EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

HUMAN MEDICINES IN **2024**

An agency of the European Union

AUTHORISATION OF NEW MEDICINES

Key figures[1] on the European Medicines Agency's (EMA) recommendations for the authorisation of new medicines in 2024:

114

POSITIVE OPINIONS

5

NEGATIVE OPINIONS

8

WITHDRAWN APPLICATIONS[3]

Among the positive opinions:

46 New active substances

6 PRIME

15 Orphan medicines[2]

1 Advanced therapy medicinal product (ATMP)

28 Biosimilars

17 Generics

3 Accelerated assessments

8 Conditional marketing approvals

4 Approval under exceptional circumstances

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

[2] This figure refers to medicines that had their orphan designation confirmed by 31 December 2024. At the time of approval, orphan designations are reviewed by EMA's Committee for Orphan Medicinal Products (COMP) to determine whether the information available to date allows maintaining the medicine's orphan status.

[3] Detailed information is available on EMA's website.

MEDICINES RECOMMENDED FOR APPROVAL[4]

THERAPEUTIC AREA/ PRODUCT NAME	New active substance	PRIME	Orphan	ATMP	Biosimilar	Generic	Accelerated assesment	Conditional approval	Exceptional circumstances
Cancer									
Apexelsin						●			
Augtyro	●							●	
Avzivi					●				
Axitinib Accord						●			
Balversa	●								
Cejemly	●								
Dasatinib Accord Healthcare						●			
Elahere	●		●						
Enzalutamide Viatris						●			
Eribulin Baxter						●			
Fruzaqla	●								
Hetronifly	●		●						
Ituxredi					●				
Korjuny									
Lazcluze	●								
Loqtorzi	●								
Nilotinib Accord						●			
Ordspono	●							●	
Pomalidomide Accord						●			
Pomalidomide Krka						●			
Pomalidomide Teva						●			
Pomalidomide Zentiva						●			
Tizveni[5]									
Truqap	●								
Tuznue					●				
Vyloy	●		●						
Welireg	●							●	
Zynyz	●		●						
Cardiovascular									
Beyonttra	●								
Jeraygo	●								
Neoatricon									
Winrevair	●	●	●						
Yuvanci									

[4] Some medicines might fall into more than one therapeutic area but have been reflected only in one.

[5] The marketing authorisation holder withdrew this medicine on 5 July 2024.

THERAPEUTIC AREA/ PRODUCT NAME	New active substance	PRIME	Orphan	ATMP	Biosimilar	Generic	Accelerated assesment	Conditional approval	Exceptional circumstances
Dermatology									
Anzupgo	●								
Nemluvio	●								
Diagnostic agents									
GalliaPharm									
Siiltibcy	●								
Tauvid	●								
Theralugand									
Endocrinology									
Awiqli	●								
Emcitate			●						
Jubbonti					●				
Obodence					●				
Osenvelt					●				
Stoboclo					●				
Wyost					●				
Xbryk					●				
Zegalogue	●								
Gastroenterology/ Hepatology									
Iqirvo	●		●					●	
Kayfanda									●
Seladelpar Gilead	●	●	●					●	
Haematology/ Haemostaseology									
Adzynma	●		●						●
Alhemo	●								
Altuvoct	●		●						
Beqvez	●	●		●				●	
Eltrombopag Viatris						●			
Fabhalta	●	●	●						
Hympavzi	●								
Piasky	●								
Rytelo	●								
Ryzneuta	●								
Voydeya	●	●	●						
Zefylti					●				

THERAPEUTIC AREA/ PRODUCT NAME	New active substance	PRIME	Orphan	ATMP	Biosimilar	Generic	Accelerated assesment	Conditional approval	Exceptional circumstances
Immunology/ Rheumatology/ Transplantation									
Absimky					●				
Andembry	●								
Apremilast Accord						●			
Avtozma					●				
Eksunbi					●				
Fymskina					●				
Imuldosa					●				
Otulfi					●				
Pyzchiva					●				
Steqeyma					●				
Tofidence					●				
Wezenla					●				
Yesintek					●				
Infections									
Akantior			●						
Emblaveo							●		
Exblifep	●								
Kavigale	●						●		
Metabolism									
Agilus									
Neurology									
Buprenorphine Neuraxpharm									
Dimethyl fumarate Accord						●			
Dimethyl fumarate Mylan						●			
Dimethyl fumarate Neuraxpharm						●			
Leqembi	●								
Qalsody	●		●						●
Wainzua	●								
Ophthalmology									
Afqlir					●				
Ahzantive					●				
Baiama					●				
Eydenzelt					●				
Lytenava									
Opuviz					●				
Ranibizumab Midas					●				
Vevizye									

