EURORDIS
THERAPEUTIC REPORT
December 2022
ISSUE 11
UPDATE ON THERAPEUTIC DEVELOPMENT AND PATIENT INVOLVEMENT IN EMA ACTIVITIES

GENERAL NEWS

CAT elects new chair!

During last January meeting, EMA's Committee for Advanced Therapies (CAT) elected **Ilona Reischl** from the **Austrian Medicines and Medical Devices Agency (AGES MEA)** as its new Chair for a three-year mandate. Congratulations Ilona!

Before becoming Chair, Dr Reischl served as Vice-Chair of the CAT for the last six years. Dr Reischl started working with the European medicines regulatory network in 2006 as a quality assessor. She subsequently created and led the AGES MEA clinical division until 2018. She replaces Dr Martina Schüssler-Lenz, from the Paul-Ehrlich Institute (PEI) in Germany.

The CAT will elect a new Vice-chair at its meeting on 16 February 2023. For more information, read *here*!

IN THIS ISSUE

Conference RNA-based medicines

The European Medicines Agency (EMA) is organising a virtual conference Thursday, 2 February 2023 from 9h to 16:30h CET. The agenda is *here*, and the event will be broadcasted on that day and link *here*. The aim of the conference is to promote the development of RNA-based medicines by:

- Identifying scientific and regulatory opportunities and challenges of RNA-based innovative medicines;
- Facilitating dialogue between industry/academia and regulators and raising awareness on scientific and regulatory aspects;
- Identifying gaps in regulatory science.

For more information, please read *here*.

CTIS becomes mandatory

Clinical Trials Information System (CTIS) is now the single-entry point for sponsors and regulators of clinical trials for the submission and assessment of clinical trial data.

Sponsors can now apply for authorisations in up to 30 EU/EEA countries at the same time and with the same documentation, and therefore, do not have to submit clinical trial applications separately to national competent authorities (NCAs) and ethics committees in each country to gain regulatory approval to run a clinical trial.

For more information, please read *here.*

EURORDIS Black Pearl Awards
21st February at 7pm CET.
Follow online here!

EURORDIS Round Table of Companies
22nd February online here

Shortages of antibiotics in the EU

A recent surge in respiratory infections has driven an increase in demand for amoxicillin which combined with other issues including manufacturing delays and production capacity challenges led to shortages affecting the majority of Member States. EMA's *Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG)*, is made up of representatives from EMA, the European Commission and the Heads of Medicines Agencies (HMA).

The MSSG and its working party, *the medicines shortages Single Point Of Contact (SPOC) working party,* have been closely monitoring the situation since November 2022 and have been engaging with key players in the supply chain of amoxicillin to take forward possible mitigating measures.

Recently, this group met to discuss the progress made and to agree on the next steps in the coordinated response to the ongoing shortages of antibiotic medicines containing amoxicillin (alone and in combination with clavulanic acid) in the EU. Progress and mitigation efforts:

- **Increase production capacity of amoxicillin:** EU regulators have met with the main parties involved in the supply chain of amoxicillin to provide regulatory support so as to increase production capacity (e.g. through rapid procedures to implement changes to alternative sources of raw materials, manufacturing sites, package materials…).
- **Increase supply:** Reviewed the possibility to increase supply to fulfil the Member States' immediate needs. EMA has received positive feedback from major manufacturers and supply is expected to be increased (through for example additional supply and increased production capacity) in the coming weeks and months.
- **Regulatory flexibilities**: such as the exceptional supply of certain medicines that may not be authorised in a particular EU Member State, or full or partial exemptions to certain labelling and packaging requirements for medicines, many national competent authorities have managed to mitigate certain shortages.

At present **this ongoing shortage situation is not considered to be a major event** and that current mitigating measures are improving the supply situation in the short-term. It is important **that antibiotics are used prudently to maintain their efficacy and avoid antimicrobial resistance.** Antibiotics, including amoxicillin, should only be prescribed to treat bacterial infections. They are not suitable for treating viral infections such as cold and flu, where they are not effective.

