EURORDIS
THERAPEUTIC REPORT

November 2022

ISSUE 9

UPDATE ON THERAPEUTIC DEVELOPMENT AND PATIENT INVOLVEMENT IN EMA ACTIVITIES

GENERAL NEWS

PCWP elects new chair!

At its exceptional December 2022 meeting, EMA's Patients' and Consumers' Working Party (PCWP) has elected *Marko Korenjak* of the *European Liver Patients' Association - ELPA* as PCWP co-chair, to succeed Marilena Vrana, European Heart Network.

Marko, together with Juan Garcia Burgos, Head of Public Engagement at EMA, will co-chair the meetings of the PCWP. Congratulations Marko!

In this issue

EURORDIS Call to Action on Rare Diseases

The *report on the Czech Presidency Expert Conference on Rare Diseases* and the Call to Action on Rare Diseases, that was discussed on the EPSCO Council on Health last Friday 9 December, under Presidency Conferences is now out, please read here. The Call to Action is now supported by the overall majority of Member States, 22 EU Member states signed up!

This report is important to shine a light on the extensive European support for a European Action Plan on Rare Diseases at the conference. There is an overall agreement that this is the right time and the right place to take continued steps towards a coordinated strategy for rare diseases that better addresses current unmet needs by setting meaningful goals for patients, families and for society at large, integrated at the national and regional levels.

For more information, please read *here*!

EURORDIS Black Pearl AWARDS

EURORDIS Photo Award!
submit your photo!

Facilitating Decentralised Clinical Trials in the EU

The European Commission (EC), the *Heads of Medicines Agencies* (HMA) and the European Medicines Agency (EMA) have published *recommendations* that aim to facilitate the conduct of decentralised *clinical trials* (DCTs) while safeguarding the rights and well-being of participants as well as the robustness and reliability of the data collected. This is an outcome of their joint initiative to *Accelerate Clinical Trials in the European Union* (ACT EU).

What is Accelerate Clinical Trials (ACT EU) in the European Union?

ACT EU is a collaboration between the EC, HMA and EMA that seeks to transform how *clinical trials* are initiated, designed and run. The initiative was launched in January 2022 and aims to further develop the EU as a focal point for clinical research, to promote the development of high-quality, safe and effective medicines, and to better integrate clinical research in the European health system.

ACT EU will strengthen the European environment for *clinical trials*, whilst maintaining the high level of protection of trial participants, data robustness and transparency that EU/EEA citizens expect. ACT EU features ten priority action areas that are the basis for the *ACT EU 2022-2026 workplan.*

Decentralised Clinical Trials

Traditionally, *clinical trials* have been conducted at specific *clinical trial* sites, to which patients had to travel to. The aim of DCTs is to make it easier for patients to participate in *clinical trials* by reducing the need to travel to central trial sites. This approach has the potential to make *clinical trials* available to a wider demographic of participants and reduce drop-out rates.

Decentralisation is enabled by the advancement of digital tools, telemedicine and more mobile and local healthcare. It includes aspects such as home health visits, remote monitoring and diagnostics, direct-to-patient shipment of study drugs and electronic informed consent.

Recommendations

The recommendations include an overview of national provisions for specific decentralised *clinical trial* elements to be used in *clinical trials*. They were put together by the *European medicines regulatory network* with experts from regulatory bodies responsible for the authorisation of *clinical trials*, members of ethic committees, *good clinical practice* inspectors, methodology experts and representatives of patient organisations. Drafting of the paper was coordinated by the *clinical trials coordination group* (CTCG).

These recommendations under ACT EU are a first and important step towards clarifying the use of decentralised *clinical trials* in the EU/EEA by the *European medicines regulatory network*.

They are expected to evolve as knowledge increases and experience is gained. In particular, the overview of national provisions will be updated on a continuous basis.

For more information, please *read here*.

