



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





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

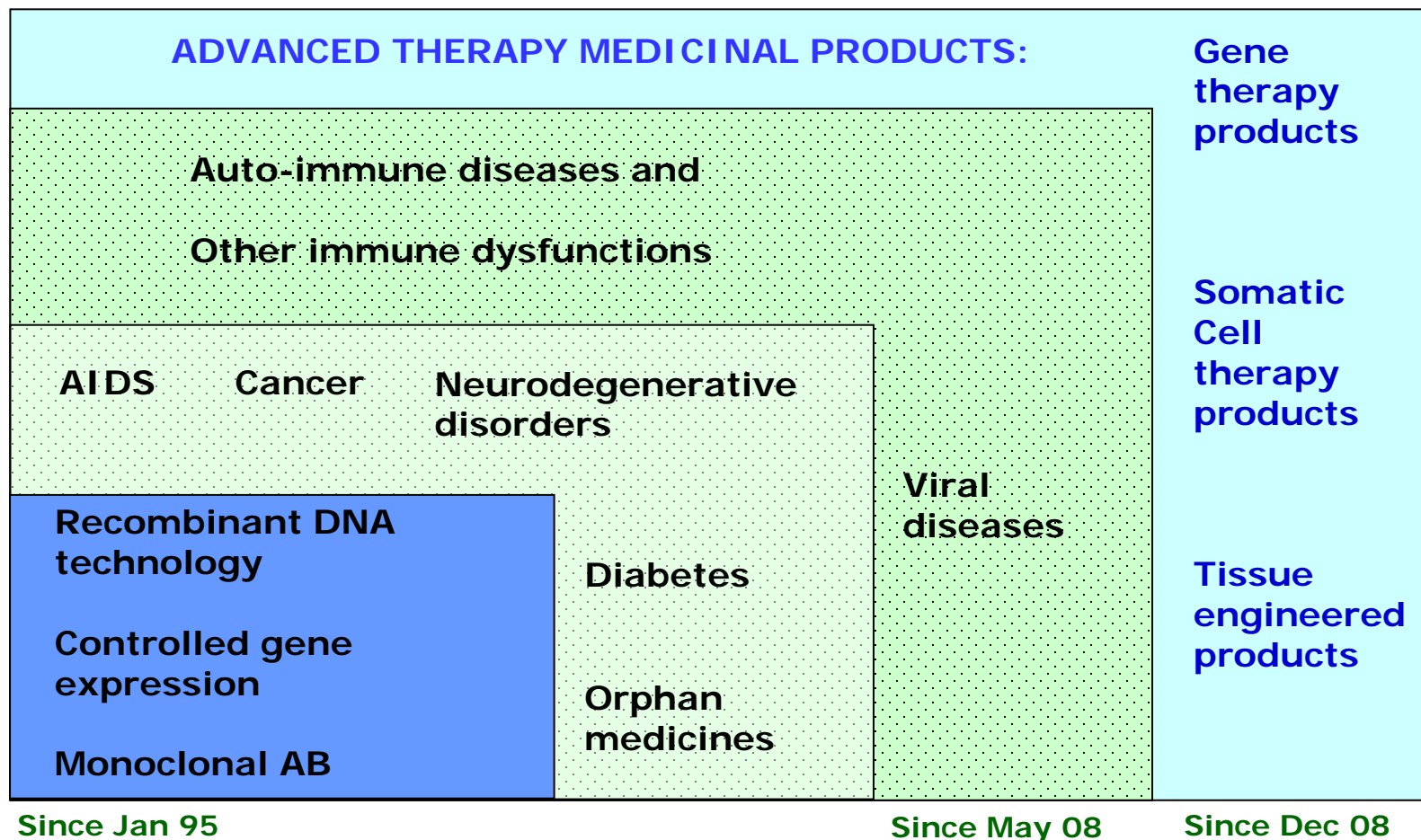


The Agency is responsible for:

- 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

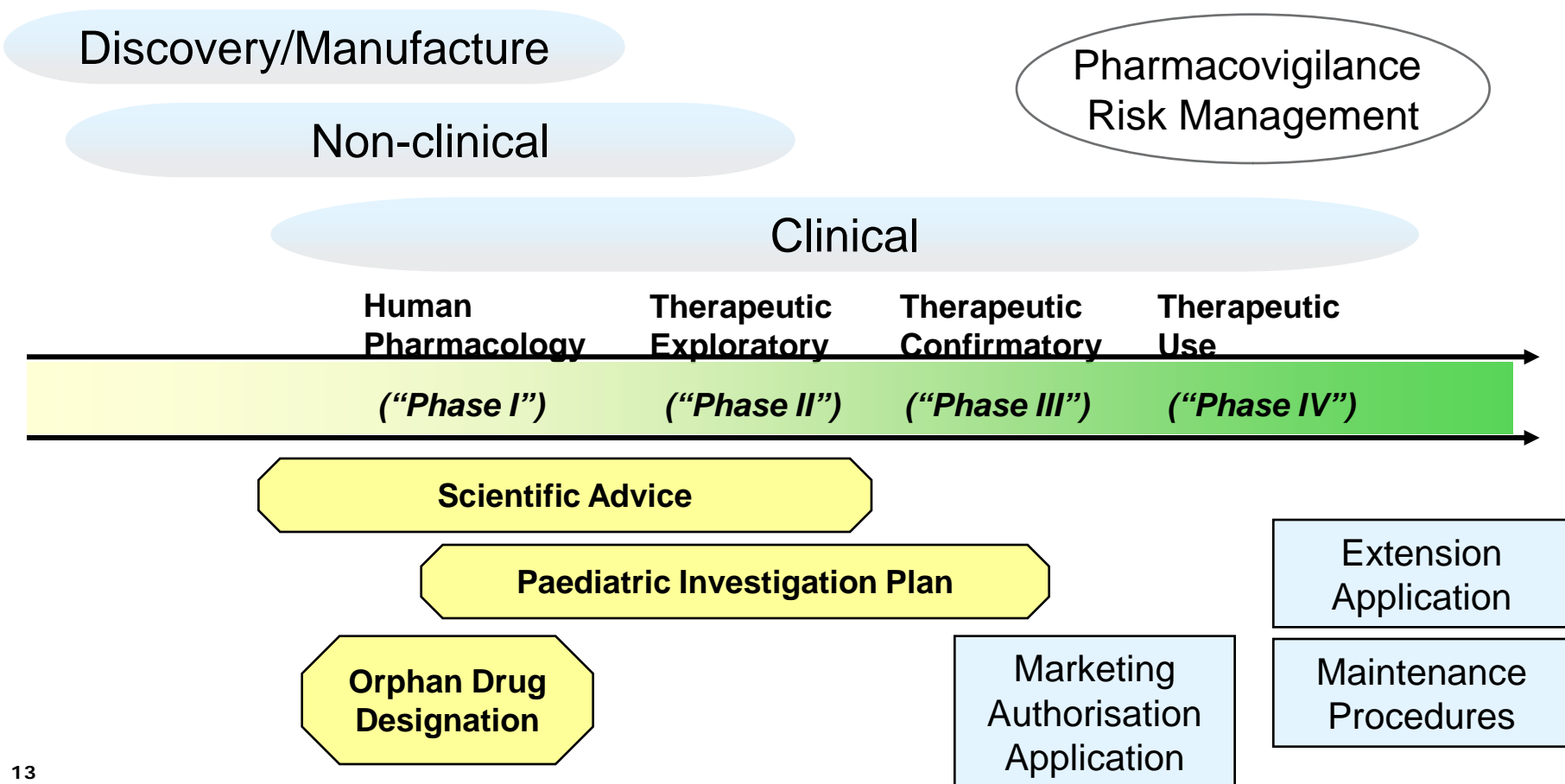


Development of Medicines





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. Norgaard

**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)**

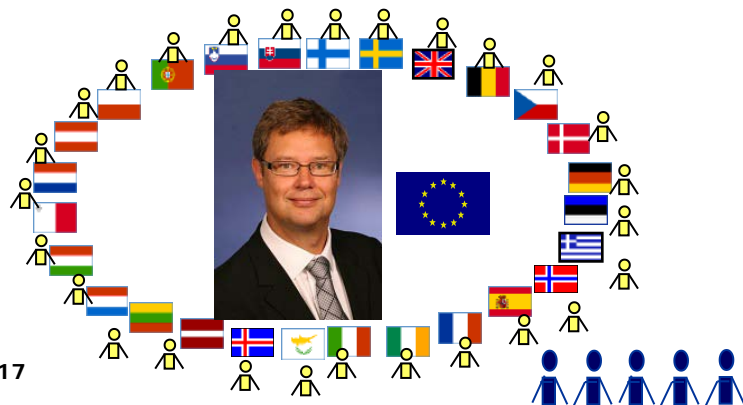


Scientific Committee CHMP



- 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

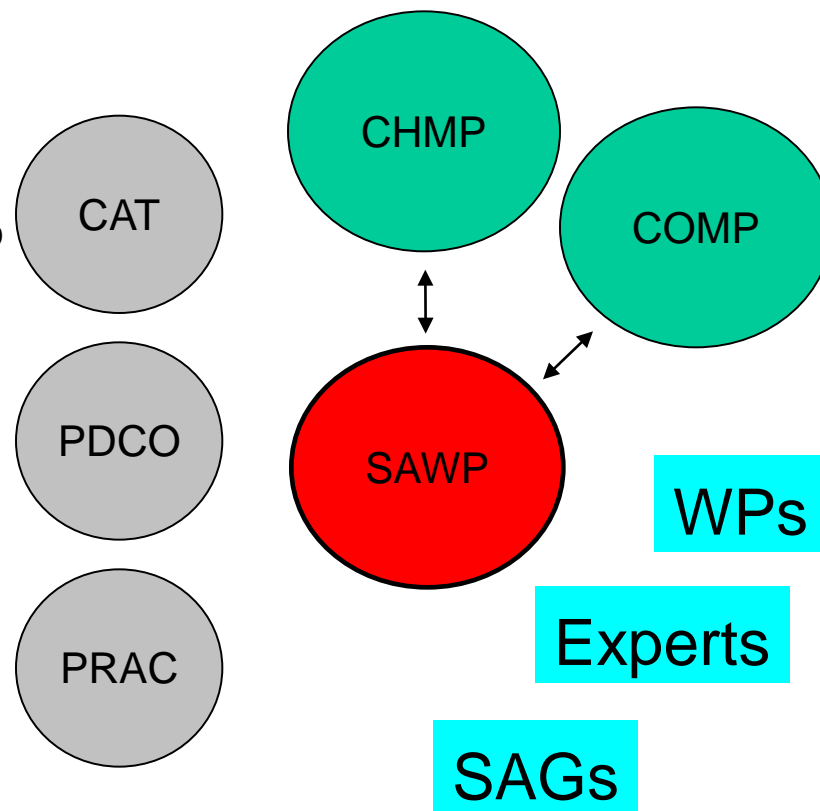




Scientific Advice

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



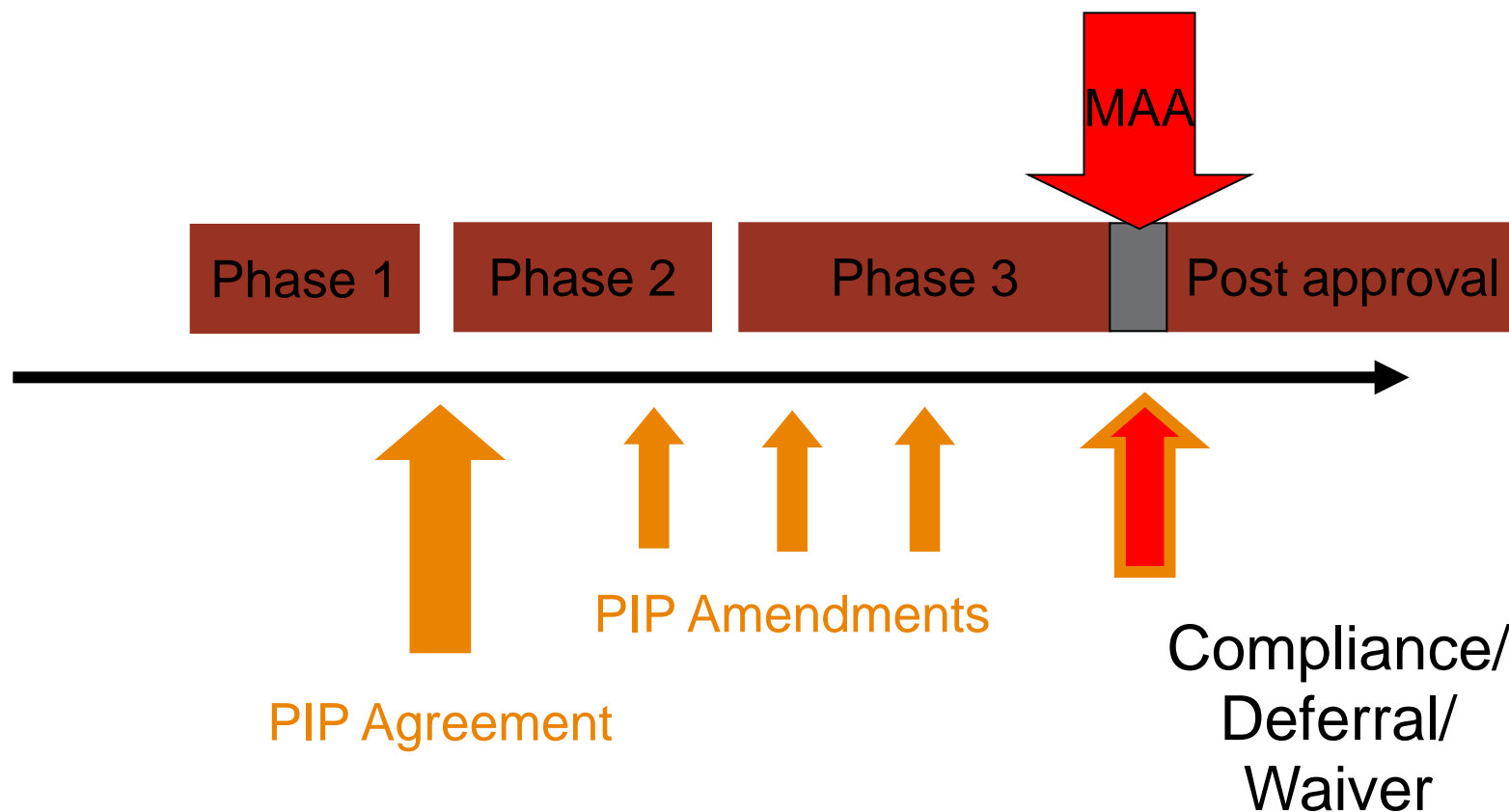


