Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Adcetris	brentuximab vedotin	L01XC12	Takeda Pharma A/S	25/10/2012	Adcetris is indicated for the treatment of adult patients with relapsed or refractory CD30+ Hodgkin lymphoma (HL): following autologous stem-cell transplant (ASCT) or; following at least two prior therapies when ASCT or multi-agent chemotherapy is not a treatment option. Adcetris is indicated for the treatment of adult patients with relapsed or refractory systemic anaplastic large-cell lymphoma (sALCL).
Adempas	riociguat	C02KX05	Bayer Pharma AG	27/03/2014	Chronic thromboembolic pulmonary hypertension (CTEPH) Adempas is indicated for the treatment of adult patients with WHO Functional Class (FC) II to III with inoperable CTEPH, persistent or recurrent CTEPH after surgical treatment, to improve exercise capacity (see section 5.1). Pulmonary arterial hypertension (PAH) Adempas, as monotherapy or in combination with endothelin receptor antagonists, is indicated for the treatment of adult patients with pulmonary arterial hypertension (PAH) with WHO Functional Class (FC) II to III to improve exercise capacity. Efficacy has been shown in a PAH population including aetiologies of idiopathic or heritable PAH or PAH associated with connective tissue disease (see section 5.1).
Arzerra	ofatumumab	L01XC10	Glaxo Group Ltd	19/04/2010	Treatment of chronic lymphocytic leukaemia (CLL) in patients who are refractory to fludarabine and alemtuzumab.
Atriance	nelarabine	L01BB07	Glaxo Group Limited	22/08/2007	Nelarabine is indicated for the 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 theseindications is based on limited data.
Bosulif	bosutinib (as monohydrate)	L01XE14	Pfizer Ltd	27/03/2013	Bosulif is indicated for the treatment of adult patients with chronic-phase, accelerated-phaseand blast-phasePhiladelphia-chromosome-positive chronic myelogenous leukaemiapreviously treated with one or more tyrosine-kinase inhibitors and for whom imatinib, nilotinib and dasatinib are not considered appropriate treatment options.
Bronchitol	mannitol	R05CB16	Pharmaxis Pharmaceuticals Ltd.	13/04/2012	Bronchitol is indicated for the treatment of cystic fibrosis (CF) in adults aged 18 years and above as an add-on therapy to best standard of care.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Carbaglu	carglumic acid	A16AA05	Orphan Europe S.A.R.L.	24/01/2003	Carbaglu is indicated in treatment of: hyperammonaemia due to N-acetylglutamate-synthase primary deficiency; hyperammonaemia due to isovaleric acidaemia; hyperammonaemia due to methymalonic acidaemia; hyperammonaemia due to propionic acidaemia.
Cayston	aztreonam lysine	J01DF01	Gilead Sciences International Limited	21/09/2009	Cayston is indicated for the suppressive therapy of chronic pulmonary infections due to Pseudomonas aeruginosa in patients with cystic fibrosis (CF) aged 6 years and older. Consideration should be given to official guidance on the appropriate use of antibacterial agents.
Ceplene	histamine dihydrochloride	L03AX14	Meda AB	07/10/2008	Ceplene maintainance 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.
Cometriq	cabozantinib	L01XE	TMC Pharma Services Ltd	21/03/2014	Treatment of adult patients with progressive, unresectable locally advanced or metastatic medullary thyroid carcinoma
Cyramza	ramucirumab	L01XC	Eli Lilly Nederland B.V.	19/12/2014	Cyramza 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. Cyramza 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.
Cystadane	betaine anhydrous	A16AA06	Orphan Europe S.A.R.L.	15/02/2007	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.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Dacogen	decitabine	L01BC08	Janssen-Cilag International N V	20/09/2012	Treatment of adult patients aged 65 years and above with newly diagnosed de novo or secondary acute myeloid leukaemia (AML), according to the World Health Organization (WHO) classification, who are not candidates for standard induction chemotherapy.
Defitelio	defibrotide	B01AX01	Gentium SpA	18/10/2013	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.
Deltyba	delamanid	J04AK06	Otsuka Novel Products GmbH	28/04/2014	Deltyba is indicated for use as part of an appropriate combination regimen for pulmonary multi-drug resistant tuberculosis (MDR-TB) in adult patients when an effective treatment regimen cannot otherwise be composed for reasons of resistance or tolerability. Consideration should be given to official guidance on the appropriate use of antibacterial agents.
Diacomit	stiripentol	N03AX17	Biocodex	04/01/2007	Diacomit is 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.
Elaprase	idursulfase	A16AB09	Shire Human Genetic Therapies AB	08/01/2007	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.
