



Enero 2019

Listados de medicamentos para enfermedades raras en Europa*

**Autorización de comercialización de la Comunidad Europea mediante el procedimiento centralizado*

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PARTE 1:

Listado de medicamentos huérfanos en Europa con designación huérfana europea y autorización de comercialización europea*

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Metodología

En esta parte del documento se ofrece un listado de todos los medicamentos huérfanos que, hasta la fecha indicada en el documento, han recibido una autorización de comercialización (AC) europea. Estos medicamentos pueden estar accesibles a partir de ahora en algunos países europeos, aunque no necesariamente en todos. En realidad, la accesibilidad de un medicamento huérfano concreto en un determinado país depende de la estrategia del laboratorio y de la decisión tomada por parte de las autoridades sanitarias nacionales respecto al reembolso.

Los medicamentos huérfanos en Europa son aquellos fármacos a los que se les ha concedido una designación huérfana europea (de acuerdo con la regulación (EC) No 141/2000), y a los que se les ha concedido también una autorización de comercialización europea y, si es aplicable, una evaluación positiva de un beneficio significativo.

Este listado de medicamentos huérfanos en Europa, con designación huérfana y autorización de comercialización europea, se obtiene cruzando el

listado de medicamentos que han recibido una designación huérfana (<http://ec.europa.eu/health/documents/community-register/html/alforphreg.htm>) con el listado de medicamentos que han recibido una autorización de comercialización (<http://ec.europa.eu/health/documents/community-register/html/alfregister.htm>). Ambos están disponibles en el sitio web de la Dirección General de Sanidad y Seguridad Alimentaria (DG SANTE) de la Comisión Europea.

Una primera clasificación por nombre comercial ofrece el nombre de la sustancia activa, la indicación de la autorización de comercialización (AC), y la fecha y el titular de la AC.

Le siguen dos tablas anexas que ofrecen:

- una tabla de medicamentos huérfanos retirados del Registro comunitario de medicamentos huérfanos

*Autorización de comercialización de la Comunidad Europea por procedimiento centralizado

(consulte el Anexo 1 – « Medicamentos huérfanos retirados del Registro comunitario de medicamentos huérfanos »; sus indicaciones están detalladas en la Parte II, “Listado de productos medicinales para enfermedades raras en Europa con autorización de comercialización europea sin designación huérfana en Europa”);

- una tabla de medicamentos retirados de su uso en la Unión Europea (consulte el Anexo 2 – Medicamentos huérfanos retirados de su uso en la Unión Europea). Más información en www.ema.europa.eu.

Tres listados adicionales proponen otras clasificaciones por:

- fecha de la AC en orden descendiente,

- categoría ATC,

- titular de la AC.

Para cada listado, los nombres comerciales se presentan en orden alfabético.

Puede encontrar información adicional de cada producto en la pestaña “Medicamentos huérfanos” del sitio web de Orphanet www.orphanet.es o en el sitio web de la EMA (Agencia Europea de Medicamentos) www.ema.europa.eu.

El listado de la EMA cubre todos los medicamentos autorizados en el mercado, no únicamente los medicamentos huérfanos.



La información oficial y actualizada sobre medicamentos huérfanos está disponible en el Registro comunitario de medicamentos huérfanos de uso humano:
<http://ec.europa.eu/health/documents/community-register/html/alforphreg.htm>

Clasificación por nombre comercial

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
ADCETRIS	brentuximab vedotin	<p>Treatment of adult patients with relapsed or refractory CD30+ Hodgkin lymphoma (HL):</p> <ul style="list-style-type: none"> -following autologous stem cell transplant (ASCT) or -following at least two prior therapies when ASCT or multi-agent chemotherapy is not a treatment option. <p>Treatment of adult patients with CD30+ HL at increased risk of relapse or progression following ASCT</p> <p>Treatment of adult patients with relapsed or refractory systemic anaplastic large cell lymphoma (sALCL).</p> <p>Treatment of adult patients with CD30+ cutaneous T-cell lymphoma (CTCL) after at least 1 prior systemic therapy.</p>	25/10/2012	Takeda Pharma A/S
ADEMPAS	riociguat	<p>Treatment of adult patients with WHO Functional Class (FC) II to III with inoperable Chronic thromboembolic pulmonary hypertension (CTEPH), persistent or recurrent CTEPH after surgical treatment, to improve exercise capacity.</p> <p>As monotherapy or in combination with endothelin receptor antagonists, for the treatment of adult patients with pulmonary arterial hypertension (PAH) with WHO Functional Class (FC) II to III to improve exercise capacity.</p> <p>Efficacy has been shown in a PAH population including etiologies of idiopathic or heritable PAH or PAH associated with connective tissue disease.</p>	27/03/2014	Bayer AG
ALOFISEL	darvadstrocel	<p>Treatment of complex perianal fistulas in adult patients with non-active/mildly active luminal Crohn's disease, when fistulas have shown an inadequate response to at least one conventional or biologic therapy. Alofisel should be used after conditioning of fistula.</p>	23/03/2018	Takeda Pharma A/S
ALPROLIX	eftrenonacog alfa	<p>Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency). ALPROLIX can be used for all age groups.</p>	12/05/2016	Swedish Orphan Biovitrum AB (publ)
AMGLIDIA	glibenclamide	<p>Treatment of neonatal diabetes mellitus, for use in newborns, infants and children.</p> <p>Sulphonylureas like Amglidia have been shown to be effective in patients with mutations in the genes coding for the β-cell ATP-sensitive potassium channel and chromosome 6q24-related transient neonatal diabetes mellitus.</p>	24/05/2018	Ammtek

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
ARZERRA	ofatumumab	In combination with chlorambucil or bendamustine, for the treatment of patients with chronic lymphocytic leukaemia (CLL) who have not received prior therapy and who are not eligible for fludarabine-based therapy. In combination with fludarabine and cyclophosphamide for the treatment of adult patients with relapsed CLL. Treatment of CLL in patients who are refractory to fludarabine and alemtuzumab.	19/04/2010	Novartis Europharm Ltd
BAVENCIO	avelumab	As monotherapy for the treatment of adult patients with metastatic Merkel cell carcinoma (MCC) .	18/09/2017	Merck Europe B.V.
BESPONSA	inotuzumab ozogamicin	As monotherapy for the treatment of adults with relapsed or refractory CD22-positive B cell precursor acute lymphoblastic leukaemia (ALL) . Adult patients with Philadelphia chromosome positive (Ph+) relapsed or refractory B cell precursor ALL should have failed treatment with at least 1 tyrosine kinase inhibitor (TKI).	29/06/2017	Pfizer Europe MA EEIG
BLINCYTO	blinatumomab	Treatment of adults with Philadelphia chromosome negative relapsed or refractory B-precursor acute lymphoblastic leukaemia (ALL) . As monotherapy for the treatment of paediatric patients aged 1 year or older with Philadelphia chromosome negative CD19 positive B cell 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.	23/11/2015	Amgen Europe B.V.
BRINEURA	cerliponase alfa	Treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease, also known as tripeptidyl peptidase 1 (TPP1) deficiency.	30/05/2017	BioMarin International Limited
BRONCHITOL	mannitol	Treatment of cystic fibrosis (CF) in adults aged 18 years and above as an add-on therapy to best standard of care.	13/04/2012	Pharmaxis Pharmaceuticals Ltd
CABLIVI	caplacizumab	Treatment of adults experiencing an episode of acquired thrombotic thrombocytopenic purpura (aTTP) , in conjunction with plasma exchange and immunosuppression.	30/08/2018	Ablynx NV
CARBAGLU	carglumic acid	Treatment of hyperammonaemia due to - isovaleric acidaemia , - methymalonic acidaemia , - propionic acidaemia .	01/06/2011	Orphan Europe S.a.r.l.
CAYSTON	aztreonam	Suppressive therapy of chronic pulmonary infections due to <i>Pseudomonas aeruginosa</i> in patients with cystic fibrosis (CF) aged 6 years and older.	21/09/2009	Gilead Sciences Ireland UC

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
CERDELGA	eliglustat	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)	19/01/2015	Genzyme Europe B.V.
CHENODEOXYCHOLIC ACID LEADIANT (previously CHENODEOXYCHOLIC ACID SIGMA-TAU)	chenodeoxycholic acid	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.	10/04/2017	Leadiant GmbH
COAGADEX	human coagulation factor X	Treatment and prophylaxis of bleeding episodes and for perioperative management in patients with hereditary factor X deficiency .	16/03/2016	Bio Products Laboratory Ltd
COMETRIQ	cabozantinib	Treatment of adult patients with progressive, unresectable locally advanced or metastatic medullary thyroid carcinoma . For patients in whom Rearranged during Transfection (RET) mutation status is not known or is negative, a possible lower benefit should be taken into account before individual treatment decision.	21/03/2014	IPSEN Pharma
CRESEMBA	isavuconazole	In adults for the treatment of: - invasive aspergillosis - mucormycosis in patients for whom amphotericin B is inappropriate	15/10/2015	Basilea Medical Ltd
CRYSVITA	burosumab	Treatment of X-linked hypophosphataemia with radiographic evidence of bone disease in children 1 year of age and older and adolescents with growing skeletons.	19/02/2018	Kyowa Kirin Holdings B.V.
CYSTADROPS	mercaptamine hydrochloride	Treatment of corneal cystine crystal deposits in adults and children from 2 years of age with cystinosis .	19/01/2017	Orphan Europe S.A.R.L.
DACOGEN	decitabine	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.	20/09/2012	Janssen-Cilag International N.V.

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DARZALEX	daratumumab	In combination with bortezomib, melphalan and prednisone for the treatment of adult patients with newly diagnosed multiple myeloma who are ineligible for autologous stem cell transplant. As monotherapy for the treatment of adult patients with relapsed and refractory multiple myeloma, whose prior therapy included a proteasome inhibitor and an immunomodulatory agent and who have demonstrated disease progression on the last therapy. In combination with lenalidomide and dexamethasone, or bortezomib and dexamethasone, for the treatment of adult patients with multiple myeloma who have received at least one prior therapy.	20/05/2016	Janssen-Cilag International N.V.
DEFITELIO	defibrotide	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.	18/10/2013	Gentium SRL
DELTYBA	delamanib	Used 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.	28/04/2014	Otsuka Novel Products GmbH
ESBRIET	pirfenidone	In adults for the treatment of mild to moderate Idiopathic Pulmonary Fibrosis (IPF) .	28/02/2011	Roche Registration GmbH
FARYDAK	panobinostat lactate anhydrous	In combination with bortezomib and dexamethasone, for the treatment of adult patients with relapsed and/or refractory multiple myeloma who have received at least two prior regimens including bortezomib and an immunomodulatory agent.	28/08/2015	Novartis Europharm Ltd
FIRAZYR	icatibant acetate	Symptomatic treatment of acute attacks of hereditary angioedema (HAE) in adults, adolescents and children aged 2 years and older, with C1-esterase-inhibitor deficiency.	11/07/2008	Shire Pharmaceuticals Ireland Limited
FIRDAPSE (previously ZENAS)	amifampridine	Symptomatic treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults.	23/12/2009	BioMarin International Limited
GALAFOLD	migalastat	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.	26/05/2016	Amicus Therapeutics UK Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
GAZYVARO	obinutuzumab	In combination with chlorambucil, treatment of adult patients with previously untreated chronic lymphocytic leukaemia (CLL) and with comorbidities making them unsuitable for full-dose fludarabine based therapy. 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 . In combination with bendamustine followed by Gazyvaro maintenance is indicated for the treatment of patients with follicular lymphoma (FL) who did not respond or who progressed during or up to 6 months after treatment with rituximab or a rituximab-containing regimen.	23/07/2014	Roche Registration GmbH
GRANUPAS (previously PARA-AMINOSALICYLIC ACID LUCANE)	para-aminosalicylic acid	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. Consideration should be given to official guidance on the appropriate use of antibacterial agents.	07/04/2014	Eurocept International B.V.
HETLIOZ	tasimelteon	Treatment of Non-24-Hour Sleep-Wake Disorder (Non-24) in totally blind adults.	03/07/2015	Vanda Pharmaceuticals Ltd
HOLOCLAR	ex vivo expanded autologous human corneal epithelial cells containing stem cells	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.	17/02/2015	Chiesi Farmaceutici SpA
ICLUSIG	ponatinib	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. Indicated in adult patients with 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.	01/07/2013	Incyte Biosciences Distribution B.V.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
IDELVION	albutrepenonacog alfa	Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency) . IDELVION can be used for all age groups.	11/05/2016	CSL Behring GmbH
IMBRUVICA	ibrutinib	As a single agent for the treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL) . As a single agent for the treatment of adult patients with previously untreated chronic lymphocytic leukaemia (CLL) . As a single agent or in combination with bendamustine and rituximab (BR) for the treatment of adult patients with CLL who have received at least one prior therapy. As a single agent 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.	21/10/2014	Janssen-Cilag International N.V.
IMNOVID (previously POMALIDOMI DE CELGENE)	pomalidomide	In combination with dexamethasone, 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.	05/08/2013	Celgene Europe B.V.
INOVELON	rufinamide	Adjunctive therapy in the treatment of seizures associated with Lennox-Gastaut syndrome in patients aged 1 year and older.	16/01/2007	Eisai GmbH
JORVEZA	budesonide	Treatment of eosinophilic esophagitis (EoE) in adults (older than 18 years of age).	08/01/2018	Dr. Falk Pharma GmbH

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
KALYDECO	ivacaftor	<p>KALYDECO tablets:</p> <p>Treatment of patients with cystic fibrosis (CF) aged 6 years and older and weighing 25kg or more who have one of the following gating (class III) mutations in the <i>CFTR</i> gene: <i>G551D</i>, <i>G1244E</i>, <i>G1349D</i>, <i>G178R</i>, <i>G551S</i>, <i>S1251N</i>, <i>S1255P</i>, <i>S549N</i> or <i>S549R</i>.</p> <p>Treatment of patients with cystic fibrosis (CF) aged 18 years and older who have an <i>R117H</i> mutation in the <i>CFTR</i> gene.</p> <p>In a combination regimen with tezacaftor 100 mg/ivacaftor 150 mg tablets for the treatment of patients with cystic fibrosis (CF) aged 12 years and older who are homozygous for the <i>F508del</i> mutation or who are heterozygous for the <i>F508del</i> mutation and have one of the following mutations in the <i>CFTR</i> gene: <i>P67L</i>, <i>R117C</i>, <i>L206W</i>, <i>R352Q</i>, <i>A455E</i>, <i>D579G</i>, <i>711+3A→G</i>, <i>S945L</i>, <i>S977F</i>, <i>R1070W</i>, <i>D1152H</i>, <i>2789+5G→A</i>, <i>3272 26A→G</i>, and <i>3849+10kbC→T</i>.</p> <p>KALYDECO granules:</p> <p>Treatment of children with cystic fibrosis (CF) aged 12 months and older and weighing 7 kg to less than 25 kg who have one of the following gating (class III) mutations in the <i>CFTR</i> gene: <i>G551D</i>, <i>G1244E</i>, <i>G1349D</i>, <i>G178R</i>, <i>G551S</i>, <i>S1251N</i>, <i>S1255P</i>, <i>S549N</i> or <i>S549R</i>.</p>	23/07/2012	Vertex Pharmaceuticals (Europe) Ltd
KANUMA	sebelipase alfa	Long-term enzyme replacement therapy (ERT) in patients of all ages with lysosomal acid lipase (LAL) deficiency	28/08/2015	Alexion Europe SAS
KETOCONAZOLE HRA	ketoconazole	Treatment of endogenous Cushing's syndrome in adults and adolescents above the age of 12 years.	19/11/2014	Laboratoire HRA Pharma
KOLBAM (previously CHOLIC ACID FGK)	cholic acid	Treatment of 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.	08/04/2014	Retrophin Europe Ltd
KUVAN	sapropterin dihydrochloride	Treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients of all ages with phenylketonuria (PKU) who have been shown to be responsive to such treatment. Treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients with tetrahydrobiopterin (BH4) deficiency who have been shown to be responsive to such treatment.	02/12/2008	Biomarin International Limited

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KYMRIAH	tisagenlecleucel	Treatment of: - Paediatric and young adult patients up to 25 years of age with B-cell acute lymphoblastic leukaemia (ALL) that is refractory, in relapse post-transplant or in second or later relapse. - Adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) after two or more lines of systemic therapy.	22/08/2018	Novartis Europharm Limited
KYPROLIS	carfilzomib	In combination with either lenalidomide and dexamethasone or dexamethasone alone is indicated for the treatment of adult patients with multiple myeloma who have received at least one prior therapy.	19/11/2015	Amgen Europe B.V.
LAMZEDE	velmanase alfa	Enzyme replacement therapy for the treatment of non-neurological manifestations in patients with mild to moderate alpha mannosidosis .	23/03/2018	Chiesi Farmaceutici S.p.A.
LARTRUVO	olaratumab	In combination with doxorubicin for the treatment of adult patients with advanced soft tissue sarcoma who are not amenable to curative treatment with surgery or radiotherapy and who have not been previously treated with doxorubicin.	09/11/2016	Eli Lilly Nederland B.V.
LEDAGA	chlormethine	Topical treatment of mycosis fungoides-type cutaneous T-cell lymphoma (MF-type CTCL) in adult patients.	03/03/2017	Actelion Registration Ltd.
LUTATHERA	lutetium (177Lu) oxodotreotide	Treatment of unresectable or metastatic, progressive, well differentiated (G1 and G2), somatostatin receptor positive gastroenteropancreatic neuroendocrine tumours (GEP NETs) in adults.	26/09/2017	Advanced Accelerator Applications
LUXTURNA	voretigene neparvovec	Treatment of adult and paediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic RPE65 mutations and who have sufficient viable retinal cells.	22/11/2018	Spark Therapeutics Ireland Ltd
MEPACT	mifamurtide	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. Safety and efficacy have been assessed in studies of patients 2 to 30 years of age at initial diagnosis.	06/03/2009	Takeda France SAS
MEPSEVII	vestronidase alfa	Treatment of non-neurological manifestations of Mucopolysaccharidosis VII (MPS VII; Sly syndrome) .	22/08/2018	Ultragenyx Germany GmbH

NEW



TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
MOZOBI	plerixafor	In combination with granulocyte-colony stimulating factor G-CSF 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.	31/07/2009	Genzyme Europe B.V.
MYALEPTA	metreleptin	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) or acquired generalised LD (Lawrence syndrome) in adults and children 2 years of age and above - with confirmed familial partial LD 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.	29/07/2018	Aegerion Pharmaceuticals B.V.
MYLOTARG	gemtuzumab ozogamicin	In 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).	19/04/2018	Pfizer Europe MA EEIG
NAMUSCLA	mexiletine hcl	Symptomatic treatment of myotonia in adult patients with non-dystrophic myotonic disorders .	18/12/2018	Lupin Europe GmbH
NATPAR	parathyroid hormone	Indicated as adjunctive treatment of adult patients with chronic hypoparathyroidism who cannot be adequately controlled with standard therapy alone.	24/04/2017	Shire Pharmaceuticals Ireland Ltd
NEXAVAR	sorafenib tosylate	Treatment of patients with progressive, locally advanced or metastatic, differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma , refractory to radioactive iodine.	19/07/2006	Bayer AG
NEXOBRID	concentrate of proteolytic enzymes enriched in bromelain	Removal of eschar in adults with deep partial- and full-thickness thermal burns .	18/12/2012	Mediound Germany GmbH
NINLARO	ixazomib	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.	21/11/2016	Takeda Pharma A/S
NPLATE	romiplostim	Indicated for chronic immune (idiopathic) thrombocytopenic purpura (ITP) patients one year of age and older who are refractory to other treatments (e.g. corticosteroids, immunoglobulins)	04/02/2009	Amgen Europe B.V.
OALIVA	obeticholic acid	Treatment of primary biliary cholangitis (also known as primary biliary cirrhosis) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA.	12/12/2016	Intercept Pharma Ltd



TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
OFEV	nintedanib	Treatment in adults of Idiopathic Pulmonary Fibrosis (IPF) .	15/01/2015	Boehringer Ingelheim International GmbH
ONIVYDE	irinotecan hydrochloride trihydrate	Treatment of 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.	14/10/2016	Baxalta Innovations GmbH
ONPATTRO	Patisiran sodium	Treatment of hereditary transthyretin - mediated amyloidosis (hATTR amyloidosis) in adult patients with stage 1 or stage 2 polyneuropathy.	26/08/2018	Alnylam Netherlands B.V.
OPSUMIT	macitentan	Used as monotherapy or in combination, 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.	20/12/2013	Janssen-Cilag International N.V.
ORPHACOL	cholic acid	Treatment of inborn errors in primary bile acid synthesis due to 3beta-hydroxy-delta5-C27- steroid oxidoreductase deficiency or delta4-3-oxosteroid-5beta-reductase deficiency in infants, children and adolescents aged 1 month to 18 years and adults.	12/09/2013	Laboratoires CTRS
OXERVATE	cenegermin	Treatment of moderate (persistent epithelial defect) or severe (corneal ulcer) neurotrophic keratitis in adults.	06/07/2017	Dompe farmaceutici s.p.a.
PEYONA (previously NYMUSA)	caffeine citrate	Treatment of primary apnea of premature newborns.	02/07/2009	Chiesi Farmaceutici SpA
PLENADREN	hydrocortisone	Treatment of adrenal insufficiency in adults.	03/11/2011	Shire Services BVBA
POTELIGEO	mogamulizumab	Treatment of adult patients with mycosis fungoides (MF) or Sézary syndrome (SS) who have received at least one prior systemic therapy.	22/11/2018	Kyowa Kirin Holdings B.V.
PREVYMIS	letermovir	Prophylaxis of cytomegalovirus (CMV) reactivation and disease in adult CMV-seropositive recipients [R+] of an allogeneic haematopoietic stem cell transplant (HSCT). Consideration should be given to official guidance on the appropriate use of antiviral agents.	08/01/2018	Merck Sharp & Dohme B.V.
PROCYSBI	mercaptopamine	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.	06/09/2013	Chiesi Farmaceutici SpA

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
QARZIBA (previously DINUTUXIMAB BETA APEIRON)	dinutuximab beta	Treatment of high-risk neuroblastoma in patients aged 12 months and above, who have previously received induction chemotherapy and achieved at least a partial response, followed by myeloablative therapy and stem cell transplantation, as well as patients with history of relapsed or refractory neuroblastoma, with or without residual disease. Prior to the treatment of relapsed neuroblastoma, any actively progressing disease should be stabilised by other suitable measures. In patients with a history of relapsed/refractory disease and in patients who have not achieved a complete response after first line therapy, Dinutuximab beta Apeiron should be combined with interleukin-2 (IL-2).	08/05/2017	EUSA Pharma (UK) Limited
RAVICTI	glycerol phenylbutyrate	Indicated for use as adjunctive therapy for chronic management of adult and paediatric patients ≥ 2 months of age with urea cycle disorders (UCDs) including: deficiencies of carbamoyl phosphate-synthase-I (CPS) - ornithine carbamoyltransferase (OTC) - argininosuccinate synthetase (ASS) , - argininosuccinate lyase (ASL) - arginase I (ARG) - ornithine translocase deficiency hyperornithinaemia -hyperammonaemia homocitrullinuria syndrome (HHH) Who cannot be managed by dietary protein restriction and/or amino acid supplementation alone. RAVICTI must be used with dietary protein restriction and, in some cases, dietary supplements (e.g., essential amino acids, arginine, citrulline, protein-free calorie supplements).	27/11/2015	HORIZON Pharma Ireland Limited
RAXONE	idebenone	Treatment of visual impairment in adolescent and adult patients with Leber's Hereditary Optic Neuropathy (LHON) .	08/09/2015	Santhera Pharmaceuticals (Deutschland) GmbH
REVESTIVE	teduglutide	Treatment of patients aged 1 year and above with Short Bowel Syndrome . Patients should be stable following a period of intestinal adaptation after surgery.	30/08/2012	Shire Pharmaceuticals Ireland Limited
REVLIMID	lenalidomide	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. Treatment of adult patients with relapsed or refractory mantle cell lymphoma .	13/06/2013	Celgene Europe B.V.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
RYDAPT	midostaurin	In combination with standard daunorubicin and cytarabine induction and high dose cytarabine consolidation chemotherapy, and for patients in complete response followed by Rydapt single agent maintenance therapy, for adult patients with newly diagnosed acute myeloid leukaemia (AML) who are FLT3 mutation positive. As monotherapy for the treatment of adult patients with aggressive systemic mastocytosis (ASM) , systemic mastocytosis with associated haematological neoplasm (SM AHN) , or mast cell leukaemia (MCL) .	18/09/2017	Novartis Europharm Limited
SCENESSE	afamelanotide	Prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP)	22/12/2014	Clinuvel UK Ltd
SIGNIFOR	pasireotide	Treatment of adult patients with Cushing's disease for whom surgery is not an option or for whom surgery has failed. 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.	24/04/2012	Novartis Europharm Ltd
SIRTURO	bedaquiline	Used 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. Consideration should be given to official guidance on the appropriate use of antibacterial agents.	05/03/2014	Janssen-Cilag International N.V.
SOLIRIS	eculizumab	Treatment of adults and children with : - Paroxysmal nocturnal haemoglobinuria (PNH) . Evidence of clinical benefit is demonstrated in patients with haemolysis with clinical symptom(s) indicative of high disease activity, regardless of transfusion history. - atypical haemolytic uraemic syndrome (aHUS) . - Refractory generalized myasthenia gravis (gMG) in patients who are anti-acetylcholine receptor (AChR) antibody-positive	20/06/2007	Alexion Europe SAS
SOMAKIT TOC	edotreotide	After radiolabelling with gallium (⁶⁸ Ga) chloride solution, the solution of gallium (⁶⁸ Ga) edotreotide obtained is indicated for Positron Emission Tomography (PET) imaging of somatostatin receptor overexpression in adult patients with confirmed or suspected well-differentiated gastro-enteropancreatic neuroendocrine tumours (GEP-NET) for localizing primary tumours and their metastases.	08/12/2016	Advanced Accelerator Applications
SPINRAZA	nusinersen sodium	Treatment of 5q Spinal Muscular Atrophy .	30/05/2017	Biogen Netherlands B.V.



TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
STRENSIQ	asfotase alfa	Long-term enzyme replacement therapy in patients with paediatric-onset hypophosphatasia to treat the bone manifestations of the disease.	28/08/2015	Alexion Europe SAS
STRIMVELIS	autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human adenosine deaminase (ADA) cDNA sequence from human haematopoietic stem/progenitor (CD34+) cells	Treatment of patients with severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID) , for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.	26/05/2016	Orchard Therapeutics (Netherlands) B.V.
SYLVANT	siltuximab	Treatment of adult patients with multicentric Castlemans disease (MCD) who are human immunodeficiency virus (HIV) negative and human herpesvirus-8 (HHV-8) negative.	22/05/2014	Janssen-Cilag International N.V.
SYMKEVI	tezacaftor/ivacaftor	In a combination regimen with ivacaftor 150 mg tablets for the treatment of patients with cystic fibrosis (CF) aged 12 years and older who are homozygous for the F508del mutation or who are heterozygous for the F508del mutation and have one of the following mutations in the cystic fibrosis transmembrane conductance regulator (<i>CFTR</i>) gene: <i>P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272 26A→G, and 3849+10kbC→T.</i>	31/10/2018	Vertex Pharmaceuticals (Europe) Limited
TAKHZYRO	lanadelumab	For routine prevention of recurrent attacks of hereditary angioedema (HAE) in patients aged 12 years and older.	22/11/2018	Shire Pharmaceuticals Ireland Limited
TASIGNA	nilotinib	Treatment of adult and paediatric patients with newly diagnosed Philadelphia chromosome positive chronic myelogenous leukaemia (CML) in the chronic phase. Adult patients with 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. Paediatric patients with chronic phase Philadelphia chromosome positive CML with resistance or intolerance to prior therapy including imatinib.	19/11/2007	Novartis Europharm Ltd
TEGSEDI	inotersen	Treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR)	10/07/2018	Akcea Therapeutics UK Ltd.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
TEPADINA	thiotepa	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.	15/03/2010	Adienne S.r.l.
TOBI PODHALER	tobramycin	Suppressive therapy of chronic pulmonary infection due to <i>Pseudomonas aeruginosa</i> in adults and children aged 6 years and older with cystic fibrosis . Consideration should be given to official guidance on the appropriate use of antibacterial agents .	20/07/2011	Novartis Europharm Ltd
TORISEL	temsirolimus	Treatment of adult patients with relapsed and / or refractory mantle cell lymphoma (MCL) .	19/11/2007	Pfizer Europe MA EEIG
TRANSLARNA	ataluren	Treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 2 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.	31/07/2014	PTC Therapeutics International Ltd
VERKAZIA	ciclosporin	Treatment of severe vernal keratoconjunctivitis (VKC) in children from 4 years of age and adolescents.	06/07/2018	Santen Oy
VIDAZA	azacitidine	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. Treatment of adult patients aged 65 years or older who are not eligible for HSCT with AML with >30% marrow blasts according to the WHO classification.	17/12/2008	Celgene Europe B.V.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
VIMIZIM	elosulfase alfa	Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome, MPS IVA) in patients of all ages.	28/04/2014	BioMarin International Limited
VOTUBIA	everolimus	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. 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 disease-related symptoms, has not been demonstrated.	02/09/2011	Novartis Europharm Ltd
VPRIV	velaglucerase alfa	Long-term enzyme replacement therapy (ERT) in patients with type 1 Gaucher disease .	26/08/2010	Shire Pharmaceuticals Ireland Ltd
VYNDAQEL	tafamidis	Treatment of transthyretin amyloidosis in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment.	16/11/2011	Pfizer Europe MA EEIG
VYXEOS	daunorubicin hydrochloride / cytarabine	Treatment of adults with newly diagnosed, therapy-related acute myeloid leukaemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC) .	22/08/2018	Jazz Pharmaceuticals Ireland Limited
WAKIX	pitolisant	Treatment in adults of narcolepsy with or without cataplexy .	31/03/2016	Bioprojet Pharma
XALUPRINE (previously MERCAPTOPURINE NOVA)	mercaptopurine	Treatment of acute lymphoblastic leukaemia (ALL) in adults, adolescents and children.	09/03/2012	Nova Laboratories Ltd
XERMELO	telotristat	Treatment of carcinoid syndrome diarrhoea in combination with somatostatin analogue (SSA) therapy in adults inadequately controlled by SSA therapy.	18/09/2017	Ipsen Pharma
YESCARTA	axicabtagene ciloleucel	Treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) and primary mediastinal large B-cell lymphoma (PMBCL) , after two or more lines of systemic therapy.	22/08/2018	Kite Pharma EU B.V.
YONDELIS	trabectedin	In combination with pegylated liposomal doxorubicin (PLD), treatment of patients with relapsed platinum-sensitive ovarian cancer .	28/10/2009	Pharma MarS.A.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
ZALMOXIS	allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (Δ LNGFR) and the herpes simplex I virus thymidine kinase (HSV-TK Mut2)	Indicated as adjunctive treatment in haploidentical haematopoietic stem cell transplantation (HSCT) of adult patients with high-risk haematological malignancies .	18/08/2016	MolMed SpA
ZAVESCA	miglustat	Treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type C disease .	28/01/2009	Janssen-Cilag International N.V.
ZEJULA	niraparib	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.	16/11/2017	Tesaro UK Limited

Anexo 1: Medicamentos huérfanos retirados del Registro comunitario de medicamentos huérfanos

Las indicaciones de los productos listados en la siguiente tabla están detallados en la Parte II “Listado de productos medicinales para enfermedades raras en Europa con autorización de comercialización europea sin designación huérfana en Europa”

Algunos productos han perdido la designación huérfana para alguna de sus indicaciones, pero no para todas. En estos casos, las indicaciones para las que han perdido la designación huérfana se mencionan más abajo.

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
AFINITOR	everolimus	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 5 June 2007.	05/08/2009	08/07/2011
ALDURAZYME	laronidase	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 14 February 2001.	12/06/2003	12/06/2013
ATRIANCE	nelarabine	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 16 June 2005.	22/08/2007	24/08/2017
BOSULIF	bosutinib	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 4 August 2010.	27/03/2013	15/03/2018
BUSILVEX	busulfan	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 29 December 2000	11/07/2003	11/07/2013
CARBAGLU	carglumic acid	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity for the following condition: - Treatment of <i>N-acetylglutamate synthetase (NAGS) deficiency</i> . It was originally designated an orphan medicine for this indication on 18 October 2000.	28/01/2003	28/01/2013
CEPLENE	histamine dihydrochloride	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 11 April 2005.	09/10/2008	09/10/2018
CUPRIOR	trientine	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 19 March 2015.	05/09/2017	20/07/2017



TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
CYRAMZA	ramucirumab	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 6 July 2012.	23/12/2014	27/01/2016
CYSTADANE	betaine anhydrous	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 9 July 2001.	15/02/2007	19/02/2017
DIACOMIT	stiripentol	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 5 December 2001.	04/01/2007	09/01/2017
ELAPRASE	idursulfase	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 11 December 2001.	08/01/2007	10/01/2017
ELMIRON	pentosan polysulfate sodium	This product was withdrawn from the Community Register of designated Orphan Medicinal Products on request of the sponsor . It was originally designated an orphan medicine on 15 January 2015.	02/06/2017	11/04/2017
ELOCTA	efmoroctocog alfa	This product was withdrawn from the Community Register of designated Orphan Medicinal Products on request of the sponsor . It was originally designated an orphan medicine on 20 September 2010.	23/11/2015	23/11/2015
EMPLICITI	elotuzumab	This product was withdrawn from the Community Register of designated orphan medicinal products by the European Commission at the time of the granting of a marketing authorisation . It was originally designated an orphan medicine on 9 August 2012.	11/05/2016	08/04/2016
EVOLTRA	clofarabine	This product was withdrawn from the Community register of orphan medicinal products at the end of the period of market exclusivity . It was originally designated an orphan medicine on 7 February 2002.	31/05/2006	31/05/2016
EXJADE	deferasirox	This product was withdrawn from the Community register of orphan medicinal products at the end of the period of market exclusivity . It was originally designated an orphan medicine on 13 mars 2002	01/09/2006	01/09/2016
FABRAZYME	agalsidase beta	This product was withdrawn from the Community register of orphan medicinal products at the end of the period of market exclusivity . It was originally designated an orphan medicine on 8 August 2000.	07/08/2001	07/08/2011

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
GLIOLAN	5-aminolevulinic acid hydrochloride	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 13 November 2002.	07/09/2007	12/09/2017
GLIVEC	imatinib mesilate	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity for the following conditions: - Treatment of chronic myeloid leukaemia (it was designated an orphan medicine on 14/02/2001). It was withdrawn from the Community register of orphan medicinal products on April 2012 on request of the sponsor for the following conditions: - Treatment of malignant gastrointestinal stromal tumours (it was designated an orphan medicine on 20/11/2001) - Treatment of dermatofibrosarcoma protuberans (it was designated an orphan medicine on 26/08/2005); - Treatment of acute lymphoblastic leukaemia (it was designated an orphan medicine on 26/08/2005); - Treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome (it was designated an orphan medicine on 28/10/2005) - Treatment of myelodysplastic / myeloproliferative diseases (it was designated an orphan medicine on 23/12/2005)	12/11/2001 27/05/2002 18/09/2006 18/09/2006 01/12/2006 01/12/2006	12/11/2011 16/04/2012
ILARIS	canakinumab	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 20 March 2007.	27/10/2009	01/12/2010
INCRELEX	mecasermin	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 22 May 2006.	03/08/2007	07/08/2017
IXIARO	Purified inactivated Japanese encephalitis SA14-4-2 virus vaccine	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 26 January 2006.	02/04/2009	12/03/2009

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
JAKAVI	ruxolitinib	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor for the following conditions: -Treatment of polycythaemia vera (19/02/2014) -Treatment of chronic idiopathic myelofibrosis (07/11/2008) -Treatment of myelofibrosis secondary to polycythaemia vera or essential thrombocythaemia (03/04/2009).	28/08/2012	20/02/2015
JINARC	tolvaptan	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 5 August 2013	29/05/2015	26/03/2015
LENVIMA	lenvatinib	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 26 April 2013.	28/05/2015	01/08/2018
LITAK	cladribine	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 18 September 2001.	19/04/2004	19/04/2014
LYNPARZA	olaparib	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 6 December 2007.	16/12/2014	16/03/2018
LYSODREN	mitotane	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 12 June 2002.	30/04/2004	30/04/2014
MYOZYME	alglucosidase alfa	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 22 February 2001.	31/03/2006	31/03/2016
NAGLAZYME	galsulfase	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 22 February 2001.	26/01/2006	26/01/2016
NEOFORDEX	dexamethasone	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 9 June 2010.	16/03/2016	25/01/2016

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
NEXAVAR	sorafenib tosylate	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity for the following conditions: - Treatment of renal cell carcinoma (it was designated an orphan medicine on 29/07/2004) - Treatment of hepatocellular carcinoma (it was designated an orphan medicine on 11/04/2006).	19/07/2006 29/10/2007	22/07/2016 01/11/2017
NOVOTHIRTEEN	catridecacog	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 12 December 2003.	05/09/2012	01/07/2012
OBIZUR	Recombinant porcine factor VIII (B-domain-deleted)	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 20 September 2010.	13/11/2015	23/10/2015
ORFADIN	nitisinone	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 29 December 2000.	24/02/2005	24/02/2015
ORKAMBI	Lumacaftor / ivacaftor	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 22 August 2014.	24/11/2015	12/10/2015
PEDEA	ibuprofen	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 14 February 2001.	02/08/2004	02/08/2014
PRIALT	ziconotide	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 9 July 2001.	24/02/2005	24/02/2015
QUINSAIR	levofloxacin	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 23 September 2008.	30/03/2015	01/02/2015
REPLAGAL	agalsidase alfa	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 8 August 2000.	07/08/2001	07/08/2011

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
REVATIO	Sildenafil citrate	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 17 December 2003.	04/11/2005	04/11/2015
REVLIMID	lenalidomide	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity for the following condition: - Treatment of multiple myeloma . It was originally designated an orphan medicine for this indication on 12 December 2003	14/06/2007	19/06/2017
REVOLADE	eltrombopag	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 3 August 2007.	15/03/2010	01/01/2012
RUBRACA	rucaparib	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 10 October 2012.	24/05/2018	4/12/2018
SAVENE	dexrazoxane	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 19 september 2001	02/08/2006	02/08/2016
SIKLOS	hydroxycarbamide	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 . This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 9 July 2003.	29/06/2007	Addmedica
SOMAVERT	pegvisomant	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 14 February 2001.	15/11/2002	15/11/2012
SPECTRILA	asparaginase	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 26 january 2005.	18/01/2016	18/01/2016
SPRYCEL	dasatinib	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 23 December 2005	20/11/2006	22/11/2016



TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
SUTENT	sunitinib malate	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 10 March 2005.	15/01/2007	23/07/2008
THALIDOMIDE CELGENE (previously THALIDOMIDE PHARMION)	thalidomide	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 20 November 2001.	16/04/2008	18/04/2018
TORISEL	temsirolimus	First-line treatment of adult patients with advanced renal cell carcinoma (RCC) who have at least three of six prognostic risk factors. This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 6 April 2006.	19/11/2007	21/11/2017
TRACLEER	bosentan monohydrate	This product is no longer an orphan medicine. It was withdrawn from the Community register of orphan medicinal products on request of the sponsor for the following condition: - Treatment of systemic sclerosis (it was designated an orphan medicine on 17/03/2003) It was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity for the following condition: - Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension (it was designated an orphan medicine on 14/02/2001)	11/06/2007 17/05/2002	04/04/2014 17/05/2012
TRISENOX	arsenic trioxide	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 18 October 2000.	07/03/2002	07/03/2012
UPTRAVI	selexipag	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor at the time of the granting of a marketing authorization . It was originally designated an orphan medicine on 26 August 2005.	12/05/2016	22/02/2016
VENCLYXTO	venetoclax	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 6 December 2012.	05/12/2016	12/10/2018

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
VENTAVIS	iloprost	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 29 December 2000.	18/09/2003	18/09/2013
VOLIBRIS	ambrisentan	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 11 April 2005.	21/04/2008	24/04/2018
WILZIN	zinc acetate dihydrate	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity . It was originally designated an orphan medicine on 31 July 2001.	18/10/2004	18/10/2014
XAGRID	anagrelide hydrochloride	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity+ 2 years for an agreed paediatric investigation plan (PIP) . It was originally designated an orphan medicine on 29 December 2000.	16/11/2004	18/11/2016
XYREM	sodium oxybate	This product was withdrawn from the Community Register of designated orphan medicinal products on request of the sponsor . It was originally designated an orphan medicine on 3 February 2003.	18/10/2005	11/01/2010
YONDELIS	trabectedin	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity for the following condition: - Treatment of soft tissue sarcoma . It was originally designated an orphan medicine for this indication on 30 May 2001.	17/09/2007	21/09/2017
ZAVESCA	miglustat	This product was withdrawn from the Community register of orphan medicinal products at the end of the 10-year period of market exclusivity for the following condition: - Treatment of type 1 Gaucher disease . It was originally designated an orphan medicine for this indication on 18 October 2000.	21/11/2002	21/11/2012