Pharmacovigilance Risk Assessment Committee (PRAC) November 2022

Minutes March 2022
Agenda November 2022
Meeting Highlights Nov 2022

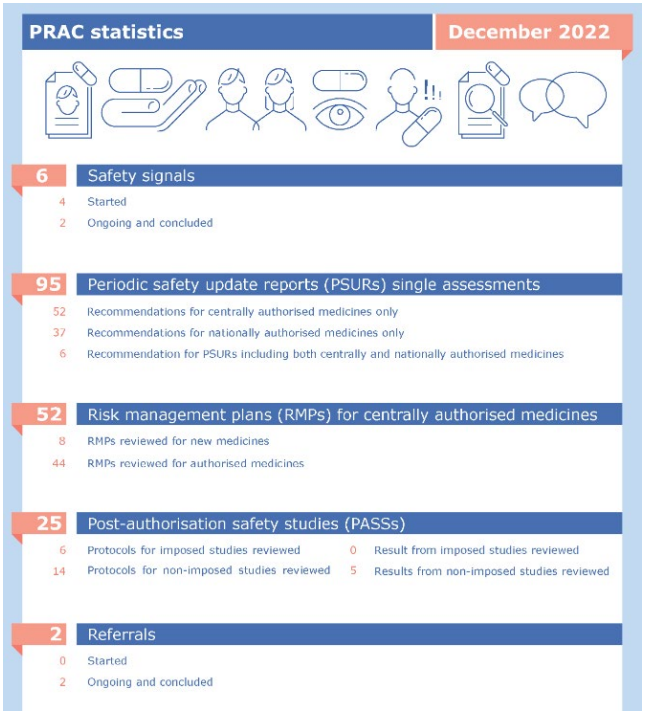
Withdrawal of pholcodine medicines from EU market

EMA's safety committee (PRAC) has confirmed its recommendation to withdraw **pholcodine-containing medicines**.

Medicines containing pholcodine are used in adults and children to treat non-productive (dry) cough and, in combination with other *active substances*, for the treatment of symptoms of cold and flu, and has recommended the revocation of the EU *marketing authorisations* for these medicines. The available data showed that use of pholcodine in the 12 months before general anaesthesia with neuromuscular blocking agents (NMBA) is a risk factor for developing an anaphylactic reaction (a sudden, severe and life-threatening allergic reaction) to NMBAs.

As it was not possible to identify effective measures to minimise this risk, nor to identify a patient population for whom the benefits of pholcodine outweigh its risks, pholcodine-containing medicines are being withdrawn from the EU market and will therefore no longer be available by prescription or over the counter.

More information is available in *EMA's public health communication.*



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

2695 Orphan designations

268 Orphan designations included in authorised indication

235 Authorised OMPs

94 To be used in children

6 Removed from the market

78 Marketed, but no longer "orphans"

To date

151

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

21 Dec 2022

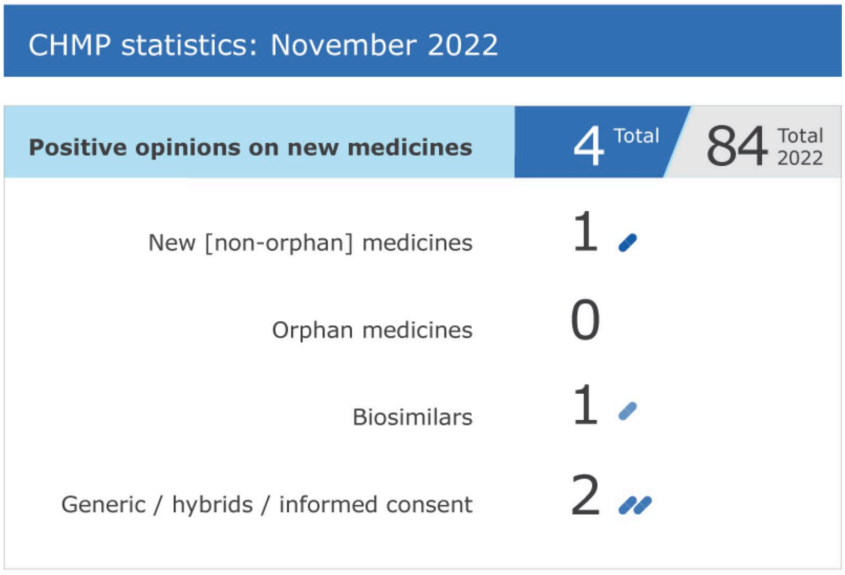
CHMP Meeting Highlights November 2022

Minutes October 2022
Agenda November 2022
Meeting Highlights Nov 2022

In November, the CHMP recommended **4 medicines for approval, non-orphan medicines**:

- **VidPrevtyn Beta** (COVID-19 vaccine (recombinant, adjuvanted)) as a booster in adults previously vaccinated with an mRNA or adenoviral vector COVID-19 vaccine.
- **Kauliv** (teriparatide), *biosimilar medicine*, for the treatment of osteoporosis, a health condition that weakens bones, making them fragile and more likely to break.
- **Pirfenidone Viatris** (pirfenidone), *generic medicine*, received a positive opinion for the treatment of idiopathic pulmonary fibrosis, a chronic and progressive condition in which the lungs become scarred and breathing becomes increasingly difficult.
- **Sugammadex Amomed** (sugammadex), *generic medicine*, intended for the reversal of neuromuscular blockade induced by rocuronium in adults and children or vecuronium in adults.

The CHMP also recommended **11 extensions of therapeutic indication,** and recommended granting marketing authorisations for **2 generics and 1 biosimilar**. For further details, read the full *CHMP meeting highlights.*



Click on the image to get the latest issue of ***Human Medicines Highlights***, a newsletter published by EMA address to organisations representing patients, consumers and healthcare professionals summarising key information on medicines for human use.

COMP September - November to be updated when info available

Minutes May 2022
Agenda July 2022
Meeting July 2022

COMP July 2022 meeting update

During the July plenary, the COMP adopted **21 positive opinions** on the designation of medicines as orphan medicinal products to the European Commission (EC). For further information, please see the *meeting report.* Please find below the list of indications covered in the medicines that were recommended for orphan designation:

- Hutchinson-Gilford progeria syndrome, Global Medical Services Sp. z o.o.;
- Urea cycle disorders , Unicyte S.r.l.;
- Graft-versus-host-disease, MDC RegAffairs GmbH;
- Cryptococcosis, Insight Drug Regulatory;
- Mucopolysaccharidosis type IV A, (Morquio A syndrome), Fondazione Telethon;
- Frontotemporal dementia, Neuroplast B.V.;
- Myasthenia gravis, Pharma Gateway AB;
- Primary sclerosing cholangitis, Amsterdam UMC;
- Prevention of acute liver failure, Egetis Therapeutics AB;
- Nontuberculous mycobacterial lung disease, Regintel Limited;
- Nontuberculous mycobacterial lung disease, Dlrc Pharma Services Limited;
- Amyotrophic lateral sclerosis, Clene Netherlands B.V.;
- Haemophilia A, S-Cubed Pharmaceutical Services ApS;
- Chondrosarcoma, TMC Pharma (EU) Limited;
- Familial adenomatous polyposis, Amsterdam UMC;
- Idiopathic hypersomnia, Propharma Group The Netherlands B.V.;
- Peripheral T-cell lymphoma, Pharma Gateway AB;
- Amyotrophic lateral sclerosis, Novartis Europharm Limited;
- Myelodysplastic syndrome, Syros Pharmaceuticals (Ireland) Limited;
- Osteosarcoma, Hephaistos-Pharma;
- Stargardt's disease, Alnylam Netherlands B.V.

Re-assessment of orphan designation at time of marketing authorisation

When a designated orphan medicinal product receives a positive opinion for marketing authorisation from EMA's Committee for Medicinal Products for Human Use (CHMP), the COMP has the responsibility to review whether or not the medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation.

The COMP adopted **three** positive opinions at time of CHMP opinion:

- **Roctavian** (valoctocogene roxaparvovec) for treatment of haemophilia A, BioMarin International Limited.
- **Scemblix** (asciminib) for treatment of chronic myeloid leukaemia, Novartis Europharm Limited.
- **Vyvgart** (efgartigimod alfa) for treatment of myasthenia gravis, Argenx.

Summaries of positive opinions on orphan designations are available on the *EMA website*.

