Orphan medicinal products with marketing authorisation

http://ec.europa.eu/health/documents/community-register/html/index_en.htm

List of Orphan Medicinal Products with Marketing Authorisation (as of 30 April 2019)

N° CHMP + opinions*a*	N° products*b*	Medicinal Product	MA Sponsor	Authorised Therapeutic Indication	New indic
2001					
1	1	**Fabrazyme** (agalsidase beta) **EXPIRED**	Genzyme BV	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency).	
2	2	**Replagal** (agalsidase alpha) **EXPIRED**	Shire Human Genetic Therapies AB	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency).	
3	3	**Glivec** (imatinib) **EXPIRED**	Novartis Europharm Limited	Treatment of adult and **paediatric** patients with newly diagnosed Philadelphia chromosome (bcrabl) positive (Ph+) chronic myeloid leukaemia (CML) for whom bone marrow transplantation is not considered as the first line of treatment. Glivec is also indicated for the treatment of adult and paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy, or in accelerated phase or blast crisis. The effect of Glivec on the outcome of bone marrow transplantation has not been determined.	
4		**Glivec** (imatinib) **WITHDRAWN**	Novartis Europharm Limited	Glivec is also indicated for the treatment of adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumours (GIST).	2002
5		**Glivec** (imatinib) **WITHDRAWN**	Novartis Europharm Limited	Treatment of adult patients with unresectable recurrent and/or metastatic dermafibrosarcoma protuberans	2006
6		**Glivec** (imatinib) **WITHDRAWN**	Novartis Europharm Limited	Treatment of adult patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) as monotherapy	2006
7		**Glivec** (imatinib) **WITHDRAWN**	Novartis Europharm Limited	Treatment of adult patients with myelodysplastic/ myeloproliferative diseases (MDS/MPD) associated with PDGFR gene re-arrangement	2006
8		**Glivec** (imatinib) **WITHDRAWN**	Novartis Europharm Limited	Treatment of adult patients with hypereosinophilic syndrome (HES) and chronic eosinophilic leukaemia (CEL)	2006
2002					
9	4	**Trisenox** (arsenic trioxide) **EXPIRED**	Cephalon Europe	"For induction of remission and consolidation in adult patients with relapsed/refractory acute promyelocytic leukaemia (APL), characterised by the presence of the t(15;17) translocation and/or the presence of the Pro-Myelocytic Leukaemia/Retinoic-Acid-Receptor-alpha (PML/RAR-alpha) gene. Previous treatment	

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				should have included a retinoid and chemotherapy. The response rate of other acute myelogenous leukaemia subtypes to TRISENOX has not been examined."	
10	5	**Tracleer** (bosentan) **EXPIRED**	Actelion Registration Limited	"Treatment of pulmonary arterial hypertension (PAH) to improve exercise capacity and symptoms in patients with WHO functional class III.Efficacy has been shown in: - Primary (idiopathic and familial) PAH. - PAH secondary to scleroderma without significant interstitial pulmonary disease. - PAH associated with congenital systemic-to-pulmonary shunts and Eisenmenger's physiology. - Some improvements have also been shown in patients with PAH WHO functional class II."	
11		**Tracleer** (bosentan) **WITHDRAWN**	Actelion Registration Limited	Indicated to reduce the number of new digital ulcers in patients with systemic sclerosis and ongoing digital ulcer disease.	2007
12	6	**Somavert** (pegvisomant) **EXPIRED**	Pfizer Limited	Treatment of patients with acromegaly who have had an inadequate response to surgery and/or radiation therapy and in whom an appropriate medical treatment with somatostatin analogues did not normalize IGF-I concentrations or was not tolerated.	
13	7	**Zavesca** (miglustat) **EXPIRED**	Actelion Registration Limited	Zavesca is indicated for the oral treatment of mild to moderate type 1 Gaucher disease. Zavesca may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable.	
14		**Zavesca** (miglustat) **EXPIRED**	Actelion Registration Limited	Extension of Indication – to include the treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type C disease.	2009
2003					
15	8	**Carbaglu** (carglumic acid) **EXPIRED**	Orphan Europe Sarl	Treatment of hyperammonaemia due to N-acetylglutamate synthase deficiency.	
16		**Carbaglu** (carglumic acid)	Orphan Europe SARL - France	This variation concerns an extension of indication of Carbaglu to add the treatment of hyperammoniemia due to isovaleric acidaemia, methylmalonic acidaemia and propionic acidaemia. 6/2021	2011
17	9	**Aldurazyme** (laronidase) **EXPIRED**	Genzyme Europe BV	Aldurazyme is indicated for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis I (MPSI; a [alpha]-L-iduronidase deficiency) to treat the non-neurological manifestations of the disease	
18	10	**Busilvex** (busulfan) **EXPIRED**	Pierre Fabre Medicament	Busilvex followed by cyclophosphamide (BuCy2) is indicated as conditioning treatment prior to conventional haematopoietic progenitor cell transplantation (HPCT) in adult patients when the combination is considered the best available option. Busilvex followed by cyclophosphamide (BuCy4) or melphalan (BuMel) is indicated as conditioning treatment prior to conventional haematopoietic progenitor cell	

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				transplantation in **paediatric** patients.	
19	11	**Ventavis** (iloprost) **EXPIRED**	Schering AG	Treatment of patients with primary pulmonary hypertension, classified as NYHA functional class III, to improve exercise capacity and symptoms.	
20	12	**Onsenal** (celecoxib) **WITHDRAWN FROM THE MARKET - SAFETY**	Pharmacia-Pfizer EEIG	For the reduction of the number of adenomatous intestinal polyps in familial adenomatous polyposis (FAP), as an adjunct to surgery and further endoscopic surveillance.	
2004					
21	13	**Photobarr** (porfimer sodium) **WITHDRAWN FROM THE MARKET - SAFETY**	Axcan Pharma International BV	Photodynamic therapy (PDT) with porfimer sodium is indicated for ablation of high grade dysplasia (HGD) in patients with Barrett's Esophagus (BE)	
22	14	**Litak** (cladribine,B) **EXPIRED**	Lipomed GmbH	Treatment of hairy cell leukaemia	
23	15	**Lysodren** (mitotane) **EXPIRED**	Laboratoire HRA Pharma	Symptomatic treatment of advanced (unresectable, metastatic or relapsed) adrenal cortical carcinoma. The effect of Lysodren on non-functional adrenal cortical carcinoma is not established.	
24	16	**Pedea** (ibuprofen) **EXPIRED**	Orphan Europe SARL	Indicated to close a patent ductus arteriosus in preterm **newborn** infants	
25	17	**Wilzin** (zinc-acetate dihydrate) **EXPIRED**	Orphan Europe SARL	Treatment of Wilson's disease	
26	18	**Xagrid** (anegrelide hydrochloride) **EXPIRED**	Shire Pharmaceutic als Ltd	Reduction of elevated platelet counts in at risk essential thrombocythaemia patients who are intolerant to their current therapy or whose elevated platelet counts are not reduced to an acceptable level by their current therapy.	
2005					
27	19	**Prialt** (ziconotide) **EXPIRED**	Elan Pharma Int.	Treatment of chronic pain requiring intrathecal (IT) analgesia in patients who fail to obtain adequate analgesia and/or suffer intolerable adverse events with systemic opioids	
28	20	**Orfadin** (nitisinone) **EXPIRED**	Swedish Orphan Int.	Treatment of patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine.	
29	21	**Xyrem** (sodium oxybate) **WITHDRAWN**	UCB Pharma Ltd	Treatment of narcolepsy with cataplexy in adult patients.	

