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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

## Antivirals/anti-infectives

### Positive CHMP opinions on new medicines

- Rezzayo (*rezafungin*) O
  Treatment of invasive candidiasis (a fungal infection caused by a yeast called Candida)

## Cancer

### Positive CHMP opinions on new medicines

- Elrexfio (*elranatamab*) C
  Treatment of multiple myeloma (cancer of the bone marrow)
- Krazati (*adagrasib*) C
  Treatment of non-small cell lung cancer

**Key to symbols used**

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

- Naveruclif (*paclitaxel*) generic of Abraxane
  Treatment of different types of cancers
- Spexotras (*trametinib*)
  Treatment of low- and high-grade glioma (a type of brain cancer)

### New medicines authorised

- Degarelix Accord (*degarelix*) generic of Firmagon
  Treatment of prostate cancer (a gland of the male reproductive system)
- Enrylaze (crisantaspase)
  Treatment of lymphoblastic leukaemia and lymphoblastic lymphoma (cancers of white blood cells called lymphoblasts)
- Herwenda (*trastuzumab*)
  Treatment of different types of cancer
- Inaqovi (*cedazuridine / decitabine*)
  Treatment of myeloid leukaemia (type of cancer affecting the white blood cells)
- Jaypirca (*pirtobrutinib*)
  Treatment of cell lymphoma (a cancer of a type of white blood cell)
- Orserdu (*elacestrant*)
  Treatment of breast cancer
- Tepkinly (*epcoritamab*)
  Treatment of blood cancer called diffuse large B-cell lymphoma ((a type of blood cancer)
- Tevimbra (*tislelizumab*)
  Treatment of oesophageal cancer (cancer of the oesophagus, the passage from the mouth to the stomach)
- Vanflyta (*quizartinib*)
  Treatment of myeloid leukaemia (cancer of the white blood cells)

### New information on authorised medicines

- Ayvakyt (*avapritinib*) - extension of indication
  Treatment of gastrointestinal stromal tumors
- Imfinzi (*durvalumab*) - new indication
  Treatment of lung cancer
- Jemperli (*dostarlimab*) - new indication
  Treatment of endometrial cancer (cancer of the lining of the womb)
- Keytruda (*pembrolizumab*) - new indication
  Treatment of several types of cancer
- Rubraca (*rucaparib*) - new indication
  Treatment of ovarian cancer
- Talzenna (*talazoparib*)
  Treatment of prostate cancer

### Withdrawal of authorised medicines

- Imatinib Koanaa (*imatinib*) generic of Glivec
  Intended for treatment of several types of cancers

**Key to symbols used**

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

### Supply shortages

- Hycamtin (*topotecan*)
  Treatment of ovary cancer and small cell lung cancer

# Cardiovascular system

### New information on authorised medicines

- Evkeeza (*evinacumab*) E - extension of indication
  Treatment of hypercholesterolemia (high levels of blood cholesterol)
- Jardiance (*empagliflozin*) - extension of indication
  Treatment of type 2 diabetes mellitus, heart failure and chronic kidney disease
- Praluent (*alirocumab*) - change of indication
  Treatment of primary hypercholesterolaemia (heterozygous familial and non-familial) or mixed dyslipidaemia, and heterozygous familial hypercholesterolaemia (treatment of abnormal levels of blood fats)
- Pradaxa (*dabigatran etexilate*) - change of indication/ removal of pharmaceutical form
  Prevention of formation of blood clots in veins, stroke, vein thrombosis (prevention of stroke and prevention and treatment of blood clots)

# Dermatology (skin conditions)

### Positive CHMP opinions on new medicines

- Uzpruvo (*ustekinumab*)
  Treatment of plaque psoriasis, psoriatic arthritis and Crohn's disease (inflammatory disorders)

### New medicines authorised

- Ebglyss (*Lebrikizumab*)
  Treatment of atopic dermatitis (also known as atopic eczema, when the skin is itchy, red and dry)

# Diabetes

### New information on authorised medicines

- Jardiance (*empagliflozin*) - extension of indication
  Treatment of type 2 diabetes mellitus, heart failure and chronic kidney disease
- Mounjaro (*tirzepatide*) - extension of indication
  Treatment of Type 2 diabetes mellitus and weight management

# Gastro-intestinal system

### Positive CHMP opinions on new medicines

- Uzpruvo (*ustekinumab*)
  Treatment of plaque psoriasis, psoriatic arthritis and Crohn's disease (inflammatory disorders)

### New information on authorised medicines

- Ayvakyt (*avapritinib*) C O - extension of indication
  Treatment of gastrointestinal stromal tumors

