EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Introduction to the European Medicines Agency

EFA training for patient experts on allergy, asthma and COPD on getting involved with the EMA, 18 February 2014

Presented by: Isabelle Moulon
Head of patients and healthcare professionals department

An agency of the European Union

Key Principles

- The EU is a Single Market for pharmaceuticals
  ~ 0.5 billion people
- In order to market a medicinal product in the EU, a company needs a Marketing Authorisation
- There are different ways ('Procedures') for a company to obtain a Marketing Authorisation
- The main scientific principle used in the evaluation of medicines is the **benefit / risk balance**, based on quality, efficacy and safety aspects
- Economic considerations are excluded from the assessment

A European agency and medicines system: Why?

- Protect and promote public and animal health
- Pooling of best scientific expertise from across Europe for evaluation of medicines
- Facilitate availability of new medicines to patients
- Same product information to patients and healthcare professionals
- Single market for pharmaceuticals
- Benefits R&D industry
- Platform for discussion of public health issues

A European agency and medicines system: How?

'One system, two routes for approval'

- **Centralised European route** - attracts nearly all innovative medicines
- **Mutual recognition + decentralised national routes -** mostly generics and some new indications for existing products

European Medicines Agency: focal point of the centralised procedure

- **1 application**
- **1 evaluation**
- **1 authorisation for all EU**
- **1 invented name**
- **1 product information (SPC, Labelling, PL)**
- **All EU languages**

The EMA is not responsible for pricing or reimbursement

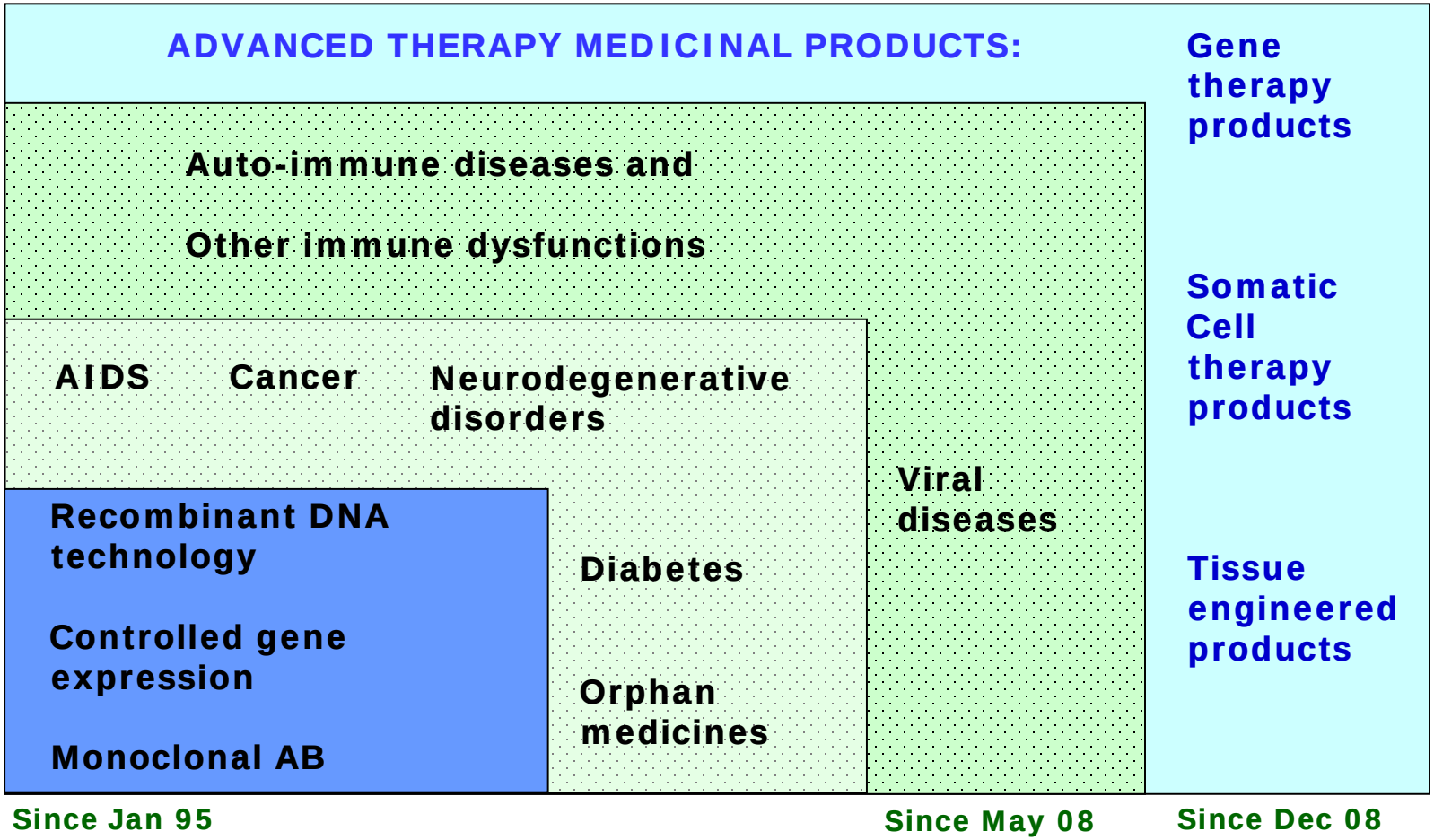
Marketing Authorisation is granted by the European Commission

The various roles of the EMA

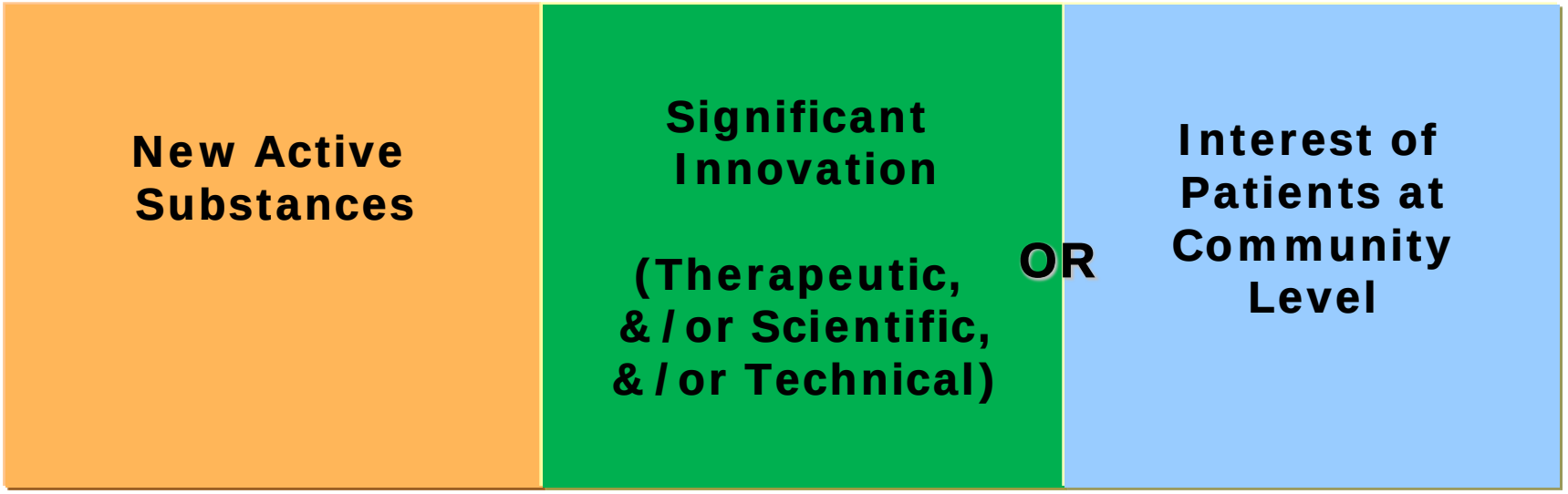
The Agency is responsible for:

- The **evaluation of marketing authorisation** for **human and veterinary** applications submitted by pharmaceutical companies
- The coordination of European **pharmacovigilance** (supervision of the medicines on the market)
- The provision of **scientific advice** on the development of medicines
- The evaluation of applications for **orphan** designation in EU
- The evaluation of **paediatric investigation** plans (or waivers)
- The evaluation of **arbitration** and **referral** procedures
- The provision of good quality and independent **information** on the medicines it evaluates to patients and health
- The coordination of Member States' **inspections** *(GMP, GCP, GLP)*

Eligibility: "Mandatory Scope"



Eligibility “Optional Scope”



Art 3(3) Generic of a product authorised via EMA

The centralised procedure attracts most innovative medicines. Decentralised and MRP mainly do generics and new indications for existing products

A networking Agency

Member States have pooled their sovereignty for authorisation of medicines

- The Agency is designed to coordinate the existing scientific resources of Member States
- It is not intended to replace national authorities, but to be a partner in the system
- It is a networking agency, providing an interface between all partners
- All parties linked by an IT network (EudraNet)

Our partners in Europe

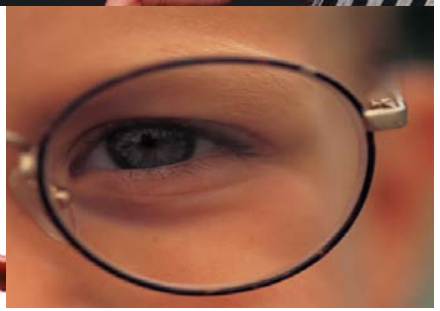
- More than 45 national competent authorities dealing with human and veterinary medicines and access to a network of more than 2,500 European experts
- EU institutions: European Commission, European Parliament, other EU agencies (EMCDDA, EFSA, ECDC, Translations Centre)
- European Pharmacopoeia (Council of Europe)
- Medicines Control Laboratories Network

A dynamic and constantly changing Agency

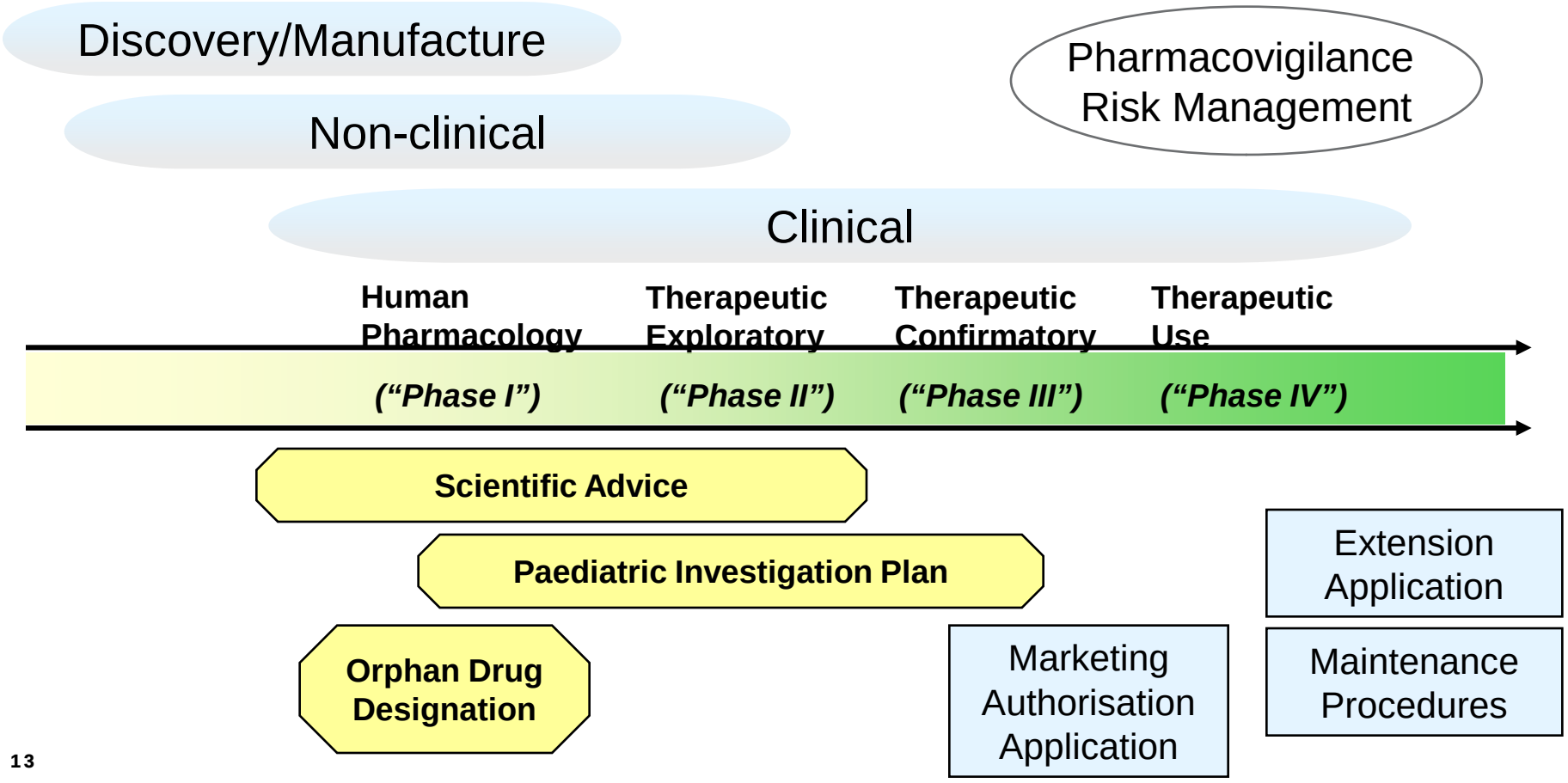
Taking on new tasks and responsibilities

- 2001: Orphan medicines (+ new committee)
- 2005 & 2008: Extended mandatory scope
- 2005: 'Biosimilar' and generic medicines
- 2005: Herbal medicines (+ new committee)
- 2007: Paediatric medicines (+ new committee)
- 2008/2009: Advanced therapies (+ new committee)
- 2012: Pharmacovigilance (+ new committee)
- 2013: Falsified medicines legislation

Development of Medicines



Drug Development Overview



EMA Scientific Committees and working parties

CHMP

(**Committee for Human Medicinal Products**)

Members: 1 per Member State + 1 alternate + 5 co-opted Members

Non voting members: ICE/NO; Chair : T. Salmonson – Vice Chair: P. Demolis

PRAC

(**Pharmacovigilance Risk Assessment Committee**)

Members: 1 per Member State + 3 additional Members + 1 Patient Organisation + 1 Healthcare Professionals Organisation

Non voting Members: ICE/NO; Chair : J. Munro Raine– Vice Chair: Almath Spooner

COMP

(**Committee for Orphan Medicinal Products**)

Members: 1 per Member State +3 additional Members + 3 Patient Organisations

Non voting Members: ICE/NO; Chair : B. Sepodes – Vice Chair: L. Greene

HMPC

(**Committee for Herbal Medicinal Products**)

Members: 1 per Member State + 1 alternate + max. 5 Co-Opted Members

Non-voting members: ICE/NO/possible intl. org.; Chair: W. Knöss - Vice-Chair: M. Delbò

PDCO

(**Paediatric Committee**)

Members: 5 CHMP, 1 per other Member States

3 HCP, 3 Patient Organisations+1 Alternate per member Non voting members: ICE/NO; Chair: D. Mentzer - Vice-Chair: K. Norga

CAT

(**Committee for Advanced Therapies**)

Members: 5 CHMP, 1 per other Member States

2 clinicians appointed by EC + 2 alternates, 2 Patient Organisations appointed by EC

Non voting members: ICE/NO; Chair: C. Schneider - Vice-Chair: P. Salmikangas

EMA-EU Network

28 EEA Member States
+ 4,500 European experts

EU institutions:
Commission - Parliament

Committee for Human Medicinal Products (CHMP)

Management Board

Committee for Veterinary Medicinal Products (CVMP)

Paediatric Committee (PDCO)

EMA

Secretariat

Committee for Orphan Medicinal Products (COMP)