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30	22	**Revatio** (sidenafil citrate) **EXPIRED**	Pfizer limited	Treatment of pulmonary arterial hypertension. Revatio has been shown to improve exercise ability and to reduce mean pulmonary arterial pressure	
2006					
31	23	**Naglazyme** (N-acetylgalactosamine 4-sulfatase,A) **EXPIRED**	BioMarin Europe	Long term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis VI (MPS VI; (N-acetylgalactosamine 4-sulfatase deficiency; Maroteaux Lamy syndrome)	
32	24	**Myozyme** (recombinant human acid alpha-glucosidase **EXPIRED**	Genzyme Europe	Myozyme is indicated for long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of Pompe disease (acid alpha-glucosidase deficiency). Myozyme is indicated in adults and paediatric patients of **all ages.** In patients with late-onset Pompe disease the evidence of efficacy is limited	
33	25	**Evoltra** (clofarabine) **EXPIRED**	Genzyme Europe BV	Treatment of acute lymphoblastic leukaemia (ALL) in **paediatric** patients who have relapsed or are refractory after receiving at least two prior regimens and where there is no other treatment option anticipated to result in a durable response. Safety and efficacy have been assessed in studies of patients ≤ 21 years old at initial diagnosis	
34	26	**Nexavar** (sorafenib tosylate) **EXPIRED**	Bayer Healthcare AG	For the treatment of patients with advanced renal cell carcinoma who have failed prior interferon-alpha or interleukin-2 based therapy or are considered unsuitable for such therapy	
35		**Nexavar** (sorafenib tosylate) **EXPIRED**	Bayer Healthcare AG	Extension of Indication to include treatment of hepatocellular carcinoma.	2007
36		**Nexavar** (sorafenib tosylate)	Bayer Healthcare AG	Extension of indication for the treatment of patients with progressive, locally advanced or metastatic, differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma, refractory to radioactive iodine. 5/2024	2014
37	27	**Savene** (dexrazoxane) **EXPIRED**	SpePharm Holding BV	Treatment of anthracycline extravasation	
38	28	**Exjade** (4-(3,5-Bis (hydroxiphenyl) -1,2,4) triazol-1-yl)benzoic acid, B) **EXPIRED**	Novartis Europharm Limited	Treatment of chronic iron overload due to blood transfusions (transfusion haemosiderosis) in adult and **paediatric** patients (aged 2 years and over)	

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39	29	**Sprycel** (dasatinib) **EXPIRED**	Bristol-Myers Squibb Pharma	Treatment of adults with Philadelphia chromosome positive (Ph+) acute lymphoblastic leukaemia (ALL) and lymphoid blast CML with resistance or intolerance to prior therapy. Treatment of adults with chronic, accelerated or blast phase chronic myeloid leukaemia (CML) with resistance or intolerance to prior therapy including imatinib mesilate.	
40	30	**Sutent** (sunitinib) **WITHDRAWN**	Pfizer Ltd.	Sutent is indicated for the treatment of advanced and/or metastatic renal cell carcinoma. Sutent is indicated for the treatment of unresectable and/or metastatic malignant gastrointestinal stromal tumour (GIST) after failure of imatinib mesylate treatment due to resistance or intolerance.	
41	31	**Thelin** (sitaxentan sodium) **WITHDRAWN FROM THE MARKET - SAFETY**	Pfizer Limited.	Treatment of patients with pulmonary arterial hypertension classified as WHO functional class III, to improve exercise capacity. Efficacy has been shown in primarily pulmonary hypertension and in pulmonary hypertension associated with connective tissue disease.	
2007					
42	32	**Diacomit** (stiripentol) **EXPIRED**	BIOCODEX	Indicated for use in conjunction with clobazam and valproate as adjunctive therapy of refractory generalized tonic-clonic seizures in patients with severe myoclonic epilepsy in **infancy** (SMEI, Dravet's syndrome) whose seizures are not adequately controlled with clobazam and valproate.	
43	33	**Elaprase** (iduronate-2-sulfatase) **EXPIRED**	Shire Human Genetic Therapies AB - Sweden	"Elaprase is indicated for the long-term treatment of patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II). Heterozygous females were not studied in the clinical trials."	
44	34	**Inovelon** (rufinamide) **EXPIRED**	Esai Limited	Adjunctive therapy in the treatment of seizures associated with Lennox-Gastaut syndrome in patients **4 years** and older.	
45	35	**Cystadane** (betaine anhydrous A) **EXPIRED**	Orphan Europe	Adjunctive treatment of homocystinuria, involving deficiencies or defects in: - cystathionine beta-synthase (CBS), - 5,10-methylene-tetrahydrofolate reductase (MTHFR), - cobalamin cofactor metabolism (cbl). Cystadane should be used as supplement to other therapies such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), folate and a specific diet.	
46	36	**Revlimid** (lenalidomide) **EXPIRED**	Celgene Europe Ltd	Revlimid is indicated for the treatment of adult patients with previously untreated multiple myeloma who are not eligible for transplant. Revlimid in combination with dexamethasone is indicated for the treatment of multiple myeloma in adult patients who have received at least one prior therapy.	