Medicinal Product	Marketing Authorisation Holder	Therapeutic Indication	Date of Marketing Authorisation
Tavneos ® (avacopan)	Vifor Fresenius Medical Care Renal Pharma France	Adult patients with severe, active granulomatosis with polyangiitis (GPA) or microscopic polyangiitis (MPA)	11/01/2022
Voraxaze ® (glucarpidase)	SERB SAS	Adults and children (aged 28 days and older) with delayed methotrexate elimination or at risk of methotrexate toxicity	11/01/2022
Skytrofa ® (lonapegsomatropin)	Ascendis Pharma Endocrinology Division A/S	Children who do not produce enough growth hormone (GHD)	11/01/2022
Oxbryta® (voxelotor)	Global Blood Therapeutics Netherlands B. V.	Haemolytic anaemia, and +12 years old sickle cell disease	14/02/2022
Ngenla® (somatrogon)	Pfizer Europe MA EEIG	Children and adolescents with growth hormone deficiency	14/02/2022
Kimmtrak® (tebentafusp)	Immunocore Ireland Limited	Adult patients with unresectable or metastatic uveal melanoma	01/04/2022
Uplizna® (inebilizumab) *Withdrawn by the company*	Viela Bio	Adults with neuromyelitis optica spectrum disorders (NMOSD)	25/04/2022
Carvykti® (ciltacabtagene autoleucel)	Janssen-Cilag International NV	Adults with multiple myeloma	25/05/2022
Lunsumio® (mosunetuzumab)	Roche Registration GmbH	Adults with follicular lymphoma	03/06/2022
Filsuvez® (birch bark extract)	Amryt Pharmaceuticals DAC	Adults and children aged 6 months or older with epidermolysis bullosa (EB)	21/06/2022
Xenpozyme® (olipudase alfa)	Genzyme Europe BV	Acid sphingomyelinase deficiency (ASMD)	24/06/2022
Kinpeygo® (budesonide)	Calliditas Therapeutics AB	Adults with primary immunoglobulin A nephropathy (IgAN)	15/07/2022
Zokinvy® (lonafarnib)	EigerBio Europe Limited	12 months and older living with progeria and laminopathies	18/07/2022

Medicinal Product	Marketing Authorisation Holder	Therapeutic Indication	Date of Marketing Authorisation
Upstaza® (eladocagene exuparvovec)	PTC Therapeutics International Limited	Adults and children aged 18 months and older with severe aromatic L-amino acid decarboxylase (AADC) deficiency	18/07/2022
Vyvgart® (efgartigimod alfa)	Argenx	Adults with myasthenia gravis	10/08/2022
Roctavian® (valoctocogene roxaparvovec)	BioMarin International Limited	Haemophilia A	24/08/2022
Scemblix® (asciminib)	Novartis Europharm Limited	Chronic myeloid leukaemia (CML)	25/08/2022
Nulibry® (fosdenopterin)	Comharsa Life Sciences Ltd	Molybdenum cofactor deficiency (MoCD) type A	15/09/2022
Enjaymo ® (sutimlimab)	Genzyme Europe BV	Haemolytic anaemia in adults with cold agglutinin disease (CAD)	15/11/2022
Livtencity® (maribavir)	Takeda Pharmaceuticals	Cytomegalovirus (CMV) in adults	9/11/2022
Pyrukynd (mitapivat)	Agios Netherlands	Adults with pyruvate kinase deficiency (PKD)	9/11/2022
Livmarli (maralixibat chloride)	Mirum Pharmaceuticals	2 months and older patients with cholestatic pruritis	9/12/2022

Please click also on the following links to see:

Orphan medicinal products authorised during 2022

Orphan medicinal products authorised since 2000

As of April 2022 and until further notice, PDCO no longer publishes meeting reports.

Minutes February 2022
Agenda March 2022
Meeting Report March 2022

PDCO March 2022 meeting update

In March, the PDCO adopted **8 positive opinions** agreeing *paediatric investigation plans (PIPs)* for the medicines below. The PIP 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.

- Ibutamoren mesylate, from Lumos Pharma, Inc., for the treatment of growth hormone deficiency;
- Zinc gluconate / alisitol / retinyl palmitate, from Vanessa Research Magyarorszag Kft, for the treatment of microvillus inclusion disease;
- Peptide derivative of glucagon-like-peptide 1 and glucagon with fatty acid side chain (BI 456906), from Boehringer Ingelheim International GmbH, for the treatment of obesity;
- Mitapivat, from Agios Netherlands B.V., for the treatment of thalassaemia;
- Deucravacitinib, from Bristol-Myers Squibb International Corporation, for the treatment of chronic idiopathic arthritis (including rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis and juvenile idiopathic arthritis);
- Adeno-associated viral vector serotype rh.10 expressing beta-galactosidase, from Lysogene, for the treatment of GM1 gangliosidosis;
- Virus-like particle of SARS-CoV-2 spike protein (recombinant, adjuvant) (CoVLP), from Medicago Inc., for the prevention of coronavirus disease 2019 (COVID-19);ç
- SARS-CoV-2 virus, beta-propiolactone inactivated, from Valneva Austria GmbH, for the prevention of coronavirus disease 2019 (COVID-19);

