EURORDIS
RARE DISEASES EUROPE

EURORDIS
THERAPEUTIC REPORT

November 2020

ISSUE 10

UPDATE ON THERAPEUTIC DEVELOPMENT AND PATIENT INVOLVEMENT IN EMA ACTIVITIES

GENERAL NEWS

EMA recommended first COVID-19 vaccine for authorization in the EU

EMA recommended granting a conditional marketing authorisation for the vaccine *Comirnaty*, developed by BioNTech and Pfizer, to prevent coronavirus disease 2019 (COVID-19) in people from 16 years of age. EMA's scientific opinion paves the way for the first marketing authorisation of a COVID-19 vaccine in the EU by the European Commission, with all the safeguards, controls and obligations this entails.

EMA's human medicines committee (CHMP) has completed its rigorous evaluation of *Comirnaty*, concluding by consensus that sufficiently robust data on the quality, safety and efficacy of the vaccine are now available to recommend a formal conditional marketing authorisation.

For more information, please see the *EMA website.*

IN THIS ISSUE

EMA organises a second public meeting about the new COVID-19 vaccines

EMA will organise a *second public meeting on 8 January 2021* (13-15:15h CET) to inform European citizens about the assessment, approval and roll-out of new COVID-19 vaccines. Together with the EU medicines regulatory network, EMA has been working around the clock to bring much needed COVID-19 vaccines to EU citizens as quickly as possible, while keeping the same rigorous standards of approval as for all vaccines. An application for a marketing authorisation for another COVID-19 vaccine, developed by Moderna, is currently ongoing and could be concluded at an extraordinary meeting of EMA's human medicines committee (CHMP) on 6 January 2021.

The *public meeting* on the 8th January will be an opportunity to inform citizens about the approval and use of the new vaccines, to explain how the safety of the vaccines will be assured, and to provide information on the role of the European Commission and the national public health authorities on the roll-out of the vaccines. As in the previous one held *last 11th December*, the meeting will be broadcast live and participants will be also able to share their views, expectations and raise any concerns. Those interested in participating and asking questions to the panellists should *fill in this form* by 31st December 2020.

For more information, please see the *agenda* of the *event*.

What is Rare 2030?

Rare 2030 is a foresight study that gathers the input of a large group of patients, practitioners and key opinion leaders to propose policy recommendations that will lead us to improved policy and a better future for people living with a rare disease in Europe. This a two-year project that will end in a presentation to EU Parliament in February 2021 with recommendations on the most critical areas needing sound policy.
For more information on the Rare 2030 project in which EURORDIS is a partner, see here.

Four stages of the Rare2030 Foresight Study

The **Rare2030 Knowledge Base** has been developed with the input of the nearly 200 members of the Panel of Experts and covers their broad range of expertise in topics relevant to rare disease policy. The knowledge collected has been organised into eight topic-based summaries, read here!

Through a series of consultations held during 2019, the Rare2030 Panel of Experts has summarised **trends and drivers** of change that have been identified as instrumental to shaping the future of rare disease policy. Read more about the trends here!

With the information collected through the knowledge base and trends identified through the Rare2030 Foresight study, the Panel of Experts put together four possible future scenarios depicting what the world may be like for people living with a rare disease in 2030, see the video!

The final Rare2030 policy recommendations will be published in February 2021.

New Rare Barometer Voices survey on the future of rare disease!

EURORDIS launched a new Rare Barometer Voices survey on *what should the future of rare diseases look like?* This survey will help to shape the recommendations of the *Rare 2030 Foresight Study in Rare Disease Policy.*

Take the *survey now* (takes around 25 minutes!), and share it within your network! All your opinions will help us to shape the future of rare disease policies regarding the access to health care, medical and social research as well as early diagnosis for children!

