EURORDIS
THERAPEUTIC REPORT
March 2025
ISSUE 3
UPDATE ON THERAPEUTIC DEVELOPMENT AND PATIENT INVOLVEMENT IN EMA ACTIVITIES

GENERAL NEWS

Joint EU medicines agencies' network strategy to 2028

EMA and the Heads of Medicines Agencies (HMA) have published their **joint EU medicines agencies' network strategy to 2028 (EMANS)**.

The strategy, is a comprehensive update of the five-year strategy which was developed to cover the period 2021 to 2025. The updated document will **guide the European medicines regulatory network** over the next few years to meet the challenges ahead.

The **six focus areas** of the strategy to 2028 build upon those in the EMANS to 2025 with the updated strategy placing more emphasis on the **competitiveness of the EU** in the development and manufacture of medicines, as well as the **use of artificial intelligence** throughout the medicines' lifecycle.

Read more *here*!

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New clinical trial map launched in the EU

A new **clinical trial map** is now **accessible from the public website of the Clinical Trials Information System (CTIS)**. The creation of the map is an action of the Accelerating Clinical Trials in the European Union (ACT EU) initiative workplan for 2025-2026. It responds to requests for a **simple, patient-friendly dashboard for CTIS to help stakeholders**, particularly patients, **locate clinical trials** of interest in Europe.

Building on the public information contained in CTIS, the map **improves how people use the system and find information** about clinical trials.

Find out more *here*.

The Cambridge Rare Disease Showcase

Don't miss the opportunity to attend the Rare Disease Showcase on **Wednesday, 18th June 2025 in Cambridge**!

This annual event celebrates the **innovative work and powerful stories emerging from the rare disease community** – uniting patient groups, researchers, clinicians, industry professionals, and more from across the UK and beyond.

Get your ticket *here* for an evening dedicated to **building community, exchanging ideas, and driving real change** in the rare disease landscape!

Get involved in
rare disease day!
RAREDISEASEDAY.ORG

In the spotlight: conect4children

About connect4children (c4c) project

c4c was a **large collaborative paediatric network that facilitated the development of new drugs and other therapies for the entire paediatric population in Europe**. It built capacity for the **implementation of multinational paediatric clinical trials** whilst ensuring the needs of babies, children, young people and their families were met. c4c provided a sustainable, integrated **infrastructure for the efficient and swift delivery of high quality clinical trials** in children and young people across all conditions and phases of the drug development process. By emphasizing inclusiveness and collaboration across geographical, specialty, sectoral, cultural and societal backgrounds, c4c improved the infrastructure to support the development of medicines for children.

This novel collaboration between the academic and the private sectors, included 46 organisations from 21 European countries. This project has received funding from the Innovative Medicines Initiative (IMI).

Completion of c4c project

The **seven year project** has been completed, with many achievements including:

- The **development** and successful **implementation of 20 National Hubs** across 21 European countries.
- **Implementation of industry and non-industry Proof of Viability Studies** using c4c infrastructure.
- The **development and launch** of the **Strategic Feasibility Advice Service**, which includes over 450 experts.
- Establishment of the **c4c Education and Training Academy** offering a variety of courses, including GCP and GCP related courses, to paediatric clinical trial staff.
- Organisation of **6 Multistakeholder Meetings**.
- In collaboration with CDISC, **developed the Pediatric User Guide** aimed at standardising paediatric data items used in clinical trials.
- Roll out of **services through the conect4children Stichting (c4c-S)** to ensure that c4c can continue to support the development of better treatments for babies, children and young people.

What's next? conect4children Stichting

The resources created will continue to benefit the paediatric landscape through **conect4children Stichting (c4c-S)**, a Dutch non-profit organisation, ensuring that the hard work from the conect4children (c4c) project can live on. Today, c4c-S is fully operational and **offers the services** listed below through their single point of contact (SPoC):

- Expert Advice
- Site Identification
- Site Feasibility

For further information about c4c-S services click *here*!