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47		**Revlimid** (lenalidomide)	Celgene Europe Limited	Revlimid is indicated for the treatment of patients with transfusion-dependent anaemia due to low- or intermediate-1-risk myelodysplastic syndromes associated with an isolated deletion 5q cytogenetic abnormality when other therapeutic options are insufficient or inadequate. 06/2023	2013
48		**Revlimid** (lenalidomide)	Celgene Europe Limited	Revlimid as monotherapy is indicated for the treatment of adult patients with relapsed or refractory mantle cell lymphoma 07/2026	2016
49	37	**Soliris** (eculizumab) **EXPIRED**	Alexion Europe	Indicated in adults and **children** for the treatment of patients with paroxysmal nocturnal haemoglobinuria (PNH). Evidence of clinical benefit demonstrated in patients with haemolysis with clinical symptom(s) indicative of high disease activity, regardless of transfusion history	
50		**Soliris** (eculizumab)	Alexion Europe SAS - France	Extension Of Indication for atypical haemolytic uremic syndrome (aHUS) 11/2023	2011
51		**Soliris** (eculizumab)	Alexion Europe SAS - France	Extension Of Indication for Refractory generalized myasthenia gravis (gMG) in patients who are anti-acetylcholine receptor (AChR) antibody-positive 08/2027	2017
52	38	**Siklos** (hydroxycarbamide)	Addmedica SAS - France	"Indicated for the prevention of recurrent painful vaso-occlusive crises including acute chest syndrome in adults, adolescents and **children** older than 2 years suffering from symptomatic Sickle Cell Syndrome."	
53	39	**Increlex** (mecasermin) **EXPIRED**	Ipsen Pharma	Long-term treatment of growth failure in **children** and adolescents with severe primary insulin like growth factor-1 deficiency (Primary IGFD). Severe Primary IGFD is defined by: • height standard deviation score ≤ –3.0 and • basal IGF-1 levels below the 2.5th percentile for age and gender and • GH sufficiency. • Exclusion of secondary forms of IGF-1 deficiency, such as malnutrition, hypothyroidism, or chronic treatment with pharmacologic doses of anti-inflammatory steroids. Severe Primary IGFD includes patients with mutations in the GH receptor (GHR), post-GHR signalling pathway, and IGF-1 gene defects; they are not GH deficient, and therefore, they cannot be expected to respond adequately to exogenous GH treatment. It is recommended to confirm the diagnosis by conducting an IGF-1 generation test.	
54	40	**Atriance** (nelarabine) **EXPIRED**	Glaxo Group Ltd	Treatment of patients with T-cell acute lymphoblastic leukaemia (T-ALL) and T-cell lymphoblastic lymphoma (T-LBL) whose disease has not responded to or has relapsed following treatment with at least two chemotherapy regimens. Due to the small patient populations in these disease settings, the information to support these indications is based on limited data.	
55	41	**Gliolan** (5 aminolevulinic	Medac GmbH	Visualisation of malignant tissue during surgery for malignant glioma	

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		acid hydrochloride L) **EXPIRED**			
56	42	**Yondelis** (trabectedin) **EXPIRED**	PharmaMar SA	Treatment of patients with advanced soft tissue sarcoma, after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents. Efficacy data are based mainly on liposarcoma and leiomyosarcoma patients	
57		**Yondelis** (trabectedin)	PharmaMar SA	EXTENSION OF INDICATION Indicated for the treatment of patients with advanced soft tissue sarcoma, after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents. Efficacy data are based mainly on liposarcoma and leiomyosarcoma patients. In combination with pegylated liposomal doxorubicin (PLD) is indicated for the treatment of patients with relapsed platinum-sensitive ovarian cancer. 30/10/2019	2009
58	43	**Torisel** (temsirolimus) **EXPIRED**	Pfizer Limited	First-line treatment of patients with advanced renal cell carcinoma who have at least three of six prognostic risk factors.	
59		**Torisel** (temsiroliums) **EXPIRED**	Pfizer Limited	EXTENSION OF INDICATION to include treatment of adult patients with relapsed and/or refractory mantle cell lymphoma.	2009
60	44	**Tasigna** (nilotinib)	Novartis Europharm Ltd	Treatment of Philadelphia chromosome positive chronic myelogenous leukaemia (CML) – *2 additional years of market exclusivity as paediatric reward granted on 17 Nov 2017 – will expire in 21/11/2019*	
2008					
61	45	**Thalidomide Celgene** (thalidomide) **EXPIRED**	Celgene Europe Limited	Thalidomide Celgene in combination with melphalan and prednisone as first line treatment of patients with untreated multiple myeloma, aged ≥ 65 years or ineligible for high dose chemotherapy.	
62	46	**Volibris** (ambrisentan) **EXPIRED**	Glaxo Group Ltd	Treatment of patients with pulmonary arterial hypertension (PAH) classified as WHO functional class II and III, to improve exercise capacity.	
63	47	**Firazyr** (icatibant acetate L)	Shire Orphan Therapies GmbH	Indicated for symptomatic treatment of acute attacks of hereditary angioedema (HAE) in adults (with C1-esterase-inhibitor deficiency). - *2 additional years of market exclusivity as paediatric reward granted on 15/07/2020*	
64	48	**Ceplene** (histamine dihydrochloride) **EXPIRED**	Meda AB	Ceplene maintenance therapy is indicated for adult patients with acute myeloid leukaemia in first remission concomitantly treated with interleukin-2 (IL-2). The efficacy of Ceplene has not been fully demonstrated in patients older than age 60.	
65	49	**Kuvan** (sapropterin dihydrochloride)	Merck Serono Europe Limited	Treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients of 4 years of age and over with phenylketonuria (PKU) who have been shown to be responsive to such treatment. Kuvan is also indicated for the treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients with	

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				tetrahydrobiopterin (BH4) deficiency who have been shown to be responsive to such treatment. *2 additional years of market exclusivity as paediatric reward granted on 24/06/2015-expires 4/12/2020*	
66	50	**Vidaza** (azacitidine) **EXPIRED**	Celgene Europe Ltd-United Kingdom	Treatment of adult patients who are not eligible for haematopoietic stem cell transplantation with: • intermediate-2 and high-risk myelodysplastic syndromes (MDS) according to the International Prognostic Scoring System (IPSS), • chronic myelomonocytic leukaemia (CMML) with 10-29% marrow blasts without myeloproliferative disorder, • acute myeloid leukaemia (AML) with 20-30% blasts and multi-lineage dysplasia, according to World Health Organisation (WHO) classification.	
2009					
67	51	**Nplate** (romiplostim) **EXPIRED**	Amgen Europe BV	Indicated for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) splenectomised patients refractory to other treatments (e.g. corticosteroids, immunoglobulins). Nplate may be considered as second line treatment for adult non-splenectomised patients where surgery is contra-indicated.	
68	52	**Mepact** (mifamurtide) **EXPIRED**	Takeda France SAS	Indicated in **children**, adolescents and young adults for the treatment of high-grade resectable non-metastatic osteosarcoma after macroscopically complete surgical resection. It is used in combination with post-operative multi-agent chemotherapy	
69	53	**Peyona** (previously Nymusa, caffeine citrate	Chiesi Farmaceutici S.P.A. - Italy	Treatment of primary apnoea of premature **F**	
70	54	**Mozobil** (plerixafor) **EXPIRED**	Genzyme BV The Netherlands	Indicated in combination with G-CSF to enhance mobilisation of haematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in adults patients with lymphoma and multiple myeloma whose cells mobilise poorly.	
71	55	**Cayston** (aztreonam lysinate inhalation use) **EXPIRED**	Gilead Sciences International Ltd – UK	Suppressive therapy of chronic pulmonary infections due to *Pseudomonas aeruginosa* in patients with cystic fibrosis (CF) aged 6 years and older.	
72	56	**Rilonacept Regeneron** (formerly Arcalyst; rilonacept) **WITHDRAWN FROM THE MARKET – SAFETY**	Regeneron UK	Treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) including Familial Cold Autoinflammatory Syndrome (FCAS) and Muckle-Wells Syndrome (MWS) in adults and **children** aged 12 years and older.	
73	57	**Firdapse**	BioMarin	Symptomatic treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults.	