Esbriet	pirfenidone	L04AX05	InterMune UK Ltd	28/02/2011	Esbriet is indicated in adults for the treatment of mild to moderate idiopathic pulmonary fibrosis.
Evoltra	clofarabine	L01BB06	Genzyme Europe B.V.	29/05/2006	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.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Exjade	deferasirox	V03AC03	Novartis Europharm Limited	28/08/2006	Exjade is indicated for the treatment of chronic iron overload due to frequent blood transfusions(7 ml/kg/month of packed red blood cells)in patients with beta thalassaemia major agedsix years and older. Exjade is also indicated for the treatment of chronic iron overload due to blood transfusions when deferoxamine therapy is contraindicated or inadequate in the following patient groups: in patients with beta thalassaemia major with iron overload due to frequent blood transfusions(7 ml/kg/month of packed red blood cells)agedtwo tofive years; in patients with beta thalassaemia major with iron overload due to infrequent blood transfusions (7 ml/kg/month of packed red blood cells) agedtwo years and older; in patients with other anaemias agedtwo years and older.
Firazyr	icatibant	C01EB19	Shire Orphan Therapies GmbH	11/07/2008	Firazyr is indicated for symptomatic treatment of acute attacks of hereditary angioedema (HAE) in adults (with C1-esterase-inhibitor deficiency).
Firdapse (previously Zenas)	amifampridine	N07XX05	BioMarin Europe Ltd	23/12/2009	Symptomatic treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults.
Gazyvaro	obinutuzumab	L01XC15	Roche Registration Ltd	23/07/2014	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
Gliolan	5-aminolevulinic acid hydrochloride	L01XD04	Medac GmbH	07/09/2007	Gliolan is indicated in adult patients for visualisation of malignant tissue during surgery for malignant glioma (World Health Organization grade III and IV).
Glybera	alipogene tiparvovec	C10 AX10	uniQure biopharma B.V.	25/10/2012	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

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Granupas (previously Para-aminosalicylic acid Lucane)	para-aminosalicylic acid	J04AA01	Lucane Pharma	07/04/2014	Granupas is indicated for use as part of an appropriate combination regimen for multi-drug resistant 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 (see section 4.4). Consideration should be given to official guidance on the appropriate use of antibacterial agents.
Iclusig	ponatinib	L01XE24	Ariad Pharma Ltd	01/07/2013	Iclusig is indicated in adult patients with: 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; 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.
Imbruvica	ibrutinib	L01XE27	Janssen-Cilag International NV	21/10/2014	Imbruvica is indicated for the treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL).Imbruvicais 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.
Imnovid (previously Pomalidomide Celgene)	pomalidomide	L04AX06	Celgene Europe Ltd	05/08/2013	Imnovid 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.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Increlex	mecasermin	H01AC03	Ipsen Pharma	03/08/2007	For the 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 insulin-like growth factor-1 (IGF-1)levels below the 2.5th percentile for age and gender and; growth hormone (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.
Inovelon	rufinamide	N03AF03	Eisai Ltd	16/01/2007	Inovelon is indicated as adjunctive therapy in the treatment of seizures associated with Lennox-Gastaut syndrome in patientsfour years and older.
Jakavi	ruxolitinib (as phosphate)	L01XE18	Novartis Europharm Ltd.	23/08/2012	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.
Kalydeco	ivacaftor	R07AX02	Vertex Pharmaceuticals (U.K.) Ltd.	23/07/2012	Kalydeco is indicated for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have one of the following gating (class III) mutations in the CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, or S549R (see sections 4.4 and 5.1).
Ketoconazol e HRA	ketoconazole	J02AB02	Laboratoire HRA Pharma	19/11/2014	Ketoconazole HRA is indicated for the treatment of endogenous Cushings syndrome in adults and adolescents above the age of 12 years.
Kolbam (previously Cholic Acid FGK)	cholic acid	A05AA03	FGK Representative Service GmbH	04/04/2014	Kolbamis indicated for the treatment of inborn errors in primary bile acid synthesis due to Sterol 27-hydroxylase (presenting as cerebrotendinous xanthomatosis, CTX) deficiency, 2- (or a-) methylacyl-CoA racemase (AMACR) deficiency or Cholesterol 7a-hydroxylase (CYP7A1) deficiency in infants, children and adolescents aged 1 month to 18 years and adults.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Kuvan	sapropterin dihydrochloride	A16AX07	Merck Serono Europe Ltd	02/12/2008	Kuvan is indicated for the 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.
