

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 21 May 2022)

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WITHDRAWN: withdrawn from the Community Register of orphan medicinal products upon request of the marketing authorisation holder

WITHDRAWN FROM THE MARKET – SAFETY withdrawn from the market in the European Union due to safety reasons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

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4		Glivec (imatinib) WITHDRAWN	Novartis Europharm Limited	Glivec is also indicated for the treatment of adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant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5		Glivec (imatinib) WITHDRAWN	Novartis Europharm Limited	Treatment of adult patients with unresectable recurrent and/or metastatic dermafibrosarcoma protuberans	2006
6		Glivec (imatinib) WITHDRAWN	Novartis Europharm Limited	Treatment of adult patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) as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7		Glivec (imatinib) WITHDRAWN	Novartis Europharm Limited	Treatment of adult patients with myelodysplastic/ myeloproliferative diseases (MDS/MPD) associated with PDGFR gene re-arrangement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 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) EXPIRED	Orphan Europe SARL - France	This variation concerns an extension of indication of Carbaglu to add the treatment of hyperammonemia due to isovaleric acidaemia, methylmalonic acidaemia and propionic acidaemia	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 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 Pharmaceuticals 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)	Swedish Orphan Int.	Treatment of patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT-1) in combination with dietary	

		EXPIRED		restriction of tyrosine and phenylalanine.	
29	21	Xyrem (sodium oxybate) WITHDRAWN	UCB Pharma Ltd	Treatment of narcolepsy with cataplexy in adult patients.	
30	22	Revatio (sildenafil 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(hydroxyphenyl)-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)	

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.

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 acid hydrochloride)	Medac GmbH	Visualisation of malignant tissue during surgery for malignant glioma	

		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) EXPIRED	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.	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 (temsirolimus) 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) EXPIRED	Novartis Europharm Ltd	Treatment of Philadelphia chromosome positive chronic myelogenous leukaemia (CML) – <i>2 additional years of market exclusivity as paediatric reward granted on 17 Nov 2017 – will expire in</i>	
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) EXPIRED	Shire Orphan Therapies GmbH	Indicated for symptomatic treatment of acute attacks of hereditary angioedema (HAE) in adults (with C1-esterase-inhibitor deficiency). - <i>2 additional years of market exclusivity as paediatric reward granted</i>	
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) EXPIRED	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 tetrahydrobiopterin (BH4) deficiency who have been shown to be responsive to such treatment. <i>2 additional years of market exclusivity as paediatric reward granted on 24/06/2015-expires</i>	

66	50	Vidaza (azacitidine) EXPIRED	Celgene Europe Ltd- United Kingdom	Treatment of adult patients who are not eligible for haematopoietic stem cell transplantation with: <ul style="list-style-type: none"> 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, <ul style="list-style-type: none"> 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 <i>Pseudomonas aeruginosa</i> 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 (amifampridine) EXPIRED	BioMarin Europe Ltd	Symptomatic treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults.
74	58	Revolade (eltrombopag) WITHDRAWN	GlaxoSmithKli ne Trading Services Limited –	Indicated for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Revolade may be

			Ireland	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."	
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 Pharmaceutic als Ireland Limited – Ireland	Treatment of type 1 Gaucher disease. <i>2 additional years of market exclusivity as paediatric reward granted on 29/08/2016</i> 08/2022	
2011					
81	64	Esbriet (perfenidone) EXPIRED	InterMune UK Ltd.	Treatment for adults with idiopathic pulmonary fibrosis	
82	65	TOBI podhaler (tobramycin)	Novartis Europharm Limited	Suppressive therapy of chronic pulmonary infection due to <i>Pseudomonas aeruginosa</i> in adults and children aged 6 years and older with cystic fibrosis 07/2023	
83	66	Votubia (everolimus) EXPIRED	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	
84	67	Plenadren (hydrocortisone modified release tablet) EXPIRED	ViroPharma SPRL	Treatment for adults with adrenal insufficiency	
85	68	Vyndaqel (tafamidis). EXPIRED	Pfizer Limited - UK	Treatment of transthyretin amyloidosis in adult patients with symptomatic polyneuropathy	
2012					
86	69	Xaluprine (previously known as Mercaptopurin e Nova Laboratories and Novapurine) EXPIRED	Nova Laboratories Limited - UK	Treatment of acute lymphoblastic leukaemia (ALL) in adults, adolescents and children	
87	70	Bronchitol (manitolum) EXPIRED	Pharmaxis Pharmaceutic als	Treatment of cystic fibrosis in adults aged 18 years and above as an add-on therapy to best standard of care	
88	71	Signifor (pasireotide) EXPIRED	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	
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 Pharmaceutic als (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 not candidates for standard induction chemotherapy" 09/2022	
95	77	Glybera (adeno- associated viral vector)	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	