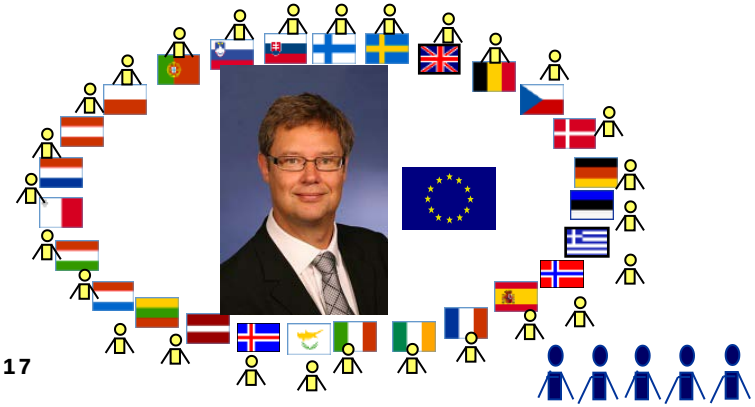
Committee for Herbal Medicinal Products (HMPC)

Pharmacovigilance Risk Assessment Committee (PRAC)

Committee for Advanced Therapies (CAT)

Scientific Committee
CHMP

- Formulate scientific opinions to the EC
  - . on medicinal products for human use (CAP)
  - . on arbitration/referral procedures
- Scientific Advice
- EU scientific and regulatory guidelines



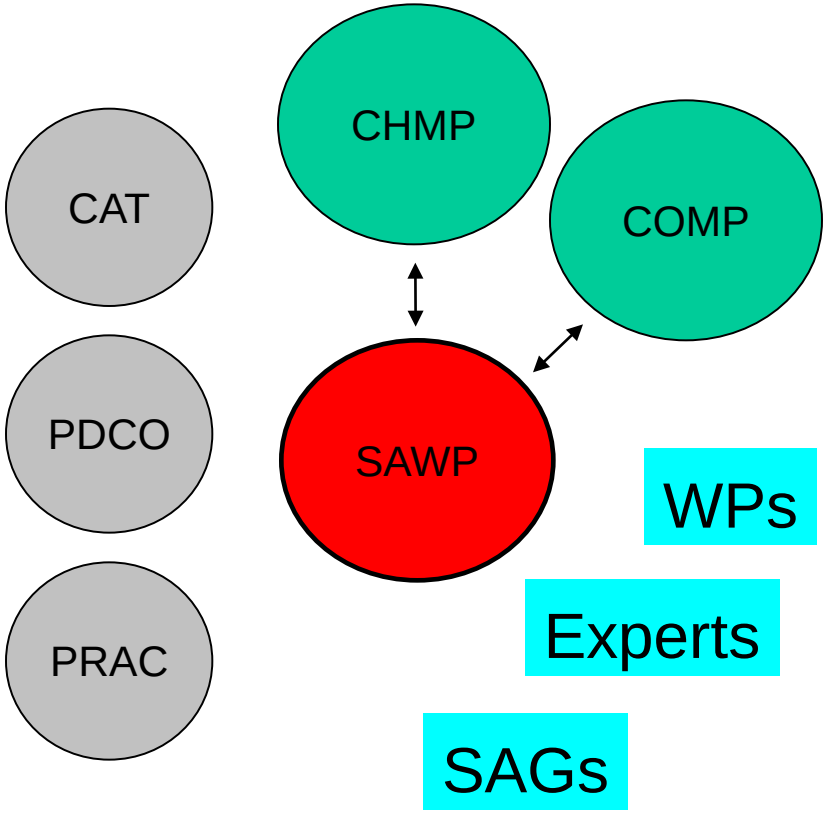
- Chair (Dr. Tomas Salmonson)
- 1 member (+ 1 alternate) per MS
- *1 member (+ 1 alternate) NO - IS*
- 5 co-opted members

Committee Plenary Meeting

Scientific Advice

Scientific Advice

- Advice to development
- Agreement on future strategy (when no guideline)
- Not a pre-evaluation
- Fee-related activity (fee reduction for orphan products)
- Working Party of the CHMP



Paediatric Development

Paediatric Initiative - Objectives

Improve the health of children

- Increase high quality, ethical research into medicines for children
- Increase availability of authorised medicines for children
- Increase information on medicines

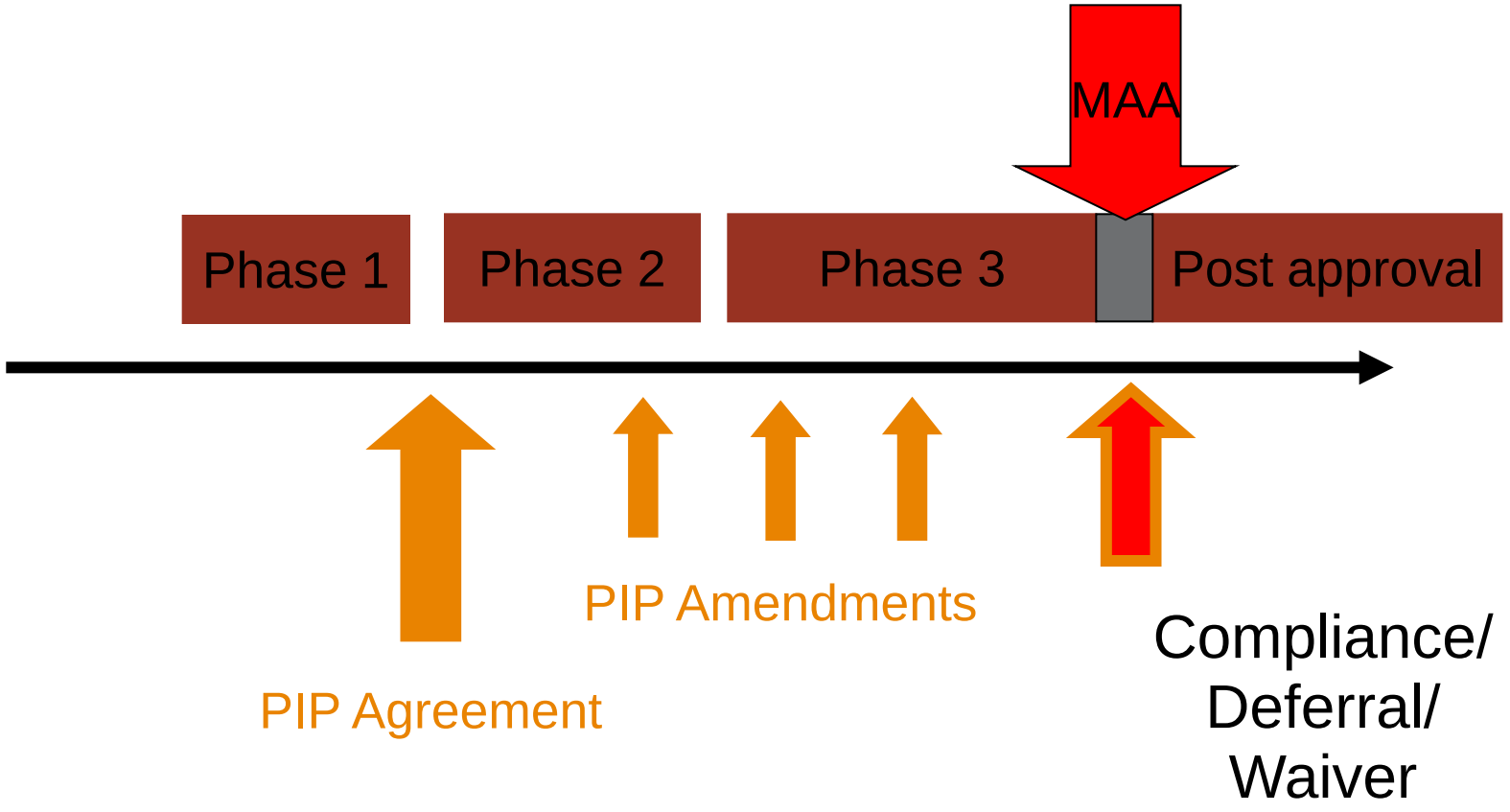
Achieve the above

- Without unnecessary studies in children
- Without delaying authorisation for adults