Pharmacovigilance Risk Assessment Committee (PRAC) December 2022

Minutes April 2022
Agenda December 2022
Meeting Highlights Dec 2022

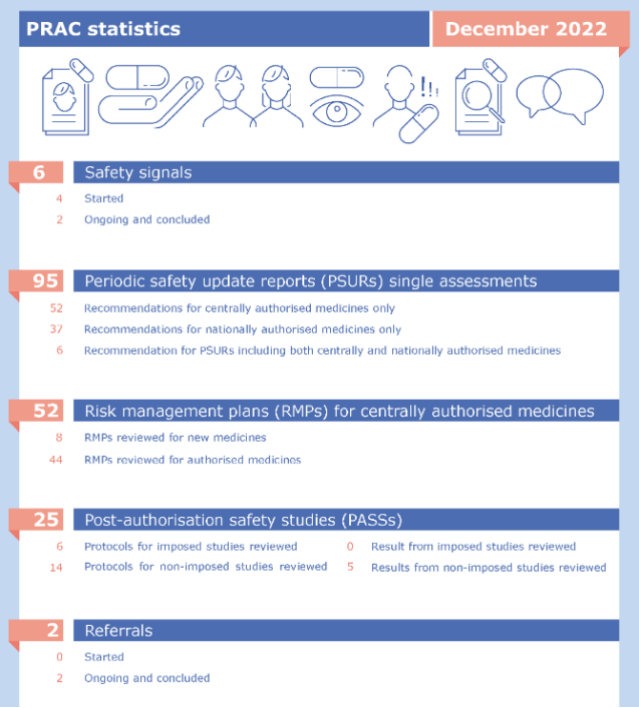
Zolgensma: fatal cases of acute liver failure

EMA's safety committee (PRAC) informed about fatal cases of acute liver failure in patients treated with **Zolgensma** *(onasemnogene abeparvovec),* a gene therapy medicine for the treatment of spinal muscular atrophy (SMA), a serious rare condition of the nerves that causes muscle wasting and weakness.

This communication informs healthcare professionals of the fatal cases of liver failure and the updated recommendations for monitoring liver function, assessing suspected liver injury after infusion and further advice regarding tapering the corticosteroid treatment.

Healthcare professionals should promptly assess patients with worsening liver function tests and/or signs or symptoms of acute liver illness. **If patients do not respond adequately to treatment with corticosteroids, treating physicians should consult a paediatric gastroenterologist or hepatologist and consider adjustment of the corticosteroid regimen**.

