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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

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

- Trogarzo (*ibalizumab*)
  Treatment of HIV infection

New information on authorised medicines

- Zerbaxa (*ceftolozane / tazobactam*) - new indication
  Treatment of hospital-acquired pneumonia

Cancer

Positive CHMP opinions on new medicines

- Vitrakvi (*larotrectinib*) O C
  Treatment of solid tumours with a specific gene mutation

Key to symbols used

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

New medicines authorised

- Libtayo (*cemiplimab*)
  Treatment of cutaneous squamous cell carcinoma (a skin cancer)
- Talzenna (*talazoparib*)
  Treatment of breast cancer

New information on authorised medicines

- Empliciti (*elotuzumab*) - new indication
  Treatment of relapsed and unresponsive multiple myeloma
- Lonsurf (*trifluridine / tipiracil*) - new indication and change to existing indication
  Treatment of stomach and colorectal cancer
- Keytruda (*pembrolizumab*) - new indication
  Treatment of renal cell carcinoma (kidney cancer)
- Tecentriq (*atezolizumab*) - new indication
  Treatment of urothelial carcinoma (cancer of the bladder and urinary system)

Safety update

- Review of cyproterone-containing medicinal products - review started (meningioma risk with cyproterone medicines)
  Treatment of various androgen-dependent conditions such as prostate cancer, excessive hair growth, hair loss, early puberty, lack of menstrual period and acne
- Review of methotrexate-containing medicinal products - PRAC recommendation (new measures to avoid dosing errors)
  Treatment of various cancers and inflammatory conditions

Cardiovascular system

New medicines authorised

- Ambrisentan Mylan (*ambrisentan*) generic of Volibris
  Treatment of pulmonary arterial hypertension (PAH) (high blood pressure in the lungs)

Dermatology

New medicines authorised

- Libtayo (*cemiplimab*)
  Treatment of cutaneous squamous cell carcinoma (skin cancer)

Gastro-intestinal system

New information on authorised medicines

- Lonsurf (*trifluridine / tipiracil*) - new indication and change to existing indication
  Treatment of stomach and colorectal cancer
- Stelara (*ustekinumab*) - new indication
  Treatment of ulcerative colitis

Haematology

Positive CHMP opinions on new medicines

- Deferasirox Mylan (*deferasirox*) generic of Exjade
  Treatment of chronic iron overload due to blood transfusions in patients with blood disorders

New medicines authorised

- Ultomiris (*ravulizumab*)
  Treatment of paroxysmal nocturnal haemoglobinuria (PNH) (a life-threatening disease including excessive breakdown of red blood cells)
- Xromi (*hydroxycarbamide*)
  Prevention of complications of sickle cell disease

HIV

Positive CHMP opinions on new medicines

- Trogarzo (*ibalizumab*)
  Treatment of HIV infection

Hormone system

Safety update

- Review of cyproterone-containing medicinal products - review started (meningioma risk with cyproterone medicines)
  Treatment of various androgen-dependent conditions such as excessive hair growth, hair loss, early puberty, lack of menstrual period, acne and prostate cancer

Immune system

New information on authorised medicines

- Stelara (*ustekinumab*) - new indication
  Treatment of ulcerative colitis

Safety update

- Review of methotrexate-containing medicinal products - PRAC recommendation (new measures to avoid dosing errors)
  Treatment of various cancers and inflammatory conditions

Metabolic disorders

New medicines authorised

- Cufence (*trientine dihydrochloride*)
  Treatment of Wilson's disease (a condition in which excessive amounts of copper accumulate in the body)

Nephrology

New medicines authorised

- LysaKare (*arginine / lysine*)
  Used to protect the kidneys against radiation during radioactive therapy with lutetium (177Lu) oxodotreotide

New information on authorised medicines

- Keytruda (*pembrolizumab*) - new indication
  Treatment of renal cell carcinoma (kidney cancer)

Nervous system

Positive CHMP opinions on new medicines

- Epidyolex (*cannabidiol*) O
  Treatment of seizures associated with Lennox-Gastaut syndrome (LGS) or Dravet syndrome (DS)
- Inbrija (*levodopa*)
  Treatment of symptoms of Parkinson's disease

New information on authorised medicines

- Soliris (*eculizumab*) - new indication
  Treatment of Neuromyelitis optica spectrum disorder (NMOSD) (an inflammatory condition of nervous system)

Safety update

- Review of Gilenya (*fingolimod*) - CHMP Opinion (not to be used in pregnancy and in women able to have children who are not using effective contraception)
  Treatment of multiple sclerosis

Ophthalmology

New information on authorised medicines

- Lucentis (*ranibizumab*) - extension to existing indication
  Treatment of retinopathy of prematurity (an eye disease in premature babies)

Respiratory system

New medicines authorised

- Ambrisentan Mylan (*ambrisentan*) generic of Volibris
  Treatment of pulmonary arterial hypertension (PAH) (high blood pressure in the lungs)

New information on authorised medicines

- Zerbaxa (*ceftolozane / tazobactam*) - new indication
  Treatment of hospital-acquired pneumonia

Rheumatology

Safety update

- Review of methotrexate-containing medicinal products - PRAC recommendation (new measures to avoid dosing errors)
  Treatment of various cancers and inflammatory conditions

Urology

New information on authorised medicines

- Tecentriq (*atezolizumab*) - new indication
  Treatment of urothelial carcinoma (cancer of the bladder and urinary system)

Other medicines

New medicines authorised

- Sixmo (*buprenorphine*)
  Treatment of opioid dependence
- Trecondi (*treosulfan*)
  Conditioning treatment for patients having blood-stem cell transplantation

Medicines under additional monitoring

- Updated list of medicines under additional monitoring

Other information

Guidelines

Guidelines open for consultation

- Draft qualification opinion of Multiple sclerosis clinical 4 outcome assessment (MSCOA)
  Deadline for comments: 20 September 2019

Adopted guidelines

- Concept paper on the need for revision of the guideline on the investigation of medicinal products in the term and preterm neonate - Revision 1

Scientific committee and working party activities

- Medicinal products for human use: monthly figures - June 2019

- CAT - agendas, minutes and reports
- CHMP - agendas, minutes and highlights
- CHMP - applications for new human medicines: July 2019
- 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: July 2019
- PRAC recommendations on safety signals

Other publications

- Medicine shortages: EU network takes steps to improve reporting and communication
- EMA takes note of the European Ombudsman's decision on pre-submission activities
- Guido Rasi elected chair of International Coalition of Medicines Regulatory Authorities (ICMRA)
- Annual accounts: Financial year 2018
- EMA tracking tool: relocation to Amsterdam - Main milestones - updated
- Public engagement highlights of 2018 - Leaflet
- Supporting medicine developers in generating quality data packages in early access approaches (PRIME and breakthrough therapies): workshop report published
- EMA and European Union payer community meeting - 18 June 2019 - Minutes
- Names of liposomal medicines to be changed to avoid medication errors
- Call for all sponsors to publish clinical trial results in EU database
- EU and US reach a milestone in mutual recognition of inspections of medicines manufacturers

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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European Medicines Agency

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Website www.ema.europa.eu **Telephone** +31 (0)88 871 6000

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