Pharmacovigilance Risk Assessment Committee (PRAC) November 2020

Minutes September 2020
Agenda November 2020
Meeting Highlights Nov 2020

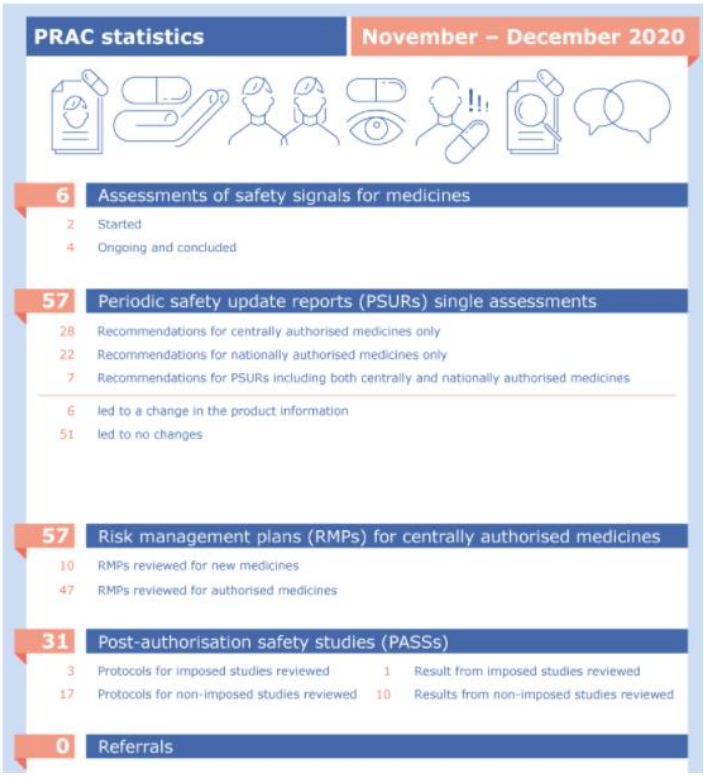
Update product information for chloroquine and hydroxychloroquine

EMA's safety committee (PRAC) EMA's safety committee (*PRAC*) has recommended updating the *product information* for all chloroquine or hydroxychloroquine-containing medicines following a review of all available data that confirmed a link between the use of these medicines and the risk of psychiatric disorders and suicidal behaviour. The review was initiated after six cases of psychiatric disorders in patients with COVID-19 who were given higher than authorised doses of hydroxychloroquine.

Chloroquine and hydroxychloroquine are authorised in the EU for the treatment of certain autoimmune diseases, such as rheumatoid arthritis and lupus, as well as for prophylaxis and treatment of malaria. They are **not authorised for the treatment of COVID-19,** but both medicines have been used as off-label treatment in patients with the disease. However, chloroquine and hydroxychloroquine *have not shown* any beneficial effects in treating COVID-19 in large randomised *clinical trials*.

PRAC recommends updating the *product information* for these medicines to provide better information to healthcare professionals and patients on the risk of suicidal behaviour and psychiatric disorders. **Patients using chloroquine or hydroxychloroquine medicines who experience mental health problems** (e.g. irrational thoughts, anxiety, hallucinations, feeling confused or feeling depressed, including thoughts of self-harm or suicide), or others around them who notice these side effects, **should contact a doctor straight away.**

For more information, please see *EMA website*.



