EURORDIS
RARE DISEASES EUROPE

EURORDIS THERAPEUTIC REPORT

December 2018

ISSUE 11

UPDATE ON THERAPEUTIC DEVELOPMENT AND PATIENT INVOLVEMENT IN EMA ACTIVITIES

GENERAL NEWS

EMA public consultation on Regulatory Science to 2025

The European Medicines Agency (EMA) has published a draft plan on the *Regulatory Science to 2025* for a six-month public consultation. This document incorporates the feedback gathered during the multi-stakeholder workshop that took place last October at the EMA. This workshop facilitated a shared reflection on the key regulatory science challenges and the opportunities to address them, and highlighting areas of particular relevance to various stakeholder groups. EURORDIS presented the patients' perspectives on regulatory science to 2025 and the primary challenges for the patients living with rare diseases in Europe. For further details, see the full workshop broadcasting at the *EMA website.*

This draft plan seeks to offer informed guidance on modern medicines development, facilitate the optimisation of regulatory science and critically assess the benefits and risks of innovative therapies and diagnostics based on new technologies. Therefore, five key goals are proposed:

- Catalysing the integration of science and technology in medicine development
- Driving collaborative evidence generation - improving the scientific quality of evaluations;
- Advancing patient-centred access to medicines in partnership with healthcare systems
- Addressing emerging health threats;
- Enabling and leveraging research and innovation in regulatory science.

This consultation encourages all the stakeholders to send their comments via an *online questionnaire* **by 30 June 2019**.

For more information, please see *EMA website*.

IN THIS ISSUE

EMA open consultation on Patient Disease Registries

The EMA has published a *discussion paper* methodological and operational considerations in the use of patient disease registries for regulatory purposes.

This consultation invites all the interested parties to send their comments and suggestions using the *Form for submission of comments* and send them back to *EMAregistries@ema.europa.eu* **by 30 June 2019.**

The final version of the document will be finished by the end of 2019.

For more information, please see *EMA website.*

Join the EURORDIS staff and paint your face to *#ShowYourRare* in support of the rare disease community for *#RareDiseaseDay2019*

RAREDISEASEDAY.ORG

In the Spotlight: The year in review

These are the **2018** achievements relevant for the **development and access of rare disease therapies and also for patient involvement in medicines development**.

EMA recommendations for authorisation of new medicines in 2018

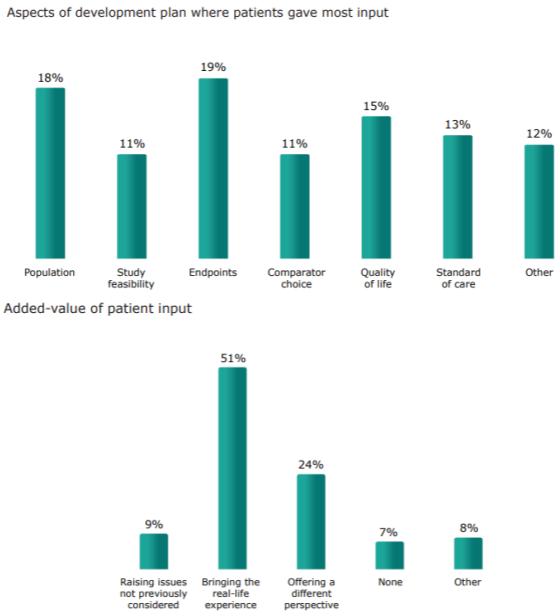
In 2018, EMA recommended **84** medicines for marketing authorisation. Of these, 42 had a new active substance which has never been authorised in the EU before. **21 had an orphan designation** at the time of CHMP opinion, with the potential to be of **significant benefit for rare diseases patients.**

For more information, please see the *Highlights 2018*

Patient involvement at EMA continues to grow

The *Stakeholder Engagement report 2017 report* on EMA's interaction with patients, consumers, healthcare professionals and their organisations published in 2018 showed that patient involvement in EMA activities has turned from incidental to systematic. **Scientific advice/protocol assistance** is an example of patient engagement in early dialogue in medicines development, and.is one of the areas showing most significant growth.

Patients most frequently provided input on issues such as study population, endpoints of the study and quality of life. In addition, scientific officers felt that patients brought the real life experience into the process as well as offering a different perspective.



