



Human medicines highlights 2015

New medicines

93 positive opinions

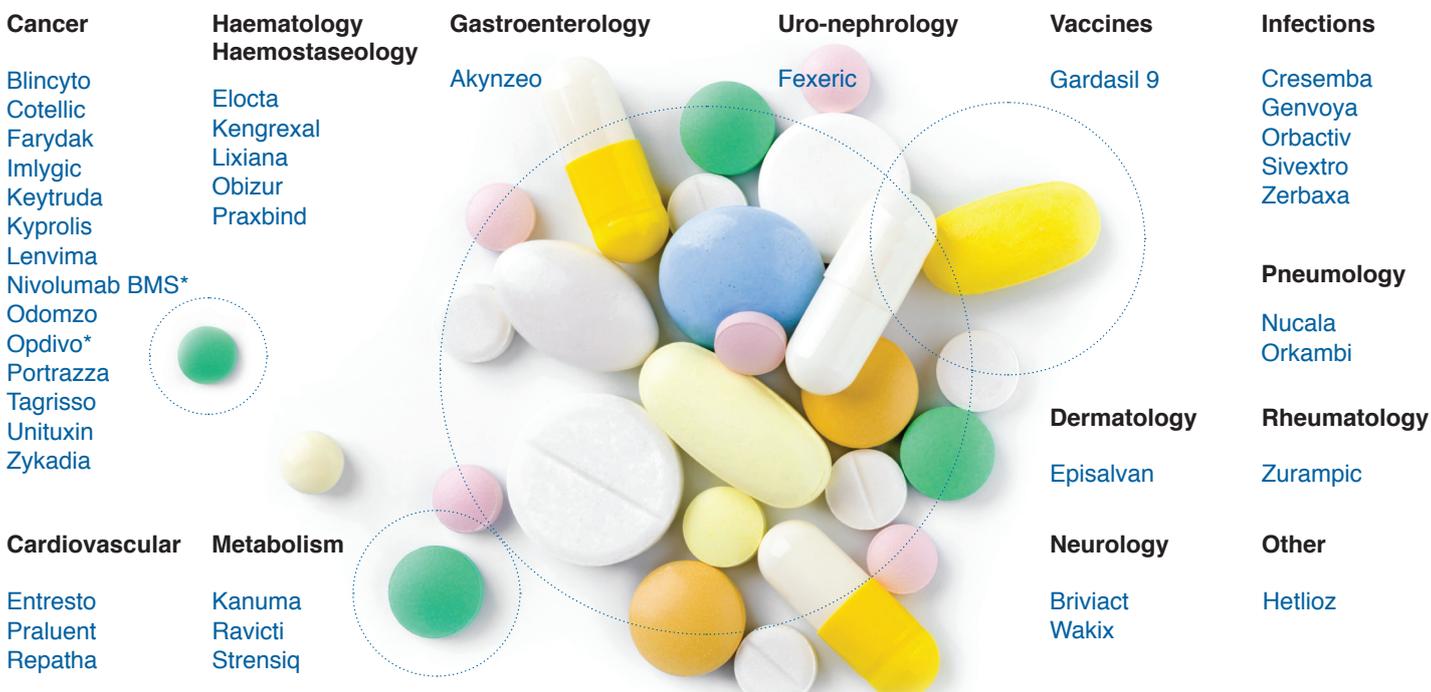
39 new active substances

4 negative opinions

5 withdrawn applications

Medicines with new active substances in 2015

In 2015, the European Medicines Agency (EMA) recommended 93 medicines for marketing authorisation. This includes recommendations for 39 new active substances. Medicines with a new active substance often have the potential to address patients' unmet medical needs. In 2015, one third of all medicines with a new active substance were for the treatment of cancer.