Paediatric Initiative

- Obligation to study products in children
- Paediatric Investigation Plan (PIP) or Waivers for products for new marketing authorisation, extension of indications, new pharmaceutical forms
- Rewards in the form of extension of supplementary patent certificate
- PIP/Waivers requests to be submitted by end of human PK studies
- Incentives for old products of paediatric interest
- Coordination of a Network of EU paediatric networks
- Paediatric Committee (PDCO)

Integration into Development Plan



Orphan Drugs

Orphan Medicinal Products

Criteria:

- Life threatening or debilitating condition
- Epidemiological – Prevalence <5/10.000
- No satisfactory methods exist or significant benefit over authorised products/methods

  Or
- Economical: unlikely to generate sufficient return on investment

- Committee for Orphan Medicinal Products (COMP) 2000

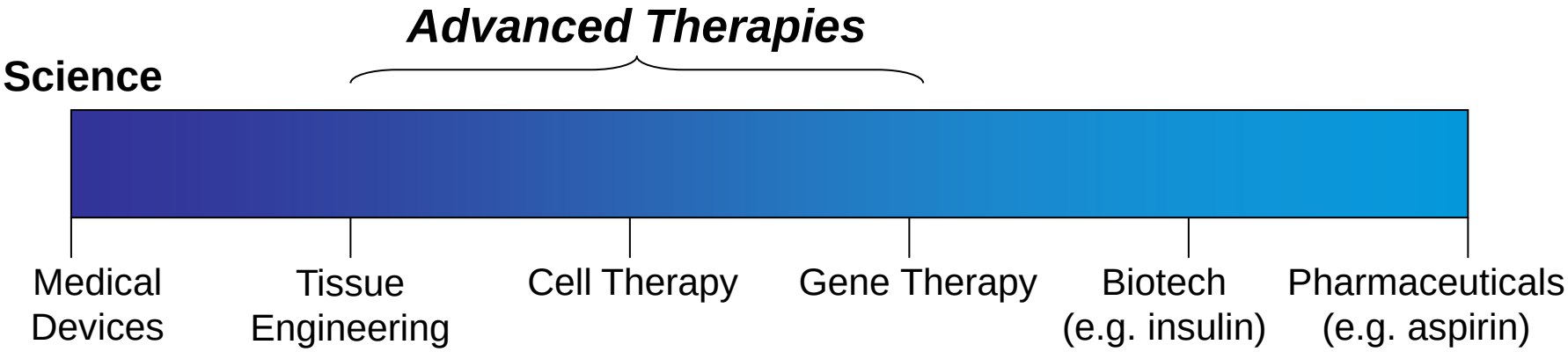
IMPORTANT:

- Designation for Orphan status (not Marketing Authorisation)
- Confirmation of Orphan status before Marketing Authorisation

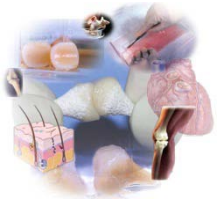
Advanced Medicines

Advanced Therapies Medicinal Products (ATMPs)

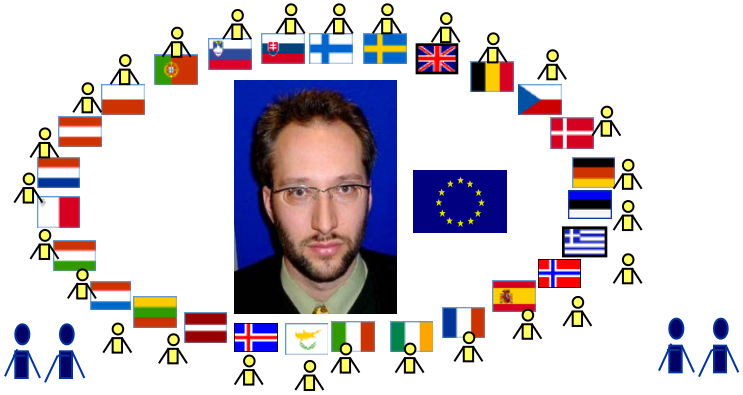
- Gene therapy products
- Somatic Cell therapy products
- Tissue engineered products



Scientific Committee
CAT



- Formulate draft opinion on Advanced Therapy
- Medicinal Products for final approval by CHMP
- ATMP classification (gene, cell, tissue)
- ATMP quality and clinical data certification



- **Chair (Dr. C. Schneider)**
- **5 CHMP members (with alternates)**
- **1 member (+ 1 alternate) per MS (not represented by CHMP member)**
- ***1 member (+ 1 alternate) NO - IS***
- **2 members (+ 2 alternates) patients organisations**
- **2 members (+ 2 alternates) healthcare professionals**

Monitoring and supervision of medicines

Pharmacovigilance and Risk Management

Pharmacovigilance

⇨ the science and activities relating to the detection, understanding and prevention of adverse drug reactions or any other drug-related problems

Risk Management System

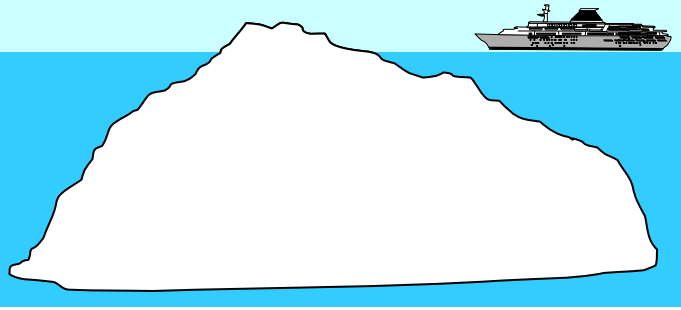
⇨ a set of pharmacovigilance activities and interventions designed to identify, characterise, prevent or minimise risks relating to medicinal products, including the assessment of the effectiveness of those interventions

Pharmacovigilance and Risk Management

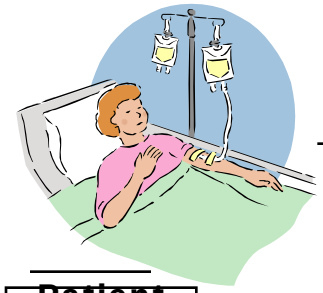
What we know at the end of the clinical trial programme…

What we don't know!

- **What happens when the medicinal product is used in normal practice?**
- **What is its adverse event profile?**