PARADIGM project launched

The *PARADIGM* project kicked-off during its First Forum held in Brussels on 10 April in Brussels. PARADIGM, which stands for Patients Active in Research and Dialogues for an Improved Generation of Medicines, is a public-private partnership led by the European Patients' Forum (EPF) and the European Federation of Pharmaceutical Industries and Associations (EFPIA). PARADIGM **aims to provide a framework for structure, effective, meaningful, ethical and sustainable patient engagement** at three key decision-making points in the development of medicinal products, i.e. the research priority setting, the design of clinical trials and the early dialogues with regulators and HTA bodies.

Second EMA public hearing

This 2018 the patients' voice was heard at EMA's second public hearing on *quinolone and fluoroquinolone antibiotics,* carried out at the June PRAC meeting.

The hearing was broadcast live and the *recording is available* on the *EMA website*

Pharmacovigilance Risk Assessment Committee (PRAC) December 2018

Minutes October 2018
Agenda November 2018
Meeting Highlights Nov 2018

List of medicines under additional monitoring

All medicines after being placed on the European Union (EU) market are carefully monitored. Some of them are monitored even more intensively by regulatory authorities, these are the medicines under additional monitoring which have a black inverted triangle (▼) displayed in their package leaflet and summary of product characteristics, together with a short sentence explaining what the triangle means.

For more information, please see the up to date *list of medicines under additional monitoring.*

New EMA Guideline for haemophilia clinical trials issued

Following a public consultation and two workshops on haemophilia registries, the EMA has published revised guidelines aimed to define the requirements for practical implementation using existing registries and to provide parameters for core data sets that should be collected to support post-authorisation observational studies of haemophilia medicines.

The revision introduces an important change in relation to the investigation of recombinant and human plasma-derived factor VIII and factor IX haemophilia medicines in previously untreated patients: for this very small subset of haemophilia patients' data should be collected from patient registries rather than from small clinical trials, that may not be fully representative of how the medicine is used day-to-day once it's on the market. This relies on data from registries as a source of high-quality, real-world data to support regulatory decision-making.

Recommendations such as appropriate governance of registries, patient consent, data collection, data quality and data sharing, and interoperability between different registries were also discussed during the workshops.

For more information, see the *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 MEDICINAL PRODUCTS AUTHORISED IN 2018

Medicinal Product	Marketing Authorisation Holder	Therapeutic Indication	Date of Marketing Authorisation
Jorveza® (budesonide)	Dr. Falk Pharma GmbH	Eosinophilic oesophagitis	08/01/2018
Premyvis® (letermovir)	Merck Sharp & Dohme Limited	Cytomegalovirus infection prevention following haematopoietic stem cell transplant	08/01/2018
Crysvita® (burosumab)	Kyowa Kirin Limited	X-linked hypophosphataemia	19/02/2018
Lamzede® (velmanase alfa)	Chiesi Farmaceutici S.p.A.	Alpha-mannosidosis	23/03/2018
Alofisel® (darvadstrocel)	Tigenix, S.A.U.	Complex anal fistulas in adults with Crohn's disease	23/03/2018
Mylotarg® (gemtuzumab ozogamicin)	Pfizer Limited	Acute myeloid leukemia	19/04/2018
Amglidia® (glibenclamide)	Ammtrek	Neonatal diabetes	24/05/2018
Rubraca® (rucaparib)	Clovis Oncology UK Limited	High-grade cancers of the ovary, fallopian tubes and peritoneum	24/05/2018
Verkazia® (ciclosporin)	Santen Oy	Keratoconjunctivitis (VKC) in children and adolescents from 4 to 18 years of age	06/07/2018
Tegsedi® (inotersen sodium)	Ionis USA Ltd	Hereditary transthyretin amyloidosis (hATTR)	06/07/2018
Myalepta® (metreleptin)	Aegerion Pharmaceuticals B.V.	Lipodystrophy	30/07/2018
Vyxeos® (daunorubicin / cytarabine)	Jazz Pharmaceuticals Ireland Limited	Adults with newly diagnosed acute myeloid leukaemia	22/08/2018
Kymriah® (tisagenlecleucel)	Novartis Europharm Limited	B-cell acute lymphoblastic leukaemia (ALL), and diffuse large B-cell lymphoma (DLBCL)	22/08/2018
Mepsevii® (vestronidase alfametreleptin)	Ultragenyx Germany GmbH	Mucopolysaccharidosis type VII (MPS VII, also known as Sly syndrome)	23/08/2018
Yescarta® (axicabtagene ciloleucel)	Kite Pharma EU B.V.	Diffuse large B-cell lymphoma (DLBCL) and primary mediastinal large B-cell lymphoma (PMBCL)	23/08/2018
Onpattro® (patisiran)	Alnylam Netherlands B.V.	Hereditary transthyretin-mediated amyloidosis (hATTR)	26/08/2018
Cablivic® (caplacizumab)	Ablynx NV	Acquired thrombotic thrombocytopenic purpura (aTTP)	31/08/2018
Symkevi® (tezacaftor/ivacaftor)	Vertex Pharmaceuticals (Europe) Ltd.	Cystic fibrosis in patients aged 12 years and above	31/10/2018
Luxturna® (voretigene neparvovec)	Spark Therapeutics Ireland Ltd	Inherited retinal dystrophy	22/11/2018