Pharmacovigilance Risk Assessment Committee (PRAC) March 2025

Minutes February 2025
Agenda March 2025
Meeting Highlights March 2025

Possible increased risk of rare eye condition in medicines containing semaglutide

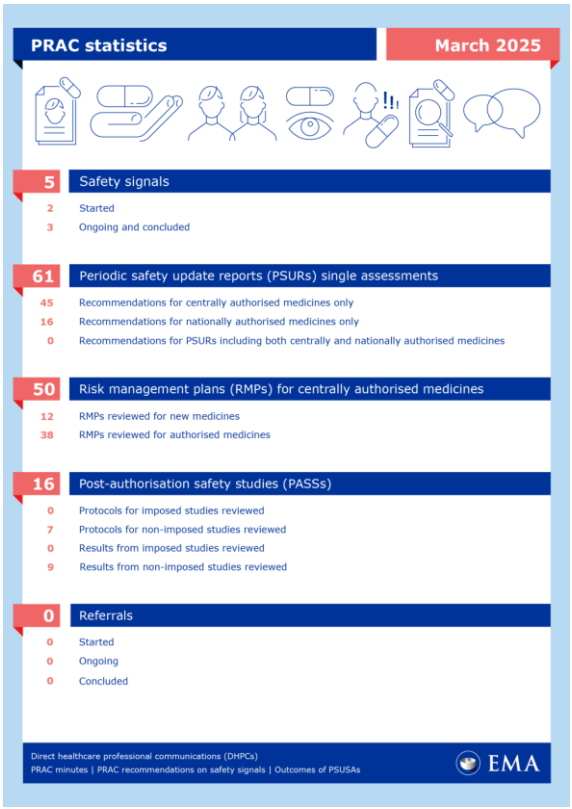
PRAC has **started a review of medicines containing semaglutide** following concerns **regarding an increased risk of developing non-arteritic anterior ischemic optic neuropathy (NAION),** a rare eye condition, as suggested in two recent observational studies.

NAION is a disorder caused by reduced blood flow to the optic nerve in the eye with potential damage to the nerve, which can lead to loss of vision in the affected eye.

Semaglutide, a GLP-1 receptor agonist, is the **active substance in certain medicines used in the treatment of diabetes and obesity** (namely Ozempic, Rybelsus and Wegovy). PRAC is assessing whether patients treated with semaglutide may have an elevated risk of developing NAION.

PRAC will now **review all available data on NAION with semaglutide** including data from **clinical trials, post-marketing surveillance, studies on the mechanism of action and the medical literature** (including the results of the observational studies).

More information is available *here*.



Medicines safety resources

- List of medicines under additional monitoring
- EudraVigilance
- Shortages catalogue
- Recommendations on medication errors
- Good Pharmacovigilance Practices
- Patient registries
- Rules of procedure on the organisation and conduct of public hearings at the

Click on the image to get the latest issue of *QPP Update*, an EMA newsletter with the latest news on EU Pharmacovigilance

Orphan medicines key figures

Since 2000

3080 Orphan designations

304 Orphan designations included in authorised indication

271 Authorised OMPs

104 To be used in children

6 Removed from the market

100 Marketed, but no longer "orphans"

To date

165

Products with a marketing authorisation and an orphan status in the European Union