Pharmacovigilance and Risk Management Data Collection and Management



Patient with ADR

Reporting physician

Original Report

MAH

O R

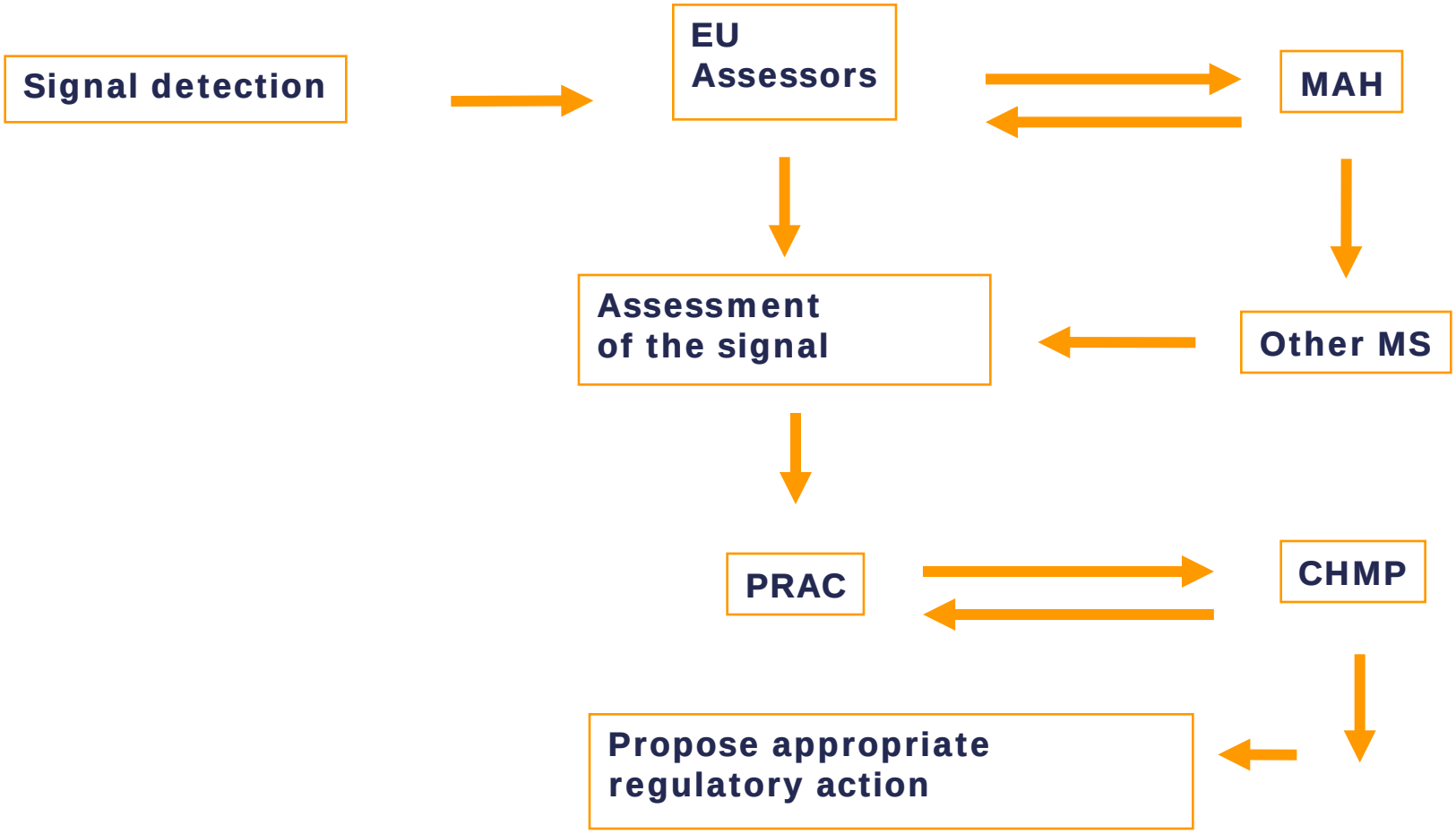
NCA

ICSR

EudraVigilance Human

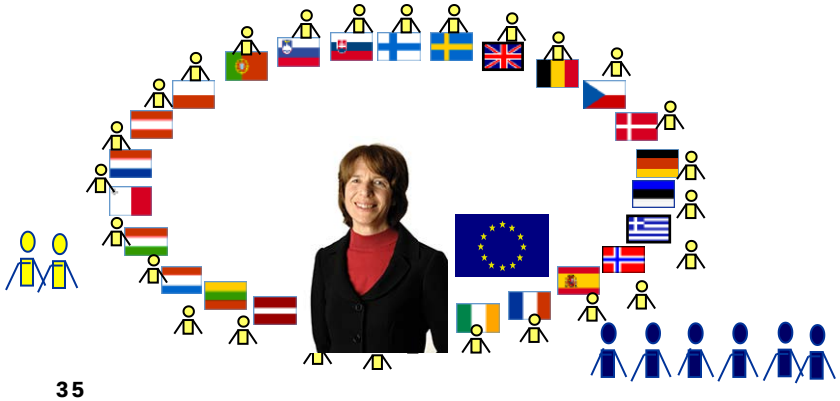
Patient Safety Monitoring Schemes?

Pharmacovigilance and Risk Management; Signal Detection and Data Analysis



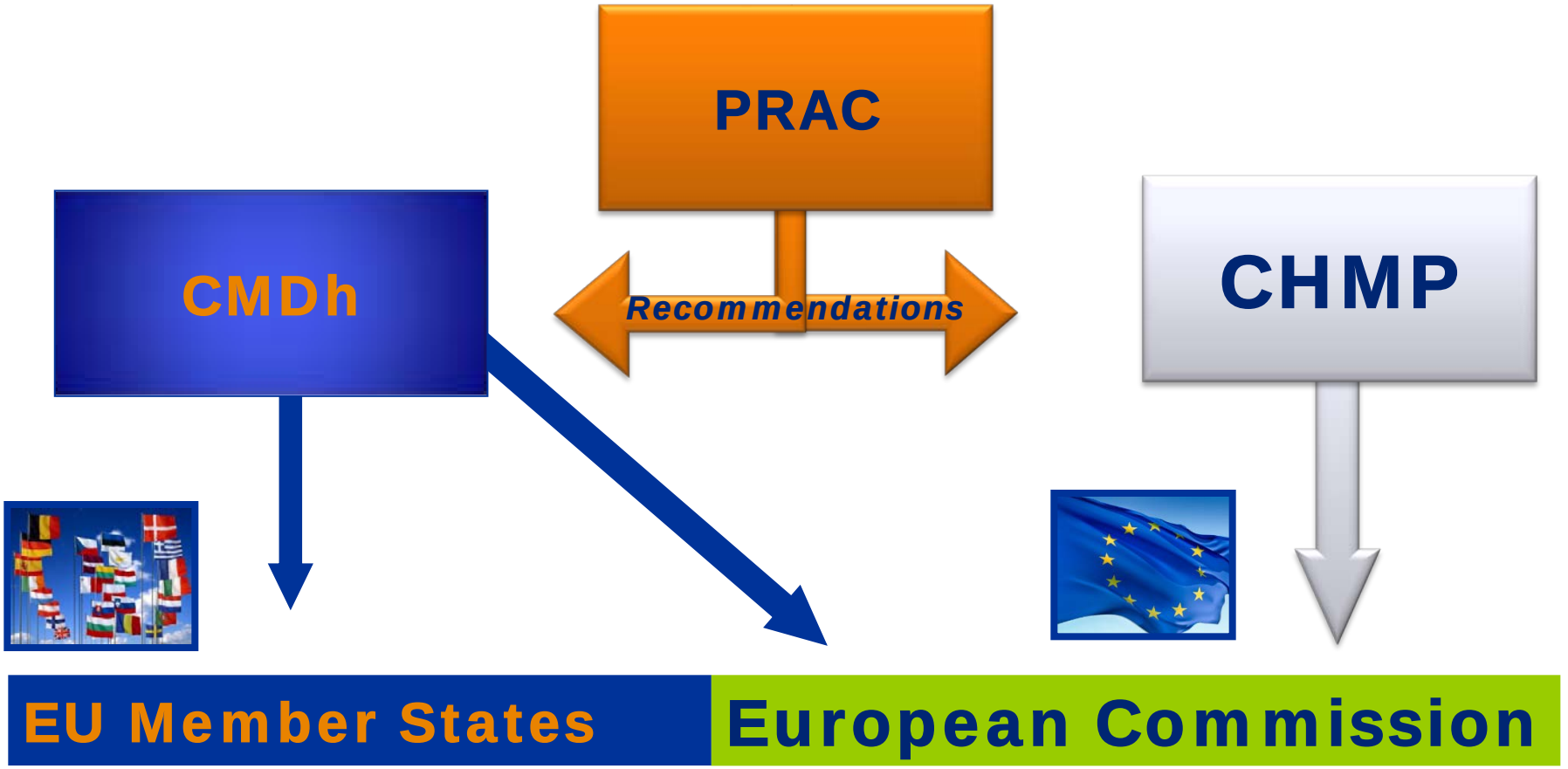
Scientific Committee
PRAC

- Assess aspects of risk management (detection, assessment, minimisation and communication of risk of adverse reactions)
- PASS and pharmacovigilance audit
- Recommendations on questions on PhV activities



- **Chair (Dr. June Raine)**
- **1 member (+ 1 alternate) per MS**
- ***1 member (+ 1 alternate) NO - IS***
- **6 experts nominated by EC**
- **1 member (+ 1 alternate) healthcare professionals**
- **1 member (+ 1 alternate) patients organisations**

How does the PRAC Work?



Transparency and communication

What information does EMA provide?