Anexo 2: Medicamentos huérfanos retirados de su uso en la Unión Europea

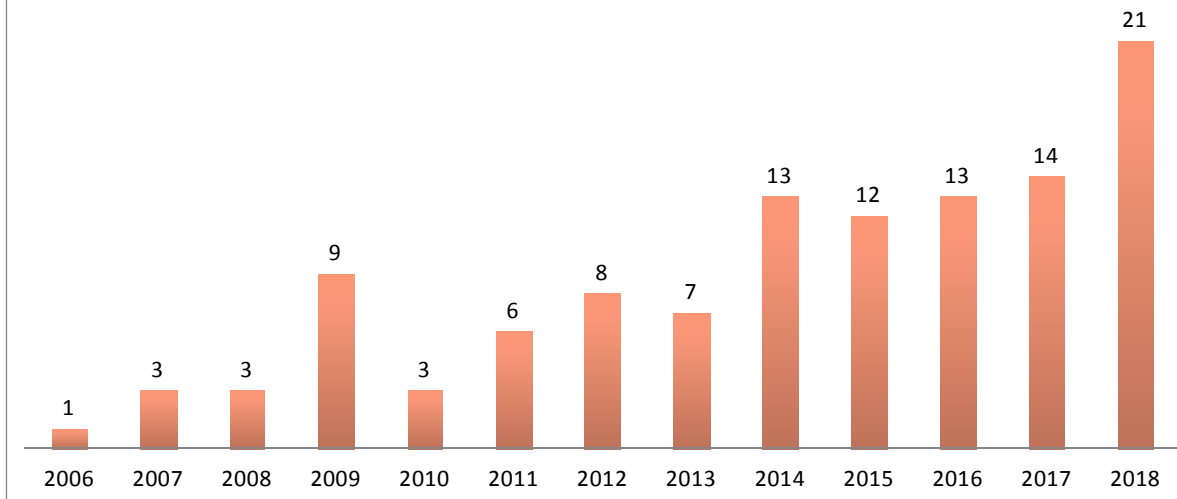
Más información: www.ema.europa.eu

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION (MA) INDICATION	MA DATE / MA HOLDER	MA WITHDRAWN DATE
GLYBERA	alipogene tiparvovec	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.	25/10/2012 uniQure biopharma B.V.	29/10/2017
ONSENAL	celecoxib	Reduction of the number of adenomatous intestinal polyps in familial adenomatous polyposis (FAP) , as an adjunct to surgery and further endoscopic surveillance.	17/10/2003 Pfizer Ltd	28/03/2011
PHOTOBARR	porfimer sodium (for use with photodynamic therapy)	Ablation of high-grade dysplasia (HGD) in patients with Barrett's oesophagus .	25/03/2004 Pinnacle Biologics B.V.	20/04/2012
RILONACEPT REGENERON (previously ARCALYST)	rilonacept	Treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) with severe symptoms, including Familial Cold Autoinflammatory Syndrome (FCAS) and Muckle-Wells Syndrome (MWS) , in adults and children aged 12 years and older.	23/10/2009 Regeneron UK Ltd	24/10/2012
THELIN	sitaxentan sodium	Treatment of patients with pulmonary arterial hypertension classified as WHO functional class III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and in pulmonary hypertension associated with connective tissue disease.	10/08/2006 Pfizer Ltd	06/01/2011
UNITUXIN	dinutuximab	Treatment of high-risk neuroblastoma in patients 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 (ASCT). It is administered in combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), interleukin-2 (IL-2), and isotretinoin.	14/08/2015 United Therapeutics Europe Ltd	20/03/2017

Clasificación por fecha de la AC en orden decreciente

2018			
ALOFISEL	RYDAPT	DELTYBA	TOBI PODHALER
AMGLIDIA	SPINRAZA	GAZYVARO	VOTUBIA
CABLIVI	XERMELO	GRANUPAS	VYNDAQEL
CRYSVITA	ZEJULA	IMBRUVICA	2010
JORVEZA	2016	KETOCONAZOLE HRA	ARZERRA
KYMRIAH	ALPROLIX	KOLBAM	TEPADINA
LAMZEDE	COAGADEX	SCENESSE	VPRIV
LUXTURNA	DARZALEX	SIRTURO	2009
MEPSEVII	GALAFOLD	SYLVANT	CAYSTON
MYALEPTA	IDELVION	TRANSLARNA	FIRDAPSE
MYLOTARG	LARTRUVO	VIMIZIM	MEPACT
NAMUSCLA	NINLARO	2013	MOZOBIL
ONPATTRO	OCALIVA	DEFITELIO	NPLATE
POTELIGEO	ONIVYDE	ICLUSIG	PEYONA
PREVYMIS	SOMAKIT TOC	IMNOVID	TORISEL
SYMKEVI	STRIMVELIS	OPSUMIT	YONDELIS
TAKHZYRO	WAKIX	ORPHACOL	ZAVESCA
TEGSEDI	ZALMOXIS	PROCYSBI	2008
VERKAZIA	2015	REVLIMID	FIRAZYR
VYXEOS	BLINCYTO	2012	KUVAN
YESCARTA	CERDELGA	ADCETRIS	VIDAZA
2017	CRESEMBA	BRONCHITOL	2007
BAVENCIO	FARYDAK	DACOGEN	INOVELON
BESPONSA	HETLIOZ	KALYDECO	SOLIRIS
BRINEURA	HOLOCLAR	NEXOBRID	TASIGNA
CHENODEOXYCHOLI C ACID LEADIANT	KANUMA	REVESTIVE	2006
CYSTADROPS	KYPROLIS	SIGNIFOR	NEXAVAR
LEDAGA	OFEV	XALUPRINE	
LUTATHERA	RAVICTI	2011	
NATPAR	RAXONE	CARBAGLU	
OXERVATE	STRENSIQ	ESBRIET	
QARZIBA	2014	PLENADREN	
	ADEMPAS		
	COMETRIQ		

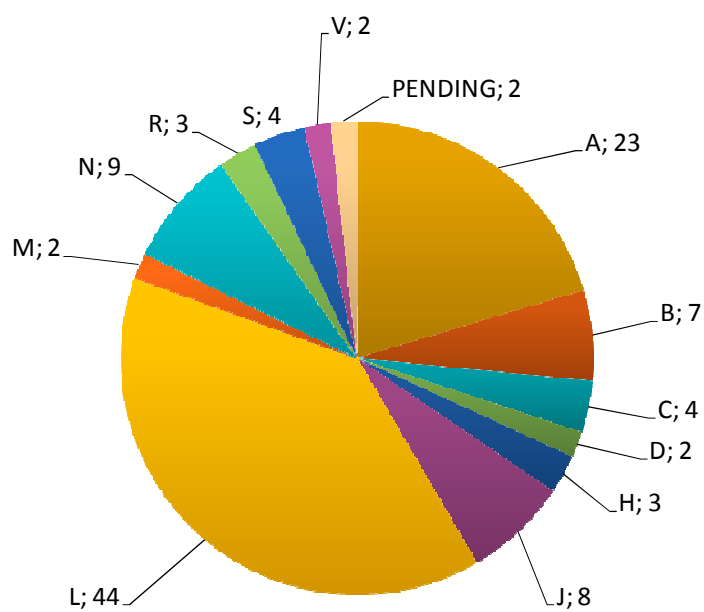
Número de medicamentos huérfanos en Europa con designación huérfana europea y autorización de comercialización europea por fecha de la AC



Clasificación por categoría ATC

A- ALIMENTARY TRACT AND METABOLISM	TAKHZYRO	COMETRIQ	YONDELIS
AMGLIDIA	C- CARDIOVASCULAR SYSTEM	DACOGEN	ZALMOXIS
BRINEURA	ADEMPAS	DARZALEX	ZEJULA
CARBAGLU	FIRAZYR	ESBRIET	M- MUSCULO-SKELETAL SYSTEM
CERDELGA	NAMUSCLA	FARYDAK	CRYSVITA
CHENODEOXYCHOLIC ACID LEADIANT	OPSUMIT	GAZYVARO	TRANSLARNA
GALAFOLD	D- DERMATOLOGICALS	ICLUSIG	N- NERVOUS SYSTEM
JORVEZA	NEXOBRID	IMBRUVICA	FIRDAPSE
KANUMA	SCENESSE	IMNOVID	HETLIOZ
KOLBAM	H- SYSTEMIC HORMONAL PREPARATIONS, EXCL. SEX HORMONES AND INSULINS	KYMRIAH	INOVELON
KUVAN	NATPAR	KYPROLIS	ONPATTRO
LAMZEDE	PLENADREN	LARTRUVO	PEYONA
MEPSEVII	SIGNIFOR	LEDAGA	RAXONE
MYALEPTA	J- GENERAL ANTIINFECTIVES FOR SYSTEMIC USE	MEPACT	SPINRAZA
OCALIVA	CAYSTON	MOZOBIL	VYNDAQEL
ORPHACOL	CRESEMBA	MYLOTARG	WAKIX
PROCYSBI	DELTYBA	NEXAVAR	R- RESPIRATORY
RAVICTI	GRANUPAS	NINLARO	BRONCHITOL
REVESTIVE	KETOCONAZOLE	OFEV	KALYDECO
STRENSIQ	PREVMIS	ONIVYDE	SYMKEVI
VIMIZIM	SIRTURO	POTELIGEO	S- SENSORY ORGANS
VPRIV	TOBI PODHALER	QARZIBA	CYSTADROPS
XERMELO	L- ANTINEOPLASTIC AND IMMUNOMODULATING	REVLIMID	HOLOCLAR
ZAVESCA	ADCETRIS	RYDAPT	OXERVATE
B- BLOOD AND BLOOD FORMING	ALOFISEL	SOLIRIS	VERKAZIA
ALPROLIX	ARZERRA	STRIMVELIS	V- VARIOUS
CABLIVI	BAVENCIO	SYLVANT	LUTATHERA
COAGADEX	BESPONSA	TASIGNA	SOMAKIT TOC
DEFITELIO	BLINCYTO	TEPADINA	PENDING
IDELVION		TORISEL	LUXTURNA
NPLATE		VIDAZA	TEGSEDI
		VOTUBIA	
		VYXEOS	
		XALUPRINE	
		YESCARTA	

Número de medicamentos huérfanos en Europa con designación huérfana europea y autorización de comercialización europea por categoría ATC



Clasificación por titular de la AC

ABLYNX N.V.	FIRDAPSE	INTERNATIONAL NV	CARBAGLU
CABLIVI	KUVAN	DACOGEN	CYSTADROPS
ACTELION	VIMIZIM	DARZALEX	OTSUKA NOVEL
REGISTRATION LTD	BIOPROJET PHARMA	IMBRUVICA	PRODUCTS GMBH
LEDAGA	WAKIX	OPSUMIT	DELTYBA
ADIENNE SRL	BOEHRINGER INGELHEIM	SIRTURO	PFIZER EUROPE MA EEIG
TEPADINA	INTERNATIONAL GMBH	SYLVANT	BESPONSА
ADVANCED	OFEV	ZAVESCA	MYLOTARG
ACCELERATOR	CELGENE EUROPE B.V.	JAZZ PHARMACEUTICALS	TORISEL
APPLICATIONS	IMNOVID	IRELAND LTD	VYNDAQEL
LUTATHERA	REVLIMID	VYXEOS	PHARMA MAR S.A.
SOMAKIT TOC	VIDAZA	KITE PHARMA EU B.V.	YONDELIS
AEGERION	CHIESI FARMACEUTICI SPA	YESCARTA	PHARMAXIS
PHARMACEUTICALS B.V.	HOLOCLAR	KYOWA KIRIN HOLDINGS	PHARMACEUTICALS LTD
MYALEPTA	LAMZEDE	B.V.	BRONCHITOL
AKCEA THERAPEUTICS UK	PEYONA	CRYSVITA	PTC THERAPEUTICS
LTD.	PROCYSBI	POTELIGEO	INTERNATIONAL LTD
TEGSEDI	CLINUVEL UK LIMITED	LABORATOIRE HRA	TRANSLARNA
ALEXION EUROPE SAS	SCENESSE	PHARMA	RETROPHIN EUROPE LTD
KANUMA	CSL BEHRING GMBH	KETOCONAZOLE HRA	KOLBAM
SOLIRIS	IDELVION	LABORATOIRES CTRS	ROCHE REGISTRATION
STRENSIQ	DOMPE FARMACEUTICI	ORPHACOL	GMBH
ALNYLAM NETHERLANDS	S.P.A.	LEADIANT GmbH	ESBRIET
B.V.	OXERVATE	CHENODEOXYCHOLIC	GAZYVARO
ONPATTRO	DR. FALK PHARMA GMBH	ACID LEADIANT	SANTEN OY
AMGEN EUROPE B.V.	JORVEZA	LUPIN EUROPE GmbH	VERKAZIA
BLINCYTO	ELI LILLY NEDERLAND B.V.	NAMUSCLA	SANTHERA
KYPROLIS	LARTRUVO	MEDIWOUND GERMANY	PHARMACEUTICALS
NPLATE	ESAI GmbH	GMBH	(DEUTSCHLAND) GMBH
AMICUS THERAPEUTICS	INOVELON	NEXOBRID	RAXONE
UK LTD	EUROCEPT	MERCK EUROPE B.V.	SHIRE PHARMACEUTICALS
GALAFOLD	INTERNATIONAL B.V.	BAVENCIO	IRELAND LTD
AMMTEK	GRANUPAS	MERCK SHARP & DOHME	FIRAZYR
AMGLIDIA	EUSA PHARMA UK LIMITED	B.V.	NATPAR
INCYTE BIOSCIENCES	QARZIBA	PREVYMIS	REVESTIVE
DISTRIBUTION B.V.	GENTIUM SRL	MolMed SpA	TAKHZYRO
ICLUSIG	DEFITELIO	ZALMOXIS	VPRIV
BASILEA MEDICAL LTD	GENZYME EUROPE B.V.	NOVA LABORATORIES LTD	SHIRE SERVICES BVBA
CRESEMBA	CERDELGA	XALUPRINE	PLENADREN
BAXALTA INNOVATIONS	MOZOBIL	NOVARTIS EUROPHARM	SPARK THERAPEUTICS
GMBH	GILEAD SCIENCES	LTD	IRELAND LTD
ONIVYDE	IRELAND UC	ARZERRA	LUXTURNA
BAYER AG	CAYSTON	FARYDAK	SWEDISH ORPHAN
ADEMPAS	HORIZON PHARMA	KYMRIAH	BIOVITRUM AB (PUBL)
NEXAVAR	IRELAND LIMITED	RYDAPT	ALPROLIX
BIO PRODUCTS	RAVICTI	SIGNIFOR	TAKEDA FRANCE SAS
LABORATORY LTD	INTERCEPT PHARMA LTD.	TASIGNA	MEPACT
COAGADEX	OICALIVA	TOBI PODHALER	TAKEDA PHARMA A/S.
BOIEN NETHERLANDS	IPSEN PHARMA	VOTUBIA	ADCETRIS
B.V.	COMETRIQ	ORCHARD THERAPEUTICS	ALOFISEL
SPINRAZA	XERMELO	(NETHERLANDS) B.V.	NINLARO
BIOMARIN	JANSSEN-CILAG	STRIMVELIS	TESARO UK LIMITED
INTERNATIONAL LIMITED		ORPHAN EUROPE SARL	ZEJULA
BRINEURA			

ULTRAGENYX GERMANY GMBH
MEPSEVII
VANDA

PHARMACEUTICALS LTD
HETLIOZ
VERTEX PHARMACEUTICALS

(EUROPE) LTD
KALYDECO
SYMKEVI

PARTE 2 :

Listado de productos medicinales para enfermedades raras en Europa con autorización de comercialización europea* sin designación huérfana en Europa

Índice

Listado de productos medicinales para enfermedades raras en Europa con autorización de comercialización europea* sin designación huérfana en Europa	36
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Metodología

En esta parte del documento se ofrece un listado de todos los medicamentos para enfermedades raras que han recibido una autorización de comercialización (AC) europea para una o más indicaciones de uso para una enfermedad rara, pero que no han recibido una designación huérfana europea o cuya designación ha sido retirada.

A estos fármacos se les puede haber concedido, o no, una designación huérfana en otras áreas geográficas del mundo. Aparecen en el listado de medicamentos de la Dirección DG SANTE a los que se les ha concedido una autorización de comercialización: <http://ec.europa.eu/health/documents/community-register/html/alfregister.htm>

Una primera clasificación por nombre comercial ofrece el nombre de la sustancia activa, la indicación rara de la autorización de

comercialización (AC), y la fecha y el titular de la AC.

Tres listados adicionales proponen otras clasificaciones por:

- fecha de la AC en orden descendiente,
- categoría ATC,
- titular de la AC.

Para cada listado, los nombres comerciales se presentan en orden alfabético.

Puede encontrar información adicional de cada producto en la pestaña "Medicamentos huérfanos" del sitio web de Orphanet www.orphanet.es o en el sitio web de la EMA (Agencia Europea de Medicamentos) <http://www.ema.europa.eu>.

**Autorización de comercialización de la Comunidad Europea por procedimiento centralizado*

Clasificación por nombre comercial

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
ABRAXANE	paclitaxel	In combination with gemcitabine is indicated for the first-line treatment of adult patients with metastatic adenocarcinoma of the pancreas .	11/01/2008	Celgene Europe B.V.
ABSEAMED	epoetin alfa	Treatment of symptomatic anaemia (haemoglobin concentration of ≤ 10 g/dl) in adults with low- or intermediate-1-risk primary myelodysplastic syndromes (MDS) who have low serum erythropoietin (< 200 mU/ml). (Indication extension)	27/08/2007	Medice Arzneimittel Pütter GmbH Co. KG
ADCIRCA	tadalafil	In adults for the treatment of pulmonary arterial hypertension (PAH) classified as WHO functional class II and III, to improve exercise capacity. Efficacy has been shown in idiopathic PAH (IPAH) and in PAH related to collagen vascular disease.	01/10/2008	Eli Lilly Nederland B.V.
ADVATE	octocog alpha	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency) . It is indicated in all age groups.	02/03/2004	Baxter AG
ADYNOVI	rurioctocog alfa pegol	Treatment and prophylaxis of bleeding in patients 12 years and above with haemophilia A (congenital factor VIII deficiency) .	08/01/2018	Baxalta Innovations GmbH
AFINITOR	everolimus	Treatment of unresectable or metastatic, well- or moderately-differentiated neuroendocrine tumours of pancreatic origin in adults with progressive disease. Treatment of unresectable or metastatic, well-differentiated (Grade 1 or Grade 2) non-functional neuroendocrine tumours of gastrointestinal or lung origin in adults with progressive disease Treatment of patients with advanced renal cell carcinoma , whose disease has progressed on or after treatment with VEGF-targeted therapy.	03/08/2009	Novartis Europharm Ltd
AFSTYLA	lonoctocog alfa	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency) .	04/01/2017	CSL Behring GmbH
ALDURAZYME	laronidase	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis I (MPS I; a [alpha]-L-iduronidase deficiency) to treat the non-neurological manifestations of the disease.	10/06/2003	Genzyme Europe B.V.
ALIMTA	pemetrexed	Treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma .	20/09/2004	Eli Lilly Nederland B.V.
ALKINDI	hydrocortisone	Replacement therapy of adrenal insufficiency in infants, children and adolescents (from birth to < 18 years old).	09/02/2018	Diurnal Europe B.V.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
AMGEVITA	Adalimumab	<p>In combination with methotrexate is indicated for the treatment of active polyarticular juvenile idiopathic arthritis, in patients from the age of 2 years who have had an inadequate response to one or more disease-modifying anti-rheumatic drugs (DMARDs). AMGEVITA can be given as monotherapy in case of intolerance to methotrexate or when continued treatment with methotrexate is inappropriate.</p> <p>Adalimumab has not been studied in patients aged less than 2 years.</p> <p>Treatment of active enthesitis-related arthritis in patients, 6 years of age and older, who have had an inadequate response to, or who are intolerant of, conventional therapy.</p> <p>Treatment of non-infectious intermediate, posterior and panuveitis in adult patients who have had an inadequate response to corticosteroids, in patients in need of corticosteroid-sparing, or in whom corticosteroid treatment is inappropriate.</p>	22/03/2017	Amgen Europe B.V.
AMMONAPS	sodium phenylbutyrate	<p>Adjunctive therapy in the chronic management of urea cycle disorders, involving deficiencies of carbamyl phosphate synthetase, ornithine transcarbamylase, or argininosuccinate synthetase. It is indicated in all patients with <i>neonatal-onset</i> presentation (complete enzyme deficiencies, presenting within the first 28 days of life). It is also indicated in patients with <i>late-onset</i> disease (partial enzyme deficiencies, presenting after the first month of life) who have a history of hyperammonaemic encephalopathy.</p>	08/12/1999	Swedish Orphan Biovitrum AB
ANAGRELIDE MYLAN	anagrelide hydrochloride	<p>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.</p> <p>An at risk essential thrombocythaemia patient is defined by one or more of the following features:</p> <ul style="list-style-type: none"> • > 60 years of age or • a platelet count > 1,000 x 10⁹/l or an history of thrombo-haemorrhagic events. 	15/02/2018	Mylan S.A.S.
ARMISARTE (previously PEMETREXED ACTAVIS)	pemetrexed	<p>In combination with cisplatin is indicated for the treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma.</p>	18/01/2016	Actavis Group PTC ehf