		expressing lipoprotein lipase WITHDRAWN		LPLD has to be confirmed by genetic testing. The indication is restricted to patients with detectable levels of LPL protein.	
96	78	Adcetris (brentuximab vedotin)	Takeda Pharma A/S, Denmark	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, Denmark	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, Denmark	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 Pharmaceutics Europe BV - The Netherlands	Procysbi 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 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 Pharmaceutic al 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 Representativ e 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 7 α -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 - Germany	Treatment of multidrug-resistant tuberculosis (MDR-TB) in adults 04/2024	
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	Treatment of adult patients with multicentric Castelman's disease (MCD who are human immunodeficiency virus (HIV)	

			NV	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 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	Prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP)	

			Limited	12/2024	
2015					
128	103	Ofev (nibtedanib) WITHDRAWN	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 (<i>ex vivo</i> 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 mm ² 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 Pharmaceutic als 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 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 Pharmaceutic	Raxone is indicated for the treatment of visual impairment in adolescent and adult patients with Leber's Hereditary Optic	

			als Gmbh	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 Pharmaceutic als	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 (albutrepenona cog alfa)	CSL Behring GmbH	Treatment and prophylaxis of bleeding in all age groups patients with haemophilia B (congenital factor IX 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) WITHDRAWN	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
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 <i>(previously Dinutuximab beta EUSA and Dinutuximab beta Apeiron)</i> (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 - cenergermin)	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) WITHDRAWN	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 (177Lu) oxodotreotide)	Advanced Accelerator Applications	Lutathera is indicated for the treatment of unresectable or metastatic, progressive, well differentiated (G1 and G2), somatostatin receptor positive gastroenteropancreatic neuroendocrine tumours (GEP NETs) in adults 09/2027
176	148	Zejula® (niraparib)	Tesaro limited UK	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 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 Pharmaceu ticals 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 therapy-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® (patisiran)	Alnylam Netherlands B.V.	Hereditary transthyretin-mediated amyloidosis (hATTR) in adult patients 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 Pharmaceutic als (Europe) Ltd	Cystic fibrosis in patients aged 12 years and above 11/2028
201	168	Takhyzyro (lanadelumab)	Shire Pharmaceutic als 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 β A-T87Q-globin gene) WITHDRAWN by company	bluebird bio (Netherlands) B.V.	beta-Thalassemia
208	175	Epidyolex® (Cannabidiol)	GW Pharma (International) B.V.	Lennox-Gastaut and Dravet syndromes
209	176	Xospata® (gilteritinib)	Astellas Pharma Europe B.V.	Acute myeloid leukaemia (AML)
2020				
210	177	Isturisa® (osilodrostat)	Novartis Europharm Limited	Cushing Syndrome
211	178	Polivy® (polatuzumab vedotin)	Roche Registration GmbH	Diffuse large B-cell lymphoma (DLBCL)
212	179	Givlaari® (givosiran)	Alnylam Netherlands B.V.	Acute hepatic porphyria
213	180	Trepulmix® (Trepstinil)	SciPharm Sàrl	Chronic thromboembolic pulmonary hypertension (CTEPH)
214	181	Zolgensma® (onasemnogene abeparvovec)	AveXis EU Limited	Spinal muscular atrophy (SMA)
215	182	Reblozyl® (luspatercept)	Celgene Europe B.V.	Beta thalassaemia & Myelodysplastic syndromes

216	183	Daurismo® (glasdegib)	Pfizer Europe MA EEIG	Newly-diagnosed acute myeloid leukaemia (AML)
217	184	Pretomanid FGK® (pretomanid)	FGK Representative Service GmbH	Adults with drug-resistant tuberculosis
218	185	Hepcludex® (bulevirtide)	MYR GmbH	Chronic (long-term) hepatitis delta virus (HDV) infection in adults
219	186	Idefirix® (imlifidase)	Hansa Biopharma AB	Prevent the body from rejecting a newly transplanted kidney
220	187	Kaftrio® (ivacaftor / tezacaftor / elexacaftor)	Vertex Pharmaceutic als (Ireland) Limited	Cystic fibrosis
221	188	Blenrep® (belantamab mafodotin)	GlaxoSmithKli ne (Ireland) Limited	Multiple Myeloma
222	189	Ayvakyt® (avapritinib)	Blueprint Medicines (Netherlands) B.V.	Gastrointestinal stromal tumour (GIST)
223	190	Arikayce liposomal® (amikacin sulfate)	Insmed Netherlands B.V.	Adults with a lung infection caused by Mycobacterium avium complex (MAC)
224	191	Adakveo® (crizanlizumab)	Novartis Europharm Limited	Sickle cell disease aged 16 years and older
225	192	Oxlumo® (lumasiran)	Alnylam Netherlands B.V.	Primary hyperoxaluria type 1
226	193	Obiltoxaximab SFL® (obiltoxaximab)	SFL Pharmaceutic als Deutschland GmbH	Inhalational anthrax due to Bacillus anthracis
227	194	Libmeldy® (autologous CD34+ cells encoding ARSA gene)	Orchard Therapeutics (Netherlands) BV	Metachromatic leukodystrophy (MLD)
228	195	Fintepla® (fenfluramine)	Zogenix ROI Limited	Seizures associated with Dravet syndrome
229	196	Tecartus® (Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced)	Kite Pharma EU B.V.	Adults with mantle cell lymphoma (a cancer of B cells, a type of white blood cell)