THERAPEUTIC AREA/ PRODUCT NAME	New active substance	PRIME	Orphan	ATMP	Biosimilar	Generic	Accelerated assesment	Conditional approval	Exceptional circumstances
Pneumology/Allergology									
Eurneffy									
Gohibic	●								●
Nintedanib Accord						●			
Omlyclo					●				
Psychiatry									
Niapelf						●			
Paxneury									
Tuzulby									
Uro-nephrology									
Filspari	●		●					●	
Obgemsa	●								
Vaccines									
Celldemic									
Fluad									
Flucelvax									
Fluenz									
Incellipan								●	
Ixchiq	●	●					●		
Kostaive	●								
mResvia	●								
Penbraya									

IMPORTANT CONTRIBUTIONS TO PUBLIC HEALTH

Authorisation of new medicines is essential to advancing public health as they bring new opportunities to treat certain diseases. Below is a selection of medicines approved in 2024 that represent significant progress in their therapeutic areas:

CANCER

Welireg (*belzutifan*)
for the treatment of tumours associated with von Hippel-Lindau disease and advanced clear cell renal cell carcinoma. This is the first medicine to treat von Hippel-Lindau disease, a rare genetic disorder causing cysts and tumours.

CARDIOVASCULAR

Winrevair (*sotatercept*)
to treat adult patients with pulmonary arterial hypertension, a rare, long-term, debilitating and life-threatening condition in which patients have abnormally high blood pressure in the arteries in the lungs.

ENDOCRINOLOGY

Emcitate (*tiratricol*)
the first treatment for peripheral thyrotoxicosis in patients with Allan-Herndon-Dudley syndrome, an ultra-rare, chronic and severely debilitating disease caused by mutations in the gene coding for the thyroid hormone transporter MCT8 protein.

HAEMATOLOGY/ HAEMOSTASEOLOGY

Beqvez (*fidanacogene elaparvovec*)
a new gene therapy treatment for haemophilia B, a rare inherited bleeding disorder.

Fabhalta (*iptacopan*)
an oral treatment for adults with paroxysmal nocturnal haemoglobinuria, a rare genetic disorder and potentially life-threatening blood disease leading to the premature destruction of red blood cells by the immune system.

Voydeya (*danicopan*)
the first oral treatment against residual haemolytic anaemia in patients with paroxysmal nocturnal haemoglobinuria.

INFECTIONS

Emblaveo (*aztreonam-avibactam*)
an antibiotic indicated for the treatment of complicated intra-abdominal and urinary tract infections, hospital-acquired pneumonia and infections caused by certain types of bacteria (aerobic Gram-negative) that are resistant to many currently available antibiotics and where patients have limited or sometimes no treatment options.

NEUROLOGY

Leqembi (*lecanemab*)
for the treatment of mild cognitive impairment (memory and thinking problems) or mild dementia due to Alzheimer's disease (early Alzheimer's disease) in patients who have only one or no copy of ApoE4, a certain form of the gene for the protein apolipoprotein E.

Qalsody (*tofersen*)
a new therapy for the treatment of adult patients with amyotrophic lateral sclerosis (ALS), a rare and often fatal disease that causes muscles to become weak and leads to paralysis. This medicine is indicated for the treatment of adults with ALS, who have a mutation in the superoxide dismutase 1 (SOD1) gene.

PNEUMOLOGY / ALLERGOLOGY

Eurneffy (*epinephrine*)
the first emergency treatment against allergic reactions that is administered as a nasal spray, not as an injection.

VACCINES

Ixchiq (*chikungunya vaccine (live)*)
the first vaccine in the EU to protect adults against disease caused by Chikungunya virus transmitted to humans by infected mosquitoes. Chikungunya is endemic in many (sub)tropical countries and causes recurrent epidemics. Due to climate change, it may also spread to regions so far spared.