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
ATRIANCE	nelarabine	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.	22/08/2007	Novartis Europharm Ltd
ATRYN	antithrombin alpha	Prophylaxis of venous thromboembolism in surgery of adult patients with congenital antithrombin deficiency . It is normally given in association with heparin or low molecular weight heparin.	28/07/2006	Laboratoire français du Fractionnement et des Biotechnologies
AVASTIN	bevacizumab	In combination with interferon alfa-2a it is indicated for first line treatment of adult patients with advanced and/or metastatic renal cell cancer . In combination with carboplatin and paclitaxel, it is indicated for the front-line treatment of adult patients with advanced (International Federation of Gynecology and Obstetrics (FIGO) stages III B, III C and IV) epithelial ovarian, fallopian tube, or primary peritoneal cancer . In combination with carboplatin and gemcitabine or in combination with carboplatin and paclitaxel, is indicated for treatment of adult patients with first recurrence of platinum-sensitive epithelial ovarian, fallopian tube or primary peritoneal cancer who have not received prior therapy with bevacizumab or other VEGF inhibitors or VEGF receptor-targeted agents. In combination with paclitaxel, topotecan, or pegylated liposomal doxorubicin it is indicated for the treatment of adult patients with platinum-resistant recurrent epithelial ovarian, fallopian tube, or primary peritoneal cancer who received no more than two prior chemotherapy regimens and who have not received prior therapy with bevacizumab or other VEGF inhibitors or VEGF receptor-targeted agents.	12/01/2005	Roche Registration GmbH
BEMFOLA	follitropin alfa	In adult men: stimulation of spermatogenesis in men who have congenital or acquired hypogonadotropic hypogonadism with concomitant human chorionic gonadotropin (hCG) therapy.	27/03/2014	Gedeon Richter Plc.
BENEFIX	nonacog alpha	Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency) .	27/08/1997	Pfizer Europe MA EEIG

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
BINOCRIT	epoetin alfa	Treatment of symptomatic anaemia (haemoglobin concentration of ≤ 10 g/dl) in adults with low- or intermediate-1-risk primary myelodysplastic syndromes (MDS) who have low serum erythropoietin (< 200 mU/ml). (Indication extension)	27/08/2007	Sandoz GmbH
BLITZIMA	rituximab	<p>Treatment of previously untreated patients with stage III-IV follicular lymphoma in combination with chemotherapy.</p> <p>As maintenance therapy indicated for the treatment of follicular lymphoma patients responding to induction therapy.</p> <p>As monotherapy indicated for the treatment of patients with stage III-IV follicular lymphoma who are chemo-resistant or are in their second or subsequent relapse after chemotherapy.</p> <p>Treatment of patients with CD20 positive diffuse large B cell non-Hodgkin's lymphoma in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy.</p> <p>In combination with chemotherapy for the treatment of patients with previously untreated and relapsed/refractory CLL. Only limited data are available on efficacy and safety for patients previously treated with monoclonal antibodies including Blitzima or patients refractory to previous Blitzima plus chemotherapy.</p>	13/07/2017	Celltrion Healthcare Hungary Kft.
BORTEZOMIB ACCORD	bortezomib	<p>As monotherapy or in combination with pegylated liposomal doxorubicin or dexamethasone for the treatment of adult patients with progressive multiple myeloma who have received at least 1 prior therapy and who have already undergone or are unsuitable for haematopoietic stem cell transplantation.</p> <p>In combination with melphalan and prednisone for the treatment of adult patients with previously untreated multiple myeloma who are not eligible for high-dose chemotherapy with haematopoietic stem cell transplantation.</p> <p>In combination with dexamethasone, or with dexamethasone and thalidomide, for the induction treatment of adult patients with previously untreated multiple myeloma who are eligible for high-dose chemotherapy with haematopoietic stem cell transplantation.</p> <p>In combination with rituximab, cyclophosphamide, doxorubicin and prednisone for the treatment of adult patients with previously untreated mantle cell lymphoma who are unsuitable for haematopoietic stem cell transplantation.</p>	20/07/2015	Accord Healthcare Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
BORTEZOMIB HOSPIRA	bortezomib	<p>As monotherapy or in combination with pegylated liposomal doxorubicin or dexamethasone is indicated for the treatment of adult patients with progressive multiple myeloma who have received at least 1 prior therapy and who have already undergone or are unsuitable for haematopoietic stem cell transplantation.</p> <p>In combination with melphalan and prednisone is indicated for the treatment of adult patients with previously untreated multiple myeloma who are not eligible for high-dose chemotherapy with haematopoietic stem cell transplantation.</p> <p>In combination with dexamethasone, or with dexamethasone and thalidomide, is indicated for the induction treatment of adult patients with previously untreated multiple myeloma who are eligible for high-dose chemotherapy with haematopoietic stem cell transplantation.</p> <p>In combination with rituximab, cyclophosphamide, doxorubicin and prednisone is indicated for the treatment of adult patients with previously untreated mantle cell lymphoma who are unsuitable for haematopoietic stem cell transplantation.</p>	22/07/2016	Hospira UK Limited
BORTEZOMIB SUN	bortezomib	<p>As monotherapy or in combination with pegylated liposomal doxorubicin or dexamethasone is indicated for the treatment of adult patients with progressive multiple myeloma who have received at least 1 prior therapy and who have already undergone or are unsuitable for haematopoietic stem cell transplantation.</p> <p>In combination with melphalan and prednisone is indicated for the treatment of adult patients with previously untreated multiple myeloma who are not eligible for high-dose chemotherapy with haematopoietic stem cell transplantation.</p> <p>In combination with dexamethasone, or with dexamethasone and thalidomide, is indicated for the induction treatment of adult patients with previously untreated multiple myeloma who are eligible for high-dose chemotherapy with haematopoietic stem cell transplantation.</p> <p>In combination with rituximab, cyclophosphamide, doxorubicin and prednisone is indicated for the treatment of adult patients with previously untreated mantle cell lymphoma who are unsuitable for haematopoietic stem cell transplantation.</p>	22/07/2016	SUN Pharmaceutical Industries (Europe) B.V.

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BOSULIF	bosutinib	Treatment of adult patients with: - newly diagnosed chronic phase (CP) Philadelphia chromosome-positive chronic myelogenous leukaemia (Ph+ CML) . - CP, accelerated phase (AP), and blast phase (BP) Ph+ CML previously treated with one or more tyrosine kinase inhibitor(s) [TKI(s)] and for whom imatinib, nilotinib and dasatinib are not considered appropriate treatment options.	27/03/2013	Pfizer Europe MA EEIG
BUCCOLAM	midazolam	Treatment of prolonged, acute, convulsive seizures in infants, toddlers, children and adolescents (from 3 months to < 18 years). Buccolam must only be used by parents/carers where the patient has been diagnosed to have epilepsy . For infants between 3-6 months of age treatment should be in a hospital setting where monitoring is possible and resuscitation equipment is available.	05/09/2011	Shire Services BVBA
BUSILVEX	busulfan	Followed by cyclophosphamide (BuCy2), conditioning treatment prior to conventional haematopoietic progenitor cell transplantation in adult patients when the combination is considered the best available option. Following fludarabine (FB), conditioning treatment prior to haematopoietic progenitor cell transplantation in adult patients who are candidates for a reduced-intensity conditioning (RIC) regimen. Followed by cyclophosphamide (BuCy4) or melphalan (BuMel), conditioning treatment prior to conventional haematopoietic progenitor cell transplantation in paediatric patients.	09/07/2003	Pierre Fabre Médicament
CABOMETYX	cabozantinib	Treatment of advanced renal cell carcinoma (RCC) : - in treatment-naïve adults with intermediate or poor risk - in adults following prior vascular endothelial growth factor (VEGF)-targeted therapy. As monotherapy for the treatment of hepatocellular carcinoma (HCC) in adults who have previously been treated with sorafenib.	09/09/2016	Ipsen Pharma

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CAELYX	doxorubicin hydrochloride (pegylated liposomal)	<p>Treatment of advanced ovarian cancer in women who have failed a first-line platinum-based chemotherapy regimen.</p> <p>In combination with bortezomib for the treatment of progressive multiple myeloma in patients who have received at least one prior therapy and who have already undergone or are unsuitable for bone marrow transplant.</p> <p>Treatment of AIDS-related Kaposi's sarcoma (KS) in patients with low CD4 counts (< 200 CD4 lymphocytes/mm³) and extensive mucocutaneous or visceral disease.</p> <p>Used as first-line systemic chemotherapy, or as second line chemotherapy in AIDS-KS patients with disease that has progressed with, or in patients intolerant to, prior combination systemic chemotherapy comprising at least two of the following agents: a vinca alkaloid, bleomycin and standard doxorubicin (or other anthracycline).</p>	21/06/1996	Janssen-Cilag International N.V.
CANCIDAS (previously CASPOFUNGIN MSD)	casposungin	<p>Treatment of invasive candidiasis in adult or paediatric patients.</p> <p>Treatment of invasive aspergillosis in adult or paediatric patients who are refractory to or intolerant of amphotericin B, lipid formulations of amphotericin B and/or itraconazole.</p> <p>Empirical therapy for presumed fungal infections (such as Candida or Aspergillus) in febrile, neutropaenic adult or paediatric patients.</p>	24/10/2001	Merck Sharp & Dohme B.V.
CAPRELSA	vandetanib	<p>Treatment of aggressive and symptomatic medullary thyroid cancer (MTC) in patients with unresectable locally advanced or metastatic disease.</p> <p>Caprelsa is indicated in adults, children and adolescents aged 5 years and older.</p> <p>For patients in whom Rearranged during Transfection (RET) mutation is not known or is negative, a possible lower benefit should be taken into account before individual treatment decision.</p>	17/02/2012	Genzyme Europe B.V.
CARBAGLU	carglumic acid	Treatment of hyperammonaemia due to N-acetylglutamate synthase (NAGS) primary deficiency	28/01/2003	Orphan Europe S.A.R.L
CARMUSTINE OBVIUS	carmustine	<p>As a single agent or in combination with other antineoplastic agents and/or other therapeutic measures (radiotherapy, surgery):</p> <ul style="list-style-type: none"> - Brain tumours (glioblastoma, Brain-stem gliomas, medulloblastoma, astrocytoma and ependymoma), brain metastases - Secondary therapy in non-Hodgkin's lymphoma and Hodgkin's disease. 	18/07/2018	Obvius Investment B.V.

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CEPLENE	histamine dihydrochloride	Maintenance therapy 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.	09/10/2008	Noventia Pharma Srl
CEPROTIN	human protein c	In purpura fulminans and coumarin-induced skin necrosis in patients with severe congenital protein C deficiency . Short-term prophylaxis in patients with severe congenital protein C deficiency : if surgery or invasive therapy is imminent, while initiating coumarin therapy, when coumarin therapy alone is not sufficient, when coumarin therapy is not feasible.	16/07/2001	Baxter AG
CEREZYME	imiglucerase	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of non-neuronopathic (Type 1) or chronic neuronopathic (Type 3) Gaucher disease and who exhibit clinically significant. The non-neurological manifestations of Gaucher disease include one or more of the following conditions: -Anaemia after exclusion of other causes, such as iron deficiency -Thrombocytopenia -Bone disease after exclusion of other causes such as Vitamin D deficiency -Hepatomegaly or splenomegaly	17/11/1997	Genzyme Europe B.V.
CINRYZE	C1 inhibitor(human)	Treatment and pre-procedure prevention of angioedema attacks in adults and adolescents with hereditary angioedema (HAE) . Routine prevention of angioedema attacks in adults and adolescents with severe and recurrent attacks of hereditary angioedema (HAE), who are intolerant to or insufficiently protected by oral prevention treatments, or patients who are inadequately managed with repeated acute treatment.	15/06/2011	Shire Services BVBA
COLOBREATHE	colistimethate sodium	Management of chronic pulmonary infections due to <i>Pseudomonas aeruginosa</i> 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.	13/02/2012	Teva B.V.
CUPRIOR	trientine	Treatment of Wilson's disease in adults, adolescents and children ≥ 5 years intolerant to D-penicillamine therapy.	05/09/2017	GMP-Orphan SA

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
CYLTEZO	adalimumab	<p>In combination with methotrexate is indicated for the treatment of active polyarticular juvenile idiopathic arthritis, in patients from the age of 2 years who have had an inadequate response to one or more disease-modifying anti-rheumatic drugs (DMARDs). Adalimumab can be given as monotherapy in case of intolerance to methotrexate or when continued treatment with methotrexate is inappropriate. Adalimumab has not been studied in patients aged less than 2 years.</p> <p>Treatment of active enthesitis-related arthritis in patients, 6 years of age and older, who have had an inadequate response to, or who are intolerant of, conventional therapy.</p> <p>Treatment of non-infectious intermediate, posterior and panuveitis in adult patients who have had an inadequate response to corticosteroids, in patients in need of corticosteroid-sparing, or in whom corticosteroid treatment is inappropriate.</p>	10/11/2017	Boehringer Ingelheim International GmbH
CYSTADANE	betaine anhydrous	<p>Adjunctive treatment of homocystinuria, involving deficiencies or defects in cystathionine beta- synthase (CBS), 5,10-methylene-tetrahydrofolate reductase (MTHFR), cobalamin cofactor metabolism (cbl).</p> <p>Cystadane should be used as supplement to other therapies such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), folate and a specific diet.</p>	15/02/2007	Orphan Europe S.a.r.l.
CYSTAGON	mercaptamine bitartrate	<p>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.</p>	23/06/1997	Orphan Europe S.A.R.L
DEFERIPRONE LIPOMED	deferiprone	<p>As monotherapy for the treatment of iron overload in patients with thalassaemia major when current chelation therapy is contraindicated or inadequate.</p> <p>Deferiprone Lipomed in combination with another chelator is indicated in patients with thalassaemia major when monotherapy with any iron chelator is ineffective, or when prevention or treatment of life-threatening consequences of iron overload justifies rapid or intensive correction.</p>	19/09/2018	Lipomed GmbH
DENGVAIXIA	dengue tetravalent vaccine (live, attenuated)	<p>Prevention of dengue disease caused by dengue virus serotypes 1, 2, 3 and 4 in individuals 9 to 45 years of age with prior dengue virus infection and living in endemic areas.</p>	12/12/2018	Sanofi Pasteur

NEW

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DIACOMIT	stiripentol	Used 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.	04/01/2007	Biocodex
DUKORAL	vibrio cholerae and recombinant cholera toxinb-subunit	Indicated for active immunisation against disease caused by Vibrio cholerae serogroup O1 in adults and children from 2 years of age who will be visiting endemic/epidemic areas. The use of Dukoral should be determined on the basis of official recommendations taking into consideration the variability of epidemiology and the risk of contracting disease in different geographical areas and travelling conditions. Dukoral should not replace standard protective measures. In the event of diarrhoea measures of rehydration should be instituted.	28/04/2004	Valneva Sweden AB
ELAPRASE	idursulfase	Long-term treatment of patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II) . Heterozygous females were not studied in the clinical trials.	08/01/2007	Shire Human Genetic Therapies AB
ELMIRON	pentosan polysulfate sodium	Treatment of bladder pain syndrome characterized by either glomerulations or Hunner's lesions in adults with moderate to severe pain, urgency and frequency of micturition.	02/06/2017	bene-Arzneimittel GmbH
ELOCTA	efmoroctocog alfa	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency). ELOCTA can be used for all age groups.	19/11/2015	Swedish Orphan Biovitrum AB (publ)
EMPLICITI	elotuzumab	In combination with lenalidomide and dexamethasone for the treatment of multiple myeloma in adult patients who have received at least one prior therapy.	11/05/2016	Bristol-Myers Squibb
ENBREL	etanercept	Treatment of polyarthritis (rheumatoid-factorpositive or -negative) and extended oligoarthritis in children and adolescents from the age of 2 years who have had an inadequate response to, or who have proved intolerant of, methotrexate. Treatment of psoriatic arthritis in adolescents from the age of 12 years who have had an inadequate response to, or who have proved intolerant of, methotrexate. Treatment of enthesitis-related arthritis in adolescents from the age of 12 years who have had an inadequate response to, or who have proved intolerant of conventional therapy. Enbrel has not been studied in children aged less than 2 years.	03/02/2000	Pfizer Europe MA EEIG

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EPOETIN ALFA HEXAL	epoetin alfa	Treatment of symptomatic anaemia (haemoglobin concentration of ≤ 10 g/dl) in adults with low- or intermediate-1-risk primary myelodysplastic syndromes (MDS) who have low serum erythropoietin (< 200 mU/ml). (Indication extension)	27/08/2007	Hexal AG
ERBITUX	cetuximab	Treatment of patients with squamous cell cancer of the head and neck : - in combination with radiation therapy for locally advanced disease, - in combination with platinum-based chemotherapy for recurrent and/or metastatic disease.	29/06/2004	Merck KGaA
ERELZI	etanercept	Treatment of polyarthritis (rheumatoid factor positive or negative) and extended oligoarthritis in children and adolescents from the age of 2 years who have had an inadequate response to, or who have proved intolerant of, methotrexate. Treatment of psoriatic arthritis in adolescents from the age of 12 years who have had an inadequate response to, or who have proved intolerant of, methotrexate. Treatment of enthesitis-related arthritis in adolescents from the age of 12 years who have had an inadequate response to, or who have proved intolerant of, conventional therapy. Etanercept has not been studied in children aged less than 2 years.	23/06/2017	Sandoz GmbH
ERIVEDGE	vismodegib	Treatment of adult patients with symptomatic metastatic basal cell carcinoma Treatment of adult patients with locally advanced basal cell carcinoma inappropriate for surgery or radiotherapy	12/07/2013	Roche Registration GmbH
EURARTESIM	piperazine tetraphosphate/ dihydroartemisinin	Treatment of uncomplicated Plasmodium falciparum malaria in adults, children and infants 6 months and over and weighing 5 kg or more. Consideration should be given to official guidance on the appropriate use of antimalarial agents.	27/10/2011	Alfasigma S.p.A
EVOLTRA	clofarabine	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.	29/05/2006	Genzyme Europe B.V.

