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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

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

Antivirals/anti-infectives

Safety update

- Review of fluoroquinolones antibiotics (*fluoroquinolone*) - review started
  Reminder of measures to reduce the risk of long-lasting, disabling and potentially irreversible side effects'

Cancer

Positive CHMP opinions on new medicines

- Pylclari (*piflufolastat (18F)*)
  Intended for the diagnosis of prostate cancer

Key to symbols used

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

New medicines authorised

- Tibsovo (*ivosidenib*)
  Treatment of acute myeloid leukaemia (a blood cancer) and biliary tract cancer (cancer of the bile ducts)

New information on authorised medicines

- Opdivo (*nivolumab*) - new indication
  Treatment of different types of cancer

Supply shortages

- Abraxane (*paclitaxel*)
  Treatment of different types of cancer

Cardiovascular system

New medicines authorised

- Dabigatran Etexilate Accord (*dabigatran etexilate*) generic of Pradaxa
  Treatment and prevention of blood clots

Supply shortages

- Actilyse (*alteplase*)
  Treatment of acute pulmonary embolism (blood clot in blood vessels supplying the lung)
- Metalyse (*tenecteplase*)
  Treatment of acute myocardial infarction (heart attack)

Gastro-intestinal system

New medicines authorised

- Tibsovo (*ivosidenib*)
  Treatment of acute myeloid leukaemia (a blood cancer) and biliary tract cancer (cancer of the bile ducts)

Gynaecology & Obstetrics (pregnancy and female reproductive)

Safety update

- Review of hydroxyprogesterone-containing medicinal products (*hydroxyprogesterone*) - review started
  Prevention of pregnancy loss or premature birth in pregnant women and treatment of various gynaecological disorders, including disorders caused by a lack of a hormone called progesterone

Haematology (blood conditions)

New medicines authorised

- Epysqli (*Eculizumab*)
  Treatment of paroxysmal nocturnal haemoglobinuria, a disease in which excessive breakdown of red blood cells results in various medical complications
- Tibsovo (*ivosidenib*)
  Treatment of acute myeloid leukaemia (a blood cancer) and biliary tract cancer (cancer of the bile ducts)
- Vafseo (*vadadustat*)
  Treatment of anaemia (low levels of red blood cells) associated with chronic kidney disease

Supply shortages

- Actilyse (*alteplase*)
  Treatment of acute pulmonary embolism (blood clot in blood vessels supplying the lung)

Safety update

- Review of Adakveo (crizanlizumab) - CHMP Opinion
  Prevention of painful crises in patients with sickle cell disease

Hormone system

New information on authorised medicines

- Sogroya (*somapacitan*) - extension of indication
  Treatment of growth hormone deficiency (lack of growth hormone)

Metabolic disorders

New medicines authorised

- Elfabrio (*pegunigalsidase alfa*)
  Treatment of Fabry disease (a rare inherited metabolic disorder)
- Pombiliti (*cipaglucosidase alfa*)
  Treatment of glycogen storage disease (a disease that causes the build up of glycogen, a complex sugar, in organs and muscles)

Musculoskeletal system

Negative CHMP opinions on new medicines

- Sohonos (*palovarotene*) - re-examination
  Intended for the treatment of fibrodysplasia ossificans progressive, a rare genetic disease that causes extra bone to form in places outside the skeleton (a process called heterotopic ossification) such as in joints, muscles, tendons and ligaments, leading to progressively decreasing mobility and other severe impairments

Nephrology (kidney conditions)

New medicines authorised

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

Nervous system

Positive CHMP opinions on new medicines

- Ztalmy (*ganaxolone*) O
  Treatment of epileptic seizures

Ophthalmology (eye conditions)

Withdrawal of applications for new medicines

- Susvimo (*ranibizumab*)
  Intended for treatment of the 'wet' form of age-related macular degeneration, a disease that affects the central part of the retina (called the macula) at the back of the eye and causes gradual loss of vision

Respiratory system

Safety update

- Review of pseudoephedrine-containing medicinal products (*pseudoephedrine*) - review started
  Treatment of nasal congestion

Vaccines

Supply shortages

- Ixiaro (*Japanese encephalitis vaccine (inactivated, adsorbed)*)
  Prevention against Japanese encephalitis, a disease that causes inflammation of the brain

Other medicines

Withdrawal of applications for new medicines

- Asimtufii (*aripiprazole*)
  Intended to treat schizophrenia

Medicines under additional monitoring

- Updated list of medicines under additional monitoring

Other information

Guidelines

Guidelines open for consultation

- Concept paper on revision of the Guideline on clinical investigation of medicinal products in the treatment of patients with acute respiratory distress syndrome
  Deadline for comments: 31 July 2023
- Draft ICH E6 (R3) Guideline on good clinical practice (GCP) - Step 2b
  Deadline for comments: 26 September 2023

Scientific committee and working party activities

- Medicinal products for human use: monthly figures - April 2023
- CAT - agendas, minutes and reports
- CHMP - agendas, minutes and highlights
- CHMP - applications for new human medicines: May 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 statistics: May 2023
- PRAC recommendations on safety signals
- PCWP
- HCPWP

Other publications

- Statement from Emer Cooke on the end of the COVID-19 public health emergency
- EMA and European medicines regulatory network lift COVID-19 business continuity status
- Global regulators agree on way forward to adapt COVID-19 vaccines to emerging variants
- Guidance for industry to prevent and mitigate medicine shortages
- Report - Moving together towards better prevention of medicine shortages in the EU

Events

- ACT EU multi-stakeholder platform kick-off workshop - 22-23 June 2023

Key to symbols used

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- 10th anniversary of European Medicines Agency (EMA) Healthcare Professionals' (HCPWP) Working Party meeting - 27 June 2023 - Agenda
- European Medicines Agency (EMA) Patients' and Consumers' (PCWP) Working Party meeting - 27 June 2023 - Agenda
- European Medicines Agency (EMA) Patients' and Consumers' (PCWP) and Healthcare Professionals' (HCPWP) Working Parties joint meeting - 28 June 2023 - Agenda
- ACT EU PA04 - Multi-stakeholder Workshop on ICH E6 R3 - Public Consultation - 13 July - Agenda
- EMA regular press briefing on public health emergencies - 6 June 2023
- Clinical Data Publication (Policy 0070) re-launch - EMA webinar - 16 May 2023 - Presentations and recording

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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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European Medicines Agency

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