COVID-19 vaccines

One new vaccine, Kostaive (zapomeran), was recommended for approval in individuals aged 18 years and older. This is the first approved self-amplifying mRNA vaccine.

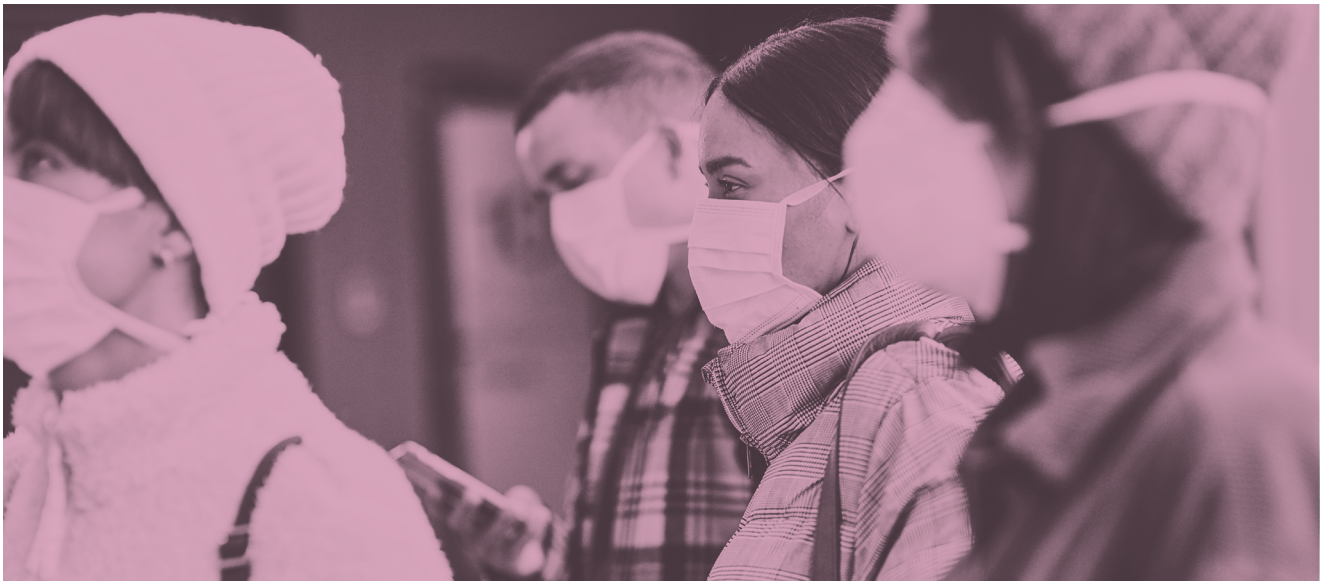
Three approved vaccines (Comirnaty, Spikevax and Nuvaxovid) were adapted to the Omicron JN.1 variant, one (Cominarty) to the KP.2 subvariant, and one (Bimervax) to the Omicron XBB.1.16 subvariant.

OPEN FRAMEWORK

Four medicines were assessed under EMA's OPEN framework that fosters international collaboration and sharing of scientific expertise to promote global public health.

	Infections
Kavigale (*sipavibart)*	
	Vaccines
Ixchiq (*chikungunya vaccine (live))*	
Kostaive (*zapomeran)*	
mResvia (*Respiratory Syncytial Virus (RSV) mRNA vaccine)*	

HIGHLIGHTS ON PUBLIC HEALTH EMERGENCIES OF INTERNATIONAL CONCERN



EMA is contributing to tackling the mpox pandemic by expediting the development and approval of safe and effective treatments and vaccines. The World Health Organization (WHO) declared a Public Health Emergency of International Concern (PHEIC) on 14 August 2024 following an outbreak of mpox in the Democratic Republic of the Congo (DRC) and in a growing number of African countries.

In September, EMA's human medicines committee (CHMP) recommended an extension of the use of the smallpox and mpox vaccine **Imvanex** (*live modified vaccinia virus Ankara*) to adolescents from 12 to 17 years of age. Imvanex protects against both the clade I and clade II mpox strains.

The Agency's assessment has important implications for the global response to the mpox outbreak. EMA is the regulatory agency of record for prequalification of this vaccine by WHO. This means that CHMP's assessment constitutes the basis for WHO prequalification approval to facilitate timely and increased access to this vaccine in communities with urgent need.