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EXJADE	deferasirox	<p>Treatment of chronic iron overload due to frequent blood transfusions ($\geq 7\text{ml/kg/month}$ of packed red blood cells) in patients with beta thalassaemia major aged 6 years and older.</p> <p>Treatment of chronic iron overload due to blood transfusions when deferoxamine therapy is contraindicated or inadequate in the following patient groups:</p> <ul style="list-style-type: none"> -in paediatric patients with beta thalassaemia major with iron overload due to frequent blood transfusions ($\geq 7\text{ml/kg/month}$ of packed red blood cells) aged 2 to 5 years, -in adult and paediatric patients with beta thalassaemia major with iron overload due to infrequent blood transfusions ($< 7\text{ml/kg/month}$ of packed red blood cells) aged 2 years and older, -in adult and paediatric patients with other anaemias aged 2 years and older. <p>Treatment of chronic iron overload requiring chelation therapy when deferoxamine therapy is contraindicated or inadequate in patients with non-transfusion dependent thalassaemia syndromes aged 10 years and older.</p>	01/09/2006	Novartis Europharm Limited
FABRAZYME	agalsidase beta	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (alphagalactosidase A deficiency) .	03/08/2001	Genzyme Europe B.V.
FERRIPROX	deferiprone	Treatment of iron overload in patients with thalassaemia major when deferoxamine therapy is contraindicated or inadequate.	25/08/1999	Apotex Europe B.V.
FILGRASTIM HEXAL	filgrastim	<p>In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/\text{l}$, and a history of severe or recurrent infections.</p> <p>Long term administration of filgrastim is indicated to increase neutrophil counts and to reduce the incidence and duration of infection-related events.</p>	06/02/2009	Hexal AG

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FLEBOGAMMA DIF	human normal immunoglobulin	<p>Replacement therapy in adults, and children and adolescents (2-18 years) in:</p> <ul style="list-style-type: none"> - Primary immunodeficiency (PID) syndromes with impaired antibody production. - Hypogammaglobulinaemia and recurrent bacterial infections in patients with chronic lymphocytic leukaemia, in whom prophylactic antibiotics have failed. - Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase multiple myeloma patients who have failed to respond to pneumococcal immunisation. - Hypogammaglobulinaemia in patients after allogeneic haematopoietic stem cell transplantation (HSCT). <p>Immunomodulation in adults, and children and adolescents (2-18 years) in</p> <ul style="list-style-type: none"> - Primary immune thrombocytopenia (ITP), in patients at high risk of bleeding or prior to surgery to correct the platelet count. - Guillain-Barré syndrome - Kawasaki disease. 	23/07/2007	Instituto Grifols S.A.
FOTIVDA	tivozanib hydrochloride monohydrate	First line treatment of adult patients with advanced renal cell carcinoma (RCC) and for adult patients who are VEGFR and mTOR pathway inhibitor-naïve following disease progression after one prior treatment with cytokine therapy for advanced RCC.	24/08/2017	EUSA Pharma (UK) Limited
GLIOLAN	5-aminolevulinic acid hydrochloride	In adult patients for visualisation of malignant tissue during surgery for malignant glioma (World Health Organization grade III and IV).	07/09/2007	medac Gesellschaft für klinische Spezialpräparate mbH

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GLIVEC	imatinib mesilate	<p>Treatment of adult and paediatric patients with newly diagnosed Philadelphia chromosome (bcr-abl) positive (Ph+) chronic myeloid leukaemia (CML) for whom bone marrow transplantation is not considered as the first line of treatment.</p> <p>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.</p> <p>Treatment of adult and paediatric patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) integrated with chemotherapy.</p> <p>Treatment of adult patients with relapsed or refractory Ph+ ALL as monotherapy.</p> <p>Treatment of adult patients with myelodysplastic / myeloproliferative diseases (MDS/MPD) associated with platelet-derived growth factor receptor (PDGFR) gene rearrangements.</p> <p>Treatment of adult patients with advanced hypereosinophilic syndrome (HES) and/or chronic eosinophilic leukaemia (CEL) with FIP1L1-PDGFRα rearrangement.</p> <p>The effect of Glivec on the outcome of bone marrow transplantation has not been determined.</p> <p>Treatment of adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumours (GIST).</p> <p>Adjuvant treatment of adult patients who are at significant risk of relapse following resection of Kit (CD117)-positive GIST. Patients who have a low or very low risk of recurrence should not receive adjuvant treatment.</p> <p>Treatment of adult patients with unresectable dermatofibrosarcoma protuberans (DFSP) and adult patients with recurrent and/or metastatic DFSP who are not eligible for surgery.</p>	07/11/2001	Novartis Europharm Ltd
GONAL-F	follitropin alpha	Stimulation of spermatogenesis in men who have congenital or acquired hypogonadotrophic hypogonadism with concomitant human Chorionic Gonadotrophin (hCG) therapy.	20/10/1995	Merck Europe B.V.
GRASTOFIL	filgrastim	In adult or children patients with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/L$, and a history of severe or recurrent infections, long term administration of Grastofil is indicated to increase neutrophil counts and to reduce the incidence and duration of infection-related events.	18/10/2013	Apotex Europe B.V.

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HALAVEN	eribulin	Treatment of adult patients with unresectable liposarcoma who have received prior anthracycline containing therapy (unless unsuitable) for advanced or metastatic disease.	17/03/2011	Eisai GmbH
HALIMATOZ	adalimumab	<p>In combination with methotrexate is indicated for the treatment of active polyarticular juvenile idiopathic arthritis, in patients from the age of 2 years who have had an inadequate response to one or more disease-modifying anti-rheumatic drugs (DMARDs). HYRIMOZ can be given as monotherapy in case of intolerance to methotrexate or when continued treatment with methotrexate is inappropriate.</p> <p>Adalimumab has not been studied in patients aged less than 2 years.</p> <p>Treatment of active enthesitis-related arthritis in patients, 6 years of age and older, who have had an inadequate response to, or who are intolerant of, conventional therapy.</p> <p>Treatment of non-infectious intermediate, posterior and panuveitis in adult patients who have had an inadequate response to corticosteroids, in patients in need of corticosteroid-sparing, or in whom corticosteroid treatment is inappropriate.</p> <p>Treatment of paediatric chronic non-infectious anterior uveitis in patients from 2 years of age who have had an inadequate response to or are intolerant to conventional therapy, or in whom conventional therapy is inappropriate.</p>	25/07/2018	Sandoz GmbH
HEFIYA	adalimumab	<p>In combination with methotrexate is indicated for the treatment of active polyarticular juvenile idiopathic arthritis, in patients from the age of 2 years who have had an inadequate response to one or more disease-modifying anti-rheumatic drugs (DMARDs). HEFIYA can be given as monotherapy in case of intolerance to methotrexate or when continued treatment with methotrexate is inappropriate.</p> <p>Adalimumab has not been studied in patients aged less than 2 years.</p> <p>Treatment of active enthesitis-related arthritis in patients, 6 years of age and older, who have had an inadequate response to, or who are intolerant of, conventional therapy.</p> <p>Treatment of non-infectious intermediate, posterior and panuveitis in adult patients who have had an inadequate response to corticosteroids, in patients in need of corticosteroid-sparing, or in whom corticosteroid treatment is inappropriate.</p> <p>Treatment of paediatric chronic non-infectious anterior uveitis in patients from 2 years of age who have had an inadequate response to or are intolerant to conventional therapy, or in whom conventional therapy is inappropriate.</p>	25/07/2018	Sandoz GmbH

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HELIXATE NEXGEN	octocog alpha	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency) . This preparation does not contain von Willebrand factor and is therefore not indicated in von Willebrand's disease.	04/08/2000	Bayer AG
HEMLIBRA	emicizumab	Indicated for routine prophylaxis of bleeding episodes in patients with haemophilia A with factor VIII inhibitors. Hemlibra can be used in all age groups.	23/02/2018	Roche Registration GmbH
HERCEPTIN	trastuzumab	In combination with capecitabine or 5-fluorouracil and cisplatin, treatment of patients with HER2-positive metastatic adenocarcinoma of the stomach or gastroesophageal junction who have not received prior anticancer treatment for their metastatic disease. Herceptin should only be used in patients with metastatic gastric cancer whose tumours have HER2 overexpression as defined by IHC2+ and a confirmatory SISH or FISH result, or by an IHC3+ result. Accurate and validated assay methods should be used.	28/08/2000	Roche Registration GmbH
HIZENTRA	human normal immunoglobulin (scig)	Replacement therapy in adults, children and adolescents (0-18 years) in: <ul style="list-style-type: none"> - Primary immunodeficiency syndromes with impaired antibody production. - Hypogammaglobulinaemia and recurrent bacterial infections in patients with chronic lymphocytic leukaemia (CLL), in whom prophylactic antibiotics have failed or are contra-indicated. - Hypogammaglobulinaemia and recurrent infections in multiple myeloma (MM) patients. - Hypogammaglobulinaemia in patients pre- and post-allogeneic haematopoietic stem cell transplantation (HSCT). Immunomodulatory therapy in adults, children and adolescents (0-18 years): - treatment of patients with chronic inflammatory demyelinating polyneuropathy (CIDP) as maintenance therapy after stabilization with IVIg.	14/04/2011	CSL Behring GmbH

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HULIO	adalimumab	<p>In combination with methotrexate is indicated for the treatment of active polyarticular juvenile idiopathic arthritis, in patients from the age of 2 years who have had an inadequate response to one or more disease-modifying anti-rheumatic drugs (DMARDs). HULIO can be given as monotherapy in case of intolerance to methotrexate or when continued treatment with methotrexate is inappropriate.</p> <p>Adalimumab has not been studied in patients aged less than 2 years.</p> <p>Treatment of active enthesitis-related arthritis in patients, 6 years of age and older, who have had an inadequate response to, or who are intolerant of, conventional therapy.</p> <p>Treatment of paediatric chronic non-infectious anterior uveitis in patients from 2 years of age who have had an inadequate response to or are intolerant to conventional therapy, or in whom conventional therapy is inappropriate.</p>	16/09/2018	Mylan S.A.S.
HUMIRA	adalimumab	<p>In combination with methotrexate is indicated for the treatment of active polyarticular juvenile idiopathic arthritis, in children and adolescents aged 2 to 17 years who have had an inadequate response to one or more disease-modifying antirheumatic drugs (DMARDs).</p> <p>As monotherapy in case of intolerance to methotrexate or when continued treatment with methotrexate is inappropriate.</p> <p>It has not been studied in children aged less than 2 years.</p> <p>Treatment of active enthesitis-related arthritis in patients, 6 years of age and older, who have had an inadequate response to, or who are intolerant of, conventional therapy.</p> <p>Treatment of non-infectious intermediate, posterior and panuveitis in adult patients who have had an inadequate response to corticosteroids, in patients in need of corticosteroid-sparing, or in whom corticosteroid treatment is inappropriate.</p> <p>Treatment of paediatric chronic non-infectious anterior uveitis in patients from 2 years of age who have had an inadequate response to or are intolerant to conventional therapy, or in whom conventional therapy is inappropriate.</p>	08/09/2003	AbbVie Deutschland GmbH & Co. KG
HYCAMTIN	topotecan	<p>As monotherapy, treatment of:</p> <ul style="list-style-type: none"> -patients with metastatic carcinoma of the ovary after failure of first-line or subsequent therapy. - patients with relapsed small cell lung cancer (SCLC) for whom retreatment with the first-line regimen is not considered appropriate. 	12/11/1996	Novartis Europharm Ltd

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HYQVIA	human normal immunoglobulin	<p>Replacement therapy in adults (≥ 18 years) in primary immunodeficiency syndromes such as:</p> <ul style="list-style-type: none"> - congenital agammaglobulinaemia and hypogammaglobulinaemia - common variable immunodeficiency - severe combined immunodeficiency - IgG subclass deficiencies with recurrent infections. <p>Replacement therapy in adults (≥ 18 years) in myeloma or chronic lymphocytic leukaemia with severe secondary hypogammaglobulinaemia and recurrent infections.</p>	16/05/2013	Baxalta Innovations GmbH
HYRIMOZ	adalimumab	<p>In combination with methotrexate is indicated for the treatment of active polyarticular juvenile idiopathic arthritis, in patients from the age of 2 years who have had an inadequate response to one or more disease-modifying anti-rheumatic drugs (DMARDs). HYRIMOZ can be given as monotherapy in case of intolerance to methotrexate or when continued treatment with methotrexate is inappropriate.</p> <p>Adalimumab has not been studied in patients aged less than 2 years.</p> <p>Treatment of active enthesitis-related arthritis in patients, 6 years of age and older, who have had an inadequate response to, or who are intolerant of, conventional therapy.</p> <p>Treatment of non-infectious intermediate, posterior and panuveitis in adult patients who have had an inadequate response to corticosteroids, in patients in need of corticosteroid-sparing, or in whom corticosteroid treatment is inappropriate.</p> <p>Treatment of paediatric chronic non-infectious anterior uveitis in patients from 2 years of age who have had an inadequate response to or are intolerant to conventional therapy, or in whom conventional therapy is inappropriate.</p>	25/07/2018	Sandoz GmbH
IBLIAS	octocog alfa	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency) . Iblias can be used for all age groups.	18/02/2016	Bayer Pharma AG

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
ILARIS	canakinumab	<p>Treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) in adults, adolescents and children aged 2 years and older with body weight of 7,5 kg or above, including:</p> <ul style="list-style-type: none"> -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. <p>Treatment of active Still's disease including Adult-Onset Still's Disease (AOSD) and Systemic Juvenile Idiopathic Arthritis (SJIA) in patients aged 2 years and older who have responded inadequately to previous therapy with non-steroidal anti-inflammatory drugs (NSAIDs) and systemic corticosteroids. Ilaris can be given as monotherapy or in combination with methotrexate.</p>	23/10/2009	Novartis Europharm Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
IMATINIB TEVA	imatinib	<p>Treatment of adult and paediatric patients with newly diagnosed Philadelphia chromosome (bcr-abl) positive (Ph+) chronic myeloid leukaemia (CML) for whom bone marrow transplantation is not considered as the first line of treatment.</p> <p>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.</p> <p>Treatment of adult and paediatric patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) integrated with chemotherapy.</p> <p>Treatment of adult patients with relapsed or refractory Ph+ ALL as monotherapy.</p> <p>Treatment of adult patients with myelodysplastic/myeloproliferative diseases (MDS/MPD) associated with platelet-derived growth factor receptor (PDGFR) gene re-arrangements.</p> <p>Treatment of adult patients with advanced hypereosinophilic syndrome (HES) and/or chronic eosinophilic leukaemia (CEL) with FIP1L1-PDGFRα rearrangement.</p> <p>The effect of imatinib on the outcome of bone marrow transplantation has not been determined.</p> <p>Treatment of adult patients with unresectable dermatofibrosarcoma protuberans (DFSP) and adult patients with recurrent and/or metastatic DFSP who are not eligible for surgery.</p>	08/01/2013	Teva B.V.
IMRALDI	adalimumab	<p>In combination with methotrexate indicated for the treatment of active polyarticular juvenile idiopathic arthritis, in patients from the age of 2 years who have had an inadequate response to one or more disease-modifying anti-rheumatic drugs (DMARDs). Imraldi can be given as monotherapy in case of intolerance to methotrexate or when continued treatment with methotrexate is inappropriate. Adalimumab has not been studied in patients aged less than 2 years.</p> <p>Treatment of active enthesitis-related arthritis in patients, 6 years of age and older, who have had an inadequate response to, or who are intolerant of, conventional therapy.</p> <p>Treatment of non-infectious intermediate, posterior and panuveitis in adult patients who have had an inadequate response to corticosteroids, in patients in need of corticosteroid-sparing, or in whom corticosteroid treatment is inappropriate.</p>	24/08/2017	Samsung Bioepis NL B.V.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
INCRELEX	mecasermin	<p>For the long-term treatment of growth failure in children and adolescents from 2 to 18 years with severe primary insulin-like growth factor-1 deficiency (Primary IGFD).</p> <p>Severe Primary IGFD is defined by:</p> <ul style="list-style-type: none"> - height standard deviation score \leq -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. <p>Severe Primary IGFD includes patients with mutations in the GH receptor (GHR), post-GHR signaling 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.</p>	03/08/2007	Ipsen Pharma
INLYTA	axitinib	Treatment of adult patients with advanced renal cell carcinoma (RCC) after failure of prior treatment with sunitinib or a cytokine.	03/09/2012	Pfizer Europe MA EEIG
INOMAX	nitric oxide	<p>In conjunction with ventilatory support and other appropriate active substances:</p> <ul style="list-style-type: none"> - for the treatment of newborn infants \geq 34 weeks gestation with hypoxic respiratory failure associated with clinical or echo cardiographic evidence of pulmonary hypertension, in order to improve oxygenation and to reduce the need for extracorporeal membrane oxygenation. - as part of the treatment of peri- and post-operative pulmonary hypertension in adults and newborn infants, infants and toddlers, children and adolescents, ages 0-17 years in conjunction to heart surgery, in order to selectively decrease pulmonary arterial pressure and improve right ventricular function and oxygenation. 	01/08/2001	Linde Healthcare AB

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
INTRONA	interferon alpha-2b	<p>Treatment of patients with hairy cell leukaemia.</p> <p>As Monotherapy for the treatment of adult patients with Philadelphia chromosome or bcr/abl translocation positive chronic myelogenous leukaemia. Clinical experience indicates that a haematological and cytogenetic major/minor response is obtainable in the majority of patients treated. A major cytogenetic response is defined by < 34 % Ph+ leukaemic cells in the bone marrow, whereas a minor response is > 34 %, but < 90 % Ph+ cells in the marrow.</p> <p>In combination with interferon alfa-2b and cytarabine (Ara-C) during the first 12 months of treatment it has been demonstrated to significantly increase the rate of major cytogenetic responses and to significantly prolong the overall survival at three years when compared to interferon alfa-2b monotherapy.</p> <p>As maintenance therapy in patients with multiple myeloma who have achieved objective remission (more than 50 % reduction in myeloma protein) following initial induction chemotherapy. Current clinical experience indicates that maintenance therapy with interferon alfa-2b prolongs the plateau phase; however, effects on overall survival have not been conclusively demonstrated.</p> <p>Treatment of high tumour burden follicular lymphoma as adjunct to appropriate combination induction chemotherapy such as a CHOP-like regimen. High tumour burden is defined as having at least one of the following: bulky tumour mass (> 7 cm), involvement of three or more nodal sites (each > 3 cm), systemic symptoms (weight loss > 10 %, pyrexia > 38°C for more than 8 days, or nocturnal sweats), splenomegaly beyond the umbilicus, major organ obstruction or compression syndrome, orbital or epidural involvement, serous effusion, or leukaemia.</p> <p>Treatment of carcinoid tumours with lymph node or liver metastases and with "carcinoid syndrome".</p>	09/03/2000	Merck Sharp & Dohme B.V.
IXIARO	japanese encephalitis vaccine (inactivated, adsorbed)	<p>Active immunisation against Japanese encephalitis in adults, adolescents, children and infants aged 2 months and older.</p> <p>IXIARO should be considered for use in individuals at risk of exposure through travel or in the course of their occupation</p>	31/03/2009	Valneva Austria GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
JAKAVI	ruxolitinib	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 . Treatment of adult patients with polycythaemia vera who are resistant to or intolerant of hydroxyurea.	23/08/2012	Novartis Europharm Ltd
JINARC	tolvaptan	Indicated to slow the progression of cyst development and renal insufficiency of autosomal dominant polycystic kidney disease (ADPKD) in adults with CKD stage 1 to 4 at initiation of treatment with evidence of rapidly progressing disease.	27/05/2015	Otsuka Pharmaceutical Europe Ltd
JIVI	damoctocog alfa pegol	Treatment and prophylaxis of bleeding in previously treated patients ≥ 12 years of age with haemophilia A (congenital factor VIII deficiency) .	22/11/2018	Bayer AG
KEPPRA	levetiracetam	As monotherapy in the treatment of partial onset seizures with or without secondary generalisation in patients from 16 years of age with newly diagnosed epilepsy . As adjunctive therapy in the treatment of partial onset seizures with or without secondary generalisation in adults, children and infants from 1 month of age with epilepsy; in the treatment of myoclonic seizures in adults and adolescents from 12 years of age with Juvenile Myoclonic Epilepsy . Treatment of primary generalised tonic-clonic seizures in adults and adolescents from 12 years of age with Idiopathic Generalised Epilepsy .	29/09/2000	UCB Pharma SA
KEYTRUDA	pembrolizumab	As monotherapy for the treatment of adult patients with relapsed or refractory classical Hodgkin lymphoma (cHL) who have failed autologous stem cell transplant (ASCT) and brentuximab vedotin (BV), or who are transplant-ineligible and have failed BV.	17/07/2015	Merck Sharp & Dohme B.V.
KIGABEQ	vigabatrin	In infants and children from 1 month to less than 7 years of age for: -Treatment in monotherapy of infantile spasms (West's syndrome) .	19/09/2018	ORPHELIA Pharma SAS

NEW

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
KINERET	anakinra	<p>Treatment in adults, adolescents, children and infants aged 8 months and older with a body weight of 10 kg or above of Cryopyrin-Associated Periodic Syndromes (CAPS), including:- Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA),- Muckle-Wells Syndrome (MWS),- Familial Cold Autoinflammatory Syndrome (FCAS).</p> <p>In adults, adolescents, children and infants aged 8 months and older with a body weight of 10 kg or above for the treatment of Still's disease, including Systemic Juvenile Idiopathic Arthritis (SJIA) and Adult-Onset Still's Disease (AOSD), with active systemic features of moderate to high disease activity, or in patients with continued disease activity after treatment with non-steroidal anti-inflammatory drugs (NSAIDs) or glucocorticoids.</p> <p>Kineret can be given as monotherapy or in combination with other anti-inflammatory drugs and disease-modifying antirheumatic drugs (DMARDs).</p>	08/03/2002	Swedish Orphan Biovitrum AB (publ)
KIOVIG	human normal immunoglobulin	<p>Replacement therapy in adults, and children and adolescents (0-18 years) in:</p> <ul style="list-style-type: none"> - Primary immunodeficiency syndromes with impaired antibody production, - Hypogammaglobulinaemia and recurrent bacterial infections in patients with chronic lymphocytic leukaemia, in whom prophylactic antibiotics have failed - Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase multiple myeloma patients who have failed to respond to pneumococcal immunisation, - Hypogammaglobulinaemia in patients after allogeneic haematopoietic stem cell transplantation (HSCT). - Congenital AIDS and recurrent bacterial infections. <p>Immunomodulation in adults, and children and adolescents (0-18 years) in:</p> <ul style="list-style-type: none"> - Primary immune thrombocytopenia (ITP), in patients at high risk of bleeding or prior to surgery to correct the platelet count - Guillain Barré syndrome - Kawasaki disease - Multifocal Motor Neuropathy (MMN). 	19/01/2006	Baxter AG