# Gynaecology & Obstetrics (pregnancy and female reproductive)

### Positive CHMP opinions on new medicines

- Veoza (*fezolinetant*)
  Treatment of hot flushes (vasomotor symptoms) associated with menopause

### New medicines authorised

- Orserdu (*elacestrant*)
  Treatment of breast cancer

### New information on authorised medicines

- Jemperli (*dostarlimab*) C - new indication
  Treatment of endometrial cancer (cancer of the lining of the womb)
- Rubraca (*rucaparib*) - new indication
  Treatment of ovarian cancer

### Safety update

- Review of Topiramate - CMDh Position
  Risk of developmental disorders in children exposed in the womb

### Direct Healthcare Professional Communication (DHPC)

- Topiramate and topiramate/phentermine combination: New restrictions to prevent exposure during pregnancy

# Haematology (blood conditions)

### New medicines authorised

- Enrylaze (crisantaspase)
  Treatment of lymphoblastic leukaemia and lymphoblastic lymphoma (cancers of white blood cells called lymphoblasts)
- Inaqovi (*cedazuridine / decitabine*)
  Treatment of myeloid leukaemia (type of cancer affecting the white blood cells)
- Tepkinly (*epcoritamab*) C O
  Treatment of blood cancer called diffuse large B-cell lymphoma ((a type of blood cancer)
- Vanflyta (*quizartinib*)
  Treatment of myeloid leukaemia (cancer of the white blood cells)

### New information on authorised medicines

- Veyvondi (vonicog alfa) O - extension of indication
  Treatment of von Willebrand disease (inherited bleeding disorder)

# Immune system

### Positive CHMP opinions on new medicines

- Omjjara (*momelotinib*)
  Treatment of enlarged spleen and myelofibrosis (a disorder of the bone marrow)
- Rystiggo (*rozanolixizumab*)
  Treatment of myasthenia gravis (a chronic autoimmune neuromuscular disease that causes weakness in the skeletal muscles)
- Uzpruvo (*ustekinumab*)
  Treatment of plaque psoriasis, psoriatic arthritis and Crohn's disease (inflammatory diseases)

### New medicines authorised

- Tyenne (*tocilizumab*)
  Treatment of rheumatoid arthritis (an immune-system disease causing inflammation of the joints)

### Withdrawal of applications for extension of indication

- RoActemra (*tocilizumab*)
  Intended for treatment of interstitial lung disease (disorder causing scarring in the lungs)

# Metabolic disorders

### New information on authorised medicines

- Veltassa (*patiromer*) - new pharmaceutical form
  Treatment of hyperkalaemia (high levels of potassium in the blood)

### Safety update

- Review of Ocaliva (*Iobeticholic acid*) - review started
  Treatment of primary biliary cholangitis, an autoimmune condition that causes gradual destruction of the small bile ducts in the liver

# Musculoskeletal system

### Positive CHMP opinions on new medicines

- Agamree (*vamorolone*)
  Treatment of Duchenne muscular dystrophy (a genetic disease that gradually causes weakness and loss of muscle function)

# Nephrology (kidney conditions)

### New information on authorised medicines

- Jardiance (*empagliflozin*) - extension of indication
  Treatment of type 2 diabetes mellitus, heart failure and chronic kidney disease

# Nervous system

### New medicines authorised

- Tyruko (*natalizumab*)
  Treatment of multiple sclerosis (disease in which inflammation destroys the sheath around the nerves)

### Positive CHMP opinions on new medicines

- Loargys (*pegzilarginase*) O E
  Treatment of hyperargininemia (a rare disease with neurological clinical signs including spasticity, ataxia, hyperreflexia, incoordination, and seizures

### Negative CHMP opinions on new medicines

- Albrioza (*sodium phenylbutyrate/ursodoxicoltaurine* ) O
  Intended for the treatment of amyotrophic lateral sclerosis (a disease of the nervous system that causes muscle weakness and paralysis)

### Safety update

- Review of Topiramate - CMDh Position
  Risk of developmental disorders in children exposed in the womb

### Direct Healthcare Professional Communication (DHPC)

- Topiramate and topiramate/phentermine combination: New restrictions to prevent exposure during pregnancy

# Ophthalmology (eye conditions)

### Positive CHMP opinions on new medicines

- Rimmyrah (*ranibizumab*)
  Treatment of different eye conditions

### New medicines authorised

- Yesafili (*aflibercept*)
  Treatment of different eye conditions

# Respiratory system

### Positive CHMP opinions on new medicines

- Krazati (*adagrasib*) C
  Treatment of non-small cell lung cancer

### New medicines authorised

- Lyfnua (*gefapixant* )
  Treatment of chronic (long-term) cough

### New information on authorised medicines

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

### Withdrawal of applications for extension of indication

- RoActemra (*tocilizumab*)
  Intended for treatment of interstitial lung disease (disorder causing scarring in the lungs)