Paediatric Initiative

- 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

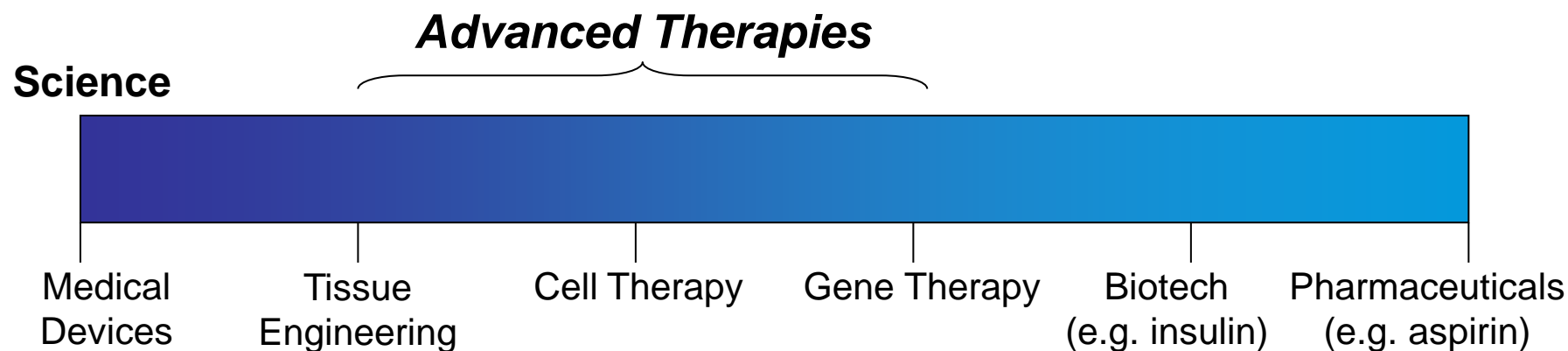


Advanced Medicines



Advanced Therapies Medicinal Products (ATMPs)

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

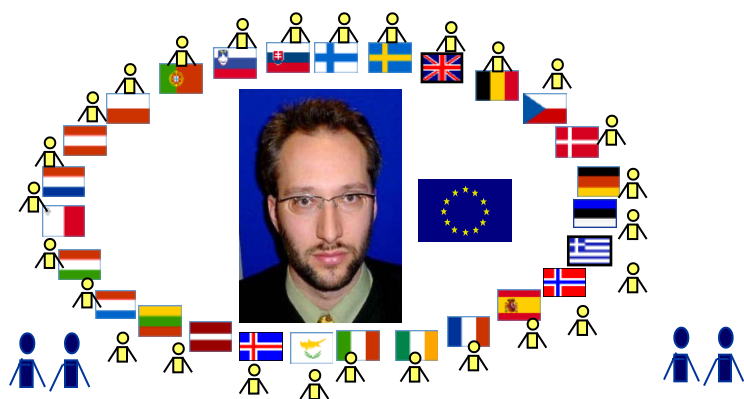




Scientific Committee CAT



- 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

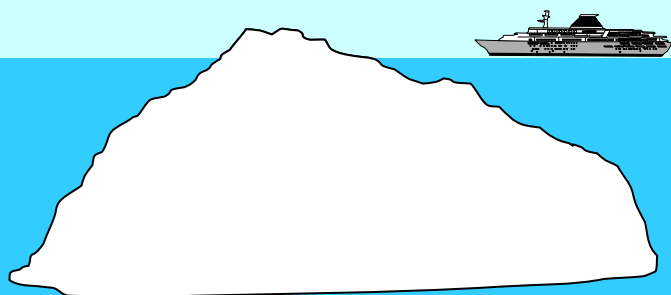


Pharmacovigilance and Risk Management

**What we know at the end
of the clinical trial
programme...**

What we don't know!

