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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

HUMAN MEDICINES HIGHLIGHTS

Key information for patients, consumers and healthcare professionals
Published monthly by the European Medicines Agency

An agency of the European Union

IN THIS ISSUE

COVID-19 vaccines and treatments	1
Antivirals/anti-infectives	2
Cancer	2
Cardiovascular system	3
Dermatology	3
Gastro-intestinal system	3
Gynaecology & Obstetrics	3
Haematology	3
Hepatology	4
Immune system	4
Metabolic disorders	4
Musculoskeletal system	4
Nephrology	5
Nervous system	5
Other medicines	5
Medicines under additional monitoring	6
Guidelines	6
Scientific committee and working party activities	6
Other publications	7
Events	7
Explanation of terms used	9

This newsletter is addressed primarily to organisations representing patients, consumers and healthcare professionals. It provides a summary of key information relating to medicines for human use published during the previous month by the European Medicines Agency.

Information is selected based on recommendations from consulted patients, consumers and healthcare professionals, and does not necessarily cover all relevant information published by the Agency.

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Information on medicines

COVID-19 vaccines and treatments

Negative CHMP opinions on new medicines

- Lagevrio (*molnupiravir*)
  Intended for the treatment of COVID-19 in adults

Direct Healthcare Professional Communication (DHPC)

- Spikevax bivalent Original/Omicron BA.1 (*elasomeran; imelasomeran and elasomeran; davesomeran and elasomeran; COVID-19 mRNA vaccine (nucleoside-modified)*)
  Prevention of COVID-19 virus infection

Key to symbols used

O Orphan medicine | Generic medicine | Biosimilar medicine | C Conditional approval | E Exceptional circumstances

Antivirals/anti-infectives

Supply shortages

- Amoxicillin and amoxicillin/clavulanic acid (*amoxicillin*)
  Treatment of wide range of bacterial infections

Cancer

Positive CHMP opinions on new medicines

- Akeega (*niraparib / abiraterone acetate*)
  Treatment of prostate cancer
- Hyftor (*sirolimus*) O
  Treatment of angiofibroma, a type of non-cancerous tumour that occurs in the nasal cavity
- Tibsovo (*ivosidenib*) O
  Treatment of myeloid leukaemia, a type of blood cancer
- Tidhesco (*ivosidenib*) O
  Treatment of myeloid leukaemia, a type of blood cancer

New medicines authorised

- Plerixafor Accord (*plerixafor*) generic of Mozobil
  Medicine used in cancer patients to obtain cells from the bone marrow for use in transplantation

New information on authorised medicines

- Libtayo (*cemiplimab*) - new indication C
  Treatment of non-small cell lung cancer

Withdrawal of applications for new medicines

- Aliqopa (*copanlisib*)
  Intended for treatment of a type of white blood cells cancer

Supply shortages

- Fasturtec (*rasburicase*)
  Prevention of high levels of uric acid in cancer patients order to prevent kidney failure

Direct Healthcare Professional Communication (DHPC)

- Caprelsa (*vandetanib*)
  Treatment of thyroid cancer
- Neofordex (*dexamethasone*)
  Treatment of multiple myeloma (cancer of the bone marrow)
- Xalkori (*crizotinib*)
  Treatment of non-small cell lung cancer

Cardiovascular system

Safety update

- Review of pseudoephedrine-containing medicines (*pseudoephedrine*) - review started
  Works by stimulating nerve endings to release the chemical noradrenaline, which causes the blood vessels to constrict (narrow)

Dermatology (skin conditions)

Positive CHMP opinions on new medicines

- Opzelura (*ruxolitinib*)
  Treatment of vitiligo, a chronic skin condition in which skin loses its pigment

New medicines authorised

- Spevigo (*spesolimab*) C
  Treatment of psoriasis (inflammatory condition of the skin)

Gastro-intestinal system

New information on authorised medicines

- Rinvoq (*upadacitinib*) - new indication
  Treatment of Crohn's disease, a small intestine inflammatory disease

Gynaecology & Obstetrics (pregnancy and female reproductive)

Supply shortages

- Cetrotide (*cetrorelix acetate*)
  Prevention of premature ovulation

Haematology (blood conditions)

Positive CHMP opinions on new medicines

- Bekemv (*eculizumab*)
  Treatment of paroxysmal nocturnal haemoglobinuria, (rare condition in which there is excessive breakdown of red blood cells and hemoglobin (red pigment) in the urine.)
- Tibsovo (*ivosidenib*) O
  Treatment of myeloid leukaemia, a type of blood cancer
- Tidhesco (*ivosidenib*) O
  Treatment of myeloid leukaemia, a type of blood cancer
- Vafseo (*vadadustat*)
  Treatment of anaemia associated with chronic kidney disease