05 May 2025

CHMP Meeting Highlights March 2025

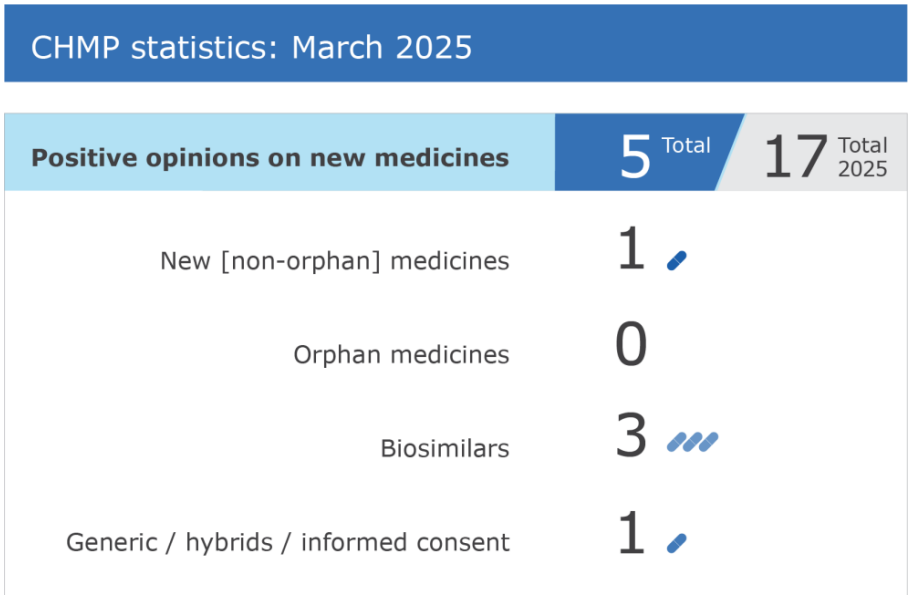
Minutes December 2024
Agenda March 2025
Meeting Highlights March 2025

In February, the CHMP recommended **5 new medicines for approval, none of them an orphan medicine**.

Non-orphan medicines, biosimilars, generics/hybrids/informed consent:

- Xoanacyl (*ferric citrate coordination complex*), for the treatment of concomitant hyperphosphataemia (high blood levels of phosphate) and iron deficiency in adults with chronic kidney disease, a condition in which the kidneys are damaged and cannot filter the blood as well as they should.
- Ryjunea *(atropine sulfate),* intended for slowing the progression of myopia (short-sightedness) in children aged 3 to 14 years.
- Jubereq (*denosumab*), for the prevention of skeletal-related events in adults with advanced malignancies involving bone and the treatment of adults and skeletally mature adolescents with giant cell tumour of bone.
- Qoyvolma (*ustekinumab*), for the treatment of plaque psoriasis in adults and children; and treatment of psoriatic arthritis, Crohn's disease and ulcerative colitis in adults.
- Osvyrti (*denosumab*), for the treatment of osteoporosis in women who have been through menopause, in men with prostate cancer who are at increased risk of fractures and whose bone loss is linked to hormone ablation, and people whose bone loss is linked to long-term treatment with systemic glucocorticoids.

For further details, read the full *CHMP meeting highlights*.



New chair elected for EMA's Human Medicines Committee

At its September 2024 meeting, EMA's Committee for Human Medicinal Products (CHMP) elected Bruno Sepodes as its new chair. Professor Sepodes started his three-year mandate on 21 September.

COMP will no longer publish meeting reports, all the information now in the minutes

COMP

The Committee for Orphan Medicinal Products (COMP) is the European Medicines Agency's (EMA) committee responsible for recommending orphan designation of medicines for rare diseases.

The COMP was established in 2000, in line with *Regulation (EC) No 141/2000* and is responsible for evaluating applications for *orphan designation and reviewing it at time of marketing authorisation*. This designation is for medicines to be developed for the diagnosis, prevention or treatment of **rare diseases** that are life-threatening or very serious. In the European Union (EU), a disease is defined as rare if it affects fewer than 5 in 10,000 people across the EU. The European Commission decides whether to grant an orphan designation for the medicine based on the COMP's opinion.

An orphan designation allows a pharmaceutical company to benefit from incentives from the EU, such as reduced fees and protection from competition once the medicine is placed on the market.

The COMP also advises and assists the European Commission on matters related to orphan medicines, including:

- developing and establishing an EU-wide policy;
- drawing up detailed guidelines;
- liaising internationally.

COMP activities for the year 2025 include (non-exhaustive list):

- Defining the requirements for major contribution to patient care (MCPC) at orphan designation as well as at marketing authorisation stage.
- Collaboration with the cross-committee Patient Experience Data initiative.
- Work on the flexibility in the definition of orphan conditions to be more in line with innovative scientific development.
- Continue the conduct of RWE studies to support COMP decision-making thanks to identification of potential use cases.
- Establishing the use of patient experience data for orphan medicines in regulatory purposes through a patient-validated methodology.
- Define criteria for when patient experts should be included in the assessment of orphan designations and orphan maintenance.
- Explore potential fit-for-purpose refinements in the CollaboRARE output.