CHMP Meeting Highlights December 2018

Minutes November 2018
Agenda December 2018
Meeting Highlights Dec 2018

In December, the CHMP recommended **7 medicines for approval,** including 2 orphan medicines:

- *Besremi* (ropeginterferon alfa-2b), for the treatment of polycythaemia vera without symptomatic splenomegaly
- *Trecondi* (treosulfan), for the conditioning treatment prior to allogeneic haematopoietic stem cell transplantation

Important: In the case of orphan medicines, please note that after the positive opinion on marketing authorisation has been issued by the CHMP, **the COMP reviews whether the medicinal product still meets the criteria of orphan designation**. If the orphan designation is maintained, the medicine benefits from the incentive of 10 years of market exclusivity.

The CHMP also recommended granting marketing authorisations for the following medicines:

- *Lusutrombopag Shionogi* (lusutrombopag), for the treatment of severe thrombocytopenia in adults with chronic liver disease undergoing invasive procedures.
- *Rizmoic* (naldemedine) for the treatment of opioid-induced constipation.

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

Recommendation on PRIME eligibility

In December, the CHMP reviewed 4 applications for eligibility to PRIME, and 2 were granted. Please see the EMA website and CHMP page for more information on the *December's recommendation on PRIME eligibility.*

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 December 2018 meeting update

Minutes September 2018
Agenda December 2018
Meeting Report Dec 2018

During the December plenary, the COMP adopted **11 positive opinions** on the designation of medicines as orphan medicinal products to the European Commission (EC). For further information, please see the *meeting report.* For further information on the work of the COMP see the *2018 work plan*

Please find below the list of indications covered in the medicines that were recommended for orphan designation:

- Ataxia telangiectasia
- Sickle cell disease
- Short bowel syndrome
- Glycogen storage disease type II (Pompe's disease)
- Immune thrombocytopenia
- Betathalassaemia intermedia and major
- Rett syndrome
- Perinatal asphyxia
- Pulmonary arterial hypertension
- Soft tissue sarcoma

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

Overview of the orphan designation process since 2000

Year	Applications submitted	Positive COMP opinions	Applications withdrawn	Negative COMP opinions	Designations granted by the Commission	Orphan medicinal products authorised	Orphan designations included in authorised therapeutic indication
2018	203	152	79	3	126	12	15
2017	260	144	100	2	147	14	15
2016	330	220	82	2	209	14	14
2015	258	177	94	1	190	14	21
2014	329	196	61	2	187	15	16
2013	201	136	60	1	136	7	8
2012	197	139	52	1	148	10	12
2011	166	111	45	2	107	5	5
2010	174	123	51	2	128	4	4
2009	164	113	23	0	106	9	9
2008	119	86	31	1	73	6	7
2007	125	97	19	1	98	13	13
2006	104	81	20	2	80	9	11
2005	118	88	30	0	88	4	4
2004	108	75	22	4	73	6	6
2003	87	54	41	1	55	5	5
2002	80	43	30	2	49	4	4
2001	83	62	27	1	64	3	3
2000	72	26	6	0	14	0	0
Total	**3209**	**2134**	**879**	**28**	**2100**	**163**	**183**

Adapted from the December 2018 Committee for Orphan Medicinal Products (COMP) meeting report

Minutes September 2018
Agenda December 2018
Meeting Report December 2018

PDCO December 2018 meeting update

In December, the PDCO adopted **6 positive opinions** agreeing *paediatric investigation plans (PIPs)* for the medicine 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.