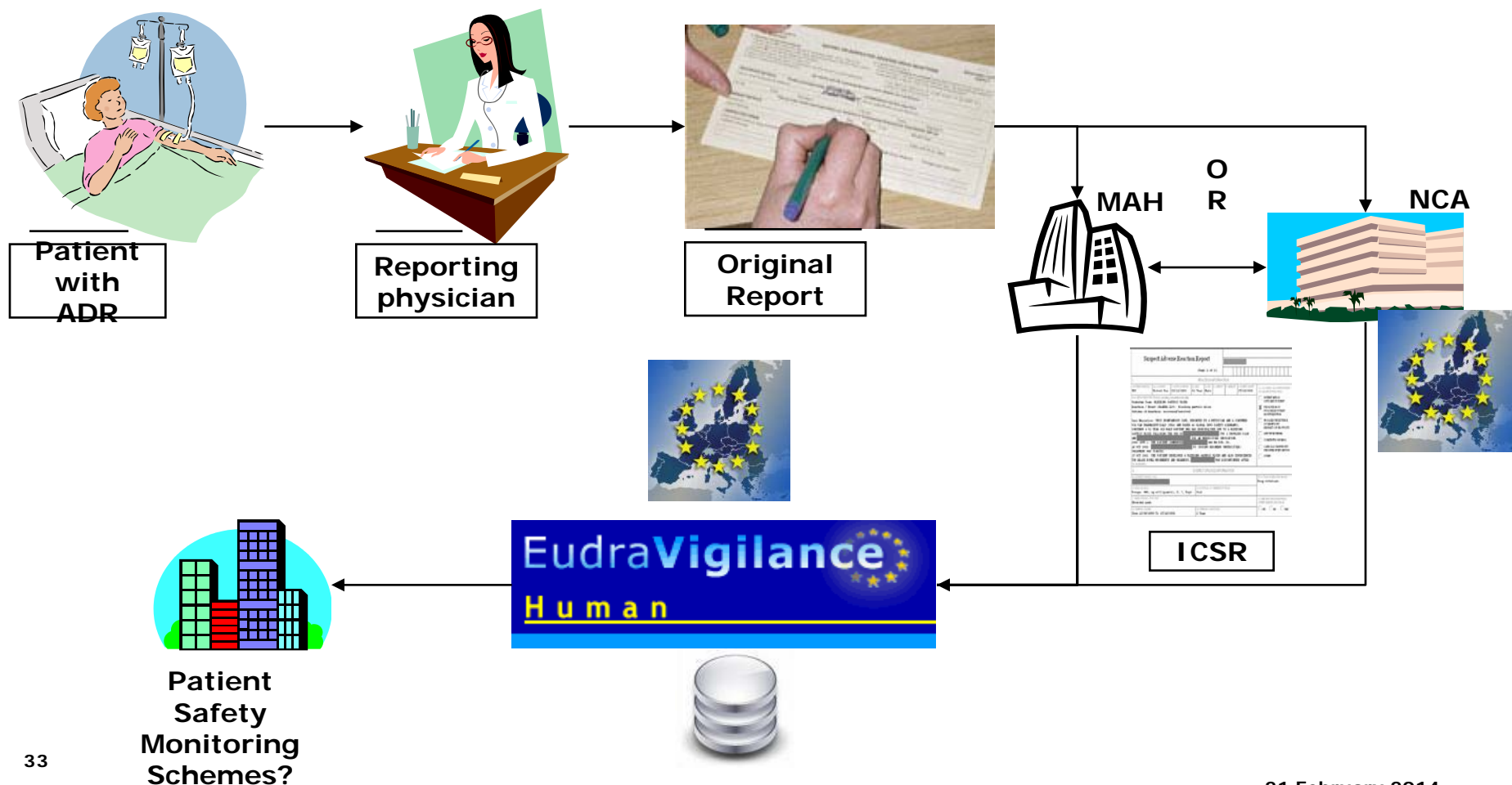
- . What happens when the medicinal product is used in normal practice?**
- . What is its adverse event profile?**