In November, EMA's Emergency Task Force (ETF) issued a recommendation on the use of Imvanex to prevent mpox in children under 12 years of age who are at risk of mpox disease during the public health emergency. The ETF considered the limited options for preventing mpox in children and data on the safety profile of Imvanex and of vaccines based on the same construct.

EARLY ACCESS TO MEDICINES THAT ADDRESS PUBLIC HEALTH NEEDS

ACCELERATED ASSESSMENTS

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

Infections

Emblaveo (*aztreonam-avibactam)*

Kavigale (*sipavibart)*

Vaccines

Ixchiq (*chikungunya vaccine (live))*

PRIORITY MEDICINES (PRIME)

The enhanced development support provided by PRIME aims at helping patients to benefit as early as possible from promising medicines that target an unmet medical need, by optimising the generation of robust data and enabling accelerated assessment. This year, **six PRIME-designated medicines** were recommended for approval:

Cardiovascular

Winrevair (*sotatercept)*

Gastroenterology

Seladelpar Gilead (*seladelpar lysine dihydrate)*

Haematology / Haemostaseology

Beqvez (*fidanacogene elaparvovec)*

Fabhalta (*iptacopan)*

Voydeya (*danicopan)*

Vaccines

Ixchiq (*chikungunya vaccine (live))*

Fourteen medicines under development were included in the scheme in 2024:

- Endocrinology - Gynaecology - Fertility - Metabolism (**4**)
- Oncology (**5**)
- Congenital, familial and genetic disorders (**2**)
- Gastroenterology - Hepatology (**1**)
- Infectious diseases (**2**)

CONDITIONAL APPROVAL

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. As these medicines address unmet medical needs the conditional authorisation allows for early approval on the basis of less complete clinical data than normally required (products for use in emergency situations may have less complete pharmaceutical or non-clinical data). These authorisations are subject to specific post-authorisation obligations to generate complete data on the medicines.

Cancer

Augtyro (*repotrectinib)*

Ordspono (*odronextamab)*

Welireg (*belzutifan)*

Gastroenterology

Iqirvo (*elafibranor)*

Seladelpar Gilead (*seladelpar lysine dihydrate)*

Haematology

Beqvez (*fidanacogene elaparvovec)*

Uro-nephrology

Filspari (*sparsentan)*

Vaccines

Incellipan (*pandemic influenza vaccine (H5N1))*

APPROVAL UNDER EXCEPTIONAL CIRCUMSTANCES

Four medicines were authorised under exceptional circumstances, a route that allows patients' access to medicines that cannot be approved under a standard authorisation as comprehensive data cannot be obtained, either because there are only very few patients with the disease, or 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.

Gastroenterology

Kayfanda (*odevixibat)*

Haematology / Haemostaseology

Adzynma (*rADAMTS13)*

Neurology

Qalsody (*tofersen)*

Pneumology / Allergology

Gohibic (*vilobelimab)*

MEDICINES FOR RARE DISEASES

The EU framework for orphan medicines aims to encourage the development and marketing of medicines for patients with rare diseases by providing incentives for developers. The assessment of orphan medicines is conducted by the Committee for Medical Products for Human Use (CHMP) using the generally applicable evaluation standards. In addition, orphan designations are reviewed by EMA's Committee for Orphan Medicinal Products (COMP) at the time of approval to determine whether the information available to date allows maintaining the medicine's orphan status and granting the medicine ten years of market exclusivity. In 2024, **15 medicines** had their orphan designation confirmed by the end of the year.