Litak	cladribine	L01BB04	Lipomed GmbH	14/04/2004	Litak is indicated for the treatment of hairy-cell leukaemia.
Lynparza	olaparib	L01	AstraZeneca AB	16/12/2014	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.
Mepact	mifamurtide	L03AX15	Takeda France SAS	06/03/2009	Mepact is 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 postoperative multi-agent chemotherapy. Safety and efficacy have been assessed in studies of patientstwo to 30 years of age at initial diagnosis.
Mozobil	plerixafor	L03AX16	Genzyme Europe B.V.	31/07/2009	Mozobil is indicated in combination with granulocyte-colony-stimulating factor to enhance mobilisation of haematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in patients with lymphoma and multiple myeloma whose cells mobilise poorly (see section 4.2).
Myozyme	alglucosidase alfa	A16AB07	Genzyme Europe B.V.	29/03/2006	Myozyme is indicated for long-term enzyme-replacement therapy (ERT) in patients with a confirmed diagnosis of Pompe disease (acid--glucosidase deficiency). In patients with late-onset Pompe disease the evidence of efficacy is limited.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Naglazyme	galsulfase	A16AB	BioMarin Europe Ltd.	24/01/2006	Naglazyme is indicated for long-term enzyme-replacement therapy in patients with a confirmed diagnosis of mucopolysaccharidosis VI (MPS VI; N-acetylgalactosamine-4-sulfatase deficiency; Maroteaux-Lamy syndrome) (see section 5.1). As for all lysosomal genetic disorders, it is of primary importance, especially in severe forms, to initiate treatment as early as possible, before appearance of non-reversible clinical manifestations of the disease. A key issue is to treat young patients aged 5 years suffering from a severe form of the disease, even though patients 5 years were not included in the pivotal phase-3 study.
Nexavar	sorafenib	L01XE05	Bayer Pharma AG	19/07/2006	Hepatocellular carcinoma Nexavar is indicated for the treatment of hepatocellular carcinoma. Renal cell carcinoma Nexavar is indicated 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. Differentiated thyroid carcinoma Nexavar is indicated for the treatment of patients with progressive, locally advanced or metastatic, differentiated (papillary/follicular/Hrthle cell) thyroid carcinoma, refractory to radioactive iodine.
NexoBrid	concentrate of proteolytic enzymes enriched in bromelain	D03BA03	MediWound Germany GmbH	18/12/2012	NexoBrid is indicated for removal of eschar in adults with deep partial- and full-thickness thermal burns.
Nplate	romiplostim	B02BX04	Amgen Europe B.V.	04/02/2009	Nplate is indicated for adult chronic-immune (idiopathic)-thrombocytopenic-purpura (ITP) splenectomised patients who are 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.
Opsumit	macitentan	C02KX04	Actelion Registration Ltd	20/12/2013	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. Efficacy has been shown in a PAH population including idiopathic and heritable PAH, PAH associated with connective tissue disorders, and PAH associated with corrected simple congenital heart disease.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Orfadin	nitisinone	A16AX04	Swedish Orphan Biovitrum International AB	21/02/2005	Treatment of patients with confirmed diagnosis of hereditary tyrosinaemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine.
Orphacol	cholic acid	A05AA03	Laboratoires CTRS	12/09/2013	Orphacol is indicated for the treatment of inborn errors in primary bile-acid synthesis due to 3-hydroxy-5-C27-steroid oxidoreductase deficiency or 4-3-oxosteroid-5-reductase deficiency in infants, children and adolescents agedone month to 18 years and adults.
Pedea	ibuprofen	C01EB16	Orphan Europe S.A.R.L.	29/07/2004	Treatment of a haemodynamically significant patent ductus arteriosus in preterm newborn infants less than 34 weeks of gestational age.
Peyona (previously Nymusa)	caffeine citrate	N06BC01	Chiesi Farmaceutici SpA	02/07/2009	Treatment of primary apnoea of premature newborns.
Plenadren	hydrocortisone	H02AB09	ViroPharma SPRL	03/11/2011	Treatment of adrenal insufficiency in adults.
Prialt	ziconotide	N02BG08	Eisai Ltd.	21/02/2005	Ziconotide is indicated for the treatment of severe, chronic pain in patients who require intrathecal (IT) analgesia.
