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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

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

Withdrawal of applications for extension of indication

- Olumiant (*baricitinib*)
  Intended for treatment of patients hospitalized with Covid-19

Cancer

Positive CHMP opinions on new medicines

- Imjudo (*tremelimumab*)
  Treatment of liver cancer
- Tremelimumab AstraZeneca (*tremelimumab*)
  Treatment of non-small cell lung cancer

Key to symbols used

Orphan medicine | Generic medicine | Biosimilar medicine | Conditional approval | Exceptional circumstances

New medicines authorised

- Locametz (*gozetotide*)
  Treatment of prostate cancer
- Padcev (*enfortumab vedotin*)
  Treatment of bladder and urinary tract cancer
- Pluvicto (*lutetium (177Lu) vipivotide tetraxetan*)
  Treatment of prostate cancer

New information on authorised medicines

- Enhertu (*trastuzumab deruxtecan*) - new indication
  Treatment of breast cancer
- Imfinzi (*durvalumab*) - new indication
  Treatment of liver cancer

Negative CHMP opinions on new medicines

- Omblastys (*iodine (131I) omburtamab*) O
  Intended for treatment of a rare type of cancer which forms from immature nerve cells

Cardiovascular system

New information on authorised medicines

- Adcirca (previously Tadalafil Lilly) (*tadalafil*) - new indication
  Treatment of pulmonary arterial hypertension (high blood pressure in lungs)
- Edistride (*dapagliflozin*) - extension of indication
  Treatment of symptomatic chronic heart failure
- Forxiga (*dapagliflozin*) - extension of indication
  Treatment of symptomatic chronic heart failure

Arbitration procedures

- Rambis and associated names (*ramipril, bisoprolol fumarate*) - outcome
  Treatment of certain long-term heart conditions and high blood pressure

Haematology (blood conditions)

Positive CHMP opinions on new medicines

- Hemgenix (*etranacogene dezaparvovec*) O C
  Treatment of inherited bleeding disorder

New medicines authorised

- Pyrukynd (*mitapivat*) O
  Treatment of a disease that causes red blood cells to break down faster than normal

New information on authorised medicines

- Hemlibra (*emicizumab*) - extension of indication
  Prevention of bleeding in patients with haemophilia A (a blood clotting disorder)

Hepatology (liver conditions)

New information on authorised medicines

- Imfinzi (*durvalumab*) - new indication
  Treatment of liver cancer

Positive CHMP opinions on new medicines

- Imjudo (*tremelimumab*)
  Treatment of liver cancer

Direct Healthcare Professional Communication (DHPC)

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

HIV

New information on authorised medicines

- Triumeq (*abacavir sulfate / dolutegravir sodium / lamivudine*) - new pharmaceutical form
  Treatment of HIV in children weighing between 14 to 25kg

Hormone system

New medicines authorised

- Mycapssa (octreotide) O
  Treatment of acromegaly (excess growth of body tissues due to excessive growth hormone)

Immune system

New information on authorised medicines

- Dupixent (*dupilumab*) - new indication
  Treatment of an inflammatory condition affecting the oesphagus, or food pipe

Metabolic disorders

Positive CHMP opinions on new medicines

- Livmarli (*Maralixibat chloride*) O
  Treatment of intense itching due to a build-up of bile in patients aged 2 months and older
- Pombiliti (*cipaglucosidase alfa*)
  Treatment of glycogen storage disease (a disease that causes the build up of glycogen, a complex sugar, in organs and muscles)

Nephrology (kidney conditions)

New information on authorised medicines

- Kerendia (*finerenone*) - extension of indication
  Treatment of chronic kidney disease

Withdrawal of applications for new medicines

- Imbarkyd (*bardoxolone methyl*)
  Treatment of chronic kidney disease caused by an inherited disease called Alport syndrome

Direct Healthcare Professional Communication (DHPC)

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

Nervous system

Positive CHMP opinions on new medicines

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

New information on authorised medicines

- Fintepla (*fenfluramine*) - new indication
  Treatment of seizures associated with Lennox-Gastaut syndrome (a severe form of epilepsy that starts in childhood)

Ophthalmology (eye conditions)

Positive CHMP opinions on new medicines

- Gelisia and associated names (*timolol maleate*)
  Treatment of high pressure inside the eye

Respiratory system

Safety update

- Review of Pholcodine-containing medicinal products (*pholcodine*) - PRAC recommendation
  Treatment of dry cough and (in combination with other active substances for treatment of) symptoms of cold and flu

Rheumatology (immune and inflammatory conditions)

New medicines authorised

- Eladynos (*abaloparatide*)
  Treatment of osteoporosis in women in menopause with increased risk of bone fractures