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		(amifampridine)	Europe Ltd	28/12/2019	
74	58	**Revolade** (eltrombopag) **WITHDRAWN**	GlaxoSmithKline Trading Services Limited – Ireland	Indicated for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Revolade may be considered as second line treatment for adult non-splenectomised patients where surgery is contraindicated.	
75	59	**Afinitor** (everolimus) **WITHDRAWN**	Novartis Europharm Ltd	Treatment of patients with advanced renal cell carcinoma, whose disease has progressed on or after treatment with VEGF-targeted therapy.	
76	60	**Ilaris** (canakinumab) **WITHDRAWN**	Novartis Europharm Ltd.	Treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) in adults, adolescents and **children** aged 4 years and older with body weight above 15 kg, including Muckle-Wells Syndrome (MWS), Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA), Severe forms of Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU) presenting with signs and symptoms beyond cold-induced urticarial skin rash.	
2010					
77	61	**Tepadina** (thiotepa)	Adienne S.r.l - Italy	Indicated, in combination with other chemotherapy medicinal products: 1) with or without total body irradiation (TBI), as conditioning treatment prior to allogeneic or autologous haematopoietic progenitor cell transplantation (HPCT) in haematological diseases in adult and **paediatric patients;** 2) when high dose chemotherapy with HPCT support is appropriate for the treatment of solid tumours in adult and paediatric patients." 03/2020	
78	62	**Arzerra** (ofatumumab) **WITHDRAWN**	Glaxo Group Limited - UK	Refractory chronic lymphocytic leukaemia (CLL): Arzerra is indicated for the treatment of CLL in patients who are refractory to fludarabine and alemtuzumab	
79		**Arzerra** (ofatumumab) **WITHDRAWN**	Glaxo Group Limited - UK	Previously untreated chronic lymphocytic leukaemia (CLL): Arzerra in combination with chlorambucil or bendamustine is indicated for the treatment of patients with CLL who have not received prior therapy and who are not eligible for fludarabine-based therapy.	2014
80	63	**VPRIV** (velaglucerase alfa)	Shire Pharmaceuticals Ireland Limited – Ireland	Treatment of type 1 Gaucher disease. *2 additional years of market exclusivity as paediatric reward granted on 29/08/2016* 08/2022	
2011					
81	64	**Esbriet** (perfenidone)	InterMune UK Ltd.	Treatment for adults with idiopathic pulmonary fibrosis 03/2021	

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82	65	**TOBI podhaler** (tobramycin)	Novartis Europharm Limited	Suppressive therapy of chronic pulmonary infection due to *Pseudomonas aeruginosa* in adults and **children** aged 6 years and older with cystic fibrosis 07/2023	
83	66	**Votubia** (everolimus)	Novartis Europharm Limited	Treatment of patients aged **3 years** and older with subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis complex (TSC) who require therapeutic intervention but are not amenable to surgery 09/2021	
84	67	**Plenadren** (hydrocortisone (modified release tablet)	ViroPharma SPRL	Treatment for adults with adrenal insufficiency 11/2021	
85	68	**Vyndaqel** (tafamidis)	Pfizer Limited - UK	Treatment of transthyretin amyloidosis in adult patients with symptomatic polyneuropathy 11/2021	
2012					
86	69	**Xaluprine** (previously known as Mercaptopurine Nova Laboratories and Novapurine)	Nova Laboratories Limited - UK	Treatment of acute lymphoblastic leukaemia (ALL) in adults, adolescents and **children** 03/2022	
87	70	**Bronchitol** (manitolum)	Pharmaxis Pharmaceuticals	Treatment of cystic fibrosis in adults aged 18 years and above as an add-on therapy to best standard of care 04/2022	
88	71	**Signifor** (pasireotide)	Novartis Europharm Limited UK	Treatment of adult patients with Cushing's disease for whom surgery is not an option or for whom surgery has failed 04/2022	
89		**Signifor** (pasireotide)	Novartis Europharm Limited UK	Treatment of adult patients with acromegaly for whom surgery is not an option or has not been curative and who are inadequately controlled on treatment with another somatostatin analogue 11/2024	2014
90	72	**Kalydeco** ivacaftor	Vertex Pharmaceuticals (U.K.) Limited	Treatment of cystic fibrosis (CF) in patients aged **12 months** (granules) and older who have mutation in the CFTR gene 07/2022	
91	73	**Jakavi** (ruxolitinib) **WITHDRAWN**	Novartis Europharm Limited - UK	Treatment of disease related splenomegaly or symptoms in adult patients with primary myelofibrosis (also known as chronic idiopathic myelofibrosis), post polycythaemia vera myelofibrosis or post essential thrombocythaemia myelofibrosis.	
92	74	**Revestive** (teduglutide)	NPS Pharma Holdings Limited	Treatment of patients **aged 1 year and above** with Short Bowel Syndrome. Patients should be stable following a period of intestinal adaptation after surgery 09/2022	
93	75	**NovoThirteen** (catridecacog) **WITHDRAWN**	Novo Nordisk A/S	Long-term prophylactic treatment of bleeding in adult and paediatric patients 6 years and above with congenital factor-XIII-A-subunit deficiency.	
94	76	**Dacogen** (decitabine)	Janssen-Cilag International NV - Belgium	Treatment of adult patients aged 65 years and above with newly diagnosed de novo or secondary acute myeloid leukaemia (AML), according to the WHO classification, who are	

