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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

- Exblifep (*cefepime/enmetazobactam*)
  Treatment of complicated urinary tract infections and lung infections in people using ventilators

New medicines authorised

- Rezzayo (*rezafungin*) O
  Treatment of invasive candidiasis

New information on authorised medicines

- Prevenar 20 (previously Apexxnar) (*pneumococcal polysaccharide conjugate vaccine (20-valent, adsorbed)*) - extension of indication
  Prevention of different infections caused by the bacteria Streptococcus pneumoniae

Key to symbols used

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

Cancer

New information on authorised medicines

- Abecma (*idecabtagene vicleucel*) O C - extension of indication
  Treatment of multiple myeloma (a cancer of the bone marrow)
- Retsevmo (*selpercatinib*) - new indication
  Treatment of different kinds of cancers

Cardiovascular system

Supply shortages

- Integrilin (*eptifibatide*)
  Prevention of myocardial infarction (heart attack)

Direct Healthcare Professional Communication (DHPC)

- Leqvio (*inclisiran*) 284 mg solution for injection in pre-filled syringe: Important information regarding instructions for use before injection

Haematology (blood conditions)

Positive CHMP opinions on new medicines

- Ryzneuta (*efbemalenograstim alfa*)
  Treatment to reduce the duration of neutropenia (low level of white blood cells) and the incidence of febrile neutropenia due to chemotherapy

New information on authorised medicines

- Aspaveli (*pegcetacoplan*) O - extension of indication
  Treatment of paroxysmal nocturnal haemoglobinuria (PNH), a condition in which there is excessive breakdown of red blood cells

Musculoskeletal system

- EMA confirms recommendation for non-renewal of authorisation of Duchenne muscular dystrophy medicine Translarna

Nervous system

Positive CHMP opinions on new medicines

- Niapelf (*paliperidone*) generic of Xeplion
  Treatment of schizophrenia

Negative CHMP opinions on new medicines

- Nezglyal (*leriglitazone*)
  Intended for the treatment of cerebral adrenoleukodystrophy (a form of an inherited disease in which fatty substances build up in tissues around the body, mainly in the brain, spinal cord and adrenal glands)

Key to symbols used

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Safety update

- Precautionary measures to address potential risk of neurodevelopmental disorders in children born to men treated with valproate medicines

Direct Healthcare Professional Communication (DHPC)

- Voxzogo (*vosoritide*): change to administration syringe and needle leading to product administration in Units (U) instead of mL

Ophthalmology (eye conditions)

Negative CHMP opinions on new medicines

- Syfovre (*pegcetacoplan*)
  Treatment of an advanced form of an eye condition known as age-related macular degeneration

Respiratory system

Positive CHMP opinions on new medicines

- Exblifep (*cefepime/enmetazobactam*)
  Treatment of complicated urinary tract infections and lung infections in people using ventilators

New information on authorised medicines

- Prevenar 20 (previously Apexxnar) (*pneumococcal polysaccharide conjugate vaccine (20-valent, adsorbed)*) - extension of indication
  Prevention of different infections caused by the bacteria Streptococcus pneumoniae

Safety update

- Review of pseudoephedrine-containing medicinal products - CHMP Opinion
  Nasal decongestants for systemic use

Urology (urinary tract conditions)

Positive CHMP opinions on new medicines

- Exblifep (*cefepime/enmetazobactam*)
  Treatment of complicated urinary tract infections and lung infections in people using ventilators

Vaccines

New information on authorised medicines

- Prevenar 20 (previously Apexxnar) (*pneumococcal polysaccharide conjugate vaccine (20-valent, adsorbed)*) - extension of indication
  Prevention of different infections caused by the bacteria Streptococcus pneumoniae

Medicines under additional monitoring

- Updated list of medicines under additional monitoring

Other information

Guidelines

Adopted guidelines

- Guideline on the clinical evaluation of anticancer medicinal products
- ICH Q5A(R2) Guideline on viral safety evaluation of biotechnology products derived from cell lines of human or animal origin - Step 5

Scientific committee and working party activities

- Medicinal products for human use: monthly figures - December 2023
- CAT - agendas, minutes and reports
- CHMP - agendas, minutes and highlights
- CHMP - applications for new human medicines: January 2024
- 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: January 2024
- PRAC recommendations on safety signals

Other publications

- Human medicines: highlights of 2023
- Clinical trials' transition to new EU system - one year left
- Questions and answers to Stakeholders on the implications of Regulation (EU) 2023/1182 for centrally authorised medicinal products for human use
- Big Data Steering Group (BDSG): 2023 report
- Major update of the SME user guide

Events

- Cancer Medicines Forum workshop - 5 April 2024
- Update webinar on Regulatory Procedure Management for Product Lifecycle Management on IRIS - 13 February
- Joint Heads of Medicines Agencies (HMA)/European Medicines Agency (EMA) Multistakeholder workshop on Patient Registries - 12-13 February - Agenda
- ACT EU Training for non-commercial sponsors: Transitioning trials to the CTR (CTIS) - 9 February
- Conversations on Cancer: Transforming Patient Lives by Therapeutic and Regulatory Innovations - 1 February 2024
- Meeting of the Executive Steering Group on Shortages and Safety of Medicinal Products (MSSG) - 29 January
- Highlights from the 12th meeting of the Nitrosamine Implementation Oversight Group (NIOG) - 22 January 2024
- Highlights from the 6th meeting of the Nitrosamine Implementation Oversight Group (NIOG) with industry stakeholders - 22 January 2024

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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