



Human medicines highlights 2016

81 Positive opinions

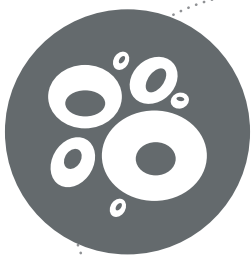
27 New active substances

2 Negative opinions*

16 Withdrawn applications

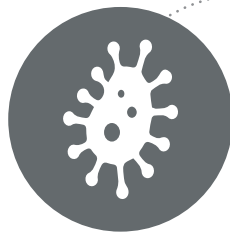
Medicines recommended for approval in 2016

Cancer



- Alecensa** ●
- Bortezomib Hospira
- Bortezomib SUN
- Cabometyx ●
- Darzalex** ● ● ●
- Empliciti** ●
- Ibrance** ●
- Kisplyx ●
- Lartruvo** ● ● ● ●
- Ledaga ●
- Lonsurf** ● ● ● ●
- Ninlaro** ● ● ●
- Onivyde ●
- Pemetrexed Fresenius Kabi
- SomaKit-TOC ●
- Truxima (biosimilar)
- Venclyxto** ● ● ●

Infections



- Atazanavir Mylan
- Darunavir Mylan
- Descovy
- Emtricitabine / Tenofovir disoproxil Mylan
- Emtricitabine / Tenofovir disoproxil Krka
- Emtricitabine - Tenofovir disoproxil Zentiva
- Epclusa** ●
- Odefsey
- Tenofovir disoproxil Mylan
- Tenofovir disoproxil Zentiva
- Vemlidy
- Zavicefta** ●
- Zepatier** ●
- Zinplava** ●

Cardiovascular



- Amlodipine-Valsartan Mylan
- Inhixa (biosimilar)
- Ivabradine JensonR
- Ivabradine Zentiva
- Mysildecard
- Neparvis
- Uptravi** ●
- Tadalafil Generics
- Thorinane (biosimilar)

Rheumatology



- Flixabi (biosimilar)
- Lifmior
- Movymia (biosimilar)
- Nordimet
- Olumiant** ●
- Sialanar
- Terrosa (biosimilar)
- Truberzi** ●

Metabolism



- Chenodeoxycholic acid ● ●
- Cystadrops ●
- Fiasp
- Galafold** ●
- Glyxambi
- Lusduna (biosimilar)
- Qtern
- Suliqua

Haematology/ Haemostaseology



- Afstyla** ●
- Alprolix** ● ●
- Coagadex ● ●
- Idelvion** ● ●
- Vihuma
- Zalmoxis** ● ●
- (advanced therapy)

Hepatology/ Gastroenterology



- Enzepi
- Ocaliva** ● ●
- Palonosetron Accord
- Palonosetron Hospira

Pneumology/ Allergology



- Aerivio Spiromax
- Airexar Spiromax
- Cinqaro** ●
- Granpidam

Neurology



- Ongentys** ●
- Pregabalin Zentiva k.s.
- Rasagiline Mylan
- Zinbryta



Endocrinology

- Parsabiv** ●
- Rekovelte



Immunology

- Strimvelis** ●
- (advanced therapy)



Psychiatry

- Zonisamide Mylan



Dermatology

- Taltz** ●



Radiolabelling agent

- EndolucinBeta



Vaccine

- Pandemic influenza vaccine (H5N1) MedImmune** ●

- Orphan medicine
- Accelerated assessment
- Conditional marketing authorisation
- Approval under exceptional circumstances

The medicines that contain a new active substance are highlighted in blue

These figures reflect EMA's recommendations which are sent to the European Commission for the adoption of an EU-wide marketing authorisation.

*Two medicines initially received a negative opinion from the CHMP: Sialanar in 2016 and Ninlaro in May 2016. Following re-examination, Sialanar received a positive opinion from the Committee in July 2016 and Ninlaro received a positive opinion in September 2016. These two medicines are included in the 81 positive opinions for 2016.

Innovations advancing public health

Innovation in healthcare brings new opportunities to treat certain diseases and is essential to advancing public health. Therapeutic innovations in 2016 included:



Haematology/ Haemostaseology

Coagadex

replaces the missing factor X, thereby helping the blood to clot and giving temporary control of bleeding in patients with hereditary factor X deficiency

Zalmoxis

an advanced therapy medicine for patients receiving a haploidentical haematopoietic stem cell transplant (HSCT), which contains T cells that have been genetically modified



Metabolism

Galafold

binds to the defective alpha-galactosidase A enzyme and restores its activity in patients with Fabry disease



Immunology

Strimvelis

a gene therapy manufactured from a patient's own immature bone marrow cells that improves their ability to fight infection



Infections

Zavicefta

inhibits the action of beta-lactamase enzymes involved in bacterial resistance to certain antibiotics



Rheumatology

Olumiant

blocks the action of Janus kinase enzymes (JAKs) reducing inflammation and other symptoms of rheumatoid arthritis

New orphan medicines for rare diseases

EMA's orphan medicines programme aims to encourage the development of medicines for patients with rare diseases by providing incentives for developers.

Among the 81 medicines recommended for centralised marketing authorisation, 16 had an orphan designation at the end of 2016.