Procysbi	mercaptamine bitartrate	A16AA04	Raptor Pharmaceuticals Europe BV	06/09/2013	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.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Revatio	sildenafil	G04BE03	Pfizer Limited	28/10/2005	Treatment of adult patients with pulmonary arterial hypertension classified as World Health Organization (WHO) functional class II and III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and pulmonary hypertension associated with connective tissue disease. Paediatric population Treatment of paediatric patients agedone year to 17 years old with pulmonary arterial hypertension. Efficacy in terms of improvement of exercise capacity or pulmonary haemodynamics has been shown in primary pulmonary hypertension and pulmonary hypertension associated with congenital heart disease. Revatio solution for injection is for the treatment of adult patients with pulmonary arterial hypertension who are currently prescribed oral Revatio and who are temporarily unable to take oral therapy, but are otherwise clinically and haemodynamically stable. Revatio (oral) is indicated for treatment of adult patients with pulmonary arterial hypertension classified as WHO functional class II and III, t□□ □□□ □□□ □□□□□□□□□□□□□□□□□□□□□□□□□□□□□□□□□
Revestive	teduglutide	A16AX08	NPS Pharma Holdings Limited	30/08/2012	Revestive is indicated for the treatment of adult patients with short-bowel syndrome. Patients should be stable following a period of intestinal adaptation after surgery.
Revlimid	lenalidomide	L04AX04	Celgene Europe Ltd	14/06/2007	Multiple myelomaRevlimid in combination with dexamethasone is indicated for the treatment of multiple myeloma in adult patients who have received at least one prior therapy. Myelodysplastic syndromesRevlimid 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.
Savene	dexrazoxane hydrochloride	V03AF02	Clinigen Healthcare Ltd	28/07/2006	Savene is indicated for the treatment of anthracycline extravasation.
Scenesse	afamelanotide	D02BB02	Clinuvel UK Limited	22/12/2014	Prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP).

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Signifor	pasireotide diaspartate	H01CB05	Novartis Europharm Ltd.	24/04/2012	Signifor is indicated for the treatment of adult patients with Cushings disease for whom surgery is not an option or for whom surgery has failed. Signifor is indicated for the 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.
Siklos	hydroxycarbamide	L01XX05	Addmedica	29/06/2007	Siklos is indicated for the prevention of recurrent painful vaso-occlusive crises including acute chest syndrome in paediatric and adult patients suffering from symptomatic sickle-cell syndrome (see section 5.1).
Sirturo	bedaquiline fumarate	J04A	Janssen-Cilag International N.V.	05/03/2014	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. See sections 4.2, 4.4 and 5.1. Consideration should be given to official guidance on the appropriate use of antibacterial agents.
Soliris	eculizumab	L04AA25	Alexion Europe SAS	20/06/2007	Soliris is indicated in adults and children for the treatment of patients with: paroxysmal nocturnal haemoglobinuria (PNH). Evidence of clinical benefit of Soliris in the treatment of patients with PNH is limited to patients with history of transfusions; atypical haemolytic uraemic syndrome (aHUS).
Sprycel	dasatinib	L01XE06	Bristol-Myers Squibb Pharma EEIG	20/11/2006	Sprycel is indicated for the treatment of adult patients with: newly diagnosed Philadelphia-chromosome-positive (Ph+) chronic myelogenous leukaemia (CML) in the chronic phase; chronic, accelerated or blast phase CML with resistance or intolerance to prior therapy including imatinib mesilate; Ph+ acute lymphoblastic leukaemia (ALL) and lymphoid blast CML with resistance or intolerance to prior therapy.
Sylvant	siltuximab	-	Janssen-Cilag International NV	22/05/2014	Sylvant is indicated for the treatment of adult patients with multicentric Castlemans disease (MCD who are human immunodeficiency virus (HIV) negative and human herpesvirus-8 (HHV-8) negative.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Tasigna	nilotinib	L01XE08	Novartis Europharm Ltd	19/11/2007	150 mg Tasigna is indicated for the treatment of adult patients with newly diagnosed Philadelphia-chromosome-positive chronic myelogenous leukaemia (CML) in the chronic phase. 200 mg Tasigna is indicated for the treatment of adult patients with: newly diagnosed Philadelphia-chromosome-positive CML in the chronic phase; chronic phase and accelerated phase Philadelphia-chromosome-positive CML with resistance or intolerance to prior therapy including imatinib. Efficacy data in patients with CML in blast crisis are not available.
Tepadina	thiotepa	L01AC01	ADIENNE S.r.l.	15/03/2010	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.". It is proposed that Tepadina must be prescribed by physicians experienced in conditioning treatment prior to haematopoietic progenitor cell transplantation.
Thalidomide Celgene (previously Thalidomide Pharmion)	thalidomide	L04AX02	Celgene Europe Limited	16/04/2008	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. Thalidomide Celgene is prescribed and dispensed according to the ThalidomideCelgene Pregnancy Prevention Programme.