Cancer

Elahere (*mirvetuximab soravtansine)*

Hetronifly (*serplulimab)*

Vyloy (*zolbetuximab)*

Zynyz (*retifanlimab)*

Cardiovascular

Winrevair (*sotatercept)*

Endocrinology

Emcitate (*tiratricol)*

Gastroenterology

Iqirvo (*elafibranor)*

Seladelpar Gilead (*seladelpar lysine dihydrate)*

Haematology / Haemostaseology

Adzynma (*rADAMTS13)*

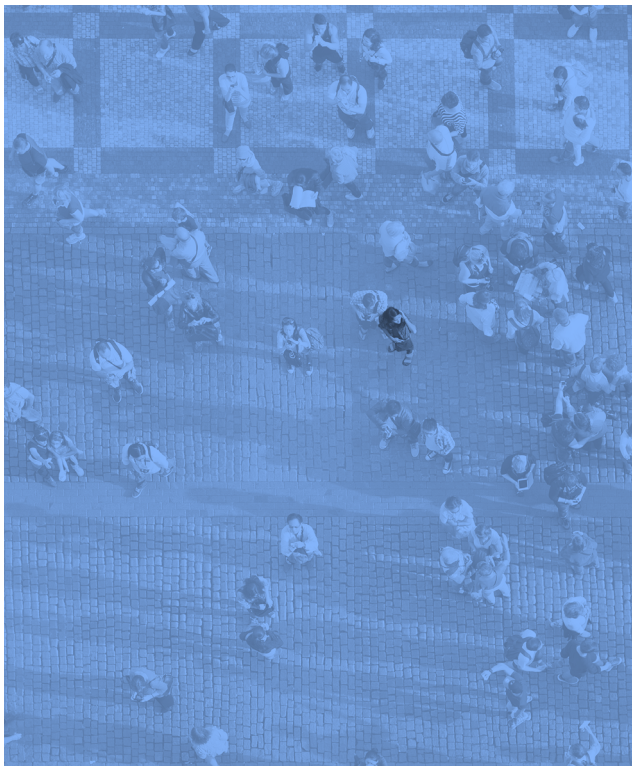
Altuvoct (*efanesoctocog alfa)*

Fabhalta (*iptacopan)*

Voydeya (*danicopan)*

Infections

Akantior (*polyhexanide)*



Neurology

Qalsody (*tofersen)*

Uro-nephrology

Filspari (*sparsentan)*

NEGATIVE OPINIONS

The Committee for Medical Products for Human Use (CHMP) adopted a negative opinion for **five medicines** in 2024. When the Committee cannot reach an agreement on a positive benefit-risk balance, it issues a negative opinion on the marketing authorisation application and elaborates on the grounds for this opinion. Applicants have the right to request a re-examination within 15 days of receipt of the notification.

Cinainu (*allium/citrus/paullinia/cacao*), for the treatment of moderate-to-severe alopecia areata, a disease causing hair loss of the scalp or other parts of the body.

Kizfizo (*temozolomide*), for the treatment of neuroblastoma, a rare cancer that forms from immature nerve cells.

Masitinib AB Science (*masitinib*), for the treatment of amyotrophic lateral sclerosis, a rare disease of the nervous system leading to loss of muscle function and paralysis.

Nezglyal (*leriglitazone*), for the treatment of paediatric and adult male patients aged two years and older with cerebral adrenoleukodystrophy, a genetic condition that damages the membrane that covers nerve cells in the brain and spinal cord.

Syfovre (*pegcetacoplan*), for the treatment of geographic atrophy secondary to age-related macular degeneration, a progressive retinal macular disease causing gradual vision impairment mainly in elderly people.

NEW USES FOR EXISTING MEDICINES

90 extensions of indication were recommended in 2024, including 40 for paediatric use[6]. The extension of the use of a medicine that is already authorised for marketing in the EU can also offer new treatment opportunities for patients. Extensions of indication included:

Ofev (*nintedanib*), for the treatment of progressive fibrosing interstitial lung diseases (ILDs) in children and adolescents from the age of six.

Pegasys (*peginterferon alfa-2a*), for the treatment of Polycythaemia Vera and essential thrombocytopenia in adults.

Xromi (*hydroxycarbamide*), for the prevention of vaso-occlusive complications of sickle-cell disease in children from the age of nine months.

[6] Most paediatric extensions of indication are based on the results of clinical studies agreed in the medicine's paediatric investigation plan (PIP).

KEEPING PATIENTS SAFE

MONITORING IN REAL-LIFE OPTIMISING SAFE AND EFFECTIVE USE

Once a medicine has been authorised, EMA and the EU Member States continuously monitor the quality, safety and the benefit-risk balance of the medicine used in clinical practice. This is to optimise how the medicine is used by patients to achieve its full benefit and to protect patients from avoidable side effects. Regulatory measures range from a change to the product information to the suspension or withdrawal of a medicine or recall of a limited number of batches.

