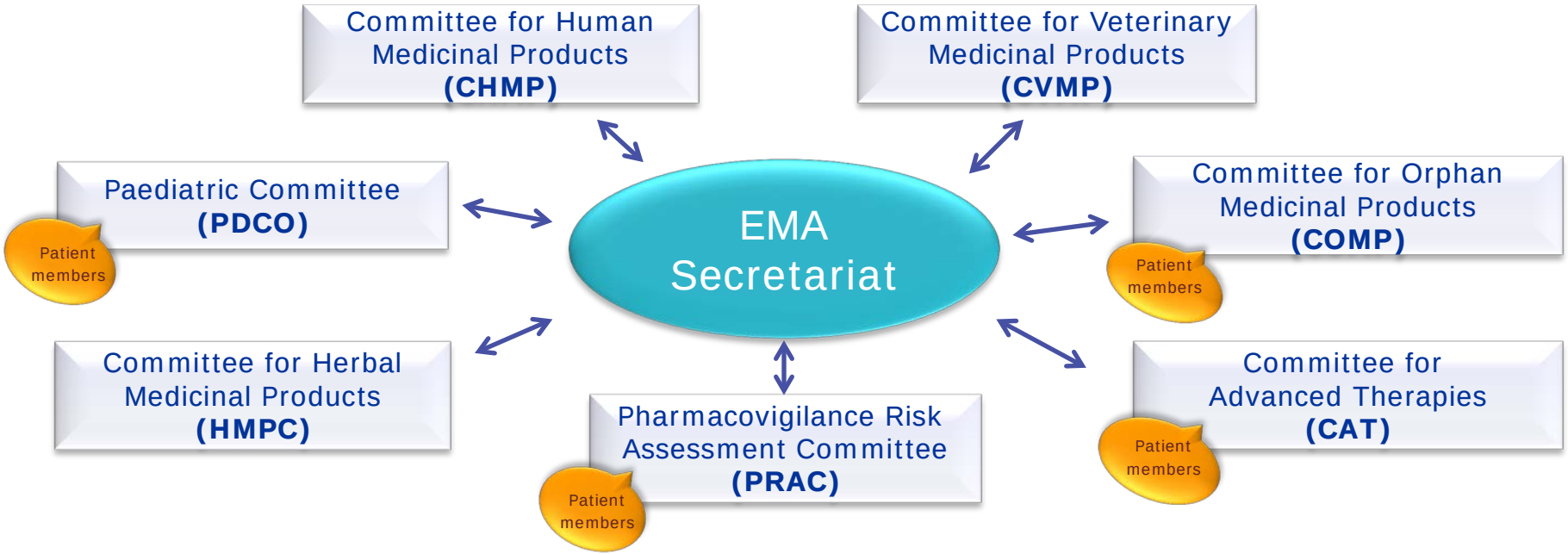
Exceptional Circumstances:

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

What the EMA does not control

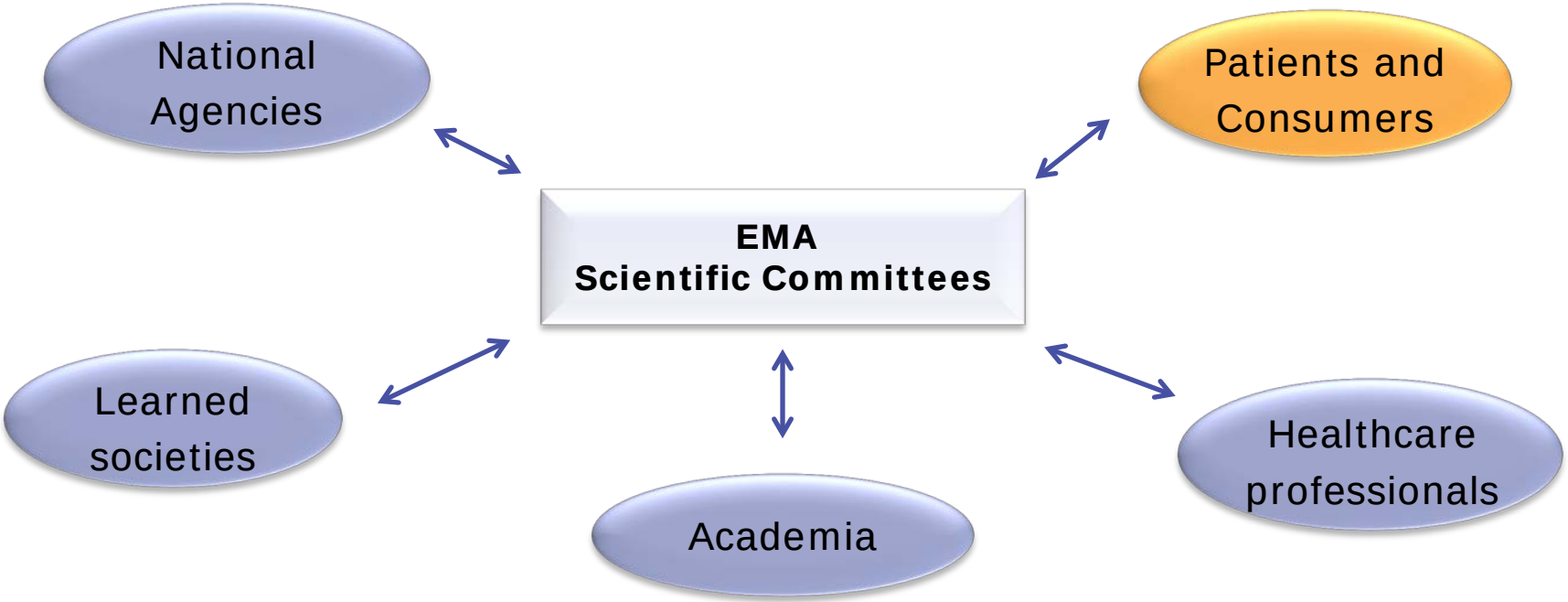
- Pricing of medicines
- Access to medicines
- Advertising of medicines
- Patents on medicines
- Medical devices
- Homoeopathic medicines
- Food supplements
- Cosmetics
- Tobacco

EMA and its scientific committees

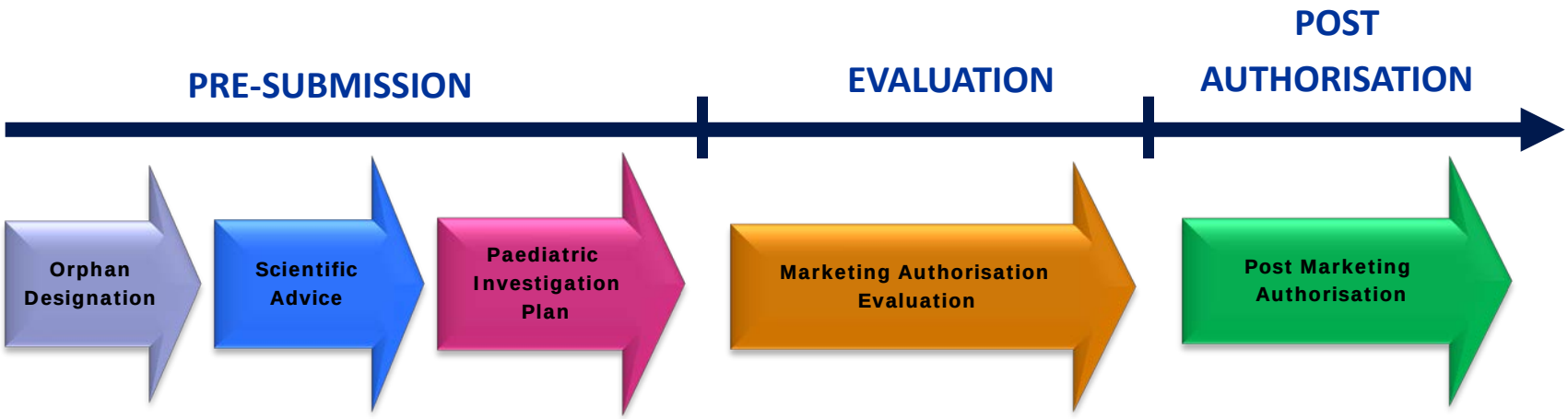


The EMA committees contain members nominated by the medicines regulatory authorities of the EU Member States (the 'national competent authorities')