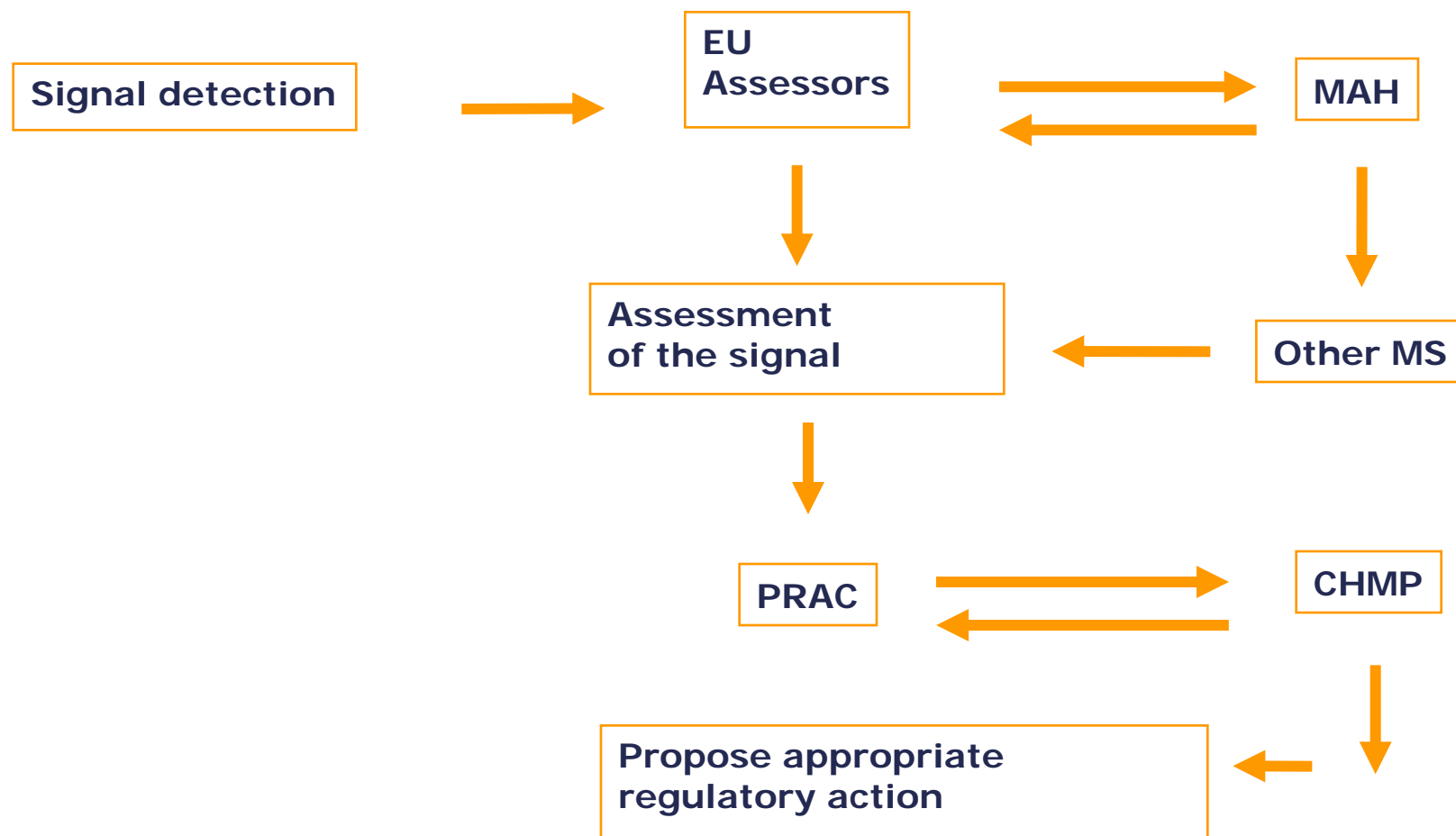
Pharmacovigilance and Risk Management

Data Collection and Management





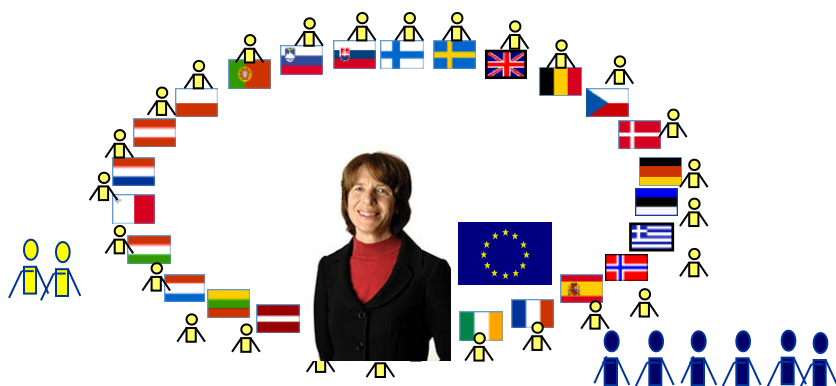
Pharmacovigilance and Risk Management; Signal Detection and Data Analysis





Scientific Committee PRAC

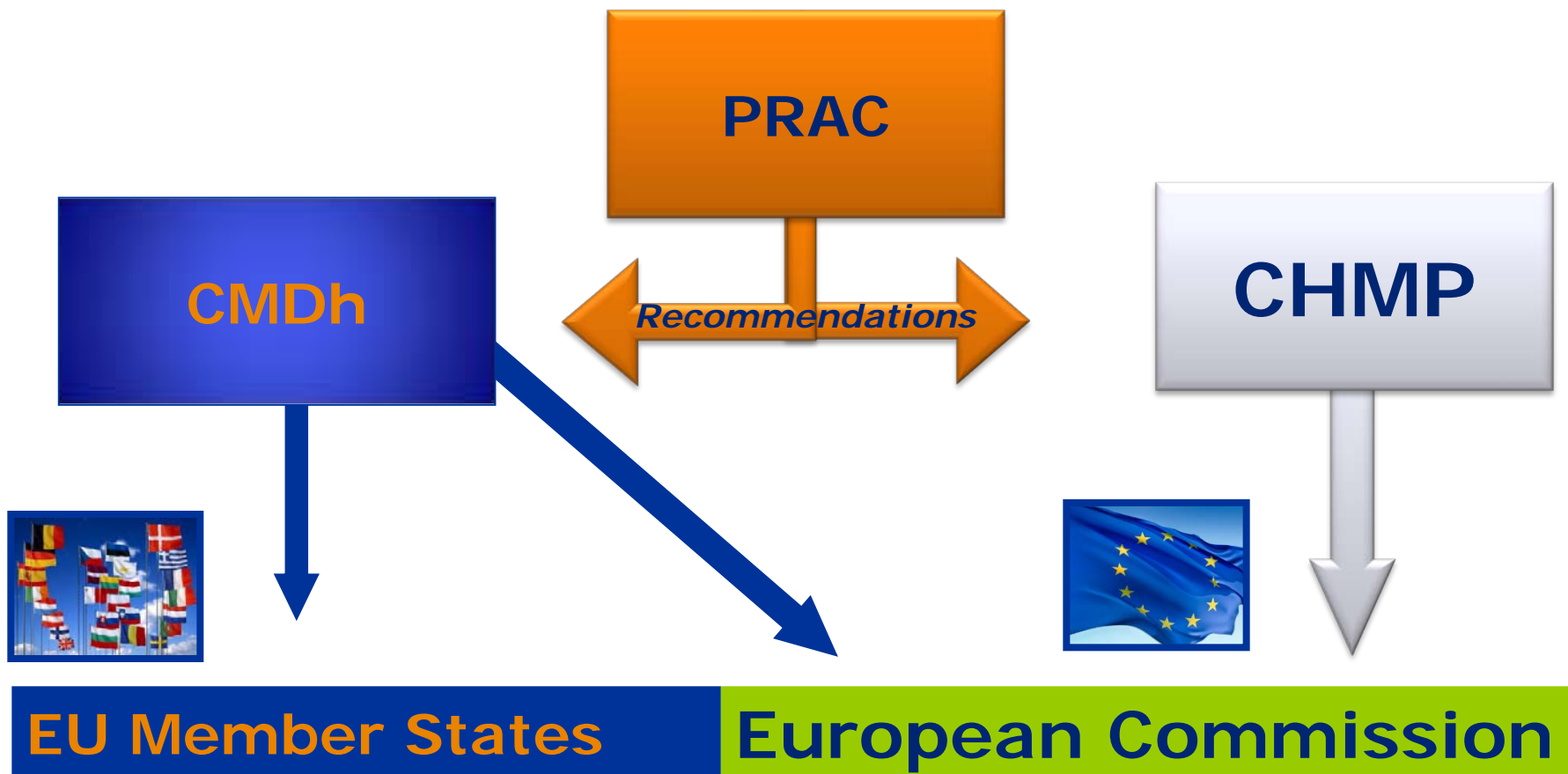
- Assess aspects of risk management (detection, assessment, minimisation and communication of risk of adverse reactions)
- PASS and pharmacovigilance audit
- Recommendations on questions on PhV activities