For more information read the full COMP Work Plan 2025 *here*.

New chair elected for EMA's Orphan Medicinal Products Committee

EMA's Committee for Orphan Medicinal Products (COMP) elected a new chair. Tim Leest started his three-year mandate on 16 September.

Medicinal Product	Marketing Authorisation Holder	Therapeutic Indication	Date of Marketing Authorisation
Hetronifly® *(Serplulimab)*	Henlius Europe GmbH	Small cell lung cancer (SCLC)	03/02/2025
Emcitate® *(Tiratricol)*	Rare Thyroid Therapeutics International AB	Peripheral thyrotoxicosis in people with monocarboxylate transporter 8 (MCT8) deficiency	12/02/2025
Rytelo *(Imetelstat)*	Geron Netherlands B.V.	Anaemia in adults with myelodysplastic syndromes	07/03/2025

Please click also on the following links to see:

Orphan medicinal products authorised during 2025

Orphan medicinal products authorised since 2000

PDCO no longer publishes meeting reports. All the information now can be found in the minutes!

Minutes February 2025
Agenda March 2025

PDCO

The *Paediatric Committee* (*PDCO*) is the European Medicines Agency's (EMA) scientific committee responsible for activities on medicines for children and to support the development of such medicines in the European Union by providing scientific expertise and defining paediatric needs.

The *PDCO* was established in line with the *Paediatric Regulation*, which came into effect in 2007, to improve the health of children in Europe by facilitating the development and **availability of medicines for children** aged 0 to 17 years.

The *PDCO*'s main role is to assess the content of *paediatric investigation plans* (PIPs), which determine the studies that companies must carry out in children when developing a medicine. This includes assessing applications for a full or partial **waiver** and for **deferrals**.

The PDCO is not responsible for *marketing authorisation applications* for medicines for use in children, which is in the remit of the CHMP.

PDCO activities for the year 2025 include (non-exhaustive list):

- Continue the conduct of RWE studies including through DARWIN EU to support PDCO decision-making thanks to the identification of potential use cases where the evidence from real word data could support the scientific assessment.
- Contribution to finalisation of a framework to use of RWE in support of extrapolation.
- Provide expert input in support of the development of guidance on use of RWE for regulatory purpose.
- To develop a focus group to explore how AI can be utilised in PIP assessments and paediatric development programmes.
- Elaborate on criteria for joint adolescent-adult early phase clinical trials.
- Evaluate the feedback to PDCO and discuss possible ways forward to further improve the work on the Paediatric Investigational Plans (PIPs)

Read *here* the full PDCO 2025 Work Plan for more information.

