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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

HUMAN MEDICINES HIGHLIGHTS

Key information for patients, consumers and healthcare professionals
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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

COVID-19 vaccines and treatments

New medicines authorised

- COVID-19 Vaccine (inactivated, adjuvanted) Valneva (*COVID-19 vaccine (inactivated, adjuvanted, adsorbed)*)
  Prevention against COVID-19

Monkeypox vaccines and treatments

New information on authorised medicines

- Imvanex (*smallpox and monkeypox vaccine (Live Modified Vaccinia Virus Ankara)*) E - extension of indication
  Prevention of monkeypox disease

Key to symbols used

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

Cancer

Positive CHMP opinions on new medicines

- Celdoxome pegylated liposomal (*doxorubicin hydrochloride*)
  Treatment of metastatic breast cancer, advanced ovarian cancer, progressive multiple myeloma (cancer of the bone marrow) and AIDS-related Kaposi's sarcoma
- Opdualag (*relatlimab / nivolumab*)
  Treatment of melanoma (skin cancer)
- Thalidomide Lipomed (*thalidomide*)
  Treatment of multiple myeloma (cancer of the bone marrow)
- Tecvayli (*teclistamab*) C
  Treatment of multiple myeloma (cancer of the bone marrow)

New information on authorised medicines

- Retsevmo (*selpercatinib*) C - extension of indication
  Treatment of cancer which is caused by changes in a gene called RET
- Tecartus (*Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured*) O C - new indication
  Treatment of mantle cell lymphoma (cancer of a type of white blood cell)

Withdrawal of applications for new medicines

- Parsaclisib Incyte Biosciences Distribution B.V. (*parsaclisib*)
  Intended for the treatment of marginal zone lymphoma (a cancer of the white blood cells)

Dermatology (skin conditions)

Positive CHMP opinions on new medicines

- Opdualag (*relatlimab / nivolumab*)
  Treatment of melanoma (skin cancer)

New medicines authorised

- Filsuvez (*birch bark extract*) O
  Treatment of epidermolysis bullosa (disease of the skin that makes the skin very fragile and causes severe blistering and scarring)

Diabetes

Positive CHMP opinions on new medicines

- Mounjaro (*tirzepatide*)
  Treatment of type 2 diabetes mellitus

Gynaecology & Obstetrics (pregnancy and female reproductive)

New medicines authorised

- Ganirelix Gedeon Richter (*ganirelix*) generic of Orgalutran
  Prevention of premature ovulation (early release of eggs from the ovary) in women having fertility treatment and who are having ovarian stimulation (stimulation of the ovaries so that they produce more eggs)

Safety update

- Medicines containing nomegestrol or chlormadinone: PRAC recommends new measures to minimise risk of meningioma

Haematology (blood conditions)

New information on authorised medicines

- Tecartus (*Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured*) O C - new indication
  Treatment of mantle cell lymphoma (cancer of a type of white blood cell)

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  Intended for the treatment of marginal zone lymphoma (a cancer of the white blood cells)

HIV

Positive opinion on medicine for use outside the European Union

- Dapivirine Vaginal Ring 25 mg (*dapivirine*)
  Treatment to reduce the risk of a woman getting infected with human immunodeficiency virus type 1 (HIV-1) through vaginal intercourse

New information on authorised medicines

- Genvoya (*elvitegravir / cobicistat / emtricitabine / tenofovir alafenamide*) - extension of indication
  Treatment of HIV-1

Metabolic disorders

Positive CHMP opinions on new medicines

- Nulibry (*osdenopterin*) E O
  Treatment of patients with molybdenum cofactor deficiency Type A, (a severe condition which affects the nervous system and is caused by changes in certain genes)

New medicines authorised

- Amversio (*betaine anhydrous*) generic of Cystadane
  Treatment of homocystinuria (an inherited disease where the amino acid homocysteine cannot be broken down and therefore builds up in the body)
- Nexviadyme (*Avalglucosidase alfa*) O
  Treatment of the Pompe disease (a rare inherited disorder caused by the lack of an enzyme called alpha - glucosidase, a rare inherited disorder that leads to the build up of glycogen, a complex sugar, in body tissues)
- Xenpozyme (*olipudase alfa*) O
  Treatment of acid sphingomyelinase deficiency (ASMD) (a genetic condition that results in the build up of a type of fat in tissues)

Musculoskeletal system

New medicines authorised

- Sugammadex Fresenius Kabi (*sugammadex*) generic of Bridion
  Treatment to speed up the recovery from the muscle relaxant, usually at the end of the operation

New information on authorised medicines

- Ultomiris (*ravulizumab*) - new indication
  Treatment of generalized myasthenia gravis (a condition that causes muscle weakness)

Nephrology (kidney conditions)

Positive CHMP opinions on new medicines

- Lupkynis (*voclosporin*)
  Treatment of lupus nephritis (a condition in which the immune system attacks the kidney cells)

Nervous system

Positive CHMP opinions on new medicines

- Nulibry (*osdenopterin*) E O
  Treatment of patients with molybdenum cofactor deficiency Type A (a severe condition which affects the nervous system and is caused by changes in certain genes)