- **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

The screenshot shows the EMA website homepage. At the top left is the EMA logo with the text "EUROPEAN MEDICINES AGENCY" and "SCIENCE MEDICINES HEALTH". To the right is the European Union flag. Below the logo is a navigation bar with links: Home, Find medicine, Regulatory, Special topics, Document search, News & events, Partners & networks, About us, and a Quick links dropdown. A search bar is also present. The main content area is divided into three columns. The left column has a "Search for medicines" section with a search box and a link to the medicines section. The middle column features a "EU Health Prize for Journalists" announcement. The right column lists various professional groups: Patients and carers, Healthcare professionals, Animal health professionals, Business, and Media professionals. Below these is a "Product emergency HOTLINE" and a "What's New" section. At the bottom left, a "Latest news" section lists three recent updates with dates and brief descriptions.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Text size: [A](#) [A](#) [A](#) Site-wide search [GO](#)

Follow us: [Twitter](#) [RSS](#)

[Home](#) [Find medicine](#) [Regulatory](#) [Special topics](#) [Document search](#) [News & events](#) [Partners & networks](#) [About us](#) [Quick links](#)

Search for medicines

Search our database of medicines - including human medicines, veterinary medicines and herbal medicines.

Quick search

Or go to the [medicines](#) section for more options to help you find what you need.

EU Health Prize for Journalists

The European Commission is inviting submissions of published articles to the fifth edition of the EU Health Prize for Journalists until 30 September 2013.

[Find out more...](#)

Find information for...

- [Patients and carers](#)
- [Healthcare professionals](#)
- [Animal health professionals](#)
- [Business](#)
- [Media professionals](#)

Product emergency **HOTLINE** (Outside working hours)

What's New on the website

[FAQs about the Agency](#)

Latest news

- 16/09/2013 Committee on Herbal Medicinal Products publishes its meeting agenda for the first time**
The European Medicines Agency has published the agenda of a Committee on Herbal Medicinal Products (HMPC) meeting for the first time today. ... [Read more](#)
- 16/09/2013 European Medicines Agency reveals new structure**
The European Medicines Agency (EMA) has announced details of its new organisational structure. ... [Read more](#)
- 13/09/2013 Committee for Medicinal Products for Veterinary Use (CVMP) meeting of 10-12 September 2013**
The Committee adopted by consensus a positive opinion for an extension of the existing authorisation for Comfortis (spinosad), from Eli Lilly and Company Limited concerning the addition of a new tablet strength of 180 mg with two presentations for dogs and cats. ... [Read more](#)



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

The screenshot displays the EMA website interface. At the top, the EMA logo and name are visible, along with a search bar and social media links. The main navigation menu includes 'Home', 'Find medicine', 'Regulatory', 'Special topics', 'Document search', 'News & events', 'Partners & networks', and 'About us'. The 'Find medicine' section is active, showing a sidebar with categories like 'Human medicines', 'Rare disease designations', and 'Veterinary medicines'. The main content area displays the public summary of opinion for orphan designation EU/3/12/1055. The summary is titled 'Orphan designation' and includes a 'Key facts' tab. The text describes the orphan designation granted to Topotarget A/S for the treatment of peripheral T-cell lymphoma. It also includes a section for 'What is peripheral T-cell lymphoma?' and 'What is the estimated number of patients affected by the condition?'. The sidebar on the right contains 'Sponsor's contact details' and 'Patients' organisations'.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Text size: A A A Site-wide search GO

Follow us:

Home Find medicine Regulatory Special topics Document search News & events Partners & networks About us Quick links

Human medicines

- European public assessment reports
- Patient safety
- Pending EC decisions
- Withdrawn applications
- Paediatrics
- Rare disease designations
- Medicines under evaluation
- Medicines for use outside the EU
- Referrals
- Veterinary medicines
- Herbal medicines for human use

Home Find medicine Human medicines Rare disease designations

EU/3/12/1055

Email Print Help Share

Orphan designation Key facts Review of designation

On 10 October 2012, orphan designation (EU/3/12/1055) was granted by the European Commission to Topotarget A/S, Denmark, for belinostat for the treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic / disseminated).

Expand all items in this list

What is peripheral T-cell lymphoma?

Peripheral T-cell lymphoma is a cancer of the lymphatic system, a network of vessels that transport fluid from tissues through the lymph nodes and into the bloodstream. In peripheral T-cell lymphoma there is uncontrolled growth of T lymphocytes (T cells), a type of white blood cell found in the lymphatic system. Different types of peripheral T-cell lymphoma have been identified and categorised as nodal, other extranodal and leukaemic / disseminated.