CAT updates are now quarterly- will be updated when EMA publishes

Minutes March 2025
Agenda March 2025
Meeting November 2024-January 2025

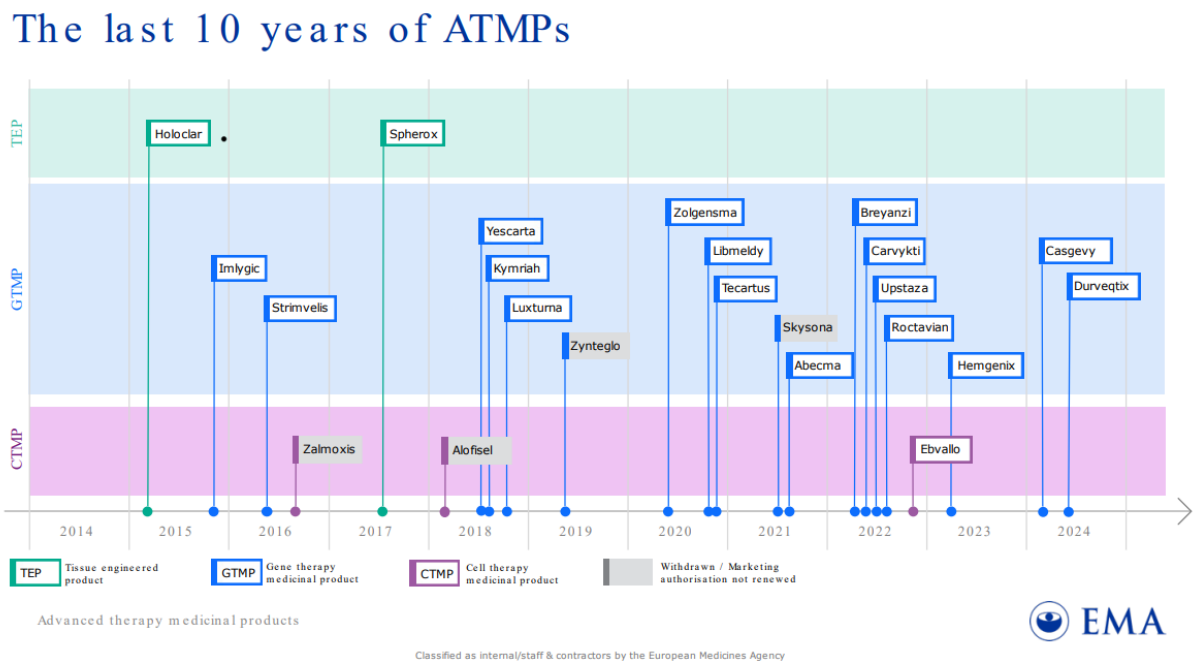
CAT highlights November 2024– January 2025 meeting update

This report provides information on ATMP approvals and extension of indications of authorised ATMPs, as well as statistical data on product-related activities.

The outcome of these assessments can be found here: *Summaries of scientific recommendations on classification of ATMPs*.

During the period covered, the CAT has not approved any new ATMP.

For more information, see also the *EMA meeting report*.



EMA's Management Board

Minutes December 2024
Agenda March 2025
Meeting Highlights March 2025

What is the EMA Management Board?

The Management Board is the European Medicines Agency's integral governance body. It has a supervisory role with general responsibility for budgetary and planning matters, the appointment of the Executive Director and the monitoring of the Agency's performance.

EMA's Management Board role

The Management Board role is to **set the Agency's budget**, approve the **annual work programme** and is responsible for **ensuring that the Agency works effectively** and **co-operates successfully** with partner organisations across the EU and beyond.

The **operational tasks** of the management board range from adopting legally binding implementing rules, to setting strategic directions for scientific networks, to reporting on the use of European Union (EU) contributions for the Agency's activities.

The Board generally meets four times a year.

EMA's Management Board composition

The Management Board consists of **36 members**, appointed to act in the public interest, who do not represent any government, organisation or sector. The members of the Management Board are appointed on the basis of their expertise in management and, if appropriate, experience in the field of human or veterinary medicines. They are selected to guarantee the highest levels of specialist qualifications, a broad spectrum of relevant expertise and the broadest possible geographical spread within the EU. Find out more about the members *here*.

The Management Board is made up of **members and observers**. **Virginie Hivert,** Therapeutic Development Director at the European Organisation For Rare Diseases EURORDIS, is one of the two **representatives of patients' organisations** in the board.

New chair elected for EMA's Management Board

EMA's Management Board elected Rui Santos Ivo as chair of the Board for a three-year period.

PATIENTS' AND CONSUMERS' WORKING PARTY

The Patients' and Consumers' Working Party (PCWP), established in 2006, serves as a platform for exchange of information and discussion of issues of common interest between EMA and patients and consumers. It provides recommendations to EMA and its human scientific committees on all matters of interest in relation to medicines.

For more information, see also the *PCWP mandate, objectives and rules of procedure*.

EMA PCWP & HCPWP meeting working parties joint meeting

Last 20th November 2024 took place *the Patients and Consumers' (PCWP) and 'Healthcare Professionals' (HCPWP) Working Parties meeting* at the EMA.

Topics discussed during the meeting included European Medicines Agency data related initiatives, digitalisation and artificial intelligence tools, sustainability of the network, working party related activities, and clinical trial updates.