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				not candidates for standard induction chemotherapy" 09/2022	
95	77	**Glybera** (adeno-associated viral vector expressing lipoprotein lipase	uniQure biopharma B.V. - The Netherlands	Glybera is indicated for adult patients diagnosed with familial lipoprotein lipase deficiency (LPLD) and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. The diagnosis of LPLD has to be confirmed by genetic testing. The indication is restricted to patients with detectable levels of LPL protein 09/2022	
96	78	**Adcetris** (brentuximab vedotin)	Takeda Pharma A/S, Danmark	Adcetris is indicated for the treatment of adult patients with relapsed or refractory CD30+ H83 (Hodgkin's lymphoma): 1.following autologous stem-cell transplant (ASCT) or; 2.following at least two prior therapies when ASCT or multi-agent chemotherapy is not a treatment option 10/2022	
97		**Adcetris** (brentuximab vedotin)	Takeda Pharma A/S, Danmark	Adcetris is indicated for the treatment of adult patients with relapsed or refractory systemic anaplastic large cell lymphoma (sALCL) 10/2022	
98		**Adcetris** (brentuximab vedotin)	Takeda Pharma A/S, Danmark	ADCETRIS is indicated for the treatment of adult patients with CD30+ cutaneous T-cell lymphoma (CTCL) after at least 1 prior systemic therapy 12/2027	2017
99	79	**NexoBrid** (concentrate of proteolytic enzymes enriched in bromelain)	MediWound Germany GmbH	NexoBrid is indicated for removal of eschar in adults with deep partial- and full-thickness thermal burns 12/2022	
2013					
100	80	**Bosulif** (bosutinib) **WITHDRAWN**	Pfizer Limited United Kingdom	Treatment of chronic myeloid leukaemia	
101	81	**Iclusig** (ponatinib)	ARIAD Pharma Ltd - UK	Iclusig is indicated in adult patients with: 1) chronic-phase, accelerated-phase or blast-phase chronic myeloid leukaemia (CML) who are resistant to dasatinib or nilotinib, who are intolerant to dasatinib or nilotinib and for whom subsequent treatment with imatinib is not clinically appropriate, or who have the T315I mutation; 07/2023	
102		**Iclusig** (ponatinib)	ARIAD Pharma Ltd - UK	2) Philadelphia-chromosome-positive acute lymphoblastic leukaemia (Ph+ ALL) who are resistant to dasatinib, who are intolerant to dasatinib and for whom subsequent treatment with imatinib is not clinically appropriate, or who have the T315I mutation 07/2023	
103	82	**Imnovid** (previously Pomalidomide Celgene; pomalidomide)	Celgene Europe Limited - UK	Pomalidomide Celgene in combination with dexamethasone is indicated in the treatment of adult patients with relapsed and refractory multiple myeloma who have received at least two prior treatment regimens, including both lenalidomide and bortezomib, and have demonstrated disease progression on the last therapy 08/2023	
104	83	**Procysbi** (cysteamine bitartrate)	Raptor Pharmaceuticals Europe BV - The	Probysbi is indicated for the treatment of proven nephropathic cystinosis. Cysteamine reduces cystine accumulation in some cells (e.g. leukocytes, muscle and liver cells) of nephropathic cystinosis patients and, when treatment is started early, it delays the	

N° CHMP + opinions*a*	N° products*b*	Medicinal Product	MA Sponsor	Authorised Therapeutic Indication	New indic
			Netherlands	development of renal failure 08/2023	
105	84	**Orphacol** (cholic acid)	Laboratoires CTRS	Orphacol is indicated for the treatment of inborn errors in primary bile-acid synthesis due to 3β-hydroxy-Δ5-C27-steroid oxidoreductase deficiency or Δ4-3-oxosteroid-5β-reductase deficiency in infants, **children** and adolescents aged 1 month to 18 years and adults 09/2023	
106	85	**Defitelio** (defibrotide)	Gentium S.p.A. - Italy	Defitelio is indicated for the treatment of severe hepatic veno-occlusive disease (VOD) also known as sinusoidal obstructive syndrome (SOS) in haematopoietic stem-cell transplantation (HSCT) therapy. It is indicated in adults and in adolescents, **children** and infants over 1 month of age 10/2023	
107	86	**Opsumit** (macitentan)	Janssen-Cilag International NV	Opsumit, as monotherapy or in combination, is indicated for the long-term treatment of pulmonary arterial hypertension (PAH) in adult patients of WHO Functional Class (FC) II to III 12/2023	
108	87	**Jinarc** (tolvaptan) **WITHDRAWN**	Otsuka Pharmaceutical Europe Ltd	Autosomal dominant polycystic kidney disease	
2014					
109	88	**Sirturo** (bedaquiline fumarate)	Janssen-Cilag International N.V. Belgium	Indicated for use as part of an appropriate combination regimen for pulmonary multidrug resistant tuberculosis (MDR TB) in adult patients when an effective treatment regimen cannot otherwise be composed for reasons of resistance or tolerability 03/2024	
110	89	**Cometriq** (cabozantinib)	TMC Pharma	Treatment of adult patients with progressive, unresectable locally advanced or metastatic medullary thyroid carcinoma 03/2024	
111	90	**Adempas** (riociguat)	Bayer Pharma AG	Treatment of adult patients with Chronic thromboembolic pulmonary hypertension (CTEPH) and Pulmonary arterial hypertension (PAH) 03/2024	
112	91	**Kolbam** (cholic acid) **WITHDRAWN**	FGK Representative Service GmbH, Germany	Inborn errors in primary bile-acid synthesis	
113		**Kolbam** (cholic acid)	Retrophin Europe Ltd	Inborn errors in primary bile-acid synthesis due to Sterol 27-hydroxylase (presenting as cerebrotendinous xanthomatosis, CTX) deficiency, 2- (or α-) methylacyl-CoA racemase (AMACR) deficiency or Cholesterol 7α-hydroxylase (CYP7A1) deficiency in infants, **children** and adolescents aged 1 month to 18 years and adults 11/2025	2015
114	92	**Granupas** (previously para-aminosalicylic acid Lucane or PAS-GR)	Lucane Pharma SA - France	Treatment of tuberculosis in adults and **paediatric** patients from 28 days of age and older when an effective treatment regimen cannot otherwise be composed for reasons of resistance or tolerability 04/2024	
115	93	**Deltyba** (delamanid)	Otsuka Novel Products GmbH -	Treatment of multidrug-resistant tuberculosis (MDR-TB) in adults 04/2024	

N° CHMP + opinions*a*	N° products*b*	Medicinal Product	MA Sponsor	Authorised Therapeutic Indication	New indic
			Germany		
116	94	**Vimizim** (elosulfase alfa)	BioMarin Europe Ltd	Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome, MPS IVA) in patients of **all ages** 04/2024	
117	95	**Sylvant** (siltuximab)	Janssen-Cilag International NV	Treatment of adult patients with multicentric Castleman's disease (MCD who are human immunodeficiency virus (HIV) negative and human herpesvirus-8 (HHV-8) negative 05/2024	
118	96	**Gazyvaro** (obinutuzumab)	Roche Registration Ltd	Gazyvaro in combination with chlorambucil is indicated for the treatment of adult patients with previously untreated chronic lymphocytic leukaemia (CLL) and with comorbidities making them unsuitable for full-dose fludarabine based therapy 07/2024	
119		**Gazyvaro** (obinutuzumab)	Roche Registration Ltd	Gazyvaro in combination with chemotherapy, followed by Gazyvaro maintenance therapy in patients achieving a response, is indicated for the treatment of patients with previously untreated advanced Follicular lymphoma FL 06/2026	2016
120	97	**Translarna** (ataluren)	PTC Therapeutics Limited	Translarna is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged **5 years** and older. Efficacy has not been demonstrated in non-ambulatory patients. The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing 08/2024	
121	98	**Imbruvica** (ibrutinib)	Janssen-Cilag International NV	Imbruvica is indicated for the treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL) 10/2024	
122		**Imbruvica** (ibrutinib)	Janssen-Cilag International NV	Imbruvica is indicated for the treatment of adult patients with chronic lymphocytic leukaemia (CLL) who have received at least one prior therapy, or in first line in the presence of 17p deletion or TP53 mutation in patients unsuitable for chemo immunotherapy 10/2024	
123		**Imbruvica** (ibrutinib)	Janssen-Cilag International NV	Imbruvica is indicated for the treatment of adult patients with Waldenström's macroglobulinaemia (WM) who have received at least one prior therapy, or in first line treatment for patients unsuitable for chemo-immunotherapy 07/2025	2015
124	99	**Ketoconazole HRA** (ketoconazole)	Laboratoire HRA Pharma	Ketoconazole HRA is indicated for the treatment of endogenous Cushing's syndrome in adults and adolescents above the **age of 12 years** 11/2024	
125	100	**Lynparza** (olaparib) **WITHDRAWN**	AstraZeneca AB	Lynparza is indicated as monotherapy for the maintenance treatment of adult patients with platinum-sensitive relapsed BRCA-mutated (germline and/or somatic) high grade serous epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in response (complete response or partial response) to platinum-based chemotherapy	
126	101	**Cyramza** (ramucirumab) **WITHDRAWN**	Eli Lilly Nederland B.V.	In combination with paclitaxel is indicated for the treatment of adult patients with advanced gastric cancer or gastro-oesophageal junction adenocarcinoma with disease progression after prior platinum and	