The symptoms of the disease vary according to the type of lymphoma, but the first sign is usually a lump in the neck, under the arm or in the groin area, which is caused by an enlarged lymph node. The lymphoma may also affect other organs in the body such as the bone marrow, liver and the skin.

Peripheral T-cell lymphoma is a long-term debilitating and life-threatening condition because in most cases the disease does not respond well to therapy and comes back within one year after initial treatment and is associated with poor overall survival.

What is the estimated number of patients affected by the condition?

Sponsor's contact details:

Topotarget A/S
Fruebjergvej 3
DK-2100 Copenhagen
Denmark
Telephone: +45 39 17 8392
Telefax: +45 39 17 94 92
E-mail: enquiries@topotarget.com

Patients' organisations

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.



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/>

EU-CTR Version: 1.2.1[Home](#) | [Search](#) | [About](#) | [Glossary](#) | [Data Quality](#) | [Joining a trial](#) | [Contacts](#) | [EudraPharm](#)

EU Clinical Trials Register[Clinicaltrialsregister.eu](https://www.clinicaltrialsregister.eu/)

Search for Clinical Trials

[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 Protoporphyrria (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

The screenshot displays the EMA website interface. At the top, the EMA logo and name are on the left, and the European Union flag is on the right. Below the logo, the text 'SCIENCE MEDICINES HEALTH' is visible. A search bar with 'Site-wide search' and a 'GO' button is present, along with social media links for Twitter and RSS. A navigation menu includes 'Home', 'Find medicine', 'Regulatory', 'Special topics', 'Document search', 'News & events', 'Partners & networks', and 'About us'. A 'Quick links' button is also available.

The 'Find medicine' section is active, showing a sidebar with categories like 'Human medicines', 'European public assessment reports', 'Patient safety', 'Pending EC decisions', 'Withdrawn applications', 'Paediatrics', 'Rare disease designations', 'Medicines under evaluation', 'Medicines for use outside the EU', 'Referrals', 'Veterinary medicines', and 'Herbal medicines for human use'. The main content area is titled 'Betaferon' with the subtitle 'interferon beta-1b'. It includes tabs for 'About', 'Authorisation details', 'Product information', and 'Assessment history'. The 'About' tab is selected, showing a summary of the EPAR and a list of related items. A green box on the right indicates 'AUTHORISED' status. Below this, there is a 'Betaferon RSS feed' link.

Betaferon
interferon beta-1b

Email Print Help Share

About Authorisation details Product information Assessment history

Next tab »

This is a summary of the European public assessment report (EPAR) for Betaferon. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for Betaferon.

Expand all items in this list

What is Betaferon?

Betaferon is a powder and solvent that are made into a solution for injection. It contains 250 micrograms (8 million international units - MIU) per millilitre of the active substance interferon beta-1b.

What is Betaferon used for?

How is Betaferon used?

How does Betaferon work?

How has Betaferon been studied?


What benefit has Betaferon shown during the studies?