The PDCO also adopted opinions on **product-specific waivers, modifications to an agreed PIP and compliance check** that can be consulted in the *meeting report.*

For a comprehensive list of opinions and decisions on PIPs, please check the *EMA website.*

CAT updates are now quarterly

Minutes October 2022
Agenda October 2022
Meeting August-October 2022

CAT highlights August – October 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*.

Advanced therapy medicinal products approvals from August-October 2022.

During its plenary meeting of **October 2022**, CAT adopted a positive draft opinion for:

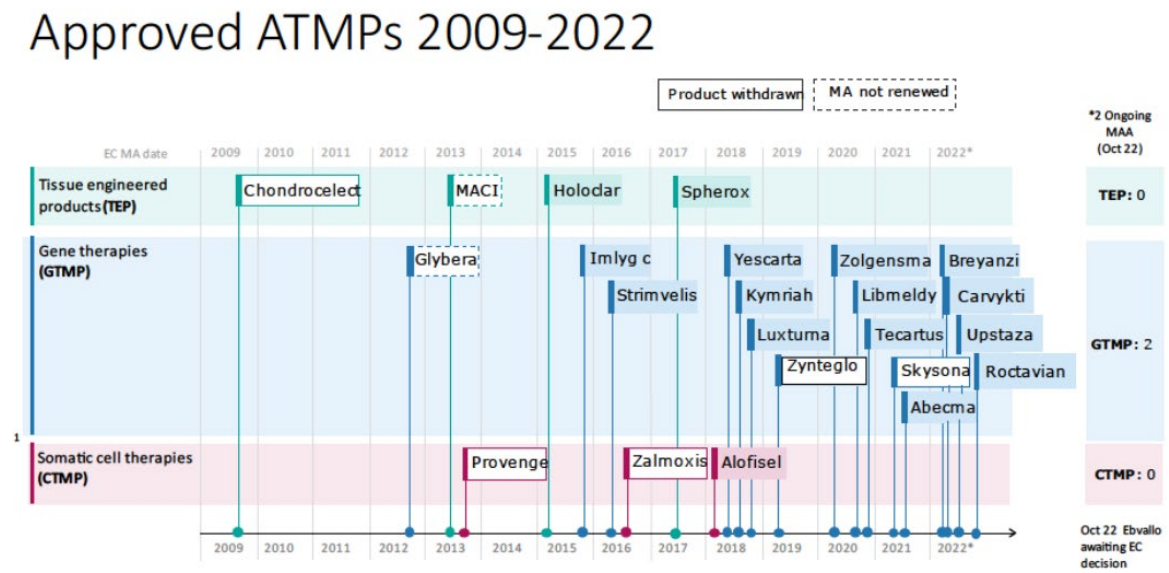
- *Ebvallo (tabelecleucel)* for the treatment of Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV+ PTLD), marketing authorisation under exceptional circumstances.

Extension of indication of authorised ATMPs

During its plenary meeting of **September 2022,** CAT adopted an extension of indication for:

- *Yescarta* for the treatment of adult patients with diffuse large B-cell lymphoma (DLBCL) and high-grade B cell lymphoma (HGBL) that relapses within 12 months from completion of, or is refractory to, first-line chemoimmunotherapy

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



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 22nd September took place face to face *the Patients and Consumers' (PCWP) and 'Healthcare Professionals' (HCPWP) Working Parties meeting.*

During the meeting new chair and co-chair was elected. EMA's Patients' and Consumers' Working Party (PCWP) has elected Marilena Vrana of the European Heart Network (EHN) as new co-chair. The Healthcare Professionals' Working Party (HCPWP) has elected Rosa Giuliani of the European Society for Medical Oncology (ESMO) as new co-chair.

A discussion on the progress report on clinical trials and contribution to ICH guidance on good clinical practice was also discussed and the EMA shared feedback from the ATMPs dedicated webinar on 28 June, as well as updates on pharmacovigilance and new initiatives for risk minimisation.

For more information, please see the agenda *here*.

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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GLOSSARY

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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