N° CHMP + opinions*a*	N° products*b*	Medicinal Product	MA Sponsor	Authorised Therapeutic Indication	New indic
				fluoropyrimidine chemotherapy. Monotherapy is indicated for the treatment of adult patients with advanced gastric cancer or gastro-oesophageal junction adenocarcinoma with disease progression after prior platinum or fluoropyrimidine chemotherapy, for whom treatment in combination with paclitaxel is not appropriate.	
127	102	**Scenesse** (afamelanotide)	Clinuvel UK Limited	Prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP) 12/2024	
2015					
128	103	**Ofev** (nibtedanib)	Boehringer Ingelheim Pharma GmbH & Co. KG	Ofev is indicated in adults for the treatment of Idiopathic Pulmonary Fibrosis (IPF) 01/2025	
129	104	**Cerdelga** (eliglustat)	Genzyme Europe BV	Cerdelga is indicated for the long-term treatment of adult patients with Gaucher disease type 1 (GD1), who are CYP2D6 poor metabolisers (PMs), intermediate metabolisers (IMs) or extensive metabolisers (EMs) 01/2025	
130	105	**Holoclar** (*ex vivo* expanded autologous human corneal epithelial cells containing stem cells)	Chiesi Farmaceutici S.p.A.	Treatment of adult patients with moderate to severe limbal stem cell deficiency (defined by the presence of superficial corneal neovascularisation in at least two corneal quadrants, with central corneal involvement, and severely impaired visual acuity), unilateral or bilateral, due to physical or chemical ocular burns. A minimum of 1-2 mm2 of undamaged limbus is required for biopsy 02/2025	
131	106	**Lenvima** (lenvatinib mesylate) **WITHDRAWN**	Eisai Europe Ltd	Lenvima is indicated for the treatment of adult patients with progressive, locally advanced or metastatic, differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma (DTC), refractory to radioactive iodine (RAI)	
132		**Lenvima** (lenvatinib mesylate) **WITHDRAWN**	Eisai Europe Ltd	Lenvima is indicated as monotherapy for the treatment of adult patients with advanced or unresectable hepatocellular carcinoma (HCC)	
133	107	**Hetlioz** (tasimelteon)	Vanda Pharmaceuticals Ltd	Hetlioz is indicated for the treatment of Non-24-Hour Sleep-Wake Disorder (Non-24) in totally blind adults 07/2025	
134	108	**Unituxin** (dinutuximab) **WITHDRAWN**	United Therapeutics Europe Ltd	Unituxin is indicated for the treatment of high-risk neuroblastoma in **children** aged 12 months to 17 years. who have previously received induction chemotherapy and achieved at least a partial response, followed by myeloablative therapy and autologous stem cell transplantation. It is administered in combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), interleukin-2 (IL-2), and isotretinoin	
135	109	**Kanuma** (sebelipase alfa)	Synageva BioPharma Ltd	Kanuma is used to treat patients of **all ages** with lysosomal acid lipase deficiency 9/2025	
136	110	**Farydak** (panobinostat)	Novartis Europharm Ltd	Farydak is indicated in combination with bortezomib and dexamethasone, for the treatment of relapsed and/or refractory multiple myeloma in adults patients who	

N° CHMP + opinions*a*	N° products*b*	Medicinal Product	MA Sponsor	Authorised Therapeutic Indication	New indic
				have received at least two prior regimens including bortezomib and an immunomodulatory agent 9/2025	
137	111	**Strensiq** (asfotase alfa)	Alexion Europe SAS	Strensiq is indicated for long-term enzyme replacement therapy in patients with **paediatric**-onset hypophosphatasia to treat the bone manifestations of the disease 9/2025	
138	112	**Raxone** (ibedenone)	Santera Pharmaceuticals Gmbh	Raxone is indicated for the treatment of visual impairment in **adolescent** and adult patients with Leber's Hereditary Optic Neuropathy 10/2025	
139	113	**Cresemba** (isavuconazole)	Basilea Medical Ltd	Cresemba is indicated for the treatment of adults with invasive aspergillosis 10/2025	
140		**Cresemba** (isavuconazole)	Basilea Medical Ltd	Treatment of mucormycosis in patients for whom amphotericin B is inappropriate 10/2025	
141	114	**Kyprolis** (carfilzomib)	Amgen Europe BV	Kyprolis is indicated for the treatment of adult patients with multiple myeloma 11/2025	
142	115	**Orkambi** (lumacaftor/ ivacaftor) **WITHDRAWN**	Vertex Pharmaceuticals	Orkambi is indicated for the treatment of cystic fibrosis	
143	116	**Obizur** (susoctog alfa) **WITHDRAWN**	Baxalta Innovations GmbH	Obizur is indicated for the treatment of haemophilia A	
144	117	**Elocta** (efmoroctocog alfa) **WITHDRAWN**	Biogen Idec Ltd	Elocta is indicated for the treatment of haemophilia A	
145	118	**Blincyto** (blinatumomab)	Amgen Europe B.V.	Blincyto is indicated as monotherapy for the treatment of **paediatric patients** aged 1 year or older with Philadelphia chromosome negative CD19 positive B-precursor ALL which is refractory or in relapse after receiving at least two prior therapies or in relapse after receiving prior allogeneic hematopoietic stem cell transplantation 11/2025	
146	119	**Ravicti** (glycerol phenylbutyrate)	Horizon Therapeutics Limited	Ravicti is indicated for the treatment of inborn urea cycle disorders (UCDs), including CPS, OTC, ASS, ASL, ARG and HHH 11/2025	
147	120	**Quinsair** (levofloxacin) **WITHDRAWN**	Regintel	Cystic fibrosis	
2016					
148	121	**Coagadex** (human coagulation factor X)	Bio Products Laboratory limited	Treatment and prophylaxis of bleeding episodes and for perioperative management in **all age groups** patients with hereditary factor X deficiency 03/2026	
149	122	**Wakix** (pitolisant)	Bioprojet Pharma	Wakix is indicated in adults for the treatment of narcolepsy with or without cataplexy 04/2026	
150	123	**Idelvion** (albutrepenonacog alfa)	CSL Behring GmbH	Treatment and prophylaxis of bleeding in **all age groups** patients with haemophilia B (congenital factor IX	