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 PRAC

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

2361 Orphan designations

226 Orphan designations included in authorised indication

193 Authorised OMPs

75 To be used in children

5 Removed from the market

66 Marketed, but no longer "orphans"

To date

122

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

20 Dec 2020

CHMP Meeting Highlights November 2020

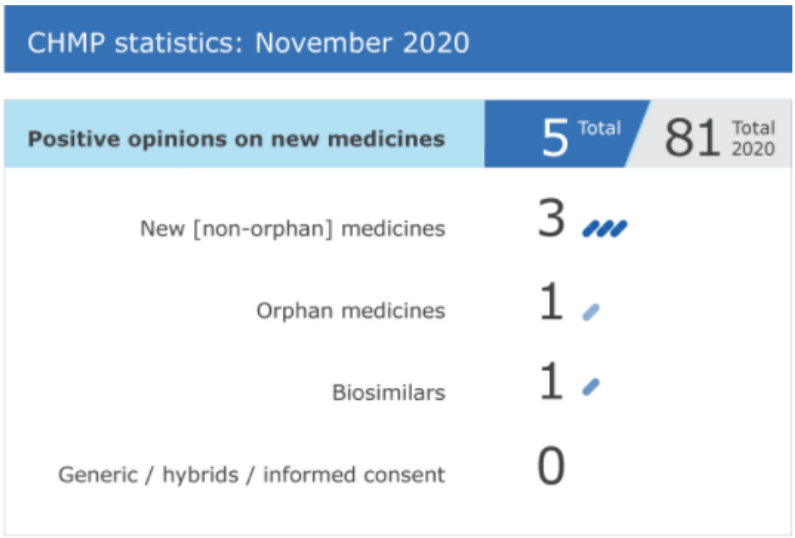
Minutes August 2020
Agenda November 2020
Meeting Highlights Nov 2020

In November, the CHMP recommended **5 medicines for approval, one orphan medicine**:

- Marketing authorisation *under exceptional circumstances* for Elzonris (tagraxofusp), for the treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN), a rare and aggressive type of acute myeloid leukaemia (blood cancer) in patients who had not yet received any treatment for BPDCN (first-line treatment).
- Phesgo (pertuzumab / trastuzumab) for the treatment of early and metastatic breast cancer.
- Roclanda (latanoprost / netarsudil) for the reduction of elevated intraocular pressure (IOP) in adult patients with primary open-angle glaucoma or ocular hypertension for whom monotherapy with a prostaglandin or netarsudil provides insufficient IOP reduction.
- Xofluza (baloxavir marboxil) received a positive opinion from the CHMP for the treatment and post-exposure prophylaxis of uncomplicated influenza.
- The biosimilar medicine Onbevzi (bevacizumab) received a positive opinion for the treatment of carcinoma of the colon or rectum, breast cancer, non-small cell lung cancer, renal cell cancer, epithelial ovarian, fallopian tube or primary peritoneal cancer and carcinoma of the cervix.

Six recommendations on extensions of therapeutic indication were also granted.

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 November 2020 meeting update

Minutes September 2020
Agenda November 2020
Meeting Report Nov 2020

During the November plenary, the COMP adopted **20 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:

- Multiple myeloma, Celgene Europe B.V.;
- Glioma, VH Regulatory Consulting GmbH & Co. KG;
- Pulmonary arterial hypertension, IDEA Innovative Drug European Associates (Ireland) Limited;
- Hepatocellular carcinoma, AstraZeneca AB;
- Prader-Willi syndrome, Helsinn Birex Pharmaceuticals Limited;
- Progressive supranuclear palsy, Granzer Regulatory Consulting & Services;
- Progressive myoclonic epilepsy type 2 (Lafora disease), Ionis Development (Ireland) Limited;
- Duchenne muscular dystrophy, Pharma Gateway AB;
- Congenital hyperinsulinism, Scendea (NL) B.V.;
- Wilson disease, Ultragenyx Germany GmbH;
- Limb-girdle muscular dystrophy, Sarepta Therapeutics Ireland Limited;
- Haemophilia A, TMC Pharma (EU) Limited;
- Acute myeloid leukaemia, Granzer Regulatory Consulting & Services;
- Glioma, Novartis Europharm Limited;
- Olmsted syndrome, Institut Des Maladies Genetiques;
- Mantle cell lymphoma, TMC Pharma (EU) Limited;
- Respiratory distress syndrome, Boyd Consultants Limited;
- Fragile X syndrome, Aparito Netherlands B.V.;
- Angelman syndrome, Roche Registration GmbH;
- Glioma, Novartis Europharm Limited.