Experts who work with the scientific committees

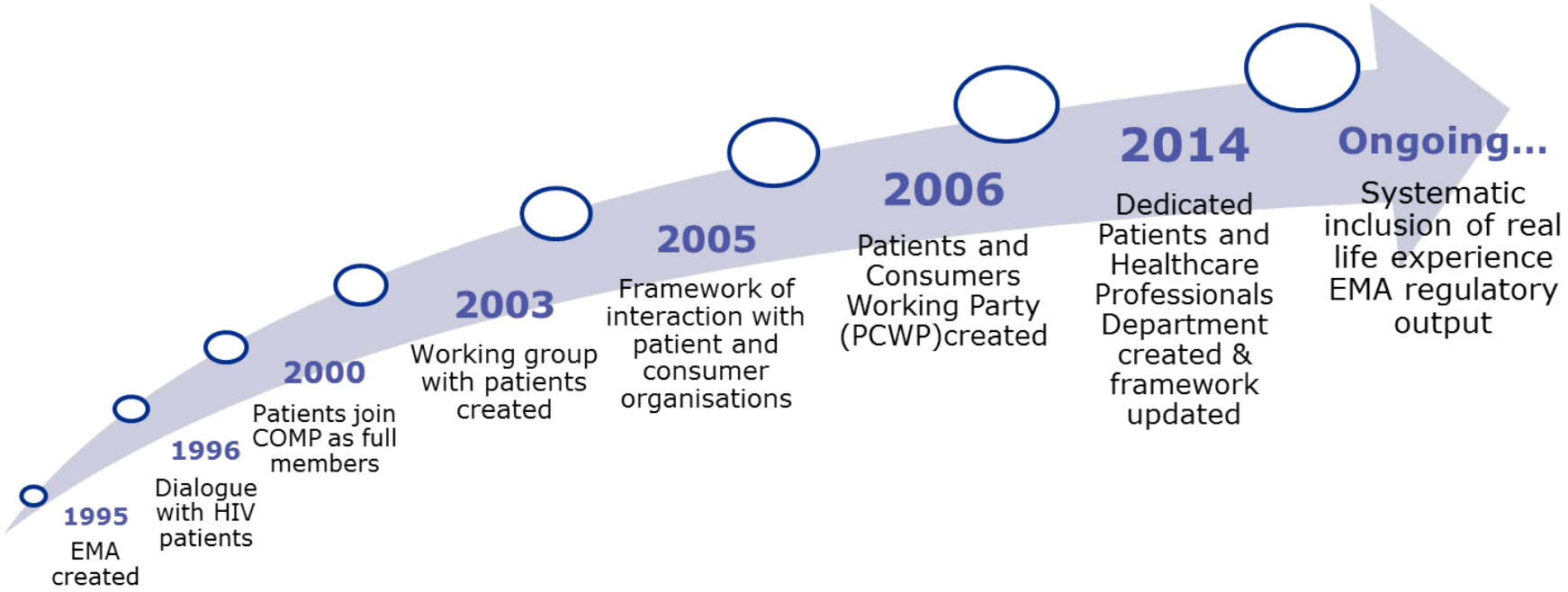


Medicines Regulatory Lifecycle



Patient/consumer involvement in the EMA

Interaction with patients: the EMA journey… so far



How are patients involved at EMA?

Non-product related

Patients representing *patients'* organisations

- Management Board
- EMA Scientific Committee(s)

Patients representing *their* organisations

- Patients' and Consumers' Working Party (PCWP)
- EMA consultations
- Workshops

Product related

Patients as *individual* experts

- Scientific Advice / Protocol Assistance Procedures
- Scientific Advisory / *ad hoc* expert Groups
- Scientific committee consultations
- Review of documents