Important new safety advice issued in 2024 included:

CAR T-cell medicines
Recommendation on the need for life-long monitoring of secondary malignancies in patients treated with these medicines.

Fluroquinolones
Recommendation to include anxiety, suicidal ideation, panic attack, neuralgia and concentration impairment as potential aspects of fluoroquinolone-induced, long-lasting and disabling adverse drug reactions.

GLP-1 receptor agonists
New measures to minimise the risk of aspiration and pneumonia aspiration in patients who undergo surgery with general anaesthesia or deep sedation.

Hydroxyprogesterone-containing medicines
Recommendation to suspend the marketing authorisations for medicines containing 17-hydroxyprogesterone caproate (17-OHPC) in the European Union (EU), because of a possible but unconfirmed risk of cancer in people exposed to 17-OHPC in the womb. In addition, the review considered new studies, which showed that 17-OHPC is not effective in preventing premature birth. There are also limited data on its effectiveness in other authorised uses.

Medroxyprogesterone acetate
New measures to minimise the risk of meningioma, a type of brain tumour. The measures include recommendations to not use this medicine in patients who have a meningioma or have had one in the past unless medroxyprogesterone acetate is needed for the treatment of an oncological indication, and monitoring symptoms of meningioma in patients taking high doses of medroxyprogesterone.

Metamizole
Updated warnings to increase awareness of agranulocytosis among patients and healthcare professionals and facilitate its early detection and diagnosis to minimise the serious outcomes of this serious side effect that can lead to serious or even fatal infections.

Mysimba (*naltrexone / bupropion*)
Recommendation to strengthen existing advice to minimise the risks from interactions with opioid-containing medicines, such as the opioid painkillers morphine and codeine, other opioids used during surgery, and certain medicines for cough, cold or diarrhoea. Opioid medicines may not work effectively in patients taking Mysimba, because naltrexone blocks the effects of opioids. There is also a risk of rare and potentially life-threatening reactions, such as seizures and serotonin syndrome (a condition that results from having too much serotonin in the body), in people taking Mysimba together with medicines for treating depression and opioids.

Ocaliva (*obeticholic acid*)
Recommendation to revoke the conditional marketing authorisation of Ocaliva, a medicine used to treat adults with a rare liver disease known as primary biliary cholangitis, because its benefits are no longer considered to outweigh its risks.

Oxycodone
New black box warning added to the existing warning in the patient leaflet stating that oxycodone is an opioid that can cause dependence and/or addiction. Dependence and addiction are important risks of oxycodone and remain of concern in the EU/EEA. The reporting rate for opioid use disorder-related events for the period 2016 to 2023 increased by around 2-fold as compared with the period from 2012 to 2015 and did not decrease in 2024.

Paxlovid (*nirmatrelvir, ritonavir*)
New warning on the co-administration of Paxlovid with certain immunosuppressants with a narrow therapeutic index such as calcineurin inhibitors (ciclosporin, tacrolimus) and mTOR inhibitors (everolimus, sirolimus) which can result in life-threatening and fatal reactions due to pharmacokinetic interactions as Paxlovid is a strong CYP3A inhibitor.

Reyataz (*atazanavir*)
New contraindications on the co-administration of Reyataz with encorafenib and ivosidenib, and with carbamazepine, phenobarbital, and phenytoin.

Valproate
New precautionary measures for the treatment of male patients with valproate medicines to address a potential increased risk of neurodevelopmental disorders in children born to men treated with valproate during the three months before conception.

Veoza (*fezolinetant*)
New recommendation to conduct liver function tests to monitor liver function before and during treatment to minimise the risk of liver injury. Treatment with Veoza should be discontinued in certain cases of transaminase and bilirubin elevations, or if liver enzyme elevations are accompanied by symptoms suggestive of liver injury.

ENSURING INTEGRITY OF CLINICAL TRIAL CONDUCT AND THE MANUFACTURE AND SUPPLY OF MEDICINES

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.

Synapse Art.31 referral re-examination
The CHMP confirmed its recommendation to suspend or not grant the marketing authorisations of a number of generic medicines tested by **Synapse Labs Pvt. Ltd**, a contract research organisation located in Pune, India. This confirmation concludes the re-examination requested by the applicants and marketing authorisation holders for some of the medicines concerned. The list of the medicines concerned is available on EMA's website.

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