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. After the October plenary, the COMP adopted by written procedure **4 positive opinions at time of CHMP opinion**:

- *Fintepla* (fenfluramine hydrochloride) for treatment of Dravet syndrome, Zogenix ROI Limited
- *Libmeldy* (autologous CD34+ cell enriched population that contains hematopoietic stem and progenitor cells transduced ex vivo using a lentiviral vector encoding the human arylsulfatase A gene) for treatment of metachromatic leukodystrophy, Orchard Therapeutics (Netherlands) B.V.
- *Oxlumo* (lumasiran) for treatment of primary hyperoxaluria type 1, Alnylam Netherlands B.V.
- *Tecartus* (autologous peripheral blood T cells CD_4 and CD8 selected and CD_3 and CD28 activated transduced with retroviral vector expressing anti-CD19 CD28/CD_3-zeta chimeric antigen receptor and cultured) for treatment of mantle cell lymphoma, Kite Pharma EU B.V.

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

ORPHAN MEDICINAL PRODUCTS AUTHORISED IN 2020

Medicinal Product	Marketing Authorisation Holder	Therapeutic Indication	Date of Marketing Authorisation
Isturisa® (*osilodrostat*)	Novartis Europharm Limited	Cushing Syndrome	09/01/2020
Polivy® *(polatuzumab vedotin)*	Roche Registration GmbH	Diffuse large B-cell lymphoma (DLBCL)	16/01/2020
Givlaari® *(Givosiran)*	Alnylam Netherlands B.V.	Acute hepatic porphyria	02/03/2020
Trepulmix® *(Treprostinil)*	SciPharm Sàrl	Chronic thromboembolic pulmonary hypertension (CTEPH)	03/04/2020
Zolgensma® *(onasemnogene abeparvovec)*	AveXis EU Limited	Spinal muscular atrophy (SMA)	18/05/2020
Reblozyl® *(luspatercept)*	Celgene Europe B.V.	Beta thalassaemia & Myelodysplastic syndromes	25/06/2020
Daurismo® *(glasdegib)*	Pfizer Europe MA EEIG	Newly-diagnosed acute myeloid leukaemia (AML)	26/06/2020
Pretomanid FGK® *(pretomanid)*	FGK Representative Service GmbH	Adults with drug-resistant tuberculosis	31/07/2020
Hepcludex® *(bulevirtide)*	MYR GmbH	Chronic (long-term) hepatitis delta virus (HDV) infection in adults	31/07/2020
Idefirix® *(imlifidase)*	Hansa Biopharma AB	Prevent the body from rejecting a newly transplanted kidney	25/08/2020
Kaftrio® *(ivacaftor / tezacaftor / elexacaftor)*	Vertex Pharmaceuticals (Ireland) Limited	Cystic fibrosis	21/08/2020
Blenrep® (belantamab mafodotin)	GlaxoSmithKline (Ireland) Limited	Multiple Myeloma	25/08/2020
Ayvakyt® (avapritinib)	Blueprint Medicines (Netherlands) B.V.	Gastrointestinal stromal tumour (GIST)	24/09/2020
Arikayce liposomal® (amikacin sulfate)	Insmed Netherlands B.V.	Adults with a lung infection caused by Mycobacterium avium complex (MAC)	27/10/2020
Adakveo® (crizanlizumab)	Novartis Europharm Limited	Sickle cell disease aged 16 years and older.	28/10/2020
Oxlumo® (lumasiran)	Alnylam Netherlands B.V.	Primary hyperoxaluria type 1	19/11/2020
Obiltoxaximab SFL ® (obiltoxaximab)	SFL Pharmaceuticals Deutschland GmbH	Inhalational anthrax due to Bacillus anthracis.	18/11/2020

Please click also on the following links to see:

Orphan medicinal products authorised during 2020

Orphan medicinal products authorised since 2000

PDCO November 2020 meeting update

Minutes October 2020
Agenda November 2020
Meeting Report November 2020

In November, the PDCO adopted **9 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.