EMA Glossaries

The EMA just published a *medical terms simplifier* that gives plain-language descriptions of medical terms commonly used in information about medicines.

A *glossary of regulatory terms* that gives definitions for the main terms used on the EMA website and in their documents has also been published.

For more information, please check the *glossaries here*.

GLOSSARY

Accelerated assessment
Rapid assessment of medicines in the centralised procedure aimed at facilitating patient access to new medicines that address an unmet medical need. Accelerated assessment usually takes 150 evaluation days, rather than 210.

Advanced therapies or advanced-therapy medicinal products (ATMPs)
ATMPs are new medical products based on genes, cells and tissues, which offer new treatment opportunities for many diseases and injuries. There are four main groups:

Gene-therapy medicines
They are medicines that contain genes leading to a therapeutic effect. They work by inserting 'recombinant' genes into cells, usually to treat a variety of diseases, including genetic disorders, cancer or long-term diseases. A recombinant gene is a stretch of DNA that is created in the laboratory, bringing together DNA from different sources.

Somatic-cell therapy medicines
These contain cells or tissues that have been manipulated to change their biological characteristics. They can be used to cure, diagnose or prevent diseases;

Tissue-engineered medicines
These contain cells or tissues that have been modified so they can be used to repair, regenerate or replace tissue.

Combined advanced-therapy medicines
These are medicines that contain one or more medical devices as an integral part of the medicine. An example of this is cells embedded in a biodegradable matrix or scaffold.

Authorisation under exceptional circumstances
It allows patients access to medicines that cannot be approved under a standard authorisation as comprehensive data cannot be obtained, either because there are only very few patients with the disease, the collection of complete information on the efficacy and safety of the medicine would be unethical, or there are gaps in the scientific knowledge. These medicines are subject to specific post-authorisation obligations and monitoring.

Compliance check
It is performed to verify that all the measures agreed in a *Paediatric Investigation Plan* (PIP) and reflected in the Agency's decision have been conducted in accordance with the decision, including the agreed timelines. Full compliance with all studies/measured contained in the PIP is one of several prerequisites for obtaining the rewards and incentives provided for in Articles 36 to 38 of the Paediatric Regulation.

Conditional marketing authorisation
It is granted to a medicine that addresses unmet medical needs of patients on the basis of less comprehensive data than normally required. The available data must indicate that the medicine's benefits outweigh its risks and the applicant should be in a position to provide the comprehensive clinical data in the future.

Designation, orphan medicinal product
A status assigned to a medicine intended for use against a rare condition. The medicine must fulfil certain criteria for designation as an orphan medicine so that it can benefit from incentives such as protection from competition once on the market.

European Public Assessment Report (EPAR)
It is a lay-language document, which provides a summary of the grounds on which the EMA/CHMP based its recommendation for the medicine to receive a marketing authorisation. This happens when a manufacturer develops a generic medicine based on a reference medicine, but with a different strength or given by a different route.

Hybrid application for marketing authorisation
Hybrid applications rely partly on the results of tests on the reference medicine and partly on new data from clinical trials.

Informed consent application for marketing authorisation
An informed consent application makes use of data from the dossier of a previously authorised medicine, with the marketing authorisation holder of that medicine giving consent for the use of their data in the application.

Orphan Legislation
Regulation (EC) No 141/2000 on orphan medicinal products

Paediatric Investigation Plan (PIP)
It sets out a programme for the development of a medicine in the paediatric population. It aims to generate the necessary quality, safety and efficacy data through studies to support the authorisation of the medicine for use in children of all ages. These data have to be submitted to the EMA, or national competent authorities, as part of an application for a marketing authorisation for a new medicine, or for one covered by a patent.

Paediatric Use Marketing Authorisation (PUMA)
It is a dedicated marketing authorisation for medicinal products indicated exclusively for use in the paediatric population, or subsets thereof, with, if necessary, an age-appropriate formulation. It has been designed to promote paediatric development of already authorised products which are no longer covered by a patent. Benefits are 8 years of data protection and 10 years market protection

Patient-reported outcomes (PROs)
Measurements based on data provided directly by patients regarding their health condition without interpretation of the patient's response by a clinician or anyone else.