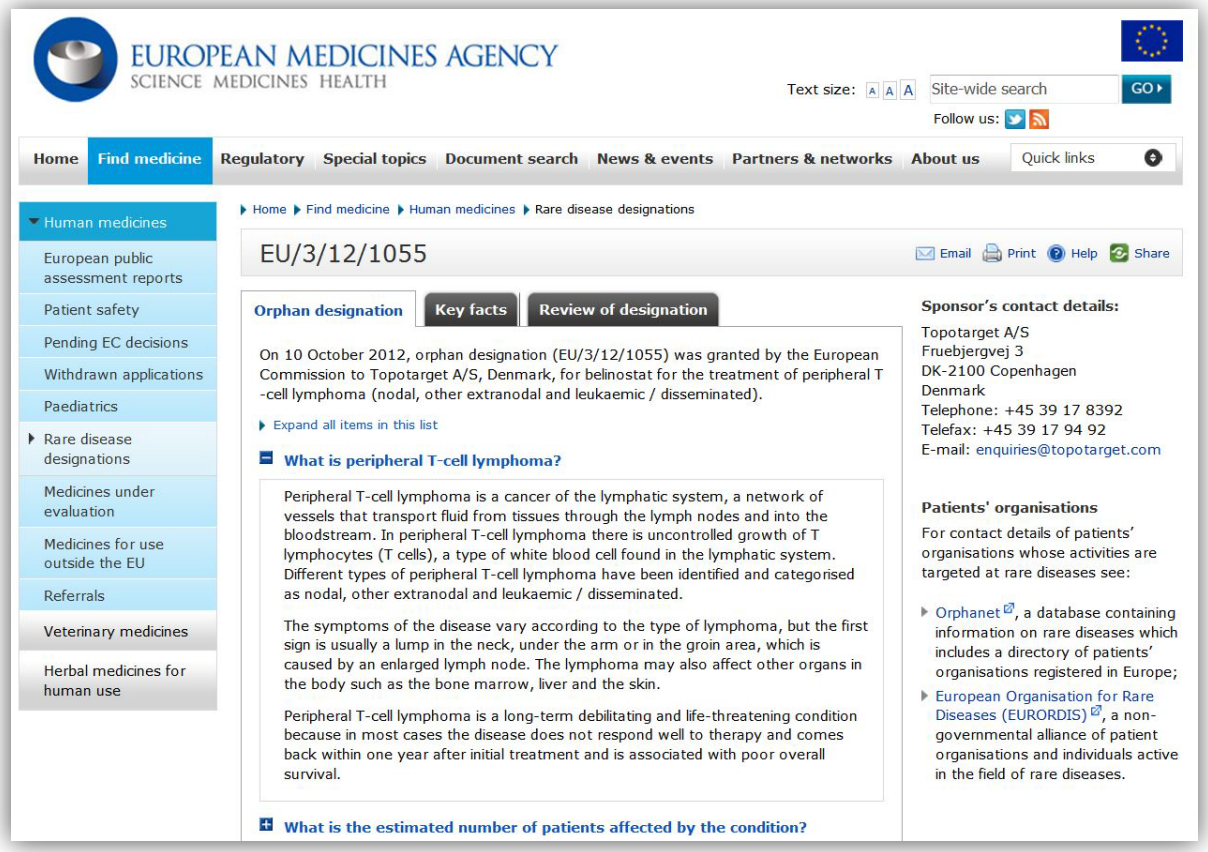
- EMA website – main channel of communication
- Different information at different stages of life-cycle of medicines
- Information on medicines authorised via EMA
- Safety communication for all medicines authorised in the EU
- Agendas and minutes of all scientific committees

EMA website: www.ema.europa.eu

Pre-authorisation (1): orphans and paediatrics

- Information on orphan designation
- Information on review of orphan designation at the time of marketing authorisation.
- Opinions and decisions on paediatric investigation plans.
- Information available in English.

Public summary of opinion on orphan designation on EMA website

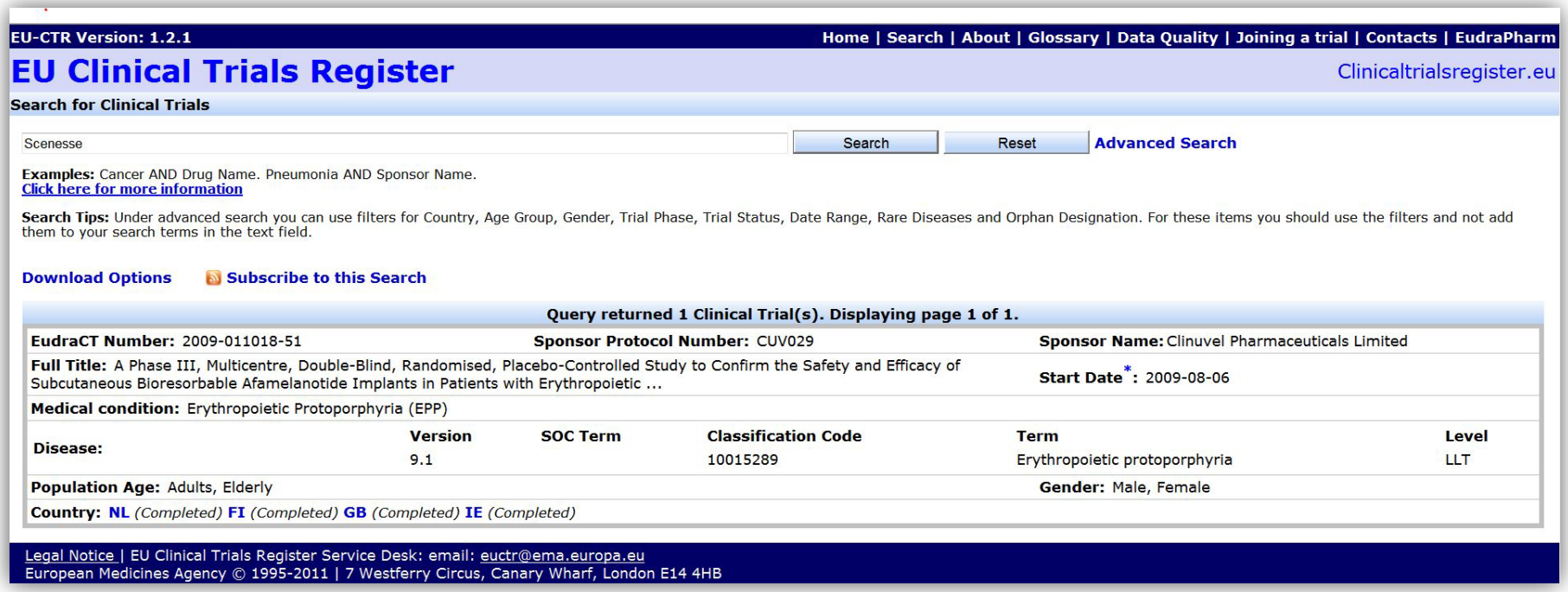


Pre-authorisation (2): clinical trials (CT)

- Information on CT – the EU Clinical Trials Register website: https://www.clinicaltrialsregister.eu/
- The Register allows to search for information on CT in the EU Member States.
- Information on:
  - trial design;
  - sponsor;
  - investigated product and therapeutic area;
  - the status of the trial.

EU Clinical Trials Register:

https://www.clinicaltrialsregister.eu/



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EU Clinical Trials Register Clinicaltrialsregister.eu

Search for Clinical Trials

Scenesse [Search] [Reset] **Advanced Search**

Examples: Cancer AND Drug Name. Pneumonia AND Sponsor Name.
Click here for more information

Search Tips: Under advanced search you can use filters for Country, Age Group, Gender, Trial Phase, Trial Status, Date Range, Rare Diseases and Orphan Designation. For these items you should use the filters and not add them to your search terms in the text field.

Download Options **Subscribe to this Search**

Query returned 1 Clinical Trial(s). Displaying page 1 of 1.