Innovation advancing public health

Innovation in healthcare brings new opportunities to treat certain diseases and is essential to advancing public health. Noteworthy therapeutic innovations in 2015 included:

- | | |
|-----------------------|--|
| Cancer | Blincyto - directing the immune system towards cancer cells |
| | Farydak - regulating the activity of genes |
| | Imlygic - using genetically engineered virus to kill cancer cells |
| | Opdivo, Nivolumab BMS and Keytruda - increasing the capacity of the immune system |
| Cardiovascular | Entresto - dual action to treat heart failure |
| | Repatha and Praluent - monoclonal antibodies to treat hypercholesterolemia |
| Haematology | Praxbind - targeted neutralisation of the anticoagulant effect of Pradaxa |
| Neurology | Wakix - action on histamine H3 receptors to treat narcolepsy |

*Opdivo and Nivolumab BMS contain the same active substance, but are authorised for different indications.



New uses for existing medicines

The use of an already approved medicine in a new therapeutic indication can also offer new opportunities for patients.



Noteworthy extensions of therapeutic indications

[Humira](#) for patients with acne inversa

[Imbruvica](#) for patients with Waldenström's macroglobulinaemia

[Perjeta](#) for patients with breast cancer undergoing surgery

[Tafinlar](#) and [Mekinist](#) a combination treatment for patients with advanced melanoma

[Xalkori](#) for previously untreated patients with non-small cell lung cancer with ALK mutation

New medicines for rare diseases

EMA's orphan designation programme gives medicine developers access to incentives and is the key instrument available in the European Union (EU) to encourage the development of medicines for patients with rare diseases.

Among the 93 medicines recommended by the Agency's Committee for Medicinal Products for Human Use (CHMP) in 2015, 18 had an orphan designation. Following the CHMP opinions, EMA's Committee for Orphan Medicinal Products (COMP) assesses whether the orphan designation should be maintained.



Noteworthy new orphan medicines

[Blinicyto](#) for patients with acute lymphoblastic leukaemia

[Farydak](#) for patients with multiple myeloma

[Hetlioz](#) for blind adults with sleep-wake disorder

[Kanuma](#) for patients with lysosomal acid lipase deficiency

[Kyprolis](#) for patients with multiple myeloma

[Lenvima](#) for patients with thyroid cancer

[Strensiq](#) for patients with childhood hypophosphatasia

[Unituxin](#) for patients with brain cancer (neuroblastoma)

Authorisations under exceptional circumstances

EMA's marketing authorisation under exceptional circumstances allows patients access to medicines that could not be approved under a standard authorisation as comprehensive data cannot be obtained, either because the disease they target is too rare, the collection of complete information on the efficacy and safety of the medicine would be unethical, or there are gaps in the scientific knowledge.

These medicines are subject to specific post-authorisation obligations and monitoring.

In 2015, three medicines were recommended for marketing authorisation under exceptional circumstances because of the rarity of the disease they target.

[Obizur](#) for patients with haemophilia

[Raxone*](#) for patients with Leber's hereditary optic neuropathy

[Strensiq](#) for patients with childhood hypophosphatasia

*Raxone is a 'hybrid medicine'. This means that it is similar to a reference medicine containing the same active substance.



Early access to medicines that address public health needs

5

Medicines
evaluated under
accelerated
assessment

Accelerated assessments

Five new 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 a faster assessment of eligible medicines by EMA's scientific committees (within up to 150 days rather than up to 210 days).

Kanuma for patients with lysosomal acid lipase deficiency

Kyprolis for patients with multiple myeloma

Lenvima for patients with thyroid cancer

Praxbind the first antidote to anticoagulant Pradaxa

Tagrisso for patients with non-small cell lung cancer with T790 mutation

3

Recommendations
for conditional
marketing
authorisations

Conditional marketing authorisations

Three medicines received a recommendation for a conditional marketing authorisation, one of EMA's early access routes to patients. This tool allows for the early approval of a medicine on the basis of less complete clinical data than normally required. It is intended for medicines that address an unmet medical need, and that target seriously debilitating or life-threatening diseases, rare diseases or are intended for use in emergency situations in response to a public health threat.

These medicines are subject to specific post-authorisation obligations that aim to obtain complete data on the medicine. EMA monitors the fulfilment of these obligations with the aim of ultimately recommending a full marketing authorisation if the complete data still demonstrate that the benefits of the medicine outweigh the risks.