		with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured)			
2021					
230	197	Elzonris® (tagraxofusp)	Stemline Therapeutics B.V.	Adults with blastic plasmacytoid dendritic cell neoplasm (BPDCN)	
231	198	Inrebic® (fedratinib)	Celgene Europe BV	Adults with myelofibrosis (a rare form of blood cancer)	
232	199	Lumoxiti® (moxetumomab pasudotox) WITHDRAWN	AstraZeneca AB	Adults with hairy cell leukaemia, a cancer of the white blood cells	
233	200	Sogroya® (somapacitan)	Novo Nordisk A/S	Growth hormone deficiency	
234	201	Koselugo® (selumetinib)	AstraZeneca AB	Neurofibromatosis type 1	
235	202	Evrysdi® (risdiplam)	Roche Registration GmbH	5q spinal muscular atrophy (SMA) in patients 2 months of age and older, with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies	
236	203	Pemazyre® (pemigatinib)	Incyte Biosciences Distribution B.V.	Adults with cholangiocarcinoma	
237	204	Enspryng® (satralizumab)	Roche Registration GmbH	Neuromyelitis Optica Spectrum disorders (NMOSD)	
238	205	Bylvay® (odevixibat)	Albireo	+6 months with progressive familial intrahepatic cholestasis (PFIC)	
239	206	Skysona® (elivaldogene autotemcel) WITHDRAWN by company	bluebird bio (Netherlands) B.V.	Under 18 years of age with early cerebral adrenoleukodystrophy (CALD)	
240	207	Imcivree® (setmelanotide)	Rhythm Pharmaceutical Limited	+6 years who have pro-opiomelanocortin (POMC) deficiency or leptin receptor (LEPR) deficiency	
241	208	Abecma® (idecabtagene vicleucel)	Celgene Europe BV	Adults with multiple myeloma (a cancer of the bone marrow)	
242	209	Voxzogo® (vosoritide)	BioMarin International Limited	Achondroplasia in patients aged +2 years	

243	210	Minjuvi® (tafasitamab)	Incyte Biosciences Distribution B.V.	Adults with diffuse large B-cell lymphoma (DLBCL)
244	211	Qinlock® (ripretinib)	Deciphera Pharmaceutic als (Netherlands) B.V.	Gastrointestinal Stromal Tumors
245	212	Artesunate Amivas® (artesunate)	Amivas Ireland Ltd	Malaria
246	213	Aspaveli® (pegcetacoplan)	Swedish Orphan Biovitrum AB (publ)	Adults with paroxysmal nocturnal haemoglobinuria (PNH)
2022				
247	214	Tavneos® (avacopan)	Vifor Fresenius Medical Care Renal Pharma France	Adult patients with severe, active granulomatosis with polyangiitis (GPA) or microscopic polyangiitis (MPA)
248	215	Voraxaze® (glucarpidase)	SERB SAS	Adults and children (aged 28 days and older) with delayed methotrexate elimination or at risk of methotrexate toxicity
249	216	Lonapegsomat ropin Ascendis Pharma® (lonapegsomatr opin)	Ascendis Pharma Endocrinology Division A/S	Children who do not produce enough growth hormone (GHD)
250	217	Oxbryta® (voxelotor)	Global Blood Therapeutics Netherlands B. V.	Haemolytic anaemia, and +12 years old sickle cell disease
251	218	Ngenla® (somatrogen)	Pfizer Europe MA EEIG	Children and adolescents with growth hormone deficiency
252	219	Kimmtrak® (tebentafusp)	Immunocore Ireland Limited	Adult patients with unresectable or metastatic uveal melanoma
253	220	Uplizna® (inebilizumab)	Viela Bio	Adults with neuromyelitis optica spectrum disorders (NMOSD)
255	222	Carvykti® (ciltacabtagene autoleucl)	Janssen-Cilag International NV	Adults with multiple myeloma



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