EudraCT Number: 2009-011018-51 **Sponsor Protocol Number:** CUV029 **Sponsor Name:** Clinuvel Pharmaceuticals Limited

Full Title: A Phase III, Multicentre, Double-Blind, Randomised, Placebo-Controlled Study to Confirm the Safety and Efficacy of Subcutaneous Bioresorbable Afamelanotide Implants in Patients with Erythropoietic ... **Start Date*:** 2009-08-06

Medical condition: Erythropoietic Protoporphyria (EPP)

Disease:	Version	SOC Term	Classification Code	Term	Level
	9.1		10015289	Erythropoietic protoporphyria	LLT

Population Age: Adults, Elderly **Gender:** Male, Female

Country: **NL** *(Completed)* **FI** *(Completed)* **GB** *(Completed)* **IE** *(Completed)*

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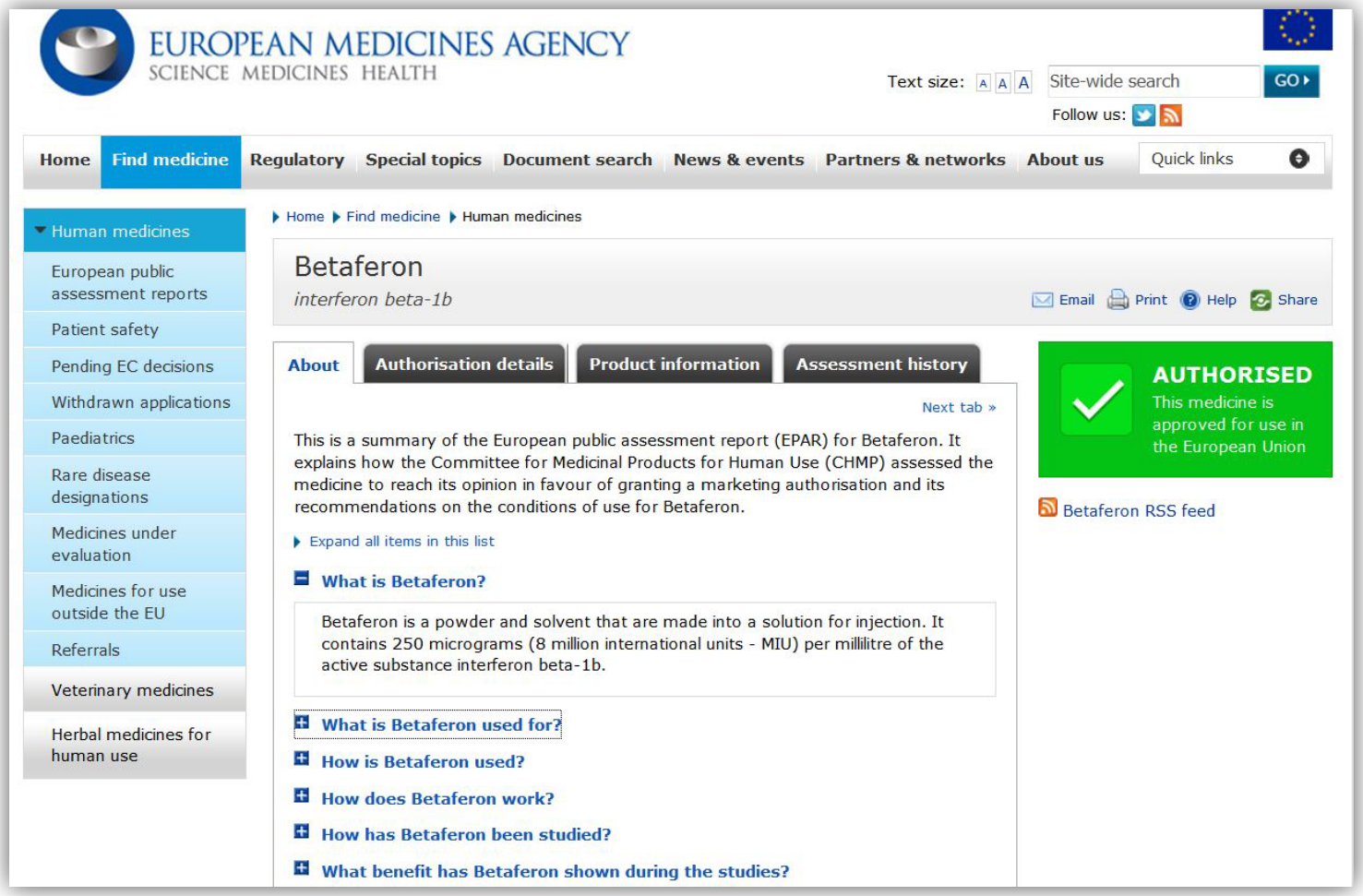
Authorisation/ Licensing

- EPAR summary – in all EU languages
- Assessment report (scientific discussion)
- Product Information – in all EU languages:
  - Summary of Product Characteristics;
  - Package leaflet;
  - Labelling.
- Risk Management Plan (soon!)

EPAR summary on the EMA website



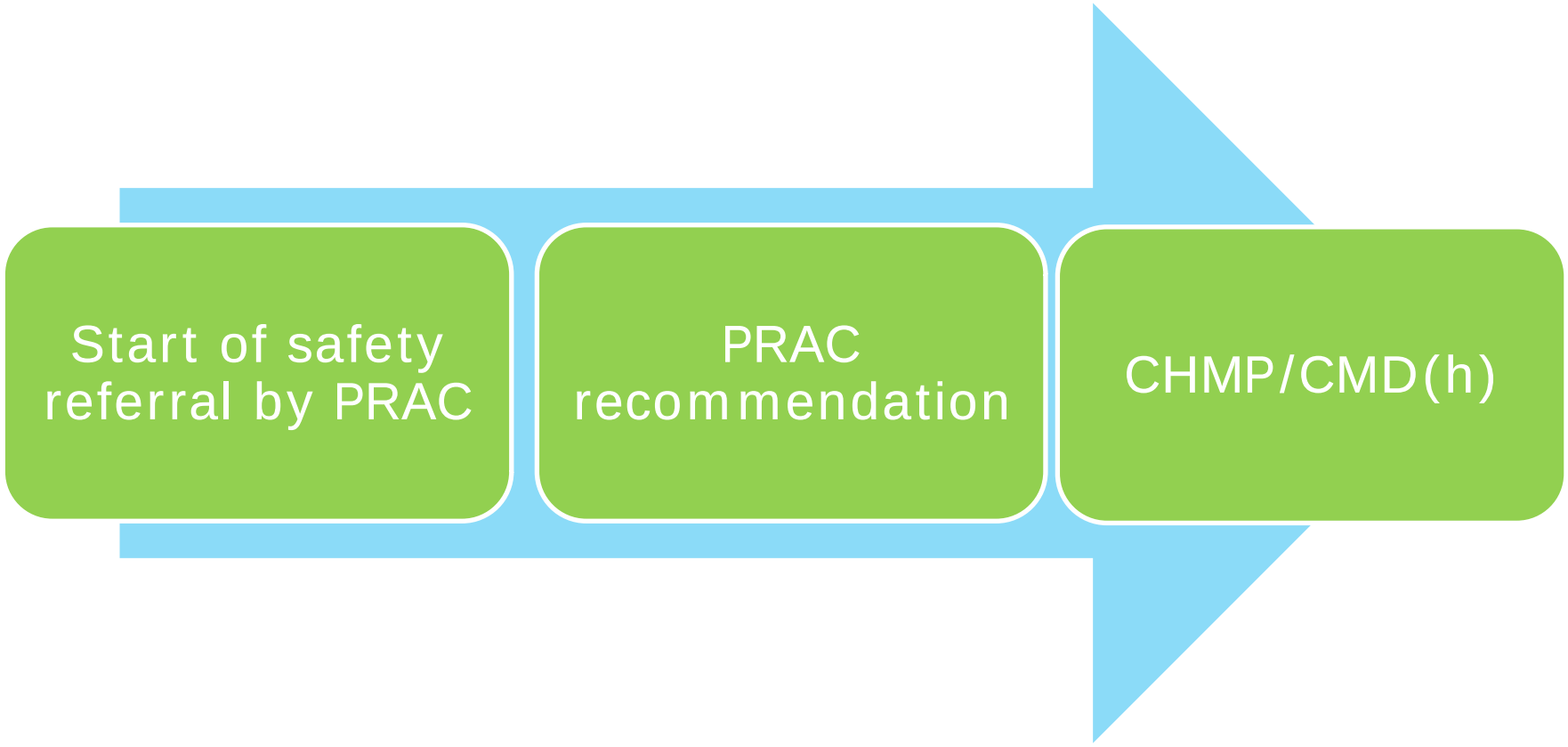
Post-authorisation

- New therapeutic indications;
- New contraindications;
- Other variations.