Patient involvement as individual experts in EMA activities

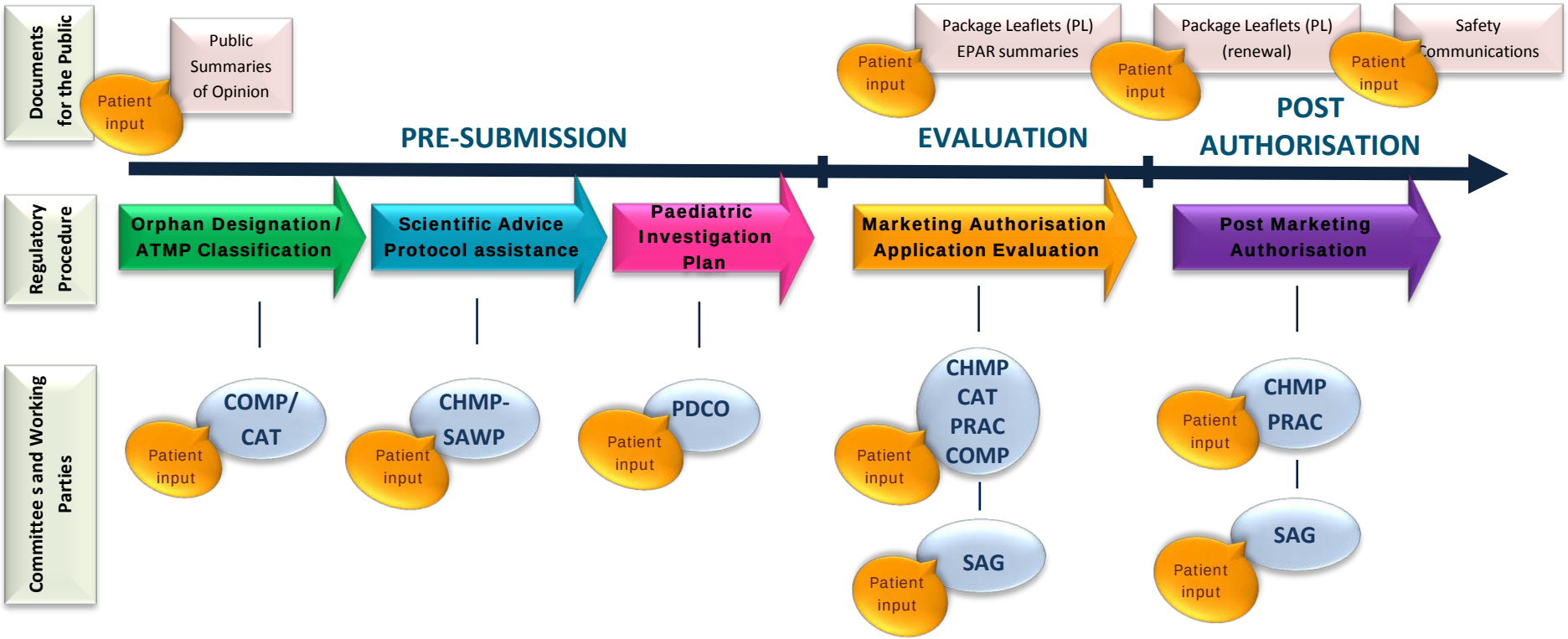
Pre-submission:

- Participation in scientific advice/protocol assistance procedures

Evaluation and Post-authorisation

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

Patient involvement along the medicine lifecycle at EMA



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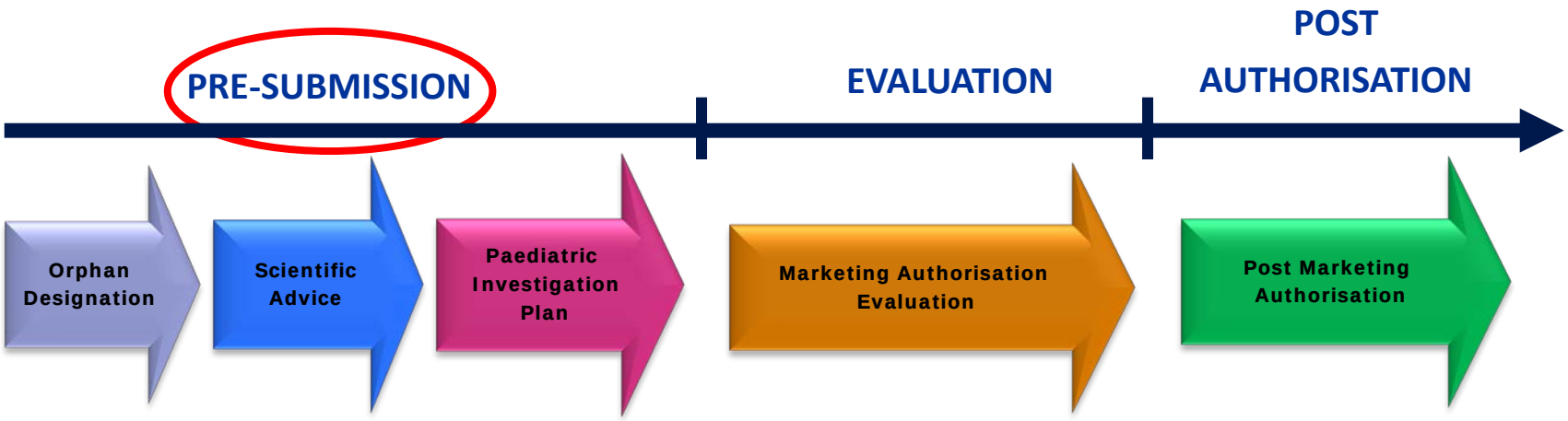
Scientific Advice at EMA


