EURORDIS RARE DISEASES EUROPE

EURORDIS

THERAPEUTIC REPORT

June 2022

ISSUE 6

UPDATE ON THERAPEUTIC DEVELOPMENT AND PATIENT INVOLVEMENT IN EMA ACTIVITIES

GENERAL NEWS

Prevention of medicines shortages in the European Union

EMA has published *a guidance for patients' and healthcare professionals' organisations* with *key principles and examples of good practices* to support them in preventing and managing shortages of human medicines. Medicine shortages and reduced availability of medicines represent an increasing issue across the EU and the globe, and it has been amplified by the COVID-19 pandemic. It may have a significant impact on patient care by causing medicine rationing and delay of critical treatments. Due to medicine shortages, patients may need to use less effective alternatives and they could risk using medication incorrectly.

Patients' organisations are encouraged to:

- **develop observatories** in collaboration with national authorities to **collect and analyse information** from patients on shortages and their early signs;
- work with national authorities on criteria and ways to develop **registries of essential and critical medicines;**
- **Communication and awareness raising** among members on causes of shortages, the safe use of alternative medicines, risks of stockpiling and where to find information on ongoing shortages.

The key recommendations have been prepared based on consultations with member organisations of the EMA Patients' and Consumers' Working Party (PCWP) and Healthcare Professionals' Working Party (HCPWP) and can be consulted in the *good practice guidance*, but also in an *infosheet* developed by EMA for patients and healthcare professionals.

IN THIS ISSUE

In the spotlight: EURORDIS Open Academy

EURORDIS Open Academy

The objective of the *EURORDIS Open Academy* is to build the capacity of rare disease patient advocates at large, as well as a select number of researchers and clinicians, so that they can go on to advocate for rare diseases at both local or international levels. By providing training, EURORDIS empowers patients and ensures they have the confidence and knowledge needed to bring their expertise to discussions on health care, research and medicines development with policy makers, industry and scientists.

You can also **take part in the free online training** where online modules are available for anyone to use. For more information, please see *EURORDIS Open Academy website*.

EURORDIS Summer School

Since its launch in 2008, the *EURORDIS Summer School on Medicines Research & Development* has taken place every year in Barcelona; however exceptionally, these past years took place online due to COVID-19. This year took place from 6-10 June and we had 22 patient advocates and 4 researchers, representing 18 countries and over 26 different rare diseases!

EURORDIS Summer School is integrated within the *EURORDIS Open Academy*, and it is a programme targeted to patient advocates and researchers to be trained in different aspects of medicines development, from clinical trial methodology and EU regulatory process to the process of health technology assessment.

Congratulations to all the participants!

EURORDIS Winter School

The *EURORDIS Winter School on Scientific Innovation and Translational Research* aims of deepening patient representatives' understanding of how pre-clinical research translates into real benefits for rare disease patients. It equips participants with knowledge and skills, so they are empowered to effectively participate in discussions with the researchers, policymakers, and companies responsible for research or research infrastructures.
You can also learn more about rare disease research at a time and place that suits you by taking the free online modules which includes a lesson and quiz!

For more information, please visit EURORDIS website.

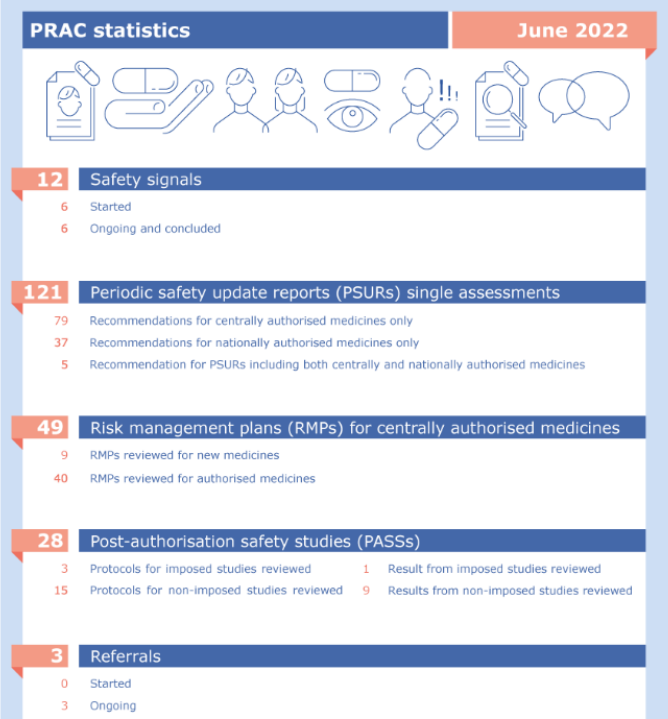
Pharmacovigilance Risk Assessment Committee (PRAC) June 2022

Minutes September 2021
Agenda June 2022
Meeting Highlights June 2022

Xalkori needs monitoring in paediatric patients due to visual loss

EMA's safety committee (PRAC) informs healthcare professionals of the risk of ocular toxicity, severe visual loss and the need for monitoring in paediatric patients with Xalkori.