New medicines authorised

- Plerixafor Accord (*plerixafor*) generic of Mozobil
  Medicine used in cancer patients to obtain cells from the bone marrow for use in transplantation

Direct Healthcare Professional Communication (DHPC)

- Adakveo (*crizanlizumab*)
  Treatment of anaemia
- Neofordex (*dexamethasone*)
  Treatment of multiple myeloma (cancer of the bone marrow)

Hepatology

Direct Healthcare Professional Communication (DHPC)

- Terlipressin-containing medicines (*terlipressin*)
  Treatment of hepatorenal syndrome (serious kidney problems in people with advanced liver disease)

Immune system

New medicines authorised

- Spevigo (*spesolimab*) C
  Treatment of psoriasis (inflammatory condition of the skin)

Withdrawal of applications for extension of indication

- Ilaris (*canakinumab*)
  Treatment of Schnitzler syndrome (a rare long-term inflammatory disease causing urticaria (hives), recurrent fever, bone and joint pain, and swollen lymph nodes)

Metabolic disorders

Positive CHMP opinions on new medicines

- Elfabrio (*pegunigalsidase alfa*) O
  Treatment of Fabry disease, a rare condition that affects the metabolism of fats in the body

Musculoskeletal system

Negative CHMP opinions on new medicines

- Sohonos (*palovarotene*)
  Intended for treatment of Fibrodysplasia ossificans progressiva (FOP), a disorder in which bone forms in other tissues

Nephrology (kidney conditions)

Positive CHMP opinions on new medicines

- Vafseo (*vadadustat*)
  Treatment of anaemia associated with chronic kidney disease

Supply shortages

- Fasturtec (*rasburicase*)
  Prevention of high levels of uric acid in cancer patients order to prevent kidney failure

Direct Healthcare Professional Communication (DHPC)

- Cystagon (*mercaptamine bitartrate*)
  Treatment of kidney cystinosis (rare disease in which excess amounts of cystine, an amino acid, build up within cells of kidneys)
- Terlipressin-containing medicines (*terlipressin*)
  Treatment of hepatorenal syndrome (serious kidney problems in people with advanced liver disease)

Nervous system

New medicines authorised

- Dimethyl fumarate Accord (*dimethyl fumarate*) generic of Tecfidera
  Treatment of multiple sclerosis

New information on authorised medicines

- Esbriet (*pirfenidone*) - change of existing indication
  Treatment of idiopathic pulmonary fibrosis (a disease in which scar tissue forms in the lungs)

Safety update

- Review of Topiramate (*topiramate*) - review started
  Prevention of epileptic seizures and migraines
- Review of pseudoephedrine-containing medicines (*pseudoephedrine*) - review started
  Stimulating of nerve endings to release the chemical noradrenaline, which causes the blood vessels to constrict (narrow)

Direct Healthcare Professional Communication (DHPC)

- Zolgensma (*onasemnogene abeparvovec*)
  Treatment of spinal muscular atrophy, a condition of the nerves that causes muscle wasting and weakness

Other medicines

New information on authorised medicines

- TachoSil (*human fibrinogen / human thrombin*) - extension of indication
  Supportive treatment in surgery as a sealant

Withdrawal of applications for extension of indication

- Buvidal (*buprenorphine*)
  Treatment of opioids dependency

Safety update

- Review of pseudoephedrine-containing medicines (*pseudoephedrine*) - review started
  Works by stimulating nerve endings to release the chemical noradrenaline, which causes the blood vessels to constrict (narrow)

Direct Healthcare Professional Communication (DHPC)

- Amfepramone-containing medicinal products (*amfepramone*)
  Treatment of obesity

Medicines under additional monitoring

- Updated list of medicines under additional monitoring

Other information

Guidelines

Guidelines open for consultation

- ICH Guideline M13A on bioequivalence for immediate-release solid oral dosage forms
  Deadline for comments: 26 May 2023

Adopted guidelines

- Guideline on clinical evaluation of vaccines
- Priority Action 3 concept paper: an EU multi-stakeholder platform for improving clinical trials
- ICH guideline Q9 (R1) on quality risk management
- Paediatric Addendum on the guidelines on clinical investigation of medicinal products for the treatment and prophylaxis of venous thromboembolic disease
- Adjuvants in vaccines for human use

Scientific committee and working party activities

- Medicinal products for human use: monthly figures - January 2023
- CAT - agendas, minutes and reports
- CHMP - agendas, minutes and highlights

- CHMP - applications for new human medicines: February 2023
- COMP - agendas, minutes and meetings reports
- HMPC - agendas, minutes and meetings reports
- PDCO - agendas, minutes and meeting reports
- PRAC - agendas, minutes and highlights
- PRAC recommendations on safety signals