PRE-SUBMISSION
EVALUATION
POST AUTHORISATION
Orphan Designation
Scientific Advice
Paediatric Investigation Plan
Marketing Authorisation Evaluation
Post Marketing Authorisation

Scientific Advice

- Pharmaceutical companies can request scientific advice from the EMA regarding the development of a medicine.
- Aimed at ensuring the most appropriate studies are conducted, avoiding major objections related to the study design during evaluation
- The Scientific Advice Working Party (SAWP) and the Committee for Medicinal Products for Human Use (CHMP) provide scientific advice by answering specific questions posed by the companies.

Types of questions

Scientific Advice can be provided on questions ranging from:

- Quality – manufacture of medicines
- Non-clinical – animal studies – interpretation and extrapolation of results
- ➢ **Clinical** – discussion of study population, endpoints, feasibility of trial
- Regulatory – including statistics
- ➢ **Significant benefit** – for orphan medicines (where applicable)

The role of patients and patient representatives

Patient representatives are invited to participate in EMA scientific advice procedures:

- Either face to face meeting or via written comments
- Share their ‘real-life’ perspective and experience with the SAWP and the pharmaceutical company, in relation to a particular medicine in their disease area.
- Provide comments on the development proposals from the company (e.g. endpoints, population, feasibility etc)

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Scientific Advisory Group (SAG) / ad hoc expert meetings


PRE-SUBMISSION
EVALUATION
POST AUTHORISATION
Orphan Designation
Scientific Advice
Paediatric Investigation Plan
Marketing Authorisation Evaluation
Post Marketing Authorisation

Scientific Advisory / Ad hoc expert Groups

- The CHMP or the PRAC can convene a SAG during the evaluation of a medicine when they encounter specific questions that are best answered by experts in the field, including patients

- SAGs exist for specific therapeutic areas and when an issue arises for which there is no SAG, an *ad hoc* expert group is organised

- Two patients, with experience of the disease/condition, are invited to participate in every SAG / ad hoc expert group meeting

- Patients contribute by providing input to the discussions on the benefits and risks, from their perspective in relation to the questions that the CHMP is asking