- Olinciguat, from Cyclerion Therapeutics Inc., for the treatment of sickle cell disease;
- Tabelecleucel, from Atara Biotherapeutics, Inc., for the treatment of Epstein-Barr virus associated post-transplant lymphoproliferative disorder;
- Venglustat, from Genzyme Europe B.V., for the treatment of autosomal dominant polycystic kidney disease;
- Fully human IgG1 RB-1 YTE anti-RSV F monoclonal antibody (MK-1654), from Merck Sharp & Dohme (Europe), Inc., for the prevention of lower respiratory tract infection caused by respiratory syncytial virus;
- Voxelotor, from Synteract GmbH, for the treatment of sickle cell disease;
- Etrasimod L-arginine, from Arena Pharmaceuticals, Inc., for the treatment of ulcerative colitis;
- (R)-2-(1-(6-Amino-5-chloropyrimidine-4- carboxamido)ethyl)-N-(5-chloro-4- (trifluoromethyl)pyridin- 2-yl)thiazole-5-carboxamide, from DOT Therapeutics-1 Inc, for the treatment of paediatric low grade glioma;
- Etranacogene dezaparvovec, from uniQure biopharma B.V., for the treatment of Haemophilia B;
- Sulbactam / durlobactam, from Entasis Therapeutics Inc., for the treatment of infections due to organisms of the Acinetobacter baumannii-calcoaceticus complex.

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 November 2020 meeting update

Minutes October 2020
Agenda November 2020
Meeting Report Nov 2020

In November the Committee for Advanced Therapies (CAT) finalised **2 scientific recommendations on the classification of advanced therapy medicinal products** (ATMPs) depicted below.

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

The following product was classified as **gene therapy medicinal product:**

- Recombinant serotype 9 adeno-associated virus encoding a codon-optimised human neuronal ceroid lipofuscinosis-5 transgene intended for the treatment of neuronal ceroid lipofuscinosis type 5.

The following product was classified as an **advanced therapy medicinal product:**

- Autologous adipose-derived mesenchymal stem cell, intended for the treatment of diabetic foot Syndrome.

CAT noted the **withdrawal** by Biomarin International Limited of the marketing authorisation application of *Roctavian* (valoctogene roxaparovec), which was intended for the treatment of severe haemophilia A. For more information, please read *here*.

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.*

PCWP and HCPWP November meeting

Last 16th November took place a virtual meeting update on *COVID-19 pandemic* which brought together all eligible patient and consumer and healthcare professionals organisations and members of the Patients' and Consumers' Working Party (PCWP) and Healthcare Professionals' Working Party (HCPWP).

The meeting focused on the following:

- Update on Agency's response to the COVID-19 pandemic
- Patient / Healthcare Professionals' participation in EMA Covid-19 Taskforce (ETF)
- Pharmacovigilance aspects on COVID therapeutics & vaccines
- Information materials on COVID-19 vaccines

For more information, please see the *agenda*.

EMA public stakeholder meeting on COVID-19 vaccines

Last 11th December the EMA organised a virtual *meeting* to explain the processes for the *development, evaluation, approval and safety* monitoring of COVID-19 vaccines in the EU, including EMA's specific role. It gave the opportunity to the public and stakeholder groups to speak and share their needs, expectations and any concerns. François Houyez, Treatment Information and Access Director and Health Policy Advisor, EURORDIS, gave the PLWRD views.

The *event* was broadcasted live, please see the *agenda*, *presentations* and *recording here*.

EURORDIS and the European Patients Forum (EPF) signed a jointly letter asking the EMA to organise a multi-stakeholder meetings open to the public on vaccines to prevent SARS-CoV-2 infection, please read the *letter 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

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GLOSSARY

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.

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.

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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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