### Supply shortages

- Hycamtin (*topotecan*)
  Treatment of ovary cancer and small cell lung cancer

## Rheumatology (immune and inflammatory conditions)

### New medicines authorised

- Tyenne (*tocilizumab*)
  Treatment of rheumatoid arthritis (an immune-system disease causing inflammation of the joints)

## Urology (urinary tract conditions)

### New information on authorised medicines

- Talzenna (*talazoparib*)
  Treatment of prostate cancer

## Vaccines

- EMA recommends approval of adapted Nuvaxovid COVID-19 vaccine targeting Omicron XBB.1.5

## Other medicines

### Positive CHMP opinions on new medicines

- Elucirem (*gadopiclenol*)
  Used for improving images from MRI body scans
- Loargys (*pegzilarginase*) O E
  Treatment of hyperargininemia (a rare disease with neurological clinical signs including spasticity, ataxia, hyperreflexia, incoordination, and seizures.
- Vueway (*gadopiclenol*)
  Used for improving images from MRI body scans

### New information on authorised medicines

- NexoBrid (*concentrate of proteolytic enzymes enriched in bromelain*) - extension of indication
  Treatment of burn wounds

**Withdrawal of applications for new medicines**

- Vijoice (*alpelisib*)
  Intended for the treatment of PIK3CA-related overgrowth spectrum (genetic condition that causes a range of symptoms, including malformations and abnormal growth or tumours affecting several tissues, such as the skin, bones, blood vessels and brain

## Medicines under additional monitoring

- Updated list of medicines under additional monitoring

# Other information

## Guidelines

**Guidelines open for consultation**

- Draft guideline on the development and manufacture of Synthetic Peptides
  Deadline for comments: 30 April 2024
- Concept paper on the development of an addendum to the Guideline on clinical development of vaccines on clinical trials for vaccines for immunocompromised individuals
  Deadline for comments: 30 January 2024
- Clinical requirements for non replacement therapy in haemophilia A and B
  Deadline for comments: 30 April 2024
- Concept paper on the revision of the Non-clinical and Clinical Module of the influenza vaccines guideline
  Deadline for comments: 30 January 2024
- Draft revised consolidated 3-year work plan for the Methodology Working Party (MWP)
  Deadline for comments: 30 November 2023

## Other scientific recommendations

## Scientific committee and working party activities

- Medicinal products for human use: monthly figures - October 2023
- CAT - agendas, minutes and reports
- CHMP - agendas, minutes and highlights
- CHMP - applications for new human medicines: November 2023
- CHMP early contact with patient and healthcare professional organisations process and FAQs
- COMP - agendas, minutes and meetings reports

- Call for expressions of interest for patients' organisations representatives to join Committee for Orphan Medicinal Products (COMP) - deadline 7 December
- HMPC - agendas, minutes and meetings reports
- PDCO - agendas, minutes and meeting reports
- PRAC - agendas, minutes and highlights
- PRAC statistics: November 2023
- PRAC statistics: December 2023
- PRAC recommendations on safety signals
- Call for expression of interest for independent scientific experts to participate in the work of EMA's Safety Committee - deadline 7 December
- European Medicines Agency (EMA) Patients' and Consumers' (PCWP) and Healthcare Professionals' (HCPWP) Working Parties meeting with all eligible organisations - 14 and 15 November

# Other publications

- EMA alerts EU patients and healthcare professionals to reports of falsified Ozempic pens
- Revised CTIS Transparency Rules
- Consumption of antimicrobials in animals reaches lowest level ever in Europe
- Global regulators celebrate 10 years of strategic leadership and cooperation
- First electronic product information (ePI) published for selected human medicines
- EMA takes further steps to address critical shortages of medicines in the EU

# Events

- EMA Management Board: highlights of October 2023 meeting
- Multi-stakeholder workshop on the guideline on clinical investigation of medicinal products in the treatment of epileptic disorders - 29 January 2024 (LINK STILL NOT WORKING)
- Multistakeholder workshop on Patient Registries - 12 and 13 February 2024
- Workshop on generating clinical evidence for treatment and prevention options for long-COVID and post-acute sequelae condition - 17 November 2023 - Agenda
- Second bilateral meeting between European Medicines Agency and Vaccines Europe - November 2023
- Sixth Industry Standing Group (ISG) meeting - 21 September 2023 - Minutes
- Seventh Industry Standing Group (ISG) meeting - 23 November 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')

### 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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