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
KISPLYX	lenvatinib	in combination with everolimus for the treatment of adult patients with advanced renal cell carcinoma (RCC) following one prior vascular endothelial growth factor (VEGF)-targeted therapy.	25/08/2016	Eisai Europe Ltd
KOGENATE BAYER	octocog alpha	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency) . This preparation does not contain von Willebrand factor and is therefore not indicated in von Willebrand's disease. This product is indicated for adults, adolescents and children of all ages.	04/08/2000	Bayer AG
KOVALTRY	octocog alfa	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency) . Kovaltry can be used for all age groups.	18/02/2016	Bayer AG
LENALIDOMIDE ACCORD	lenalidomide	As monotherapy for the maintenance treatment of adult patients with newly diagnosed multiple myeloma who have undergone autologous stem cell transplantation. As combination therapy for the treatment of adult patients with previously untreated multiple myeloma who are not eligible for transplant. In combination with dexamethasone for the treatment of multiple myeloma in adult patients who have received at least one prior therapy.	19/09/2018	Accord Healthcare Limited
LENVIMA	lenvatinib	As monotherapy 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). As monotherapy for the treatment of adult patients with advanced or unresectable hepatocellular carcinoma (HCC) who have received no prior systemic therapy.	28/05/2015	Eisai Europe Limited
LITAK	cladribine	Treatment of hairy cell leukaemia .	14/04/2004	Lipomed GmbH
LOJUXTA	lomitapide	Adjunct to a low-fat diet and other lipid-lowering medicinal products with or without low density lipoprotein (LDL) apheresis in adult patients with homozygous familial hypercholesterolaemia (HoFH) . Genetic confirmation of HoFH should be obtained whenever possible. Other forms of primary hyperlipoproteinemia and secondary causes of hypercholesterolaemia (e.g., nephrotic syndrome, hypothyroidism) must be excluded.	31/07/2013	Aegerion Pharmaceuticals SAS
LYNPARZA	olaparib	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.	16/12/2014	AstraZeneca AB

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
LYSODREN	mitotane	Symptomatic treatment of advanced (unresectable, metastatic or relapsed) adrenal cortical carcinoma . The effect of Lysodren on non functional adrenal cortical carcinoma is not established.	28/04/2004	Laboratoire HRA Pharma
MABTHERA	rituximab	Non-Hodgkin's lymphoma (NHL) - Treatment of previously untreated patients with stage III-IV follicular lymphoma in combination with chemotherapy. - As maintenance therapy, the treatment of follicular lymphoma patients responding to induction therapy. - In monotherapy, treatment of patients with stage III-IV follicular lymphoma who are chemoresistant or are in their second or subsequent relapse after chemotherapy. - Treatment of patients with CD20 positive diffuse large B cell non- Hodgkin's lymphoma in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy. In combination with chemotherapy, treatment of patients with previously untreated and relapsed/ refractory chronic lymphocytic leukaemia . Only limited data are available on efficacy and safety for patients previously treated with monoclonal antibodies including MabThera or patients refractory to previous MabThera plus chemotherapy. Granulomatosis with polyangiitis and Microscopic polyangiitis in combination with glucocorticoids, it is indicated for the induction of remission in adult patients with severe, active Granulomatosis with polyangiitis (Wegener's) (GPA) and Microscopic polyangiitis (MPA).	02/06/1998	Roche Registration GmbH
MIGLUSTAT GEN ORPH	miglustat	Oral treatment of adult patients with mild to moderate type 1 Gaucher disease . Miglustat Gen.Orph may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable.	10/11/2017	Gen.Orph

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
MVASI	bevacizumab	<p>In combination with interferon alfa-2a indicated for first-line treatment of adult patients with advanced and/or metastatic renal cell cancer.</p> <p>In combination with carboplatin and paclitaxel indicated for the front-line treatment of adult patients with advanced (International Federation of Gynecology and Obstetrics (FIGO) stages IIIB, IIIC and IV) epithelial ovarian, fallopian tube, or primary peritoneal cancer.</p> <p>In combination with carboplatin and gemcitabine or in combination with carboplatin and paclitaxel, indicated for treatment of adult patients with first recurrence of platinum-sensitive epithelial ovarian, fallopian tube or primary peritoneal cancer who have not received prior therapy with bevacizumab or other VEGF inhibitors or VEGF receptor-targeted agents.</p> <p>In combination with paclitaxel, topotecan, or pegylated liposomal doxorubicin indicated for the treatment of adult patients with platinum-resistant recurrent epithelial ovarian, fallopian tube, or primary peritoneal cancer who received no more than two prior chemotherapy regimens and who have not received prior therapy with bevacizumab or other VEGF inhibitors or VEGF receptor-targeted agents.</p>	15/01/2018	Amgen Europe B.V.
MYOZYME	alglucosidase alpha	<p>Long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of Pompe disease (acid α-glucosidase deficiency).</p> <p>Myozyme is indicated in adults and paediatric patients of all ages</p>	29/03/2006	Genzyme Europe B.V.
MYSILDECARD	sildenafil	<p>Treatment of adult patients with pulmonary arterial hypertension classified as 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.</p> <p>Treatment of paediatric patients aged 1 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.</p>	15/09/2016	MYLAN S.A.S.
NAGLAZYME	galsulfase	<p>Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis VI (MPS VI; N-acetylgalactosamine 4-sulfatase deficiency; Maroteaux-Lamy syndrome)</p> <p>A key issue is to treat children aged <5 years suffering from a severe form of the disease, even though children <5 years were not included in the pivotal phase 3 study. Limited data are available in patients < 1 year of age.</p>	24/01/2006	BioMarin International Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
NEOFORDEX	dexamethasone	Indicated in adults for the treatment of symptomatic multiple myeloma in combination with other medicinal products.	16/03/2016	Laboratoires CTRS
NEXAVAR	sorafenib tosylate	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.	19/07/2006	Bayer AG
NITISINONE MDK (previously NITISINONE MENDELIKABS)	nitisinone	Treatment of adult and paediatric (in any age range) patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT 1) in combination with dietary restriction of tyrosine and phenylalanine.	24/08/2017	MendeliKABS Europe Ltd
NITYR	nitisinone	Treatment of adult and paediatric patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine.	26/07/2018	Cycle Pharmaceuticals Ltd
NIVESTIM	filgrastim	In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/l$, and a history of severe or recurrent infections.	08/06/2010	Hospira UK Ltd
NONAFACT	human coagulation factor IX	Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency) .	03/07/2001	Sanquin Plasma Products B.V.
NORDIMET	methotrexate	Treatment of polyarthritic forms of severe, active juvenile idiopathic arthritis (JIA) , when the response to nonsteroidal anti-inflammatory drugs (NSAIDs) has been inadequate.	18/08/2016	Nordic Group B.V.
NOVOEIGHT	turoctocog alpha	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency) . NovoEight can be used for all age groups.	13/11/2013	Novo Nordisk A/S
NOVOSEVEN	eptacog alpha (activated)	Treatment of bleeding episodes and for the prevention of bleeding in those undergoing surgery or invasive procedures in the following patient groups : -patients with congenital haemophilia with inhibitors to coagulation factors VIII or IX > 5 BU -patients with congenital haemophilia who are expected to have a high anamnestic response to factor VIII or factor IX administration -patients with acquired haemophilia -patients with congenital FVII deficiency ; -patients with Glanzmann's thrombasthenia with past or present refractoriness to platelet transfusions, or where platelets are not readily available.	23/02/1996	Novo Nordisk A/S
NOVOTHIRTEEN	catridecacog	Long term prophylactic treatment of bleeding in in adult and paediatric patients with congenital factor XIII A-subunit deficiency	03/09/2012	Novo Nordisk A/S

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
NOXAFIL	posaconazole	<p>Treatment of the fungal infections in adults:</p> <ul style="list-style-type: none"> - Invasive aspergillosis in patients with disease that is refractory to amphotericin B or itraconazole or in patients who are intolerant of these medicinal products - Fusariosis in patients with disease that is refractory to amphotericin B or in patients who are intolerant of amphotericin B. - Chromoblastomycosis and mycetoma in patients with disease that is refractory to itraconazole or in patients who are intolerant of itraconazole - Coccidioidomycosis in patients with disease that is refractory to amphotericin B, itraconazole or fluconazole or in patients who are intolerant of these medicinal products. <p>Refractoriness is defined as progression of infection or failure to improve after a minimum of 7 days of prior therapeutic doses of effective antifungal therapy.</p> <p>Prophylaxis of invasive fungal infections in :</p> <ul style="list-style-type: none"> - Patients receiving remission-induction chemotherapy for acute myelogenous leukemia (AML) or myelodysplastic syndromes (MDS) expected to result in prolonged neutropenia and who are at high risk of developing invasive fungal infections - Hematopoietic stem cell transplant (HSCT) recipients who are undergoing high-dose immunosuppressive therapy for graft versus host disease and who are at high risk of developing invasive fungal infections. 	25/10/2005	Merck Sharp & Dohme B.V.
NUWIQ	simoctocog alfa	<p>Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency).</p> <p>Nuwiq can be used for all age groups.</p>	21/07/2014	Octapharma AB
OBIZUR	susoctocog alfa	<p>Treatment of bleeding episodes in patients with acquired haemophilia caused by antibodies to Factor VIII.</p>	11/11/2015	Baxalta Innovations GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
OMNITROPE	somatropin	<p>Infants, children and adolescents:</p> <ul style="list-style-type: none"> - Growth disturbance due to insufficient secretion of growth hormone (growth hormone deficiency, GHD). - Growth disturbance associated with Turner syndrome. - Growth disturbance associated with chronic renal insufficiency. - Growth disturbance (current height standard deviation score (SDS) < -2.5 and parental adjusted height SDS < -1) in short children/adolescents born small for gestational age (SGA), with a birth weight and/or length below -2 standard deviation (SD), who failed to show catch-up growth (height velocity (HV) SDS < 0 during the last year) by 4 years of age or later. - Prader-Willi syndrome (PWS), for improvement of growth and body composition. The diagnosis of PWS should be confirmed by appropriate genetic testing. <p>Adults</p> <ul style="list-style-type: none"> - Replacement therapy in adults with pronounced growth hormone deficiency. - <i>Adult onset</i>: Patients who have severe growth hormone deficiency associated with multiple hormone deficiencies as a result of known hypothalamic or pituitary pathology, and who have at least one known deficiency of a pituitary hormone not being prolactin. These patients should undergo an appropriate dynamic test in order to diagnose or exclude a growth hormone deficiency. - <i>Childhood onset</i>: Patients who were growth hormone deficient during childhood as a result of congenital, genetic, acquired, or idiopathic causes. Patients with childhood onset GHD should be re-evaluated for growth hormone secretory capacity after completion of longitudinal growth. In patients with a high likelihood for persistent GHD, i.e. a congenital cause or GHD secondary to a hypothalamic-pituitary disease or insult, an insulin-like growth factor-I (IGF-I) SDS < -2 off growth hormone treatment for at least 4 weeks should be considered sufficient evidence of profound GHD. <p>All other patients will require IGF-I assay and one growth hormone stimulation test.</p>	12/04/2006	Sandoz GmbH
ONCASPAR	pegaspargase	<p>Indicated as a component of antineoplastic combination therapy in acute lymphoblastic leukaemia (ALL) in paediatric patients from birth to 18 years, and adult patients.</p>	14/01/2016	Baxalta Innovations GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
OPDIVO	nivolumab	As monotherapy indicated for the treatment of advanced renal cell carcinoma after prior therapy in adults. As monotherapy for the treatment of adult patients with relapsed or refractory classical Hodgkin lymphoma after autologous stem cell transplant (ASCT) and treatment with brentuximab vedotin. As monotherapy for the treatment of squamous cell cancer of the head and neck in adults progressing on or after platinum-based therapy.	19/06/2015	Bristol-Myers Squibb Pharma EEIG
ORENCIA	abatacept	In combination with methotrexate, for the treatment of moderate to severe active polyarticular juvenile idiopathic arthritis (JIA) in paediatric patients 6 years of age and older who have had an insufficient response to other DMARDs including at least one TNF inhibitor.	21/05/2007	Bristol-Myers SquibbPharma EEIG
ORFADIN	nitisinone	Treatment of adult and paediatric (in any age range) patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine.	21/02/2005	Swedish Orphan Biovitrum AB
ORKAMBI	lumacaftor / ivacaftor	Treatment of cystic fibrosis (CF) in patients aged 6 years and older who are homozygous for the F508del mutation in the CFTR gene	19/11/2015	Vertex Pharmaceuticals (Europe) Ltd
OVALEAP	follitropin alpha	Indicated for the stimulation of spermatogenesis in adult men who have congenital or acquired hypogonadotropic hypogonadism with concomitant human chorionic gonadotropin (hCG) therapy.	27/09/2013	Teva B.V.
OZURDEX	dexamethasone	For the treatment of adult patients with inflammation of the posterior segment of the eye presenting as non-infectious uveitis .	27/07/2010	Allergan Pharmaceuticals Ireland
PANRETIN	alitretinoin	Topical treatment of cutaneous lesions in patients with AIDS-related Kaposi's sarcoma (KS) : - when lesions are not ulcerated or lymphoedematous, and -treatment of visceral KS is not required, and -when lesions are not responding to systemic antiretroviral therapy, and -radiotherapy or chemotherapy are not appropriate.	11/10/2000	Eisai Ltd
PEDEA	ibuprofen	Treatment of a haemodynamically significant patent ductus arteriosus in preterm newborn infants less than 34 weeks of gestational age.	29/07/2004	Orphan Europe S.A.R.L
PEMETREXED ACCORD	pemetrexed disodium hemipentahydrate	In combination with cisplatin is indicated for the treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma .	18/01/2016	Accord Healthcare Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
PEMETREXED FRESENIUS KABI	pemetrexed diacid	In combination with cisplatin is indicated for the treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma .	22/07/2016	Fresenius Kabi Deutschland GmbH
PEMETREXED HOSPIRA	pemetrexed disodium hemipentahydrate	In combination with cisplatin indicated for the treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma .	20/11/2015	Hospira UK Ltd
PEMETREXED Krka	pemetrexed disodium	In combination with cisplatin for the treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma .	22/05/2018	KRKA d.d.
PEMETREXED LILLY	pemetrexed disodium	In combination with cisplatin for the treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma .	14/09/2015	Eli Lilly Netherlands
PEMETREXED MEDAC	pemetrexed disodium hemipentahydrate	In combination with cisplatin indicated for the treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma .	27/11/2015	medac Gesellschaft für klinische Spezialpräparate mbH
PEMETREXED SANDOZ	pemetrexed disodium hemipentahydrate	In combination with cisplatin for the treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma .	18/09/2015	Sandoz GmbH
PIXUVRI	pixantrone dimaleate	As monotherapy for the treatment of adult patients with multiply relapsed or refractory aggressive Non-Hodgkin B-cell Lymphomas (NHL) . The benefit of pixantrone treatment has not been established in patients when used as fifth line or greater chemotherapy in patients who are refractory to last therapy.	10/05/2012	CTI life Sciences Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
PRIVIGEN	human normal immunoglobulin (IVIg)	<p>Replacement therapy in adults, and children and adolescents (0-18 years) in:</p> <ul style="list-style-type: none"> - Primary immunodeficiency (PID) syndromes with impaired antibody production - Hypogammaglobulinaemia and recurrent bacterial infections in patients with chronic lymphocytic leukaemia, in whom prophylactic antibiotics have failed. - Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase multiple myeloma patients who have failed to respond to pneumococcal immunisation. - Hypogammaglobulinaemia in patients after allogeneic haematopoietic stem cell transplantation (HSCT). - Congenital AIDS with recurrent bacterial infections. <p>Immunomodulation in adults, and children and adolescents (0-18 years) in:</p> <ul style="list-style-type: none"> - Primary immune thrombocytopenia (ITP), in patients at high risk of bleeding or prior to surgery to correct the platelet count. - Guillain-Barré syndrome. - Kawasaki disease. - Chronic inflammatory demyelinating polyneuropathy (CIDP). <p>Only limited experience is available of use of intravenous immunoglobulins in children with CIDP.</p>	25/04/2008	CSL Behring GmbH
PUREGON	follitropin beta	Indicated in adult males with deficient spermatogenesis due to hypogonadotrophic hypogonadism .	03/05/1996	Merck Sharp & Dohme B.V.
QUINSAIR	levofloxacin	Management of chronic pulmonary infections due to <i>Pseudomonas aeruginosa</i> in adult patients with cystic fibrosis	26/03/2015	Chiesi Farmaceutici S.p.A.
RAPAMUNE	sirolimus	Treatment of patients with sporadic lymphangiomyomatosis with moderate lung disease or declining lung function. (Indication extension)	12/03/2001	Pfizer Europe MA EEIG
RATIOGRASTIM	filgrastim	In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/l$, and a history of severe or recurrent infections.	15/09/2008	Ratiopharm GmbH
REFACTO AF	moroctocog alpha	<p>Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency) in adults and children of all ages, including newborns.</p> <p>ReFacto AF is appropriate for use in adults and children of all ages, including newborns.</p> <p>ReFacto AF does not contain von Willebrand factor, and hence is not indicated in von Willebrand's disease.</p>	13/04/1999	Pfizer Europe MA EEIG

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
REFIXIA	nonacog beta pegol	Treatment and prophylaxis of bleeding in patients 12 years and above with haemophilia B (congenital factor IX deficiency).	02/06/2017	Novo Nordisk A/S
REPATHA	evolocumab	Indicated in adults and adolescents aged 12 years and over with homozygous familial hypercholesterolaemia in combination with other lipid-lowering therapies. The effect of Repatha on cardiovascular morbidity and mortality has not yet been determined.	17/07/2015	Amgen Europe B.V.
REPLAGAL	agalsidase alfa	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency)	03/08/2001	Shire Human Genetic Therapies AB
RESPREEZA	human alpha1-proteinase inhibitor	For maintenance treatment, to slow the progression of emphysema in adults with documented severe alpha1-proteinase inhibitor deficiency (e.g.genotypes PiZZ, PiZ(null), Pi(null,null), PiSZ). Patients are to be under optimal pharmacologic and non-pharmacologic treatment and show evidence of progressive lung disease (e.g.lower forced expiratory volume per second (FEV1) predicted, impaired walking capacity or increased number of exacerbations) as evaluated by a healthcare professional experienced in the treatment of alpha1-proteinase inhibitor deficiency.	20/08/2015	CSL Behring GmbH
REVATIO	sildenafil citrate	Treatment of adult patients with pulmonary arterial hypertension classified as 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. Treatment of paediatric patients aged 1 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.	28/10/2005	Pfizer Europe MA EEIG
REVLIMID	lenalidomide	As monotherapy for the maintenance treatment of adult patients with newly diagnosed multiple myeloma who have undergone autologous stem cell transplantation. As combination therapy for the treatment of adult patients with previously untreated multiple myeloma who are not eligible for transplant. Treatment in combination with dexamethasone of multiple myeloma in adult patients who have received at least one prior therapy.	14/06/2007	Celgene Europe B.V.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
REVOLADE	eltrombopag	<p>Indicated for chronic immune (idiopathic) thrombocytopenic purpura (ITP) patients aged 1 year and above who are refractory to other treatments.</p> <p>Indicated in adult patients with acquired severe aplastic anaemia (SAA) who were either refractory to prior immunosuppressive therapy or heavily pretreated and are unsuitable for haematopoietic stem cell transplantation.</p>	11/03/2010	Novartis Europharm Ltd
RILUTEK	riluzole	<p>To extend life or the time to mechanical ventilation for patients with amyotrophic lateral sclerosis (ALS).</p> <p>Clinical trials have demonstrated that RILUTEK extends survival for patients with ALS. Survival was defined as patients who were alive, not intubated for mechanical ventilation and tracheotomy-free. There is no evidence that RILUTEK exerts a therapeutic effect on motor function, lung function, fasciculations, muscle strength and motor symptoms. RILUTEK has not been shown to be effective in the late stages of ALS.</p> <p>Safety and efficacy of RILUTEK has only been studied in ALS. Therefore, RILUTEK should not be used in patients with any other form of motor neurone disease.</p>	10/06/1996	Aventis Pharma S.A.
RITEMVIA	rituximab	<p>Treatment of previously untreated patients with stage III, IV follicular lymphoma in combination with chemotherapy.</p> <p>As maintenance therapy for the treatment of follicular lymphoma patients responding to induction therapy.</p> <p>As monotherapy for the treatment of patients with stage III, IV follicular lymphoma who are chemo resistant or are in their second or subsequent relapse after chemotherapy.</p> <p>Treatment of patients with CD20 positive diffuse large B cell non Hodgkin's lymphoma in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy.</p> <p>In combination with glucocorticoids, is indicated for the induction of remission in adult patients with severe, active granulomatosis with polyangiitis (Wegener's) (GPA) and microscopic polyangiitis (MPA).</p>	13/07/2017	Celltrion Healthcare Hungary Kft