- *Filgotinib*, from Gilead Sciences International Ltd., for the treatment of Crohn's disease and treatment of ulcerative colitis
- *Rilpivirine,* from Janssen-Cilag International N.V., for the treatment of human immunodeficiency virus (HIV-1) infection
- *Ozanimod,* from Celgene Europe Limited, for the treatment of Crohn's disease
- *Etripamil,* from Milestone Pharmaceuticals Inc, for the treatment of supraventricular arrhythmia
- *Ianalumab,* from Novartis Europharm Limited, for the treatment of autoimmune hepatitis
- *Split influenza virus,* inactivated containing antigens equivalent to the A/H1N1-like strain, A/H3N2-like strain, B-like strain (Victoria lineage) and B-like strain (Yamagata lineage), from Sanofi Pasteur, for prevention of influenza infection

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*

EMA gives guidance on safety monitoring of medicines used in children

EMA has published a new *good pharmacovigilance practice (GVP) chapter IV* on specific considerations for the paediatric population. It provides guidance on **how to make best use of existing tools and processes to address the specific needs and challenges of safety monitoring of medicines used in children**. In addition, it advises on how to adapt regulatory requirements to the paediatric population in the European Union.

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

CAT December 2018 meeting to be updated in January issue

Minutes November 2018
Agenda Dec 2018
Meeting Report Nov 2018

CAT November 2018 meeting update

In November, the Committee for Advanced Therapies (CAT) finalised **5 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 products:**

- Codon-optimised human cystic fibrosis transmembrane conductance regulator mRNA complexed with lipid based nanoparticles, intended for the **treatment of cystic fibrosis**
- Adeno-associated virus vector containing a human neuronal ceroid lipofuscinosis expression cassette encoding for the soluble lysosomal enzyme tripeptidyl-peptidase 1, intended for the **treatment of late-infantile neuronal ceroid lipofuscinosis type 2 disease**

The following product was classified as a **somatic cell therapy medicinal product**:

- Allogeneic Epstein-Barr virus (EBV)-specific cytotoxic T cells, intended for the **treatment of refractory / relapsed EBV-associated post-transplant lymphoproliferative disease**

The following products were classified as **tissue engineered products**:

- Autologous bone marrow derived mesenchymal stem cells, intended for the **treatment of ischemic stroke**
- Autologous bone marrow derived mesenchymal stem cells, intended for the **regeneration of cartilage, ligamentum, bone and muscle defects**

Guideline on requirements for investigational ATMPs

This October, the CAT Committee discussed the Guideline on requirements for investigational ATMPs. This guideline is expected to be released for **public consultation by end of 2018.** For more information, *see the EMA website.*

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 2018-2019 Work Plan

At the last *Plenary with all eligible organisations* held on 22 November 2017, the draft joint work programme for PCWP and HCPWP for 2018 and 2019 was presented. This draft was sent for consultation during December to the drafting group and in it in the final review process by both working. For more information please see the *meeting summary* and the *draft joint work programme 2018-2019 presentation*

PCWP 2018 meetings

The first *PCWP – HCPWP joint meeting* of the year was held at the EMA on 17-18 April 2018. See *agenda* and *summary report.* All presentations are available in the *PCWP-HCPWP joint meeting event page*.

The second *PCWP – HCPWP joint meeting* of the year was held at the EMA on 25 September 2018. They discussed topics such as the results of the 2017 EMA perception survey, also the agency regulatory science initiative to 2025 and an update on Good Pharmacovigilance Practices (GVP). A feedback on the ongoing work on electronic product information and on availability of authorised medicines was given to the working parties' members.

EMA is also **supporting public health campaigns** with the aim to engage more with patients and HCPs. During this meeting a *case study* was presented where EMA has shared EURORDIS #RareDiseaseDay campaign.

For more information, see the *agenda.*

All presentations are available in the *PCWP-HCPWP joint meeting event page*.

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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