Ophthalmology (eye conditions)

Positive CHMP opinions on new medicines

- Vabysmo (*faricimab*)
  Treatment of neovascular age-related macular degeneration and diabetic macular oedema (conditions that affect part of the retina, the light sensitive part of the eye)

Respiratory system

Positive CHMP opinions on new medicines

- Tezspire (*tezepelumab*)
  Intended as add-on treatment of severe asthma

Vaccines

New information on authorised medicines

- Imvanex (*smallpox and monkeypox vaccine (Live Modified Vaccinia Virus Ankara)*) E - extension of indication
  Prevention of monkeypox disease

Other medicines

Positive CHMP opinions on new medicines

- Amvuttra (*vutrisiran*) O
  Treatment of hereditary transthyretin-mediated (hATTR) amyloidosis (a disease in which abnormal proteins called amyloids build up in tissues around the body including around the nerves)
- Illuzyce (*lutetium (177Lu) chloride*)
  Radiopharmaceutical precursor used for radiolabelling of carrier medicines

New medicines authorised

- Amversio (*betaine anhydrous*) generic of Cystadane
  Treatment of homocystinuria (an inherited disease where the amino acid homocysteine cannot be broken down and therefore builds up in the body)
- Imcivree (*setmelanotide*) O - new indication
  Treatment of obesity and hunger control in patients with Bardet-Biedl syndrome

Withdrawal of applications for extension of indication

- Imcivree (*setmelanotide*) O
  Intended to treat obesity and control hunger associated with genetically confirmed Alström syndrome

Withdrawal of authorised medicines

- Senstend (*lidocaine / prilocaine*)
  Treatment of lifelong premature ejaculation

Medicines under additional monitoring

- Updated list of medicines under additional monitoring

Other information

Guidelines

Guidelines open for consultation

- Draft ICH guideline M12 on drug interaction studies - Step 2b
  Deadline for comments: 21 November 2022

Adopted guidelines

- Guidance on the procedural aspects for the consultation to the European Medicines Agency by a notified body on companion diagnostics

Scientific committee and working party activities

- Medicinal products for human use: monthly figures - June 2022
- CAT - agendas, minutes and reports
- CAT quarterly highlights and approved ATMPs
- CHMP - agendas, minutes and highlights
- CHMP - applications for new human medicines: July 2022
- Annex to CHMP highlights: Recommendations on eligibility to PRIME scheme - Adopted at the CHMP meeting of 18-21 July 2022
- COMP - agendas, minutes and meetings reports
- HMPC - agendas, minutes and meetings reports
- PDCO - agendas, minutes and meeting reports
- EMA Paediatric Committee elects Brian Aylward as its new Chair
- PRAC - agendas, minutes and highlights
- PRAC statistics: July 2022
- PRAC recommendations on safety signals
- PCWP & HCPWP joint meeting - 22 September 2022: Agenda

Other publications on COVID-19

- EMA reviewing data on sabizabulin for COVID-19
- Global regulators agree on key principles on adapting vaccines to tackle virus variants
- ECDC and EMA update recommendations on additional booster doses of mRNA COVID-19 vaccines

Other publications

- EMA response to the monkeypox public health emergency
- EMA recommends restricting use of cancer medicine Rubraca
- Further measures to identify and address medicine shortages during public health emergencies adopted
- List of the main therapeutic groups (MTGs) in crisis preparedness
- Info-cards: What can you do when it comes to shortages of medicines?
- Factsheet: Towards better prevention of medicine shortages
- Towards better prevention of medicine shortages in the EU
- Good practice guidance for patient and healthcare professional organisations on the prevention of shortages of medicines for human use
- OPEN Pilot: One-year review and recommendations
- EMA launches pilot project on analysis of raw data from clinical trials
- Information about the raw data proof-of-concept pilot for industry
- EMA re-elected as chair of ICMRA from October 2022
- Big data use for public health: publication of Big Data Steering Group workplan 2022-25
- Joint controllership arrangement with regard to EudraVigilance Human (EV)
- Report: Technology Capability Investment Plan - Becoming a digital hub for the European medicines regulator network
- Global regulators call for international collaboration to integrate real-world evidence into regulatory decision-making
- Key performance indicators (KPIs) to monitor the European clinical trials environment

Events

- Multi-stakeholder workshop: Patient experience data in medicines development and regulatory decision-making - 21 September 2022 - Agenda
- Ad-hoc meeting of the Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG) - 7 July 2022
- European Medicines Agency's interaction with industry stakeholders - Biennial report 2010-2021
- Eight industry stakeholder platform on research and development support - 11 July 2022
- EMA workshop on thrombosis with thrombocytopenia syndrome - 27 June
- Digital application dataset integration (DADI) Q&A webinar - variations form for human medicinal products - 12 July 2022
- DARWIN EU Advisory Board meeting - 6 July 2022 - Agenda
- Industry Standing Group meeting - 21 June 2022 - Highlights
- Meeting of the Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG) - 25 July 2022

Key to symbols used

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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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