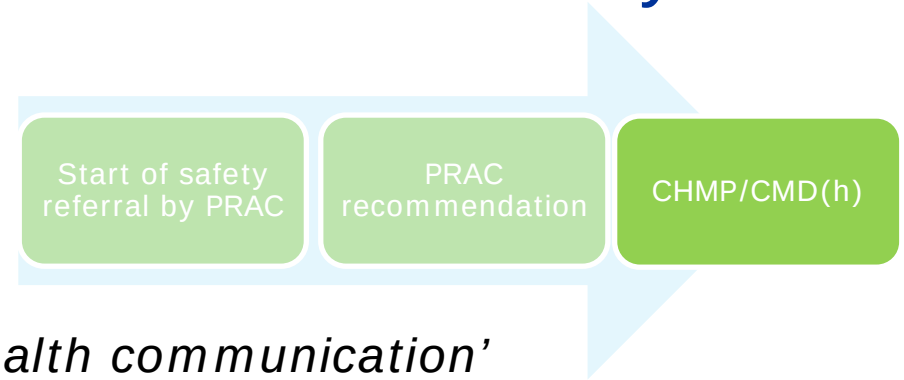
- Update of the EPAR summary;
- Update of Product information;
- Publication of relevant assessment report.

Communication about safety referrals

Procedure



Communication about safety referrals



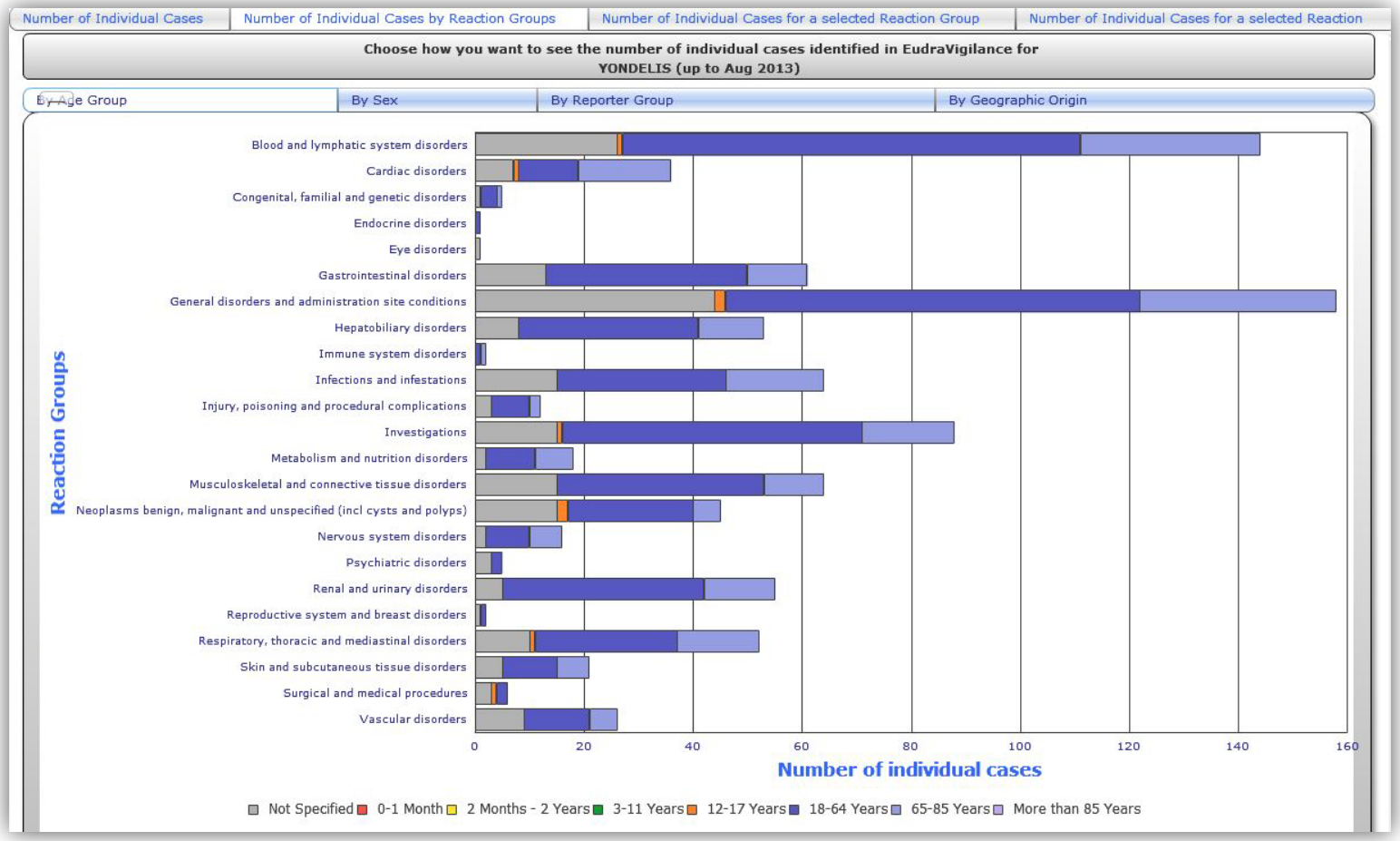
- *'EMA public health communication'*
- Single piece of information (integrates PR+Q&A into one document), composed of three sections:
  - Summary of the issue (for press and general public)
  - Information to patients
  - Information to healthcare professionals
- Explain any divergence with PRAC recommendation if applicable
- Syndicated to press, patients and healthcare professionals contacts

Example: Tredaptive

Information on adverse drug reactions: http://www.adrreports.eu/

- This EU database displays information on ‘suspected adverse drug reactions’ for medicines authorised in the EU.
- A phased development: so far, only for medicines approved via centralised procedure.
- The reports are constantly updated.

European database of suspected adverse drug reaction reports: http://www.adrreports.eu/



Human medicines highlights Newsletter

54 Issue 54 July 2013

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

HUMAN MEDICINES
HIGHLIGHTS

Key information for patients, consumers and healthcare professionals
Published monthly by the European Medicines Agency

An agency of the European Union

IN THIS ISSUE

This newsletter is addressed primarily to organisations representing patients, consumers and healthcare professionals. It provides a summary of key information relating to medicines for human use published during the previous month by the European Medicines Agency.

Information is selected based on recommendations from consulted patients, consumers and healthcare professionals, and does not necessarily cover all relevant information published by the Agency.

To receive an e-mail alert when each new issue of the newsletter is published, send a request to: HMHnewsletter@ema.europa.eu

Information on medicines

Antivirals/anti-infectives

Negative CHMP opinions on new medicines

- Delamanid (*delamanid*)
  Intended for the treatment of multidrug-resistant tuberculosis

Conclusions

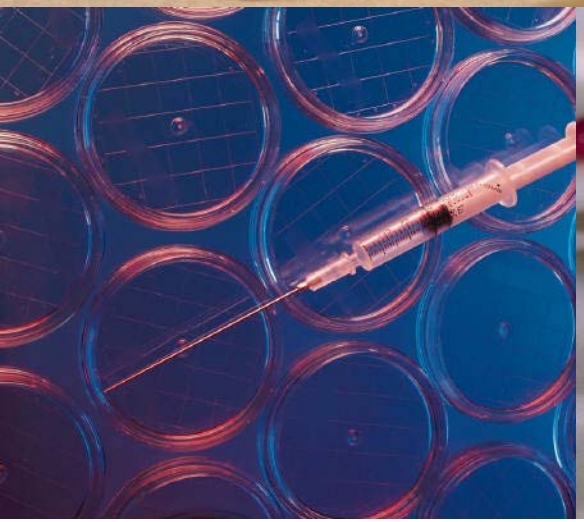
Information on medicines is a key factor of the safe and rational use of medicines. EMA provides understandable, up-to-date, evidence-based information on medicines.

Patients play a key role in the evaluation of medicines and the provision of information by the EMA.

Acronyms

- ADR = Adverse Reaction
- AR = Assessment Report
- CHMP = Committee for Medicinal Products for Human Use
- CD = Commission Decision
- D1, etc = Day 1 (procedural timeline)
- 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**
- **SmPC = Summary of Product Characteristics**

Any Questions?



Thank you for your attention