Tobi Podhaler	tobramycin	J01GB01	Novartis Europharm Ltd.	20/07/2011	Tobi Podhaler is indicated for the suppressive therapy of chronic pulmonary infection due to Pseudomonas aeruginosa in adults and children aged 6 years and older with cystic fibrosis. See sections 4.4 and 5.1 regarding data in different age groups.Consideration should be given to official guidance on the appropriate use of antibacterial agents.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Torisel	temsirolimus	L01XE09	Pfizer Limited	19/11/2007	Renal-cell carcinoma Torisel is indicated for the first-line treatment of adult patients with advanced renal-cell carcinoma (RCC) who have at least three of six prognostic risk factors. Mantle-cell lymphoma Torisel is indicated for the treatment of adult patients with relapsed and / or refractory mantle-cell lymphoma (MCL).
Translarna	ataluren		PTC Therapeutics International Limited	31/07/2014	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.
Vidaza	azacitidine	L01BC07	Celgene Europe Ltd.	17/12/2008	Vidaza is indicated for the 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 Organization (WHO) classification.
Vimizim	recombinant human n-acetylgalactosamine-6-sulfatase (rhgalns)	A16AB12	BioMarin Europe Ltd	28/04/2014	Vimizim is indicated for the treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome, MPS IVA) in patients of all ages.
Volibris	ambrisentan	C02KX02	Glaxo Group Ltd.	21/04/2008	Volibris is indicated for the treatment of patients with pulmonary arterial hypertension (PAH) classified as World Health Organization functional class II and III, to improve exercise capacity. Efficacy has been shown in idiopathic PAH (IPAH) and in PAH associated with connective-tissue disease.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Votubia	everolimus	L01XE10	Novartis Europharm Ltd.	02/09/2011	Renal angiomyolipoma associated with tuberous sclerosis complex (TSC) Votubia is indicated for the treatment of adult patients with renal angiomyolipoma associated with tuberous sclerosis complex (TSC) who are at risk of complications (based on factors such as tumour size or presence of aneurysm, or presence of multiple or bilateral tumours) but who do not require immediate surgery. The evidence is based on analysis of change in sum of angiomyolipoma volume. Subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis complex (TSC) Votubia is indicated for the treatment of patients with subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis complex (TSC) who require therapeutic intervention but are not amenable to surgery. The evidence is based on analysis of change in SEGA volume. Further clinical benefit, such as improvement in diseaserelated symptoms, has not been demonstrated.
Vpriv	velaglucerase alfa	A16AB10	Shire Pharmaceuticals Ireland Ltd.	26/08/2010	Vpriv is indicated for long-term enzyme-replacement therapy (ERT) in patients with type-1 Gaucher disease.
Vyndaqel	tafamidis	N07XX08	Pfizer Ltd.	16/11/2011	Vyndaqel is indicated for the treatment of transthyretin amyloidosis in adult patients with stage-1 symptomatic polyneuropathy to delay peripheral neurologic impairment.
Wilzin	zinc	A16AX05	Orphan Europe S.A.R.L.	13/10/2004	Treatment of Wilson's disease.
Xagrid	anagrelide	L01XX35	Shire Pharmaceutical Contracts Limited	16/11/2004	Xagrid is indicated for the reduction of elevated platelet counts in at-risk essential-thrombocythaemia (ET) patients who are intolerant to their current therapy or whose elevated platelet counts are not reduced to an acceptable level by their current therapy. An at-risk patient An at-risk ET is defined by one or more of the following features: 60 years of age or; aplatelet count 1000 x 109/l or; ahistory of thrombohaemorrhagic events.

Medicine Name	Active Substance	Atc code	Marketing Authorisation Holder	Authorisation date	Indication
Xaluprine (previously Mercaptopurine Nova Laboratories)	6-mercaptopurine monohydrate	L01BB02	Nova Laboratories Ltd	09/03/2012	Xaluprineis indicated for the treatment of acute lymphoblastic leukaemia (ALL) in adults, adolescents and children.
Yondelis	trabectedin	L01CX01	Pharma Mar S.A.	17/09/2007	Yondelis is 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. Yondelis in combination with pegylated liposomal doxorubicin (PLD) is indicated for the treatment of patients with relapsed platinum-sensitive ovarian cancer.
Zavesca	miglustat	A16AX06	Actelion Registration Ltd.	20/11/2002	Zavesca is indicated for the oral treatment of adult patients with mild to moderate type-1 Gaucher disease. Zavesca may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable.Zavesca is indicated for the treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type-C disease.