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

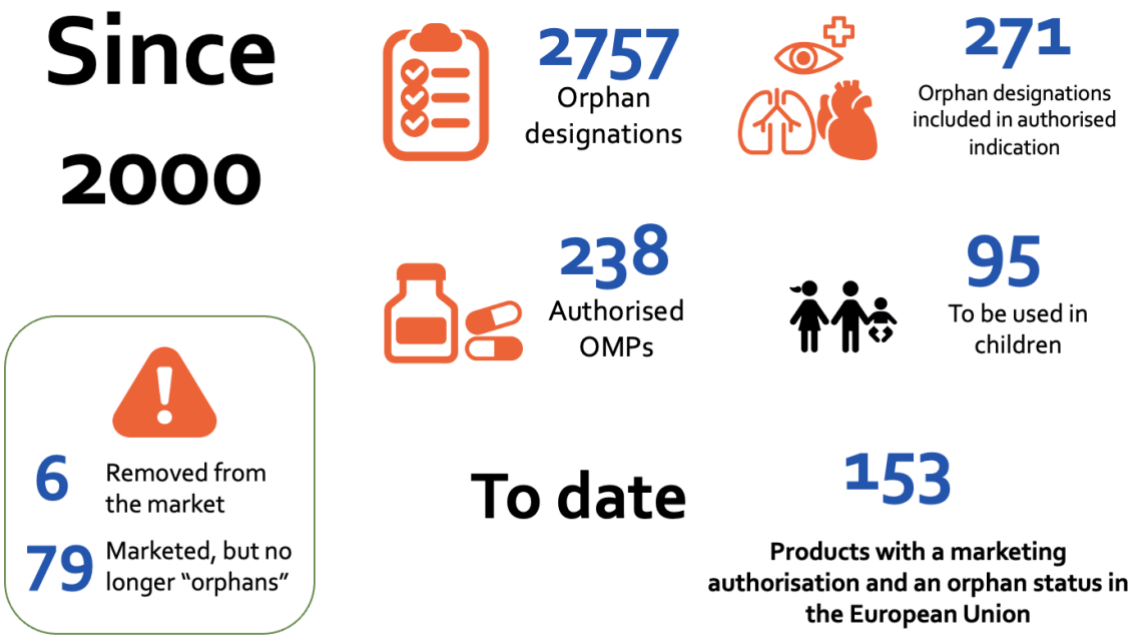


Medicines safety resources

- 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



21 January 2023

CHMP Meeting Highlights December 2022

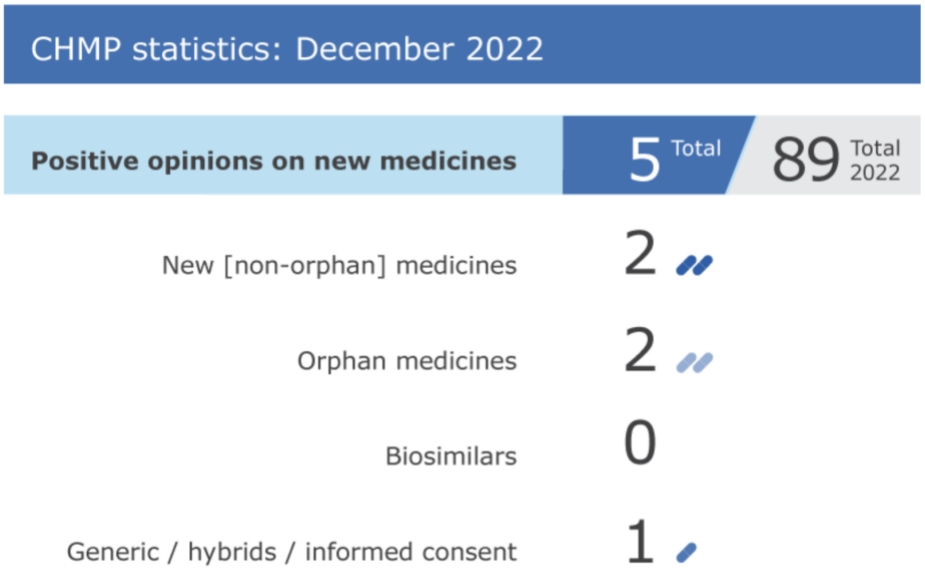
Minutes November 2022
Agenda December 2022
Meeting Highlights Dec 2022

In December, the CHMP recommended **5 medicines for approval, two orphan medicines**:

- *Conditional Marketing Authorisation* for **Hemgenix** (etranacogene dezaparvovec), the first gene therapy for the treatment of severe and moderately severe Haemophilia B.
- **Pombiliti** (cipaglucosidase alfa) for the treatment of glycogen storage disease type II, also known as Pompe disease.
- **Imjudo** (tremelimumab), to be used in combination with Imfinzi (durvalumab) for the treatment of adults with hepatocellular carcinoma, a type of liver cancer.
- **Tremelimumab AstraZeneca** (tremelimumab), for the treatment of metastatic non-small-cell lung cancer in combination with Imfinzi (durvalumab) and platinum-based chemotherapy.

The CHMP also recommended **12 extensions of therapeutic indication,** and recommended granting marketing authorisations for **1 generic for Multiple Sclerosis**.

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 - December 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
Amvuttra® (vutrisiran)	Alnylam Netherlands B.V.	Polyneuropathy caused by hereditary transthyretin-mediated (hATTR) amyloidosis	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
Ebvallo® (tabelecleucel)	Atara Biotherapeutics Ireland Limited	Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV+ PTLD)	16/12/2022
Mycapssa® (octreotide)	Amryt Pharmaceuticals DAC	Acromegalia	2/12/2022

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:

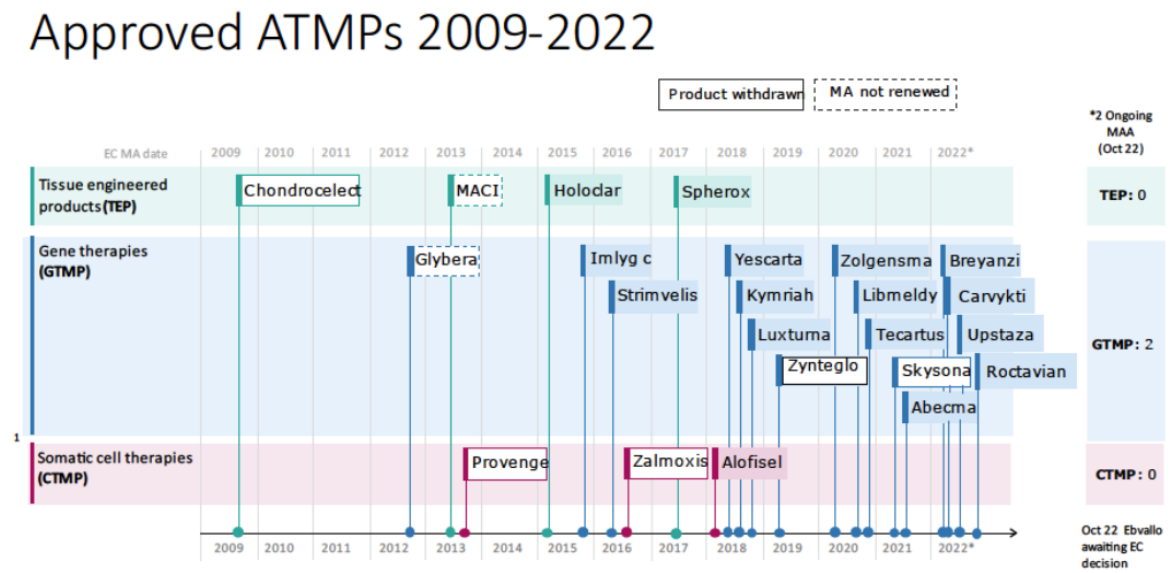
- *Ebvallo (tabelecleucel)* for the treatment of Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV+ PTLD), marketing authorisation under exceptional circumstances.

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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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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