Blincyto for patients with acute lymphoblastic leukaemia

Tagrisso for patients with non-small cell lung cancer with T790 mutation

Zykadia for patients with non-small cell lung cancer with ALK mutation

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

Medicines 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 2015, EMA adopted a positive scientific opinion for **Mosquirix**, the first vaccine for malaria to be assessed by a regulatory authority worldwide.





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. The regulatory measures range from the change to the product information to the suspension or withdrawal of a medicine.

Important new safety advice issued in 2015

Bisphosphonates and denosumab

New recommendations included in product information and patient reminder card introduced to minimise risk of osteonecrosis of the jaw

Harvoni, Sovaldi and Daklinza

New recommendations included in product information to avoid risk of slow heart rate when used with antiarrhythmic amiodarone

Adrenaline auto-injectors

Introduction of training device and audio-visual materials to ensure appropriate use of the devices

Mycophenolate

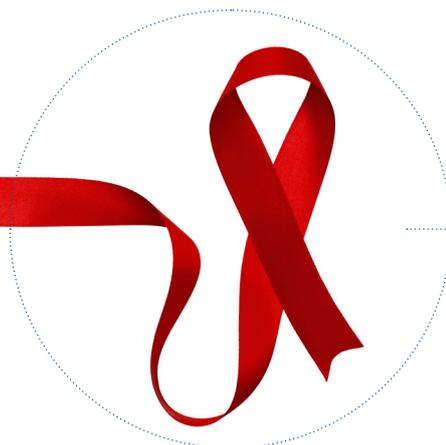
Warning included in product information to restrict use during pregnancy

Tecfidera and Gilenya

Advice to doctors to conduct certain tests before and throughout treatment to minimise the risk of progressive multifocal leukoencephalopathy (a rare brain infection) with Tecfidera and Gilenya and the risk of basal cell carcinoma with Gilenya

Updated advice for HIV patients

In 2015, EMA reviewed the product information of all [HIV medicines](#) and for most of them decided to remove certain warnings in relation to the impact on body fat changes and lactic acidosis, in light of new data available. These changes allow patients and healthcare professionals to use and prescribe these medicines in the best possible way.



Human papillomavirus (HPV) vaccines referral

EMA completed a review of the evidence surrounding reports of two syndromes, complex regional pain syndrome (CRPS) and postural orthostatic tachycardia syndrome (POTS) in young women who received [human papillomavirus vaccines](#). These vaccines are given to protect them from cervical cancer and other HPV-related cancers and pre-cancerous conditions.

EMA concluded that the available evidence does not support a causal link between the vaccines (Cervarix, Gardasil/Silgard and Gardasil 9) and development of CRPS or POTS. Therefore there is no reason to change the way the vaccines are used or amend the current product information.



Protecting EU patients – ensuring integrity of medicines’ development and manufacturing

The EU operates strict control over the supply chain bringing medicines from manufacturers to pharmacies and patients.

Companies’ systems for reporting on clinical trials or monitoring adverse drug reactions are checked by EU regulators. These checks ensure that the information supplied by pharmaceutical companies to regulatory authorities is accurate and complete to enable robust decision-making. Inspections are an important part of these controls.

Inspections conducted by EU regulatory authorities in 2015

Over 2,590 good manufacturing practice (GMP) inspections
Over 270 good clinical practice (GCP) inspections
Over 190 pharmacovigilance inspections

Where inspections have negative findings the companies have to implement strict corrective action plans agreed with inspectors.

GMP inspections led to 20 GMP non-compliance statements preventing the manufacturing sites from supplying medicines authorised in the EU, including one for a centrally authorised medicine.



High impact actions for centrally authorised medicines

A GMP inspection triggered a referral procedure resulting in the suspension of a marketing authorisation ([Inductos](#))

GCP inspections led to the withdrawal of three marketing authorisation applications prior to an opinion by the CHMP ([Aripiprazole Mylan](#), [Duloxetine Sandoz](#), [Veraseal](#))

European Medicines Agency

30 Churchill Place
Canary Wharf
London E14 5EU
United Kingdom

Telephone +44 (0)20 3660 6000

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