N° CHMP + opinions*a*	N° products*b*	Medicinal Product	MA Sponsor	Authorised Therapeutic Indication	New indic
				deficiency) 06/2026	
151	124	**Uptravi** (selexipag) **WITHDRAWN**	Actelion Registration Ltd	Pulmonary arterial hypertension	
152	125	**Alprolix** (eftrenacog alfa)	Biogen Idec Ltd	Treatment and prophylaxis of bleeding in **all age groups** patients with haemophilia B (congenital factor IX deficiency) 05/2026	
153	126	**Darzalex** (daratumumab)	Janssen-Cilag International N.V.	Treatment of plasma cell myeloma in adults patients 05/2026	
154	127	**Galafold** (migalastat hydrochloride)	Amicus Therapeutics UK Ltd	Galafold is indicated for long-term treatment of adults and **adolescents** aged 16 years and older with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation 05/2026	
155	128	**Strimvelis** (autologous CD34+ + cells transduced with retroviral vector encoding for the human adenosine deaminase (ADA))	GlaxoSmithKline Trading Services Limited	Severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID) 05/2028	
156	129	**Zalmoxis** (allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor and the herpes simplex I virus thymidine kinase)	MolMed SpA	Adjunctive treatment in haematopoietic stem cell transplantation (HSCT) of adult patients with high-risk haematological malignancies 08/2026	
157	130	**Onivivyde** (irinotecan hydrochloride trihydrate)	Baxalta Innovations GmbH	Metastatic adenocarcinoma of the pancreas, in combination with 5 fluorouracil (5 FU) and leucovorin (LV), in adult patients who have progressed following gemcitabine based therapy 10/2026	
158	131	**Lartruvo** (olaratumab) **WITHDRAWN FROM THE MARKET**	Eli Lilly Nederland B.V.	Treatment of adult patients advanced soft tissue sarcoma 11/2026	
159	132	**Ninlaro** (ixazomib)	Takeda Pharma A/S	NINLARO in combination with lenalidomide and dexamethasone is indicated for the treatment of adult patients with multiple myeloma who have received at least one prior therapy 11/2026	
160	133	**Venclyxto** (venetoclax) **WITHDRAWN**	AbbVie Ltd	Chronic lymphocytic leukemia	

N° CHMP + opinions*a*	N° products*b*	Medicinal Product	MA Sponsor	Authorised Therapeutic Indication	New indic
161	134	**Ocaliva** (obeticholic acid)	Intercept Pharma Ltd	Primary biliary cholangitis in adults 12/2026	
162	135	**SomaKit TOC** (edotreotide)	Advanced Accelerator Applications	Diagnosis of gastro-entero-pancreatic neuroendocrine tumours 12/2026	
2017					
163	136	**Cystadrops** (mercaptamine)	Orphan Europe S.A.R.L.	Treatment of corneal cystine crystal deposits in adults and **children** from 2 years of age with cystinosis 01/2027	
164	137	**Ledaga** (chlormethine)	Actelion Registration Ltd	Ledaga is indicated for the topical treatment of mycosis fungoides-type cutaneous T-cell lymphoma (MF type CTCL) in adult patients 03/2027	
165	138	**Natpar** (parathyroid hormone)	Shire Pharmaceutic als Ireland Ltd	Natpar is indicated as adjunctive treatment of adult patients with chronic hypoparathyroidism who cannot be adequately controlled with standard therapy alone 04/2027	
166	139	**Qarziba** *(previously Dinutuximab beta EUSA and Dinutuximab beta Apeiron)* (dinutuximab beta)	EUSA Pharma (Netherlands) B.V.	Neuroblastoma (in patients over **1 year** of age) 11/2027	
167	140	**Spinraza** (nusinersen)	Biogen Idec Ltd	5q Spinal muscular atrophy 06/2027	
168	141	**Brineura** (cerliponase alfa)	Biomarin International Limited	Neuronal ceroid lipofuscinosis type 2 (CLN2) disease 06/2027	
169	142	**Besponsa** (inotuzumab ozogamicin)	Pfizer Limited	Treatment of adults with Philadelphia chromosome positive (Ph+) relapsed or refractory CD22-positive B cell precursor acute lymphoblastic leukaemia (ALL) 03/2027	
170	143	**Oxervate** (recombinant human nerve growth factor - cenegermin)	Dompe farmaceutici s.p.a.	Treatment of moderate (persistent epithelial defect) or severe (corneal ulcer) neurotrophic keratitis in adults 10/2027	
171	144	**Bavencio** (avelumab)	Merck Serono Europe Limited	Bavencio is indicated as monotherapy for the treatment of adult patients with metastatic Merkel cell carcinoma (MCC) 09/2027	
172	145	**Rydapt®** (midostaurin)	Novartis Europharm Ltd	Adult patients with acute myeloid leukemia (AML) 09/2027	
173		**Rydapt®** (midostaurin)	Novartis Europharm Ltd	Adult patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with associated haematological neoplasm (SM AHN), or mast cell leukaemia (MCL) 09/2027	
174	146	**Xermelo®** (telotristat etiprate)	Ipsen Pharma	Treatment of carcinoid syndrome diarrhoea in combination with somatostatin analogue (SSA) therapy in adults inadequately controlled by SSA therapy 09/2027	
175	147	**Lutathera** (lutetium	Advanced Accelerator	Lutathera is indicated for the treatment of unresectable or metastatic,	