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
RITUZENA (previously TUXELLA)	rituximab	<p>Treatment of previously untreated patients with stage III IV follicular lymphoma in combination with chemotherapy.</p> <p>As monotherapy indicated for treatment of patients with stage III IV follicular lymphoma who are chemo resistant or are in their second or subsequent relapse after chemotherapy.</p> <p>Treatment of patients with CD20 positive diffuse large B cell non Hodgkin's lymphoma in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy.</p> <p>In combination with chemotherapy for the treatment of patients with previously untreated and relapsed/refractory Chronic lymphocytic leukaemia (CLL). Only limited data are available on efficacy and safety for patients previously treated with monoclonal antibodies including Rituzena or patients refractory to previous Rituzena plus chemotherapy.</p>	13/07/2017	Celltrion Healthcare Hungary Kft
RIXATHON	rituximab	<p>Treatment of previously untreated patients with stage III-IV follicular lymphoma in combination with chemotherapy.</p> <p>As maintenance therapy for the treatment of follicular lymphoma patients responding to induction therapy.</p> <p>As monotherapy for the treatment of patients with stage III-IV follicular lymphoma who are chemoresistant or are in their second or subsequent relapse after chemotherapy.</p> <p>For the treatment of patients with CD20 positive diffuse large B cell non Hodgkin's lymphoma in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy.</p> <p>In combination with chemotherapy is indicated for the treatment of patients with previously untreated and relapsed/refractory chronic lymphocytic leukaemia. Only limited data are available on efficacy and safety for patients previously treated with monoclonal antibodies including rituximab or patients refractory to previous rituximab plus chemotherapy.</p> <p>In combination with glucocorticoids, is indicated for the induction of remission in adult patients with severe, active granulomatosis with polyangiitis (Wegener's) (GPA) and microscopic polyangiitis (MPA).</p>	15/06/2017	Sandoz GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
RIXIMYO	rituximab	<p>Treatment of previously untreated patients with stage III-IV follicular lymphoma in combination with chemotherapy.</p> <p>As maintenance therapy for the treatment of follicular lymphoma patients responding to induction therapy.</p> <p>As monotherapy for the treatment of patients with stage III-IV follicular lymphoma who are chemoresistant or are in their second or subsequent relapse after chemotherapy.</p> <p>For the treatment of patients with CD20 positive diffuse large B cell non Hodgkin's lymphoma in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy.</p> <p>In combination with glucocorticoids, is indicated for the induction of remission in adult patients with severe, active granulomatosis with polyangiitis (Wegener's) (GPA) and microscopic polyangiitis (MPA).</p>	15/06/2017	Sandoz GmbH
RIXUBIS	nonacog gamma	<p>Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency).</p> <p>RIXUBIS is indicated in patients of all age groups.</p>	19/12/2014	Baxalta Innovations GmbH
ROACTEMRA	tocilizumab	<p>RoActemra 20 mg/ml concentrate for solution for infusion:</p> <p>Treatment of active systemic juvenile idiopathic arthritis (sJIA) in patients 2 years of age and older, who have responded inadequately to previous therapy with NSAIDs and systemic corticosteroids. RoActemra can be given as monotherapy (in case of intolerance to MTX or where treatment with MTX is inappropriate) or in combination with MTX.</p> <p>In combination with methotrexate (MTX) it is indicated for the treatment of juvenile idiopathic polyarthritis (pJIA; rheumatoid factor positive or negative and extended oligoarthritis) in patients 2 years of age and older, who have responded inadequately to previous therapy with MTX.</p> <p>RoActemra 162 mg solution for injection in pre-filled syringe :</p> <p>Treatment of Giant Cell Arteritis (GCA) in adult patients.</p>	16/01/2009	Roche Registration GmbH
RUBRACA	rucaparib	<p>As monotherapy for the treatment of adult patients with platinum sensitive, relapsed or progressive, BRCA mutated (germline and/or somatic), high-grade epithelial ovarian, fallopian tube, or primary peritoneal cancer, who have been treated with two or more prior lines of platinum based chemotherapy, and who are unable to tolerate further platinum based chemotherapy.</p>	24/05/2018	Clovis Oncology Ireland Limited

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
RUCONEST	conestat alfa	Treatment of acute angioedema attacks in adults and adolescents with hereditary angioedema (HAE) due to C1 esterase inhibitor deficiency .	28/10/2010	Pharming Group N.V.
SAVENE	dexrazoxane	Treatment of anthracycline extravasation in adults.	28/07/2006	Clinigen Healthcare Ltd
SIKLOS	hydroxycarbamide	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 .	29/06/2007	Addmedica
SIMPONI	golimumab	In combination with methotrexate (MTX) for the treatment of polyarticular juvenile idiopathic arthritis in children with a body weight of at least 40 kg, who have responded inadequately to previous therapy with MTX	01/10/2009	Janssen Biologics B.V.
SLENYTO	melatonin	Treatment of insomnia in children and adolescents aged 2-18 with Autism Spectrum Disorder (ASD) and / or Smith-Magenis syndrome , where sleep hygiene measures have been insufficient.	19/09/2018	RAD Neurim Pharmaceuticals EEC Ltd.
SOMAVERT	pegvisomant	Treatment of adult 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.	13/11/2002	Pfizer Europe MA EEIG
SPECTRILA	asparaginase	Indicated as a component of antineoplastic combination therapy for the treatment of acute lymphoblastic leukaemia (ALL) in paediatric patients from birth to 18 years and adults.	14/01/2016	Medac Gesellschaft fuer klinische Spezialpraeparate mbH
SPRYCEL	dasatinib	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. Treatment of paediatric patients with: newly diagnosed Ph+ CML in chronic phase (Ph+ CML-CP) or Ph+ CML-CP resistant or intolerant to prior therapy including imatinib.	20/11/2006	Bristol-Myers SquibbPharma EEIG

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
STAYVEER	bosentan monohydrate	<p>Treatment of pulmonary arterial hypertension (PAH) to improve exercise capacity and symptoms in patients with WHO functional class III. Efficacy has been shown in:</p> <ul style="list-style-type: none"> • Primary (idiopathic and heritable) pulmonary arterial hypertension • Pulmonary arterial hypertension secondary to scleroderma without significant interstitial pulmonary disease • Pulmonary arterial hypertension associated with congenital systemic-to-pulmonary shunts and Eisenmenger's physiology. <p>Some improvements have also been shown in patients with pulmonary arterial hypertension WHO functional class II.</p> <p>Indicated to reduce the number of new digital ulcers in patients with systemic sclerosis and ongoing digital ulcer disease</p>	24/06/2013	Marklas Nederland BV
SUTENT	sunitinib	<p>Treatment of unresectable and/or metastatic malignant gastrointestinal stromal tumour (GIST) in adults after failure of imatinib treatment due to resistance or intolerance.</p> <p>Treatment of advanced/metastatic renal cell carcinoma (MRCC) in adults.</p> <p>Treatment of unresectable or metastatic, well-differentiated pancreatic neuroendocrine tumours (pNET) with disease progression in adults.</p> <p>Experience with SUTENT as first-line treatment is limited</p>	19/07/2006	Pfizer Europe MA EEIG
TADALAFIL GENERICS	tadalafil	<p>Indicated in adults for the treatment of pulmonary arterial hypertension (PAH) classified as WHO functional class II and III, to improve exercise capacity.</p> <p>Efficacy has been shown in idiopathic PAH (IPAH) and in PAH related to collagen vascular disease.</p>	09/01/2017	MYLAN S.A.S
TARCEVA	erlotinib	<p>In combination with gemcitabine, for the treatment of patients with metastatic pancreatic cancer. When prescribing Tarceva, factors associated with prolonged survival should be taken into account. No survival advantage could be shown for patients with locally advanced disease.</p>	19/09/2005	Roche Registration GmbH
TARGRETIN	bexarotene	<p>Treatment of skin manifestations of advanced stage cutaneous T-cell lymphoma (CTCL) patients refractory to at least one systemic treatment.</p>	29/03/2001	Eisai GmbH
TAXESPIRA (previously DOCETAXEL HOSPIRA UK LIMITED)	docetaxel	<p>In combination with cisplatin and 5-fluorouracil for the induction treatment of patients with locally advanced squamous cell carcinoma of the head and neck.</p>	28/08/2015	Hospira UK Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
TAXOTERE	docetaxel	In combination with cisplatin and 5-fluorouracil for the induction treatment of patients with locally advanced squamous cell carcinoma of the head and neck .	27/11/1995	Aventis Pharma S.A.
TEMODAL	temozolomide	Treatment of adult patients with newly-diagnosed glioblastoma multiforme concomitantly with radiotherapy (RT) and subsequently as monotherapy treatment. Treatment of children from the age of three years, adolescents and adult patients with malignant glioma , such as glioblastoma multiforme or anaplastic astrocytoma , showing recurrence or progression after standard therapy.	26/01/1999	Merck Sharp & Dohme B.V.
TEVAGRASTIM	filgrastim	In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/l$, and a history of severe or recurrent infections.	15/09/2008	Teva GmbH
TEYSUNO	tegafur/gimeracil/oteracil	In adults for the treatment of advanced gastric cancer when given in combination with cisplatin.	14/03/2011	Nordic Group BV
THALIDOMIDE CELGENE (previously THALIDOMIDE PHARMION)	thalidomide	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 Thalidomide Celgene Pregnancy Prevention Programme	16/04/2008	Celgene Europe B.V.
THYROGEN	thyrotropin alfa	For use with serum thyroglobulin (Tg) testing with or without radioiodine imaging for the detection of thyroid remnants and well-differentiated thyroid cancer in post-thyroidectomy patients maintained on hormone suppression therapy (THST). Low-risk patients with well-differentiated thyroid carcinoma who have undetectable serum Tg levels on THST and no rh (recombinant human) TSH-stimulated increase of Tg levels may be followed-up by assaying rh TSH-stimulated Tg levels. For pre-therapeutic stimulation in combination with a range of 30 mCi (1.1 GBq) to 100 mCi (3.7 GBq) radioiodine for ablation of thyroid tissue remnants in patients who have undergone a near-total or total thyroidectomy for well-differentiated thyroid cancer and who do not have evidence of distant metastatic thyroid cancer.	09/03/2000	Genzyme Europe B.V.
TORISEL	temsirolimus	First-line treatment of adult patients with advanced renal cell carcinoma (RCC) who have at least three of six prognostic risk factors.	19/11/2007	Pfizer Europe MA EEIG

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
TRACLEER	bosentan monohydrate	<p>Treatment of pulmonary arterial hypertension (PAH) to improve exercise capacity and symptoms in patients with WHO functional class III. Efficacy has been shown in :</p> <ul style="list-style-type: none"> - primary (idiopathic and heritable) 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. <p>To reduce the number of new digital ulcers in patients with systemic sclerosis and ongoing digital ulcer disease.</p>	15/05/2002	Janssen-Cilag International NV
TRISENOX	arsenic trioxide	<p>Indicated for induction of remission, and consolidation in adult patients with:</p> <ul style="list-style-type: none"> • Newly diagnosed low-to-intermediate risk acute promyelocytic leukaemia (APL) (white blood cell count, $\leq 10 \times 10^3/\mu\text{l}$) in combination with all-trans-retinoic acid (ATRA) • Relapsed/refractory acute promyelocytic leukaemia (APL)(Previous treatment should have included a retinoid and chemotherapy) 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. <p>The response rate of other acute myelogenous leukaemia subtypes to arsenic trioxide has not been examined.</p>	05/03/2002	Teva B.V.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
TRUXIMA	rituximab	<p>Treatment of previously untreated patients with stage III-IV follicular lymphoma in combination with chemotherapy.</p> <p>Truxima maintenance therapy is indicated for the treatment of follicular lymphoma patients responding to induction therapy.</p> <p>Truxima monotherapy is indicated for treatment of patients with stage III-IV follicular lymphoma who are chemo-resistant or are in their second or subsequent relapse after chemotherapy.</p> <p>Treatment of patients with CD20 positive diffuse large B cell non-Hodgkin's lymphoma in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy.</p> <p>In combination with chemotherapy is indicated for the treatment of patients with previously untreated and relapsed/refractory Chronic lymphocytic leukaemia (CLL) . Only limited data are available on efficacy and safety for patients previously treated with monoclonal antibodies including Truxima or patients refractory to previous Truxima plus chemotherapy.</p> <p>In combination with glucocorticoids, is indicated for the induction of remission in adult patients with severe, active granulomatosis with polyangiitis (Wegener's) (GPA) and microscopic polyangiitis (MPA).</p>	17/02/2017	Celltrion Healthcare Hungary Kft.
UCEDANE	carglumic acid	Treatment of hyperammonaemia due to N-acetylglutamate synthase primary deficiency .	23/06/2017	Lucane Pharma
UPTRAVI	selexipag	<p>Long-term treatment of pulmonary arterial hypertension (PAH) in adult patients with WHO functional class (FC) II–III, either as combination therapy in patients insufficiently controlled with an endothelin receptor antagonist (ERA) and/or a phosphodiesterase type 5 (PDE-5) inhibitor, or as monotherapy in patients who are not candidates for these therapies.</p> <p>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.</p>	12/05/2016	Janssen-Cilag International NV
VEDROP	tocofersolan	Indicated in vitamin E deficiency due to digestive malabsorption in paediatric patients with congenital chronic cholestasis or hereditary chronic cholestasis , from birth (full term newborns) up to 18 years of age.	24/07/2009	Orphan Europe S.A.R.L

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
VELCADE	bortezomib	<p>As monotherapy or in combination with pegylated liposomal doxorubicin or dexamethasone is indicated for the treatment of adult patients with progressive multiple myeloma who have received at least 1 prior therapy and who have already undergone or are unsuitable for haematopoietic stem cell transplantation.</p> <p>In combination with melphalan and prednisone is indicated for the treatment of adult patients with previously untreated multiple myeloma who are not eligible for high-dose chemotherapy with haematopoietic stem cell transplantation.</p> <p>In combination with dexamethasone, or with dexamethasone and thalidomide, is indicated for the induction treatment of adult patients with previously untreated multiple myeloma who are eligible for high-dose chemotherapy with haematopoietic stem cell transplantation.</p> <p>In combination with rituximab, cyclophosphamide, doxorubicin and prednisone is indicated for the treatment of adult patients with previously untreated mantle cell lymphoma who are unsuitable for haematopoietic stem cell transplantation.</p>	26/04/2004	Janssen-Cilag International N.V.
VENCLYXTO	venetoclax	<p>In combination with rituximab for the treatment of adult patients with chronic lymphocytic leukaemia (CLL) who have received at least one prior therapy.</p> <p>As monotherapy for the treatment of CLL:</p> <ul style="list-style-type: none"> - in the presence of 17p deletion or TP53 mutation in adult patients who are unsuitable for or have failed a B cell receptor pathway inhibitor, or - in the absence of 17p deletion or TP53 mutation in adult patients who have failed both chemoimmunotherapy and a B cell receptor pathway inhibitor. 	05/12/2016	AbbVie Deutschland GmbH & Co. KG
VENTAVIS	iloprost	Treatment of patients with primary pulmonary hypertension , classified as NYHA functional class III, to improve exercise capacity and symptoms.	16/09/2003	Bayer AG
VEYVONDI	vonico g alfa	<p>In adults (age 18 and older) with von Willebrand Disease (VWD), when desmopressin (DDAVP) treatment alone is ineffective or not indicated for the:</p> <ul style="list-style-type: none"> - Treatment of haemorrhage and surgical bleeding - Prevention of surgical bleeding. <p>VEYVONDI should not be used in the treatment of Haemophilia A</p>	30/08/2018	Baxalta Innovations GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
VFEND	voriconazole	In adults and children aged 2 years and above as follows: - treatment of invasive aspergillosis . - treatment of serious fungal infections caused by <i>Scedosporium spp.</i> and <i>Fusarium spp.</i> Vfend should be administered primarily to patients with progressive, possibly life-threatening infections. Prophylaxis of invasive fungal infections in high risk allogeneic hematopoietic stem cell transplant (HSCT) recipients.	19/03/2002	Pfizer Europe MA EEIG
VOLIBRIS	ambrisentan	Treatment of pulmonary arterial hypertension (PAH) in adult patients of WHO Functional Class (FC) II to III, including use in combination treatment. Efficacy has been shown in idiopathic PAH (IPAH) and in PAH associated with connective tissue disease.	21/04/2008	GlaxoSmithKline (Ireland) Limited
VONCENTO	human coagulation factor viii/ von willebrand factor	Treatment of haemorrhage or prevention and treatment of surgical bleeding in patients with von Willebrand disease (VWD) , when desmopressin (DDAVP) treatment alone is ineffective or contraindicated. Prophylaxis and treatment of bleeding in patients with haemophilia A (congenital FVIII deficiency) .	12/08/2013	CSL Behring GmbH
VORICONAZOLE HOSPIRA	voriconazole	In adults and children aged 2 years and above as follows: - treatment of invasive aspergillosis . - treatment of serious fungal infections caused by <i>Scedosporium spp.</i> and <i>Fusarium spp.</i> Voriconazole should be administered primarily to patients with progressive, possibly life – threatening infections.	27/05/2015	Hospira UK Ltd
VOTRIENT	pazopanib	In adults for the first-line treatment of advanced renal cell carcinoma (RCC) and for patients who have received prior cytokine therapy for advanced disease. For the treatment of adult patients with selective subtypes of advanced soft-tissue sarcoma (STS) who have received prior chemotherapy for metastatic disease or who have progressed within 12 months after (neo)-adjuvant therapy. Efficacy and safety have only been established in certain STS histological tumour subtypes.	14/06/2010	Novartis Europharm Ltd
WILZIN	zinc acetate dihydrate	Treatment of Wilson's disease .	13/10/2004	Orphan Europe S.A.R.L

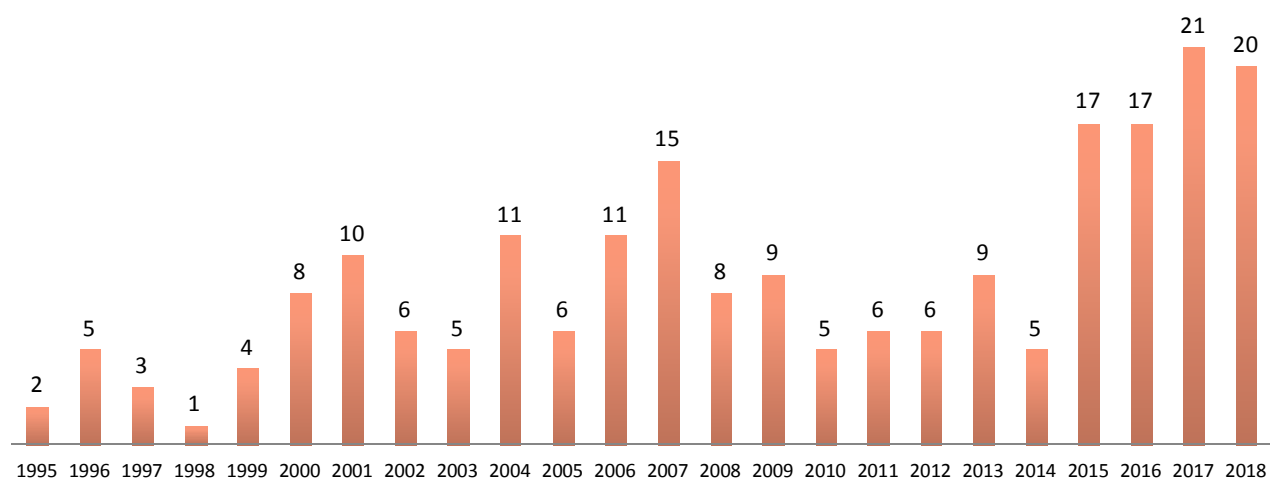
Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
XAGRID	anagrelide hydrochloride	Reduction of elevated platelet counts in at-risk essential-thrombocythaemia (ET) patients who are intolerant to their current therapy or whose elevated platelet counts are not reduced to an acceptable level by their current therapy. An at risk ET is defined by one or more of the following features: - > 60 years of age or - a platelet count > 1000 x 10 ⁹ /l or - a history of thrombo-haemorrhagic events.	16/11/2004	Shire Pharmaceuticals Ireland Limited
XELODA	capecitabine	First-line treatment of advanced gastric cancer in combination with a platinum-based regimen	02/02/2001	Roche Registration GmbH
XYREM	sodium oxybate	Treatment of narcolepsy with cataplexy in adult patients.	13/10/2005	UCB Pharma S.A.
YARGESA	miglustat	For the oral treatment of adult patients with mild to moderate type 1 Gaucher disease . Yargesa may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable	22/03/2017	JensonR+ Limited
YONDELIS	trabectedin	Treatment of adult 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.	17/09/2007	Pharma MarS.A.
ZARZIO	filgrastim	In children and adults with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/l$, and a history of severe or recurrent infections, long term administration of filgrastim is indicated to increase neutrophil counts and to reduce the incidence and duration of infection-related events.	06/02/2009	Sandoz GmbH
ZAVESCA	miglustat	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.	21/11/2002	Janssen-Cilag International NV
ZEVALIN	ibritumomab tiuxetan	Consolidation therapy after remission induction in previously untreated patients with follicular lymphoma . Treatment of adult patients with rituximab relapsed or refractory CD20+ follicular B-cell non-Hodgkin's lymphoma (NHL) .	16/01/2004	Spectrum Pharmaceuticals B.V.
ZUTECTRA	human hepatitis b immunoglobulin	Prevention of hepatitis B virus (HBV) re-infection in HBV-DNA negative patients over 6 months after liver transplantation for hepatitis B induced liver failure . Zutectra is indicated in adults only. The concomitant use of adequate virostatic agents should be considered, if appropriate, as standard of hepatitis B re-infection prophylaxis.	30/11/2009	Biotest Pharma GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
ZYDELIG	idelalisib	<p>In combination with rituximab, treatment of adult patients with chronic lymphocytic leukaemia (CLL):</p> <ul style="list-style-type: none"> - who have received at least one prior therapy, or - as first line treatment in the presence of 17p deletion or TP53 mutation in patients unsuitable for chemo-immunotherapy. <p>As monotherapy, treatment of adult patients with follicular lymphoma (FL) that is refractory to two prior lines of treatment.</p>	18/09/2014	Gilead Sciences Ireland UC