Xalkori (crizotinib) is a cancer medicine used to treat adults with a type of lung cancer called non-small cell lung cancer (NSCLC), when the disease is advanced. Xalkori has been studied in children from 6 to 18 years of age as a monotherapy for the treatment of relapsed or refractory systemic anaplastic large cell lymphoma (ALCL) that is ALK positive or patients with unresectable, recurrent, or refractory ALK positive inflammatory myofibroblastic tumour (IMT). Vision disorders have been reported in 61% of paediatric patients treated with crizotinib in clinical trials for these indications. Paediatric patients should be monitored for ocular toxicity, including the risk of severe vision loss. They should receive a baseline ophthalmologic examination prior to starting Xalkori with follow-up examinations. Healthcare professionals are advised to inform patients and caregivers of the symptoms and remind them to contact their doctor if any of these symptoms develop. Any visual symptoms should be referred to an eye specialist.



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

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

2639 Orphan designations

257 Orphan designations included in authorised indication

224 Authorised OMPs

90 To be used in children

6 Removed from the market

74 Marketed, but no longer "orphans"

To date

144

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

21 July 2022

CHMP Meeting Highlights June 2022

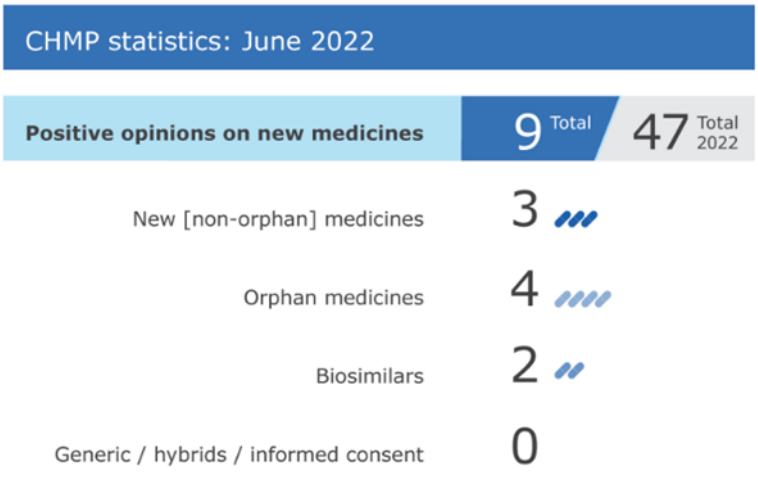
Minutes May 2022
Agenda June 2022
Meeting Highlights June 2022

In June, the CHMP recommended **9 medicines for approval, 4 orphan medicines**:

- *Pepaxti (melphalan flufenamide)* for the treatment of multiple myeloma, a rare cancer of the bone marrow that affects plasma cells, a type of white blood cell that produces antibodies.
- *Roctavian (valoctocogene roxaparvovec)* the first gene therapy to treat severe haemophilia A, a rare inherited bleeding disorder caused by lack of factor VIII.
- *Scemblix (asciminib)* for the treatment of adults with Philadelphia chromosome-positive chronic myeloid leukaemia in chronic phase (Ph+CML-CP), previously treated with two or more tyrosine kinase inhibitors. This is a new therapeutic option for patients with this type of rare blood cancer.
- *Vyvgart (efgartigimod alfa)* intended for the treatment of anti-acetylcholine receptor (AChR) antibody positive generalised myasthenia gravis.
- *Rayvow (lasmiditan)* intended for the treatment of migraine in adults.
- *Sunlenca (lenacapavir)* intended for the treatment of human immunodeficiency virus type 1 (HIV-1) infection in adults with multidrug-resistant HIV-1 infection.
- *Valneva for COVID-19 Vaccine* (inactivated, adjuvanted) for use in people from 18 to 50 years of age as primary vaccination. It is the sixth vaccine recommended in the European Union (EU) for protecting against COVID-19.

The CHMP also recommended **8 extensions of therapeutic indication,** and recommended granting marketing authorisations for **2 biosimilar medicines**.