Other publications

- Crisis preparedness and management
- Public consultation on a multi-stakeholder platform to improve clinical trials in the EU
- Actions to support the development of medicines for children
- EMA update on shortages of antibiotics in the EU
- Updated EMA emerging health threats plan
- Note on European Medicines Agency's involvement in HORIZON-HLTH-2023-TOOL-05-09: Developing a Data Quality and Utility Label for the European Health Data Space
- Questions and answers – Clinical Trials Information System (CTIS) and Clinical Trials Regulation (CTR)
- Multilingualism on the EMA website and in external communications
- Considerations for research / project teams seeking competent authority participation in externally funded regulatory science and public health research projects related to medicinal products
- Mandate, objectives and rules of procedure for the Oncology European Specialised Expert Communities (ESEC)

Events

- Quarterly system demo - Q1 2023 - 22 March 2023
- European Medicines Agency (EMA) Patients' and Consumers' (PCWP) and Healthcare Professionals' (HCPWP) Working Parties joint meeting - 3 March 2023
- HMA/EMA multi-stakeholder workshop on shortages - 1 - 2 March 2023
- Clinical Trials Information System (CTIS) bitesize talk: Document and personal data in CTIS - 23 February 2023
- Meeting of the Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG) - 23 February 2023
- Product Management Service (PMS) Webinar on Data Migration - 23 February 2023
- EMA regular press briefing on public health emergencies - 15 February 2023
- DARWIN EU Advisory Board meeting - 6 February 2023
- EMA virtual technical media briefing on the RNA technology - 3 February 2023
- Regulatory and scientific virtual conference on RNA-based medicines - 2 February 2023 - Agenda

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- EIC / EMA Info Day: Regulatory support for the development of innovative medicines and technologies - 31 January 2023
- European Union (EU) International Organisation for Standardization (ISO) for identification of medical products (IDMP)/ Substance, Product, Organisation and Referential (SPOR) data Task Force meeting - January 2023 - 26 January 2023
- Information session on the pilot for expert panels' scientific advice to manufacturers of high-risk medical devices - 25 January 2023 - Agenda
- Meeting of the Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG) - 11 January 2023 - Minutes
- EMA/HMA Big Data Stakeholder Forum 2022 - 1 December 2022 - Report
- Management Board meeting - 14-15 December 2022
- Meeting of the Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG) - 26 January 2023

Key to symbols used

O Orphan medicine | Generic medicine | Biosimilar medicine | C Conditional approval | E Exceptional circumstances

Explanation of terms used

Orphan medicine

A medicine intended for the treatment of a rare, serious disease. These medicines are granted orphan status during their development; at time of approval, orphan designations are reviewed to determine whether the information available to date allows maintaining the medicine's orphan status.

Generic medicine

A medicine that is essentially the same as one that has already been authorised for use.
(The latter is known as the 'reference medicine')

Biosimilar medicine

A biological medicine that is similar to another biological medicine which has already been authorised for use.
(Biosimilar medicines are also known as 'similar biological' medicines)

Conditional approval

A medicine that fulfils an unmet medical need may, if its immediate availability is in the interest of public health, be granted a conditional marketing authorisation on the basis of less complete clinical data than are normally required, subject to specific obligations being imposed on the authorisation holder to generate complete data on the medicine.

Exceptional circumstances

A medicine may be approved in some cases where the applicant cannot provide comprehensive data on the safety or efficacy of the medicine under normal conditions of use, due to exceptional circumstances such as ethical issues or the rarity of the disease concerned. These medicines are subject to specific post-authorisation obligations and monitoring.

Medicines assessed under Article 58

Article 58 of Regulation (EC) No 726/2004 allows the CHMP to give opinions, in co-operation with the World Health Organization, on medicinal products that are intended exclusively for markets outside of the European Union.

Note on the centralised authorisation procedure

To obtain a single marketing authorisation (licence) for a medicine that is valid in all Member States of the European Union (EU) – via a process known as the 'centralised procedure' – the company or person developing the medicine must submit an application to the European Medicines Agency.

The Agency's Committee for Medicinal Products for Human Use (CHMP) carries out a scientific evaluation of the information contained in the application and prepares an opinion (scientific recommendation). The Agency transmits this (positive or negative) opinion to the European Commission, which then issues a Decision granting or refusing the marketing authorisation.

When the CHMP adopts a positive opinion on a medicine, the Agency publishes on its website a 'summary of opinion', in the first instance, followed by more detailed information in a 'European public assessment report (EPAR)' after the marketing authorisation has been granted.

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http://www.ema.europa.eu

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European Medicines Agency

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