Part II

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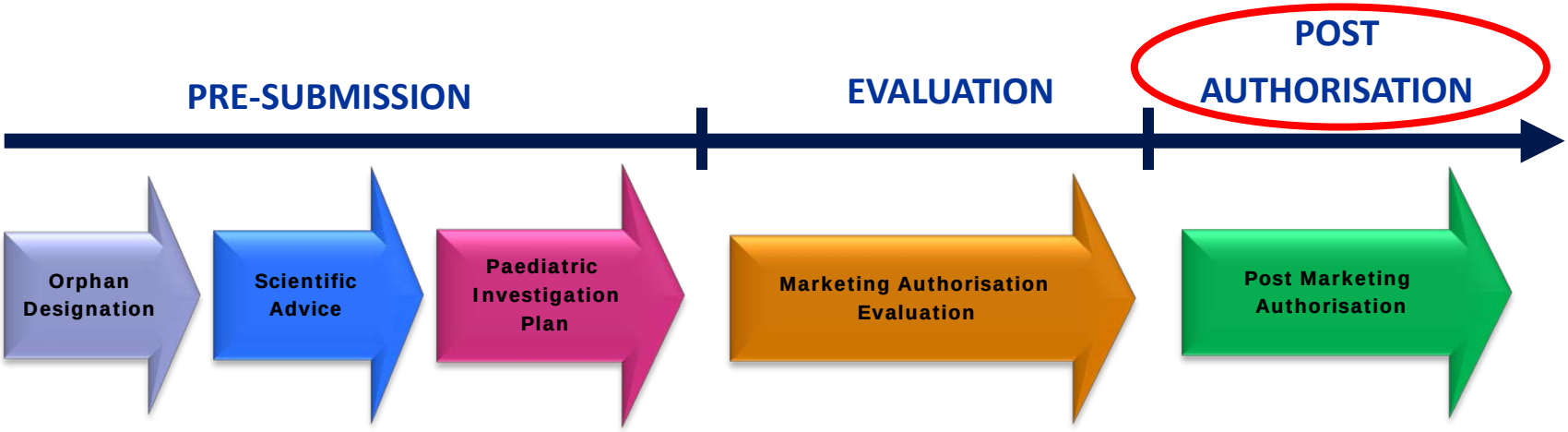
Pharmacovigilance at EMA

Pharmacovigilance

Pharmacovigilance is the process and science of monitoring the safety of medicines and taking action to reduce the risks and increase the benefits of medicines.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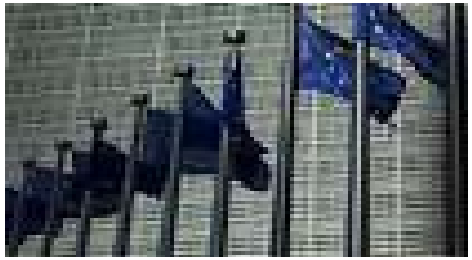


What now?

Authorised!

Pharmacovigilance overview

- Collect information on the potential side effects
- Decide if new or changing side effects are observed
- Decide if action is needed to optimise the safe and effective use of the medicine
- Take action and communicate to users
- Has action been effective?

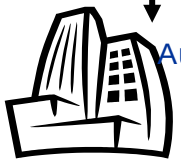
How do we monitor the risks?

Patient with Adverse Drug Reaction (ADR)

Healthcare professional

ADR report

OR



Marketing Authorisation Holder

National Competent Authority (NCA)

EudraVigilance Human

What safety actions can be taken

When new information arises that warrants action, regulators have several tools available:

- Update patient information/Summary of Product Characteristics (SmPC)
- Inform patients and/or healthcare professionals (Safety Communications, Direct healthcare professional communication (DHPC), educational material)
- Review of benefit-risk profile of medicine (referral)
- Restrict access to medicine

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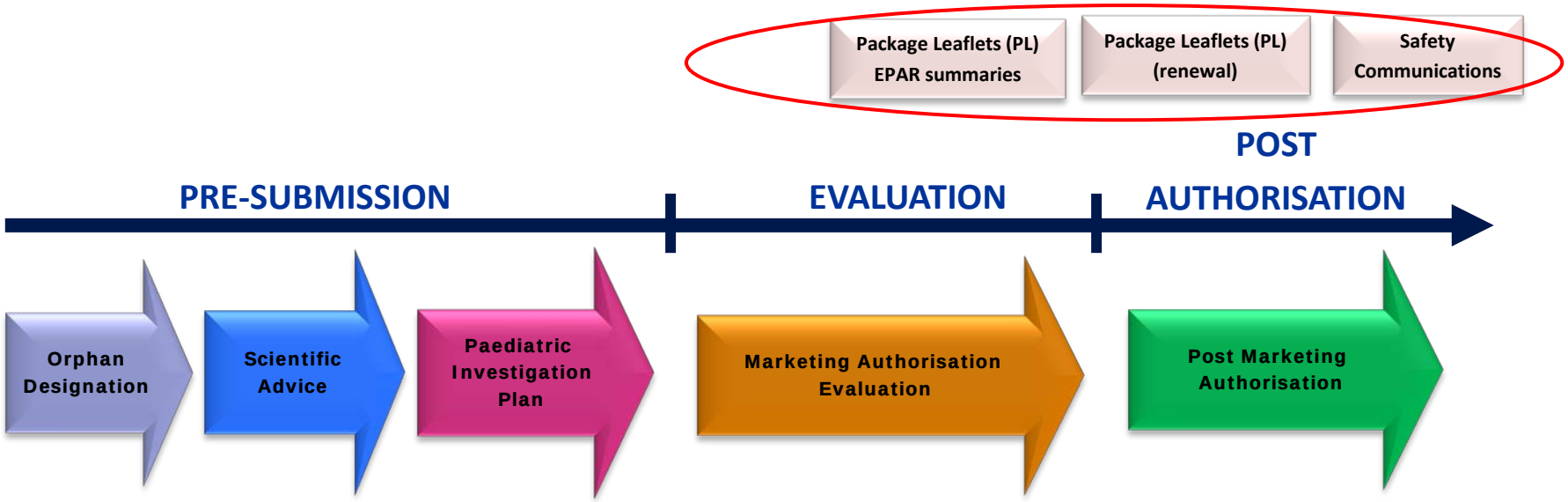
Review of Documents