AUTHORISED
This medicine is approved for use in the European Union

Betaferon RSS feed



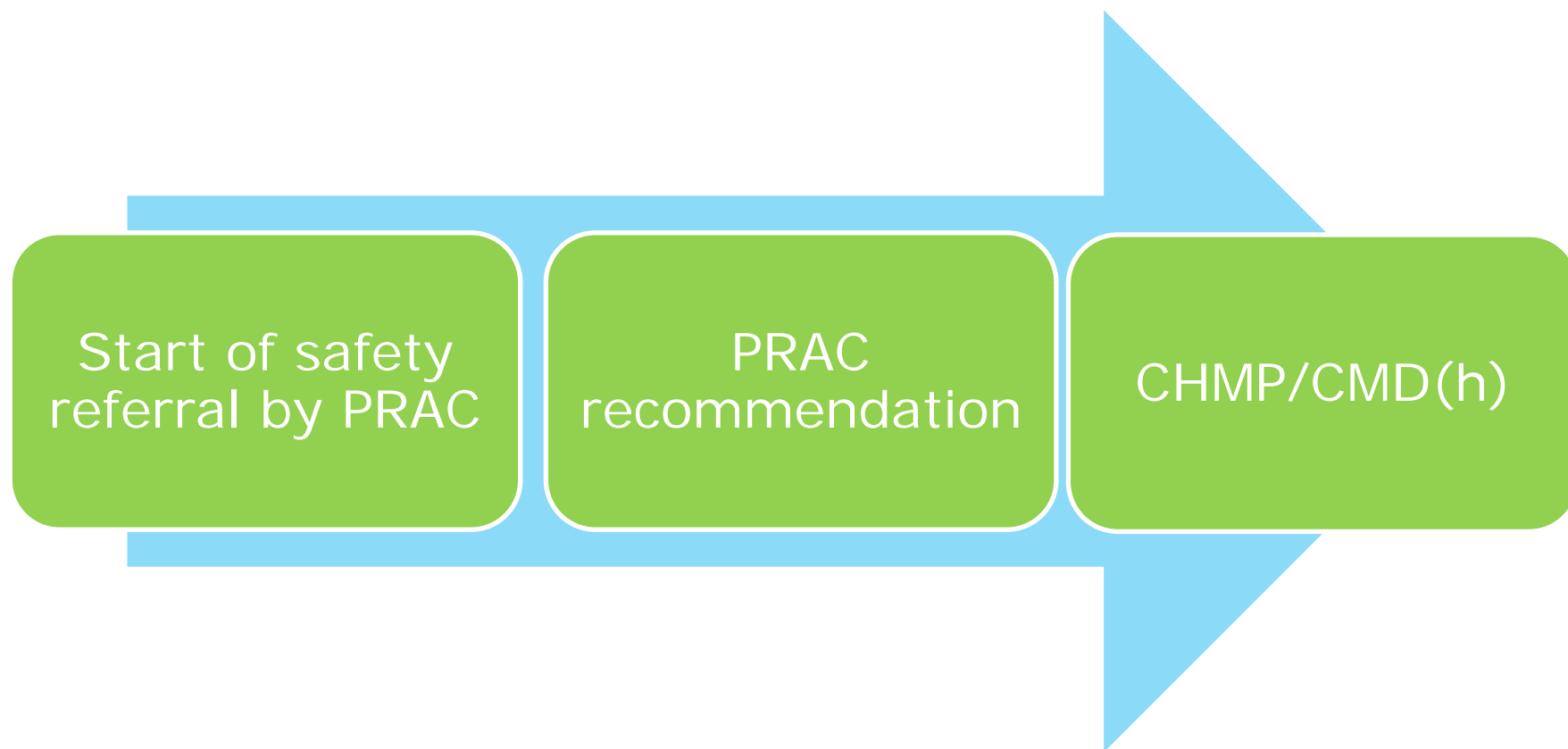
Post-authorisation

- New therapeutic indications;
 - New contraindications;
 - Other variations.
- 
- Update of the EPAR summary;
 - Update of Product information;
 - Publication of relevant assessment report.



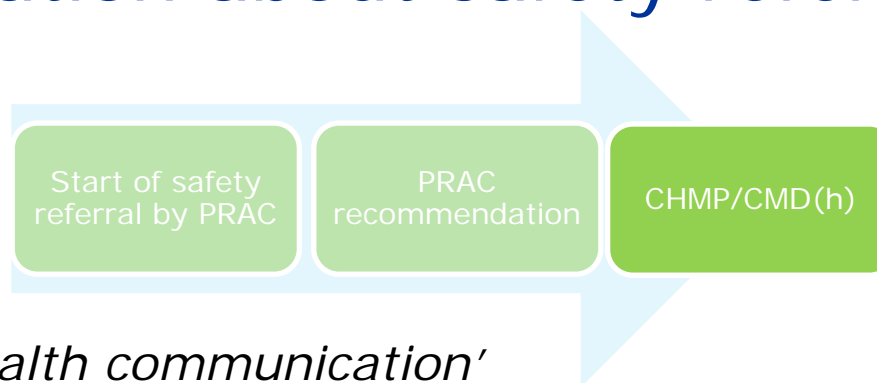
Communication about safety referrals

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](#)

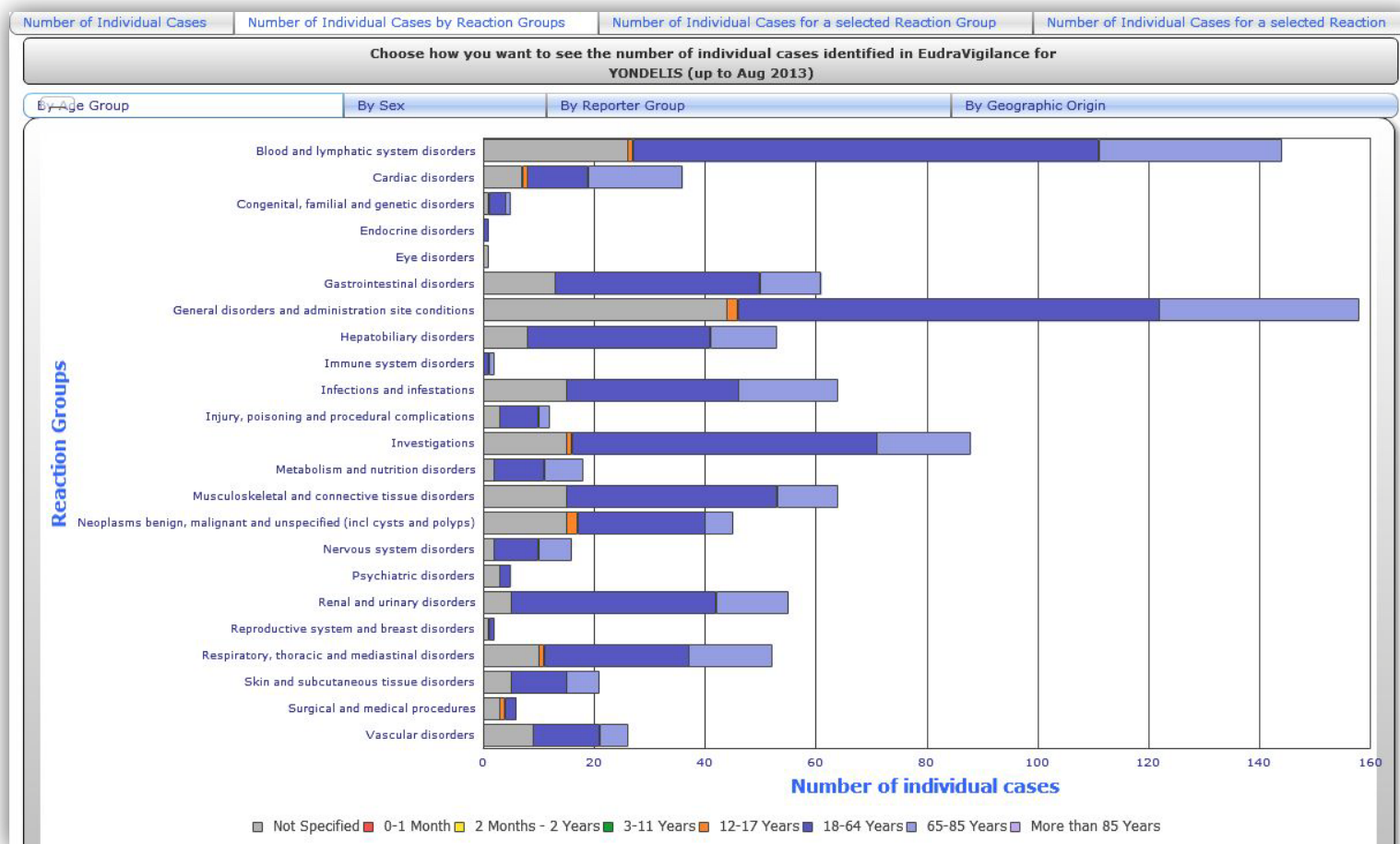


Information on adverse drug reactions: <http://www.adrreports.eu/>

- 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 

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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