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 June 2022 meeting update

Minutes May 2022
Agenda June 2022
Meeting June 2022

During the June plenary, the COMP adopted **28 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:

- Familial adenomatous polyposis, Diamond Pharma Services Ireland Limited;
- Fibrodysplasia ossificans progressive, Biocryst Ireland Limited;
- Glioma, Chimerix IRL Limited;
- Multiple system atrophy, Teva B.V.;
- Solid organ transplantation, Sangamo Therapeutics France S.A.S.;
- Small-cell lung cancer, Merck Europe B.V.;
- Pemphigus, Argenx;
- Pachyonychia congenita, Imagine Institut Des Maladies Génétiques Necker Enfants Malades;
- Invasive aspergillosis, Pfizer Europe MA EEIG;
- Invasive candidiasis, Pfizer Europe MA EEIG;
- Solid organ transplantation, Xenothera;
- Epidermolysis bullosa, Tampereen Korkeakoulusäätiö Sr;
- Autoimmune haemolytic anaemia, Genzyme Europe B.V.
- Brugada syndrome, Teofarma S.r.l.;
- Wolfram syndrome, Pietro Maffei;
- Ornithine transcarbamylase deficiency, Arcturus Therapeutics Europe B.V.;
- Diffuse large B-cell lymphoma, Regeneron Ireland Designated Activity Company;
- Follicular lymphoma, Regeneron Ireland Designated Activity Company;
- Glioma, Scendea (NL) B.V.;
- Autoimmune haemolytic anaemia, Incyte Biosciences Distribution B.V.;
- Pyridoxal 5'-phosphate homeostasis protein deficiency, Amsterdam UMC;
- Schwannoma, Premier Research Group S.L.;
- Maple syrup urine disease, Renantos Pharmavertriebsgesellschaft mbH;
- Malignant mesothelioma, EMA Regulatory Submissions Expediter Limited;
- Nasopharyngeal cancer, TMC Pharma (EU) Limited;
- Pyoderma gangrenosum, InflaRx GmbH;
- Non-traumatic spontaneous intracerebral haemorrhage, Op2Lysis;
- Myasthenia gravis, UCB Pharma.

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 five positive opinions at time of CHMP opinion:

- **Crysvita** (burosumab) for treatment of phosphaturic mesenchymal tumour, Kyowa Kirin Holdings B.V.
- **Kinpeygo** (budesonide) for treatment of primary IgA nephropathy, Calliditas Therapeutics AB.
- **Upstaza** (eladocagene exuparvovec) for treatment of aromatic L-amino acid decarboxylase deficiency, PTC Therapeutics International Limited.
- **Xenpozyme** (olipudase alfa) for treatment of Niemann-Pick disease, Genzyme Europe B.V.
- **Zokinvy** (lonafarnib) for treatment of Hutchinson-Gilford Progeria Syndrome, Eigerbio Europe Limited.

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
Lonapegsomatropin Ascendis Pharma® (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
Kinpeygo® (budesonide)	Calliditas Therapeutics AB	Adults with primary immunoglobulin A nephropathy (IgAN)	15/07/2022

Please click also on the following links to see:

Orphan medicinal products authorised during 2022

Orphan medicinal products authorised since 2000

PDCO April to June meeting to be updated when info available

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 May & June meeting to be updated when info available

Minutes May 2022
Agenda July 2022
Meeting Report Jan-April 2022

CAT highlights January – April meeting update

From January to April the Committee for Advanced Therapies (CAT) finalised **14 scientific recommendations on the classification of advanced therapy medicinal products** (ATMPs).

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

Advanced therapy medicinal products approvals from January-April 2022.

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

- *Breyanzi (lisocabtagene maraleucel)* for the treatment of adult with relapsed or refractory diffuse large B cell lymphoma (DLBCL), primary mediastinal large B-cell lymphoma (PMBCL) and follicular lymphoma grade 3B (FL3B), after at least two previous lines of treatments. Based on the assessment of the CAT, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product *Breyanzi*.

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

- *Carvykti (ciltacabtagene autoleucel)* for the treatment of adult patients with relapsed and refractory multiple myeloma, who have received at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody, and have demonstrated disease progression on the last therapy. Based on the assessment of the CAT, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product *Carvykti*.

Extension of indication of authorised ATMPs

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

- *Kymriah* to include the treatment of adult patients with follicular lymphoma after two or more lines of therapy who are refractory or released during or within 6 months after completion of anti-CD20 antibody maintenance or relapsed after autologous haematopoietic stem cell transplantation.

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

- *Yescarta* to include the treatment of adult patients with relapsed or refractory follicular lymphoma after three or more lines of systematic therapy.

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 1st and 2nd of June took place online *the first joint meeting of the new mandate (2022-2025) of the 'Patients and Consumers' (PCWP) and 'Healthcare Professionals' (HCPWP) Working Parties.*

The first day of the meeting began with an introduction of the members followed by a discussion on issues related with clinical trials and the ongoing review of the ICH E6(R3) Good Clinical Practice. The second day continued with an update on COVID-19, Big Data activities and also the use of communications by patients and healthcare professionals. The final session looked ahead to the new mandate with the adoption of the PCWP/HCPWP work plan 2022-2025 and a call for Co-chairs of the working parties.

For more information, please see the *agenda, the presentations and the recordings 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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