Patient involvement along the medicine lifecycle at EMA



Which documents are reviewed by patients?

- European public assessment report (EPAR) summaries
- Package leaflets (PL)
- Safety communications
- Herbal summaries

Why the review by patients?

To make sure message is clear and all relevant information is included

- Complicated/oversimplified language
- Unexplained scientific terms
- Inappropriate explanations
- Unnecessary/missing information
- Confusing numbers
- Do you understand the main message?

Package Leaflet (PL)

- PL is part of the “product information” that is approved at the time of marketing authorisation
- Initially prepared by applicant when requesting a marketing authorisation
- All new and renewal PLs are sent for review to relevant patients with 10 days to comment
- Patients review in parallel to other scientific/linguistic reviewers
- Committee adopts the PL as part of its opinion
- Final PL published with Commission Decision
- After approval of the medicine, the PL is regulary updated

European Public Assessment Report (EPAR) summary

- At the time of marketing authorisation, the Agency publishes a European Public Assessment Report (EPAR) for the medicine which reflects the scientific conclusions reached by the CHMP
- It contains an EPAR summary written in a manner that is understandable to the public
- Drafted by the EMA immediately after the CHMP opinion and sent for review to the EMA project managers, CHMP rapporteurs, patients and the applicant
- All new EPAR summaries are sent for review to relevant patients with 10 days to comment
- The EPAR summary is finalised within about one month, adopted by the CHMP and then translated into all official EU languages before publication.
- EMA implements patient comments where possible

Safety communications (SC)

- SCs concern authorised medicines and tend to relate to major safety issues, often within ‘referral’ procedures
- Preparation of SCs implies short timelines with multiple stages of review and input from internal and external experts with limited predictability
- Once finalised SCs are published on the Agency website
- All SCs are sent to patients for review, if feasible within timelines (usually 24 hrs)
- Once aware of an upcoming safety concern EMA will contact organisation(s) requesting availability to review the communication
- Draft document is forwarded to the expert(s), usually with 12-24 hours deadline, in some urgent cases only 3-4 hours may be available for consultation.

Acronyms

- ADR : Adverse Reaction
- AR : Assessment Report
- CHMP : Committee for Medicinal Products for Human Use
- LoQ : List of Questions
- LoOIs : List of Outstanding Issues
- MAH : Marketing Authorisation Holder
- PRAC : Pharmacovigilance Risk Assessment Committee
- PSUR : Periodic Safety Update Report
- RMP : Risk Management Plan
- SAG : Scientific Advisory Group
- COMP: Committee for Orphan Medicinal Products;
- CHMP: Committee for Human Medicinal Products;
- CAT: Committee for Advanced Therapies;
- PDCO: Paediatric Committee;
- SAWP: Scientific Advice Working Party;
- SAG: Scientific Advisory Group;
- PRAC: Pharmacovigilance and Risk Assessment Committee;
- EPAR: European Public Assessment Report;
- ATMP: Advanced Therapy Medicinal Product
- SmPC : Summary of Product Characteristics

Contact

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