New orphan medicines with the potential to significantly benefit patients included:



Haematology/ Haemostaseology

Coagadex

for patients with factor X deficiency, a rare inherited bleeding disorder

Zalmoxis

an advanced therapy medicine for patients receiving a haploidentical haematopoietic stem cell transplant (HSCT)



Hepatology

Ocaliva

for patients with primary biliary cholangitis



Immunology

Strimvelis

for patients with ADA-SCID who have no suitable stem cell donor



Cancer

Darzalex

for patients with multiple myeloma

Lartuvo

for patients with soft tissue sarcoma



Metabolism

Galafold

for patients with Fabry disease, a rare genetic disorder

Early access to medicines that address specific public health needs

Accelerated assessments

Seven medicines received a recommendation for marketing authorisation following an accelerated assessment. This mechanism is reserved for medicines that have the potential to address unmet medical needs. It allows for faster assessment of eligible medicines by EMA's scientific committees (within up to 150 days rather than up to 210 days).



Cancer

Darzalex

for patients with multiple myeloma

Kisplyx

for patients with advanced renal cell carcinoma

Cabometyx

for patients with advanced renal cell carcinoma

Empliciti

for patients with multiple myeloma

Lartruvo

for patients with soft tissue sarcoma



Haematology/ Haemostaseology

Coagadex

for patients with factor X deficiency



Infections

Epclusa

for patients with chronic hepatitis C virus infection

Conditional marketing authorisations

Eight medicines received a recommendation for a conditional marketing authorisation, one of the possibilities in the EU to give patients early access to new medicines. This tool allows for the early approval of a medicine on the basis of less complete clinical data than normally required if the medicine addresses an urgent unmet medical need. These medicines are subject to specific post-authorisation obligations for medicines developers that aim to obtain complete data on the medicine.



Cancer

Alecensa

for patients with anaplastic lymphoma kinase (ALK)-positive advanced non-small cell lung cancer

Darzalex

for patients with multiple myeloma

Ninlaro

for patients with multiple myeloma

Lartruvo

for patients with soft tissue sarcoma

Venclyxto

for patients with chronic lymphocytic leukaemia



Vaccine

Pandemic influenza vaccine H5N1 MedImmune

to protect children (12 months to 18 years) against influenza during a flu pandemic



Haematology/ Haemostaseology

Zalmoxis

an advanced therapy medicine for patients receiving a haploidentical haematopoietic stem cell transplant (HSCT)




Hepatology

Ocaliva

for patients with primary biliary cholangitis

A new medicine for use outside the European Union



In cooperation with the World Health Organization (WHO) EMA supports the availability of important medicines for use outside the EU through its Article 58 procedure. This procedure allows EMA to work closely with experts from WHO and the regulatory authorities from countries where the medicine will be used.

In 2016, EMA adopted a positive scientific opinion for **Umbipro**, an antiseptic gel to prevent umbilical cord infections (omphalitis) in newborn babies, under its accelerated assessment programme. It contains chlorhexidine, an antibacterial agent, commonly used as a mouthwash and topical antiseptic.

New uses for existing medicines

59 extensions of indication were recommended in 2016. The extension of the use of a medicine that is already approved in a new therapeutic indication can also offer new opportunities for patients. Extensions of indication included:

Truvada

for pre-exposure prophylaxis (PrEP) in combination with safer sex practices to reduce the risk of sexually-acquired human immunodeficiency virus type 1 (HIV-1) infection in adults at high risk

Zontivity

for patients with peripheral arterial disease

Opdivo

for patients with advanced renal cell carcinoma

Gazyvaro

for patients with follicular lymphoma



Monitoring in real-life – optimising safe and effective use

Once a medicine has been put on the market, EMA and the EU Member States continuously monitor the benefits and risks that patients experience with this medicine in real life. This is to ensure that the medicine is used in the best possible way by patients in the EU. Regulatory measures range from a change to the product information to the suspension or withdrawal of a medicine.

Important new safety advice issued in 2016 included:

New contraindication for **Adempas** in patients with symptomatic pulmonary hypertension associated with idiopathic interstitial pneumonia or PH-IIP

Product information to be updated to strengthen existing warnings for **Noxafil** that the two dose forms given by mouth cannot be simply interchanged as this may lead to under-dosing and to a potential lack of efficacy

New recommendations to minimise the risk of diabetic ketoacidosis in patients taking **SGLT2 inhibitors** (a class of type 2 diabetes medicines)

New recommendations to minimise the risk of progressive multifocal leukoencephalopathy (a rare brain infection) in patients taking the multiple sclerosis medicine **Tysabri**

Updated recommendations for use of **Zydelig** to minimise the risk of serious infections in cancer patients treated with this medicine

Use of **metformin** to treat diabetes expanded to patients with moderately-reduced kidney function



Ensuring integrity of global development and manufacturing chains

Medicine development and manufacturing is global. It is important for regulators to ensure that EU standards are adhered to no matter where clinical trials or manufacturing takes place. In 2016, EMA made the following recommendations based on the results of inspections:

Alkem Laboratories Ltd. review: recommendations to suspend a medicine (Riluzole Alkem) for amyotrophic lateral sclerosis (ALS) for which studies were conducted at the Alkem Laboratories Ltd. site in Talaja, India

Pharmaceutics International Inc., US: supply of non-critical medicines to the EU stopped due to manufacturing failings

Recommendation to suspend medicines over flawed studies at **Semler Research Centre Private Ltd.** Bangalore, India



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