Patient-reported outcome measures (PROMs)
They are instruments, scales, or single-item measures that have been developed to measure PROs, for example a self-completed questionnaire to assess pain.

Periodic Safety Update Reports (PSURs)
Periodic reports that the evaluate the benefit-risk balance of a medicine as evidence is gathered in clinical use. They are submitted by marketing authorisation holders at defined time points after the authorisation.

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Post-authorisation efficacy studies (PAES)
PAES are studies relating to authorised medicinal products conducted within the therapeutic indication with the aim of addressing well-reasoned scientific uncertainties on aspects of the evidence of benefits of a medicine that could not be resolved before authorisation or were identified afterwards.

Post-authorisation safety studies (PASS)
A PASS is carried out after a medicine has been authorised to obtain further information on its safety, or to measure the effectiveness of risk-management measures. The PRAC is responsible for assessing the protocols of imposed PASSs and for assessing their results.

Prevalence
In the context of the Orphan Legislation, the prevalence refers to the number of persons with the condition at the time the application is made, divided by the population of the European Union (EU) at that time. It requires demonstration through authoritative references that the disease or condition for which the medicinal product is intended affects not more than 5 in 10,000 persons in the EU, when the application is made.

Public summaries of PDCO evaluations of PIPs
They describe the applicant's proposal for the development of their medicine in children, the PDCO's conclusion on the potential use of the medicine in the paediatric population, the plan agreed between the committee and the applicant at the completion of the procedure (including any partial waivers or deferrals) and the next steps.

Referral procedures for safety reasons
A referral is a procedure used to resolve issues such as concerns over the safety or benefit-risk balance of a medicine or a class of medicines. In a referral, the EMA is requested to conduct a scientific assessment of a particular medicine or a class of medicines on behalf of the European Commission or a Member State.

Risk Management Plans (RMPs)
RMPs are regulatory documents submitted by medicine developers when they apply for marketing authorisation and include information on the medicine's safety profile; how its risks will be prevented or minimised in patients; plans for studies and other activities to gain more knowledge about the safety and efficacy of the medicine; risk factors for developing adverse reactions; measuring the effectiveness of risk-minimisation measures.

Scientific advice/protocol assistance
Through scientific advice, companies can ask the EMA for advice on whether they are conducting the appropriate tests and studies during the clinical development of a given product. In the case of orphan medicines for the treatment of rare diseases, it also includes advice on1) the demonstration of significant benefit for the designated orphan indication and on 2) similarity or clinical superiority over other medicines; which are criteria for the authorisation of an orphan medicine.

Significant benefit
Demonstrating a significant benefit, this is demonstrating a "clinically relevant advantage or a major contribution to patients" is one of the criteria that medicines for the treatment of rare diseases must fulfil to benefit from 10 years of market exclusivity once they have been authorised. For further information, read the *workshop report: Demonstrating significant benefit of orphan medicines*, held at the EMA in December 2015.

Safety signal
A safety signal is information on a new or incompletely documented adverse event that is potentially caused by a medicine and that warrants further investigation. Signals are generated from several sources such as spontaneous reports, clinical studies and the scientific literature, but their presence does not mean that a medicine has caused the reported adverse event. The adverse event could be a symptom of another illness or caused by another medicine taken by the patient. The evaluation of a safety signal is required to establish whether or not there is a causal relationship between the medicine and the adverse event.

Similar active substance
It means an identical active substance, or an active substance with the same principal molecular structural features (but not necessarily all of them) and which acts via the same mechanism.

Scientific Advisory Group (SAG)
SAGs have been established to provide an independent recommendation on scientific/technical matters related to products under evaluation through centralised regulatory procedures and referrals by the CHMP or any other scientific issue relevant to the work of the Committee.

Waiver
A waiver can be issued if there is evidence that the medicine concerned is likely to be ineffective or unsafe in the paediatric population, or that the disease or condition targeted occurs only in adult populations, or that the medicine, or the performance of trials, does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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