Clasificación por fecha de la AC en orden decreciente

2018	VENCLYXTO	SIMPONI	2003
ADYNOVI	2015	VEDROP	ALDURAZYME
ALKINDI	BORTEZOMIB ACCORD	ZARZIO	BUSILVEX
ANAGRELIDE MYLAN	TAXESPIRA	ZUTECTRA	CARBAGLU
CARMUSTINE OBVIUS	ELOCTA	2008	HUMIRA
DEFERIPRONE	JINARC	ABRAXANE	VENTAVIS
DENGVAZIA	KEYTRUDA	ADCIRCA	2002
HALIMATOZ	LENVIMA	CEPLENE	KINERET
HEFIYA	OBIZUR	PRIVIGEN	SOMAVERT
HEMLIBRA	OPDIVO	RATIOGRASTIM	TRACLEER
HULIO	ORKAMBI	TEVAGRASTIM	TRISENOX
HYRIMOZ	PEMETREXED HOSPIRA	THALIDOMIDE	VFEND
JIVI	PEMETREXED LILLY	CELGENE	ZAVESCA
KIGABEQ	PEMETREXED MEDAC	VOLIBRIS	2001
LENALIDOMIDE ACCORD	PEMETREXED SANDOZ	2007	CANCIDAS
MVASI	QUINSAIR	ABSAMEAD	CEPROTIN
NITYR	REPATHA	ATRIANCE	FABRAZYME
PEMETREXED Krka	RESPREEZA	BINOCRIT	GLIVEC
RUBRACA	VORICONAZOLE HOSPIRA	CYSTADANE	INOMAX
SLENYTO	2014	DIACOMIT	NONAFAC
VEYVONDI	BEMFOLA	ELAPRASE	RAPAMUNE
2017	LYNPARZA	EPOETIN ALFA HEXAL	REPLAGAL
AFSTYLA	NUWIQ	FLEBOGAMMA DIF	TARGETIN
AMGEVITA	RIXUBIS	GLIOLAN	XELODA
BLITZIMA	ZYDELIG	INCRELEX	2000
CUPRIOR	2013	ORENCIA	ENBREL
CYLTEZO	BOSULIF	REVLIMID	HELIXATE NEXGEN
ELMIRON	ERIVEDGE	SIKLOS	HERCEPTIN
ERELZI	GRASTOFIL	TORISEL	INTRONA
FOTIVDA	HYQVIA	YONDELIS	KEPPRA
IMATINIB TEVA	LOJUXTA	2006	KOGENATE BAYER
IMRALDI	NOVOEIGHT	ATRYN	PANRETIN
MIGLUSTAT GEN ORPH	OVALEAP	KIOVIG	THYROGEN
NITISINONE MDK	STAYVEER	EVOLTRA	1999
REFIXIA	VONCENTO	EXJADE	AMMONAPS
RITEMVIA	2012	NEXAVAR	FERRIPROX
RITUZENA	CAPRELSA	OMNITROPE	REFACTO AF
RIXATHON	COLOBRETHE	MYOZYME	TEMODAL
RIXIMYO	INLYTA	NAGLAZYME	1998
TADALAFIL GENERICS	JAKAVI	SAVENE	MABTHERA
TRUXIMA	NOVOTHIRTEEN	SPRYCEL	1997
UCEDANE	PIXUVRI	SUTENT	BENEFIX
YARGESA	2011	2005	CEREZYME
2016	BUCCOLAM	AVASTIN	CYSTAGON
ARMISARTE	CINRYZE	NOXAFIL	1996
BORTEZOMIB HOSPIRA	EURARTESIM	ORFADIN	CAELYX
BORTEZOMIB SUN	HALAVEN	REVATIO	HYCANTIN
CABOMETYX	HIZENTRA	TARCEVA	NOVOSEVEN
EMPLICITI	TEYSUNO	XYREM	PUREGON
IBLIAS	2010	2004	RILUTEK
KISPLYX	NIVESTIM	ADVATE	1995
KOVALTRY	OZURDEX	ALIMTA	GONAL-F
MYSILDECARD	REVOLADE	DUKORAL	TAXOTERE
NEOFORDEX	RUCONEST	ERBITUX	
NORDIMET	VOTRIENT	LITAK	
ONCASPAR	2009	LYSODREN	
PEMETREXED ACCORD	AFINITOR	PEDEA	
PEMETREXED FRESENIUS KABI	FILGRASTIM HEXAL	VELCADE	
SPECTRILA	ILARIS	WILZIN	
UPTRAVI	IXIARO	XAGRID	
	ROACTEMRA	ZEVALIN	

Número de medicamentos huérfanos en Europa con autorización de comercialización europea, sin designación huérfana en Europa por fecha de la AC



Clasificación por categoría ATC

A- ALIMENTARY TRACT AND METABOLISM	REVOLADE	PRIVIGEN	IMRALDI
ALDURAZYME	RIXUBIS	QUINSAIR	INLYTA
AMMONAPS	RUCONEST	VFEND	INTRONA
CARBAGLU	UPTRAVI	VORICONAZOLE	JAKAVI
CEREZYME	VENTAVIS	HOSPIRA	KEYTRUDA
CUPRIOR	VEYVONDI	ZUTECTRA	KINERET
CYSTADANE	VONCENTO	L- ANTINEOPLASTIC AND IMMUNOMODULATING AGENTS	KISPLYX
CYSTAGON	C- CARDIOVASCULAR SYSTEM	ABRAXANE	LENALIDOMIDE
ELAPRASE	JINARC	AFINITOR	ACCORD
FABRAZYME	LOJUXTA	ALIMTA	LENVIMA
MIGLUSTAT GEN ORPH	PEDEA	AMGEVITA	LITAK
MYOZYME	REPATHA	ANAGRELIDE	LYNPARZA
NAGLAZYME	STAYVEER	MYLAN	LYSODREN
NITISINONE MDK	TRACLEER	ARMISARTE	MABTHERA
NITYR	VOLIBRIS	ATRIANCE	MVASI
ORFADIN	G- GENITO URINARY SYSTEM AND SEX HORMONES	AVASTIN	NEXAVAR
REPLAGAL	ADCIRCA	BLITZIMA	NIVESTIM
UCEDANE	BEMFOLA	BORTEZOMIB	NORDIMET
VEDROP	ELMIRON	ACCORD	ONCASPAR
WILZIN	GONAL-F	BORTEZOMIB	OPDIVO
YARGESA	GONAL-F	HOSPIRA	ORENCIA
ZAVESCA	MYSILDECARD	BORTEZOMIB SUN	PANRETIN
B- BLOOD AND BLOOD FORMING ORGANS	OVALEAP	BOSULIF	PEMETREXED
ABSEAMED	PUREGON	BUSILVEX	ACCORD
ADVATE	REVATIO	CABOMETYX	FRESENIUS KABI
ADYNOVI	TADALAFIL	CAELYX	PEMETREXED
AFSTYLA	GENERICS	CAPRELSA	HOSPIRA
ATRYN	H- SYSTEMIC HORMONAL PREPARATIONS, EXCL, SEX HORMONES AND INSULINS	CARMUSTINE	PEMETREXED Krka
BENEFIX	ALKINDI	OBVIUS	PEMETREXED
BINOCRIT	INCRELEX	CEPLENE	LILLY
CEPROTIN	NEOFORDEX	CYLTEZO	PEMETREXED
CINRYZE	OMNITROPE	EMPLICITI	MEDAC
EPOETIN ALFA	SOMAVERT	ENBREL	SANDOZ
HEXAL	THYROGEN	ERBITUX	PIXUVRI
ELOCTA	J- GENERAL ANTIINFECTIVES FOR SYSTEMIC USE	ERELZI	RAPAMUNE
HELIXATE NEXGEN	CANCIDAS	ERIVEDGE	RATIOGRASTIM
HEMLIBRA	DENGVAIXA	EVOLTRA	REVLIMID
IBLIAS	DUKORAL	FILGRASTIM HEXAL	RITEMVIA
JIVI	FLEBOGAMMA DIF	FOTIVDA	RITUZENA
KOGENATE BAYER	HIZENTRA	GLIOLAN	RIXATHON
KOVALTRY	HYZENTRA	GLIVEC	RIXIMYO
NONAFACT	HYQVIA	GRASTOFIL	ROACTEMRA
NOVOEIGHT	IXIARO	HALAVEN	RUBRACA
NOVOSEVEN	KIOVIG	HALIMATOZ	SIKLOS
NOVOTHIRTEEN	NOXAFIL	HEFIYA	SIMPONI
NUWIQ		HERCEPTIN	SPECTRILA
OBIZUR		HULIO	SPRYCEL
REFACTO AF		HUMIRA	SUTENT
REFIXIA		HYCANTIN	TARCEVA
RESPREEZA		HYRIMOZ	TARGETIN
		ILARIS	TAXESPIRA
		IMATINIB TEVA	TAXOTERE
			TEMODAL

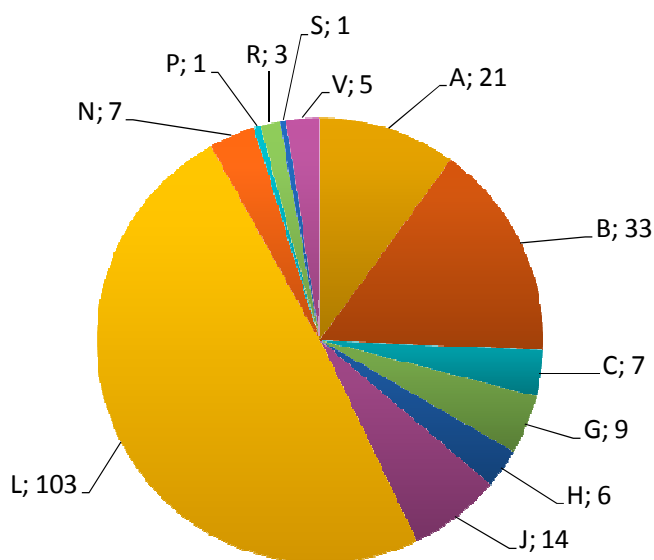
TEVAGRASTIM
TEYSUNO
THALIDOMIDE
CELGENE
TORISEL
TRISENOX
TRUXIMA
VELCADE
VENCLYXTO
VOTRIENT
XAGRID

XELODA
YONDELIS
ZARZIO
ZYDELIG
N- NERVOUS SYSTEM
BUCCOLAM
DIACOMIT
KEPPRA
KIGABEQ
RILUTEK

SLENYTO
XYREM
P- ANTIPARASITIC PRODUCTS, INSECTICIDES AND REPELLENTS
EURARTESIM
R- RESPIRATORY SYSTEM
COLOBREATHE
INOMAX

ORKAMBI
S- SENSORY ORGANS
OZURDEX
V- VARIOUS
DEFERIPRONE
EXJADE
FERRIPROX
SAVENE
ZEVALIN

Número de medicamentos huérfanos en Europa con autorización de comercialización europea, sin previa designación huérfana en Europa por categoría ATC



Clasificación por el titular de la AC

ABBVIE DEUTSCHLAND GMBH & CO. KG	BRISTOL-MYERS SQUIBB PHARMA EEIG	FABRAZYME	Medice Arzneimittel Pütter GmbH & Co KG
HUMIRA	EMPLICITI	MYOZYME	ABSEAMED
VENCLYXTO	OPDIVO	THYROGEN	MERCK KGAA
ADDMEDICA	ORENCIA	GILEAD SCIENCES IRELAND UC	ERBITUX
SIKLOS	SPRYCEL	ZYDELIG	MERCK EUROPE B.V.
ACCORD HEALTHCARE LTD	CELGENE EUROPE B.V.	GLAXOSMITHKLINE (IRELAND) LIMITED	GONAL-F
BORTEZOMIB ACCORD	ABRAXANE	VOLIBRIS	MERCK SHARP & DOHME B.V.
LENALIDOMIDE ACCORD	REVLIMID	GMP-ORPHAN SA	CANCIDAS
PEMETREXED ACCORD	THALIDOMIDE CELGENE	CUPRIOR	INTRONA
ACTAVIS GROUP PTC EHF	CELLTRION HEALTHCARE HUNGARY KFT.	HEXAL AG	KEYTRUDA
ARMISARTE	BLITZIMA	EPOETIN ALFA HEXAL	NOXAFIL
AGERION PHARMACEUTICALS SAS	RITEMVIA	FILGRASTIM HEXAL	PUREGON
LOJUXTA	RITUZENA	HOSPIRA UK LTD	TEMODAL
ALFASIGMA S.P.A	TRUXIMA	BORTEZOMIB HOSPIRA	MYLAN SAS
EURARTESIM	CHIESI FARMACEUTICI S.P.A.	TAXESPIRA	ANAGRELIDE MYLAN
ALLERGAN PHARMACEUTICALS IRELAND	QUINSAIR	PEMETREXED HOSPIRA	HULIO
OZURDEX	CLINIGEN HEALTHCARE LTD	NIVESTIM	MYSILDECARD
AMGEN EUROPE BV	SAVENE	VORICONAZOLE HOSPIRA	TADALAFIL GENERICS
AMGEVITA	CLOVIS ONCOLOGY IRELAND LIMITED	INSTITUTO GRIFOLS S.A.	NORDIC GROUP BV
MVASI	RUBRACA	FLEBOGAMMA DIF	NORDIMET
REPATHA	CSL BEHRING GMBH	IPSEN PHARMA	TEYSUNO
APOTEX EUROPE B.V.	AFSTYLA	CABOMETYX	NOVARTIS EUROPHARM LTD
FERRIPROX	HIZENTRA	INCRELEX	AFINITOR
GRASTOFIL	PRIVIGEN	JANSSEN-CILAG INTERNATIONAL NV	ATRIANCE
ASTRAZENECA AB	RESPREEZA	CAELYX	EXJADE
LYNPARZA	VONCENTO	SIMPONI	GLIVEC
AVENTIS PHARMA S.A.	CTI LIFE SCIENCES LTD	TRACLEER	HYCANTIN
RILUTEK	PIXUVRI	UPTRAVI	ILARIS
TAXOTERE	CYCLE PHARMACEUTICALS LTD	VELCADE	JAKAVI
BAXALTA INNOVATIONS GMBH	NITYR	ZAVESCA	REVOLADE
ADYNOVI	DIURNAL EUROPE B.V.	JENSON PHARMACEUTICALS SERVICES LIMITED	VOTRIENT
HYQVIA	ALKINDI	YARGESA	NOVENTIA PHARMA SRL
OBIZUR	EISAI GmbH	KRKA d.d.	CEPLENE
ONCASPAR	HALAVEN	PEMETREXED Krka	NOVO NORDISK A/S
RIXUBIS	TARGRETIN	LABORATOIRES CTRS	NOVOEIGHT
VEYVONDI	EISAI EUROPE LTD	NEOFORDEX	NOVOSEVEN
BAXTER AG	KISPLYX	LABORATOIRE FRANÇAIS DU FRACTIONNEMENT ET DES BIOTECHNOLOGIES	NOVOTHIRTEEN
ADVATE	LENVIMA	ATRYN	REFIXIA
CEPROTIN	PANRETIN	LABORATOIRE HRA PHARMA	OBVIUS INVESTMENT B.V..
KIOVIG	ELI LILLY NEDERLAND B.V.	LYSODREN	CARMUSTINE OBVIUS
BAYER AG	ADCIRCA	LINDE HEALTHCARE AB	OCTAPHARMA AB
HELIXATE NEXGEN	ALIMTA	INOMAX	NUWIQ
IBLIAS	PEMETREXED LILLY	LIPOMED GMBH	ORPHAN EUROPE S.A.R.L.
JIVI	EUSA Pharma (UK) Limited	DEFERIPRONE LIPOMED	CARBAGLU
KOGENATE BAYER	FOTIVDA	LITAK	CYSTADANE
KOVALTRY	FRESENIUS KABI DEUTSCHLAND GMBH	LUCANE PHARMA	CYSTAGON
NEXAVAR	PEMETREXED FRESENIUS	UCEDANE	PEDEA
VENTAVIS	GEDEON RICHTER PLC.	MARKLAS NEDERLAND BV	VEDROP
BENE- ARZNEIMITTEL GMBH	BEMFOLA	STAYVEER	WILZIN
ELMIRON	GEN.ORPH	MEDAC GESELLSCHAFT FÜR KLINISCHE SPEZIALPRÄPARATE MBH	ORPHELIA PHARMA SAS
BIOCODEX	MIGLUSTAT GEN ORPH	GLIOLAN	KIGABEQ
DIACOMIT	GENZYME EUROPE B.V.	PEMETREXED MEDAC	OTSUKA PHARMACEUTICAL EUROPE LTD
BIOMARIN INTERNATIONAL Limited	ALDURAZYME	SPECTRILA	JINARC
NAGLAZYME	CAPRELSA	MENDELKABS EUROPE LTD	PFIZER EUROPE MA EEIG
BIOTEST PHARMA GMBH	CEREZYME	NITISINONE MDK	BENEFIX
ZUTECTRA	EVOLTRA		BOSULIF
BOEHRINGER INGELHEIM INTERNATIONAL GMBH			ENBREL
CYLTEZO			INLYTA
			RAPAMUNE
			REFACTO AF
			REVATIO
			SOMAVERT

SUTENT	ROACTEMRA	SHIRE PHARMACEUTICALS IRELAND LIMITED	SWEDISH ORPHAN BIOVITRUM INTERNATIONAL AB
TORISEL	TARCEVA	XAGRID	AMMONAPS
VFEND	XELODA	SHIRE HUMAN GENETIC THERAPIES AB	ORFADIN
PHARMA MAR S.A.	SAMSUNG BIOEPIS NL B.V.	ELAPRASE	TEVA GMBH
YONDELIS	IMRALDI	REPLAGAL	TEVAGRASTIM
PHARMING GROUP N.V.	SANDOZ GMBH	SHIRE SERVICES BVBA	TEVA BV
RUCONEST	BINOCRIT	BUCCOLAM	COLOBREATHE
PIERRE FABRE MÉDICAMENTS	ERELZI	CINRYZE	IMATINIB TEVA
BUSILVEX	HALIMATOZ	SPECTRUM PHARMACEUTICALS B.V.	OVALEAP
RATIOPHARM GMBH	HEFIYA	ZEVALIN	TRISENOX
RATIOGRASTIM	HYRIMOZ	SUN Pharmaceutical Industries (Europe) B.V.	UCB PHARMA SA
RAD NEURIM PHARMACEUTICALS EEC LIMITED	OMNITROPE	BORTEZOMIB SUN	KEPPRA
SLENYTO	PEMETREXED SANDOZ	SWEDISH ORPHAN BIOVITRUM AB (PUBL)	XYREM
ROCHE REGISTRATION GMBH	RIXATHON	ELOCTA	VALNEVA AUSTRIA GMBH
AVASTIN	RIXIMYO	KINERET	IXIARO
ERIVEDGE	ZARZIO		VALNEVA SWEDEN AB
HEMLIBRA	SANOFI PASTEUR		DUKORAL
HERCEPTIN	DENGVAXIA		VERTEX PHARMACEUTICALS (EUROPE) LTD
MABTHERA	SANQUIN PLASMA PRODUCTS B.V.		ORKAMBI
	NONAFAC		

Por favor, tenga en cuenta que todos los datos presentados en este informe están disponibles para su descarga en [Orphadata](#)

Redactoras : Ana Rath & Valérie Salamon ● Fotografía : M. Depardieu/Inserm

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