N° CHMP + opinions*a*	N° products*b*	Medicinal Product	MA Sponsor	Authorised Therapeutic Indication	New indic
		(177Lu) oxodotreotide)	Applications	progressive, well differentiated (G1 and G2), somatostatin receptor positive gastroenteropancreatic neuroendocrine tumours (GEP NETs) in adults 09/2027	
176	148	**Zejula**® (niraparib)	Tesaro UK limited	Zejula is indicated as monotherapy for the maintenance treatment of adult patients with platinum sensitive relapsed high grade serous epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in response (complete or partial) to platinum based chemotherapy 11/2027	
177	149	**Chenodeoxycholic acid Leadiant** (chenodeoxycholic acid)	Leadiant GmbH	Treatment of inborn errors of primary bile acid synthesis due to sterol 27 hydroxylase deficiency (presenting as cerebrotendinous xanthomatosis (CTX)) in infants, **children** and adolescents aged 1 month to 18 years and adults 12/2027	
2018					
178	150	**Jorveza**® (budesonide)	Dr. Falk Pharma GmbH	Eosinophilic esophagitis (EoE) in adults 1/2028	
179	151	**Prevymis**® (letermovir)	Merck Sharp & Dohme Limited	Prophylaxis of cytomegalovirus (CMV) reactivation and disease in adult CMV-seropositive recipients [R+] of an allogeneic haematopoietic stem cell transplant (HSCT) 1/2028	
180	152	**Crysvita**® (burosumab)	Kyowa Kirin Limited	X-linked hypophosphataemia (to be used in **children** over 1 year of age and adolescents) 3/2028	
181	153	**Lamzede**® (velmanase alfa)	Chiesi Farmaceutici S.p.A.	Enzyme replacement therapy for the treatment of non-neurological manifestations in patients with mild to moderate alpha-mannosidosis (to be used in **children** aged 6 years and older, and adults) 3/2028	
182	154	**Alofisel**® (darvadstrocel)	Tigenix, S.A.U.	Complex perianal fistulas in adults with Crohn's disease 4/2028	
183	155	**Mylotarg**® (gemtuzumab ozogamicin)	Pfizer Limited	Indicated for combination therapy with daunorubicin (DNR) and cytarabine (AraC) for the treatment of patients age 15 years and above with previously untreated, de novo CD33 positive acute myeloid leukaemia (AML), except acute promyelocytic leukaemia (APL) 4/2028	
184	156	**Amglidia**® (glibenclamide)	Ammtek	Neonatal diabetes mellitus **(newborns, infants and children)** 5/2028	
185	157	**Rubraca**® (rucaparib) **WITHDRAWN and waiting for EC decision for a new indication**	Clovis Oncology UK Limited	High-grade cancers of the ovary, fallopian tubes and peritoneum. New indication 2018_ maintenance treatment of adult patients with platinum-sensitive relapsed high-grade epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in response (complete or partial) to platinum-based chemotherapy	2018?
186	158	**Verkazia**® (ciclosporin)	Santen Oy	Treatment of severe vernal keratoconjunctivitis (VKC) in **children** from 4 years of age and adolescents	

N° CHMP + opinions*a*	N° products*b*	Medicinal Product	MA Sponsor	Authorised Therapeutic Indication	New indic
				7/2030	
187	159	**Tegsedi®** (inotersen sodium)	Akcea Therapeutics Ireland Ltd	Treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR) 07/2028	
188	160	**Myalepta®** (metreleptin)	Aegerion Pharmaceutic als B.V.	Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients with or acquired partial LD (**Barraquer-Simons syndrome**), in adults and children 12 years of age and above for whom standard treatments have failed to achieve adequate metabolic control 08/2028	
189		**Myalepta®** (metreleptin)	Aegerion Pharmaceutic als B.V.	Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients with confirmed congenital generalised LD (**Berardinelli-Seip syndrome**) 08/2028	
190		**Myalepta®** (metreleptin)	Aegerion Pharmaceutic als B.V.	Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients with or acquired generalised LD (**Lawrence syndrome**) in adults and children 2 years of age and above 08/2028	
191		**Myalepta®** (metreleptin)	Aegerion Pharmaceutic als B.V.	Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients with confirmed **familial partial LD** 08/2028	
192	161	**Vyxeos®** (daunorubicin / cytarabine)	Jazz Pharmaceutic als Ireland Limited	Adults with newly diagnosed, t herapy-related acute myeloid leukaemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC) 08/2028	
193	162	**Mepsevii®** (vestronidase alfametreleptin)	Ultragenyx Germany GmbH	Non-neurological manifestations Mucopolysaccharidosis type VII (MPS VII, also known as Sly syndrome) 08/2028	
194	163	**Kymriah®** (tisagenlecleucel)	Novartis Europharm Limited	Kymriah is indicated for the treatment of **paediatric** and young adult patients up to 25 years of age with B-cell acute lymphoblastic leukaemia (ALL) 8/2028	
195		**Kymriah®** (tisagenlecleucel)	Novartis Europharm Limited	Adult patients with relapsed or refractory diffuse large B cell lymphoma (DLBCL) 08/2028	
196	164	**Yescarta®** (axicabtagene ciloleucel)	Kite Pharma EU B.V.	Treatment of adult primary mediastinal large B-cell lymphoma (PMBCL), after two or more lines of systemic therapy.lymphoma (PMBCL) 08/2028	
197		**Yescarta®** (axicabtagene ciloleucel)	Kite Pharma EU B.V.	Treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) 08/2028	
198	165	**Onpattro®**	Alnylam Netherlands	Hereditary transthyretin-mediated amyloidosis (hATTR) in adult patients	

N° CHMP + opinions*a*	N° products*b*	Medicinal Product	MA Sponsor	Authorised Therapeutic Indication	New indic
		(patisiran)	B.V.	with stage 1 or stage 2 polyneuropathy 08/2028	
199	166	**Cablivi®** (caplacizumab)	Ablynx NV	Adults experiencing an episode of acquired thrombotic thrombocytopenic purpura (aTTP), in conjunction with plasma exchange and immunosuppression 09/2028	
200	167	**Symkevi®** (patisiran)	Vertex Pharmaceuticals (Europe) Ltd	Cystic fibrosis in patients aged **12 years** and above 11/2028	
201	168	**Takhzyro** (lanadelumab)	Shire Pharmaceuticals Ireland Limited	Indicated for routine prevention of recurrent attacks of hereditary angioedema (HAE) in patients aged **12 years and older** 11/2028	
202	169	**Poteligeo** (Mogamulizumab)	Kyowa Kirin Holdings B.V.	Treatment of adult patients with mycosis fungoides (MF) or Sézary syndrome (SS) who have received at least one prior systemic therapy 11/2028	
203	170	**Luxturna®** (voretigene neparvovec)	Spark Therapeutics Ireland Ltd	Adults and **children** with loss of vision due to inherited retinal dystrophies 1) Leber's congenital amaurosis 2) retinitis pigmentosa 12/2028	
204	171	**Namuscla®** (mexiletine hcl)	Lupin Europe GmbH	Symptomatic treatment of myotonia in adult patients with non-dystrophic myotonic disorders 12/2028	
2019					
205	172	**Palynziq®** (pegvaliase)	BioMarin International Limited	Phenylketonuria (PKU)	
206	173	**Waylivra®** (volanesorsen)	Akcea Therapeutics Ireland Limited	Familial chylomicronaemia syndrome (FCS)	
207	174	**Zynteglo®** (Autologous CD34+ cells encoding βA-T87Q-globin gene)	bluebird bio (Netherlands) B.V.	beta-Thalassemia	
208	175	**Epidyolex®** (Cannabidiol)	GW Pharma (International) B.V.	Lennox-Gastaut and Dravet syndromes	

a = Number of positive CHMP opinions; ***b*** =Number of *different* products ***c*** = International Non-proprietary Name (INN)

EXPIRED product reached the end of the period of market exclusivity

WITHDRAWN: withdrawn from the Community Register of orphan medicinal products upon request of the marketing authorisation holder

WITHDRAWN FROM THE MARKET – SAFETY withdrawn from the market in the European Union due to safety reasons

XX/XXXX Date that the market exclusivity will end

Executive Agency for Health and Consumers

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