Urology

New medicines authorised

- Locametz (*gozetotide*)
  Treatment of prostate cancer
- Padcev (*enfortumab vedotin*)
  Treatment of bladder and urinary tract cancer
- Pluvicto (*lutetium (177Lu) vipivotide tetraxetan*)
  Treatment of prostate cancer

Vaccines

New medicines authorised

- Odenga (*dengue tetravalent vaccine (live, attenuated)*)
  Protection against dengue disease in patients from 4 years of age

Other medicines

Safety update

- Review of Synchron (*INN*) - European Commission final decision (Scope of safety referral)
  Suspension of medicines over flawed studies

Medicines under additional monitoring

- Updated list of medicines under additional monitoring

Other information

Guidelines

Adopted guidelines

- Liposomal amphotericin B product-specific bioequivalence guidance - Scientific guideline
- Lanreotide acetate, prolonged-release solution for injection in prefilled syringe 60, 90 and 120 mg product-specific bioequivalence guidance - Scientific guideline

Scientific committee and working party activities

- Download here https://www.ema.europa.eu/en/about-us/what-we-do/authorisation-medicines/medicine-evaluation-figures

Key to symbols used

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

- CAT - agendas, minutes and reports
- CHMP - agendas, minutes and highlights
- 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
- PCWP

Other publications on COVID-19

- COVID -19 vaccines safety update
- COVID-19 vaccines: key facts
- ETF concludes that bivalent original/Omicron BA.4-5 mRNA vaccines may be used for primary vaccination
- ETF warns that monoclonal antibodies may not be effective against emerging strains of SARS-CoV-2

Other publications on Monkeypox

- Possible use of the medicinal product TPOXX for the treatment of monkeypox

Other publications

- EMA recommends withdrawal of pholcodine medicines from EU market
- Synchron Research Service: re-examination confirms suspension of medicines over flawed studies
- ECDC and EMA collaborate on vaccine safety and effectiveness monitoring studies
- Letter of support for TREAT-NMD Core Dataset for Spinal Muscular Atrophy (SMA)
- Big Data Highlights - Issue 4
- First gene therapy to treat haemophilia B
- Letter of Support of Model-based Clinical Trial Simulation Platform (CTSP) for Duchenne Muscular Dystrophy
- Facilitating Decentralised Clinical Trials in the EU
- Clinical Trials Highlights
- Key performance indicators (KPIs) to monitor the European clinical trials environment
- Questions and answers for marketing authorisation holders/applicants on the CHMP Opinion for the Article 5(3) of Regulation (EC) No 726/2004 referral on nitrosamine impurities in human medicinal products

Events

- ACT EU multi-stakeholder meeting on decentralised clinical trials - 4 October 2022 - Report
- Second European Medicines Agency and Affordable Medicines Europe bilateral meeting - 16 November 2022
- Ninth Nitrosamine Implementation Oversight Group (NIOG) meeting - 21 November 2022
- Ninth meeting of the industry stakeholder platform on the operation of the centralised procedure for human medicine - 24 November 2022
- First European Medicines Agency - Vaccines Europe meeting - 28 November 2022 - Highlights
- Ninth industry stakeholder platform on research and development support - 5 December 2022
- Management Board meeting - 14-15 December 2022 - Agenda, Highlights
- Clinical Trials Information System (CTIS) bitesize talk: Annual safety report (ASR) - 15 December 2022
- Joint EMA-FDA workshop: Efficacy of monoclonal antibodies in the context of rapidly evolving SARS-CoV-2 variants - 15 December 2022
- EMA regular press briefing on public health emergencies - 16 December 2022
- Cancer Medicines Forum December 2022 - 20 December 2022
- Clinical Trials Information System (CTIS): Walk-in clinic - 18 January 2023
- Regulatory and scientific virtual conference on RNA-based medicines - 2 February 2023 - Agenda
- Clinical Trials Information System (CTIS) sponsor end user training programme 7-10 February 2023 - Agenda
- eXtended EudraVigilance Medicinal Product Dictionary (XEVMPD) training course - February 2023 - 13-15 February 2023
- Clinical Trials Information System (CTIS): Walk-in clinic - 16 February 2023
- HMA/EMA multi-stakeholder workshop on shortages - 1-2 March 2023
- Clinical Trials Information System (CTIS): Walk-in clinic - 16 March 2023
- Clinical Trials Information System (CTIS) sponsor end user training programme - 2-5 May 2023 - Agenda
- eXtended EudraVigilance Medicinal Product Dictionary (XEVMPD) training course - May 2023 - 10-12 May 2023
- Clinical Trials Information System (CTIS) sponsor end user training programme 27-30 June 2023 - Agenda
- eXtended EudraVigilance Medicinal Product Dictionary (XEVMPD) training course - July 2023 - 3-5 July 2023

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')

Highly similar 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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Further information about the European Medicines Agency and the work it does is available on our website:

http://www.ema.europa.eu

In particular, you may be interested in these links:

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European Medicines Agency

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