Informes Periódicos de Orphanet

serie Medicamentos Huérfanos

Octubre 2017

Listados de medicamentos para enfermedades raras en Europa*

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

www.orpha.net www.orphadata.org

Liberté • Égalité • Fraternité
RÉPUBLIQUE FRANÇAISE
MINISTÈRE DES AFFAIRES SOCIALES ET DE LA SANTÉ

Instituts thématiques Inserm
Institut national de la santé et de la recherche médicale

AFMTÉLÉTHON INNOVER POUR GUERIR

Co-funded by the Health Programme of the European Union

Índice general

Para cualquier pregunta o comentario, por favor contacte con: *contact.orphanet@inserm.fr*

PARTE 1:

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

Índice

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 (consulte el Anexo 1 – « Medicamentos huérfanos retirados del Registro comunitario de medicamentos

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

huérfanos »; sus indicaciones están detalladas en la Parte II, « Lista de medicamentos destinados a enfermedades raras en Europa con una 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. Los medicamentos huérfanos con designación huérfana europea están indicados con el logo

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	Treatment of adult patients with relapsed or refractory CD30+ **Hodgkin lymphoma** (**HL**): -following autologous stem cell transplant (ASCT) or -following at least two prior therapies when ASCT or multi-agent chemotherapy is not a treatment option. Treatment of adult patients with **CD30+ HL** at increased risk of relapse or progression following ASCT Treatment of adult patients with relapsed or refractory systemic **anaplastic large cell lymphoma** (**sALCL**).	25/10/2012	Takeda Pharma A/S
ADEMPAS	riociguat	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. 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. Efficacy has been shown in a PAH population including etiologies of idiopathic or heritable PAH or PAH associated with connective tissue disease.	27/03/2014	Bayer Pharma AG
ALPROLIX	eftrenonacog alfa	Treatment and prophylaxis of bleeding in patients with **haemophilia B** (congenital factor IX deficiency). ALPROLIX can be used for all age groups.	12/05/2016	Biogen Idec Ltd
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
NEW BAVENCIO	avelumab	As monotherapy for the treatment of adult patients with metastatic **Merkel cell carcinoma** (**MCC**).	18/09/2017	Merck Serono Europe Limited

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
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 Limited
BLINCYTO	blinatumomab	Treatment of adults with Philadelphia chromosome negative relapsed or refractory **B -precursor acute lymphoblastic leukaemia** (**ALL**).	23/11/2015	Amgen Europe B.V.
BOSULIF	bosutinib	Treatment of adult patients with chronic phase (CP), accelerated phase (AP), and blast phase (BP) **Philadelphia chromosome positive chronic myelogenous leukaemia** (**Ph+ CML**) previously treated with one or more tyrosine kinase inhibitor(s) and for whom imatinib, nilotinib and dasatinib are not considered appropriate treatment options.	27/03/2013	Pfizer Ltd
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
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 *Pseudomonas aeruginosa* in patients with **cystic fibrosis** (**CF**) aged 6 years and older.	21/09/2009	Gilead Sciences International Ltd
CEPLENE	histamine dihydrochloride	Maintainance 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.	07/10/2008	Meda AB
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

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
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	TMC PharmaServices Ltd.
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
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.
DARZALEX	daratumumab	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 S.p.A.
DELTYBA	delamanib	Used as part of an appropriate combination regimen for **pulmonary multi-drugresistant 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

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
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	Apeiron Biologics AG
ESBRIET	pirfenidone	In adults for the treatment of mild to moderate **Idiopathic Pulmonary Fibrosis** (**IPF**).	28/02/2011	Roche Registration Ltd
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 (with C1-esterase-inhibitor deficiency).	11/07/2008	Shire Orphan Therapies GmbH
FIRDAPSE (previously ZENAS)	amifampridine	Symptomatic treatment of **Lambert-Eaton myasthenic syndrome** (**LEMS**) in adults.	23/12/2009	BioMarin Europe Ltd
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
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.	23/07/2014	Roche Registration Ltd
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.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
GRANUPAS (previously PARA-AMINOSALICYLIC ACID LUCANE)	para-aminosali- cylic 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	Lucane Pharma
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	ARIAD Pharma Ltd
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.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
IMNOVID (previously POMALIDOMIDE 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 Ltd
INOVELON	rufinamide	Adjunctive therapy in the treatment of seizures associated with **Lennox-Gastaut syndrome** in patients aged 4 years and older.	16/01/2007	Eisai Ltd
KALYDECO	ivacaftor	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 CFTR gene:*G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R*. Treatment of patients with cystic fibrosis (CF) aged 18 years and older who have an *R117H* mutation in the CFTR gene	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	Synageva BioPharma Ltd
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 ascerebrotendinous 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	Merck Serono Europe Ltd
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.
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.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
LEDAGA	chlormethine	Topical treatment of **mycosis fungoides-type cutaneous T-cell lymphoma** (**MF-type CTCL**) in adult patients.	03/03/2017	Actelion Registration Ltd.
LENVIMA	lenvatinib	Treatment of adult patients with progressive, locally advanced or metastatic, **differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma** (**DTC**) refractory to radioactive iodine (RAI).	28/05/2015	Eisai Ltd
NEW LUTATHERA	lutetium (177Lu) oxodotreotide	Treatment of unresectable or metastatic, progressive, well differentiated (G1 and G2), somatostatin receptor positive **gastroenteropancreatic neuroendocrine tumours** (**GEP NET**s) in adults.	26/09/2017	Advanced Accelerator Applications
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
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
MOZOBIL	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.
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 **hepatocellular carcinoma.** Treatment of patients with progressive, locally advanced or metastatic, **differentiated (papillary/ follicular/Hürthle cell) thyroid carcinoma**, refractory to radioactive iodine.	19/07/2006	Bayer Pharma AG
NEXOBRID	concentrate of proteolytic en- zymes enriched in bromelain	Removal of eschar in adults with **deep partial-** and **full-thickness thermal burns.**	18/12/2012	Mediwound 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

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
NPLATE	romiplostim	Indicated for adult **chronic immune (idiopathic) thrombocytopenic purpura** (**ITP**) patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins)	04/02/2009	Amgen Europe B.V.
OCALIVA	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
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
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	Actelion Registration Ltd
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	ViroPharmaSPRL
PROCYSBI	mercaptamine	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	Raptor Pharmaceuticals Europe BV

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
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 Therapeutics Ltd
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	NPS Pharma Holdings Ltd
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 Ltd
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.	24/04/2012	Novartis Europharm Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
SIKLOS	hydroxycarba- mide	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
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 Idec Ltd
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	GlaxoSmithKline Trading Services Limited

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
SYLVANT	siltuximab	Treatment of adult patients with **multicentric Castleman's disease** (**MCD**) who are human immunodeficiency virus (HIV) negative and human herpesvirus-8 (HHV-8) negative.	22/05/2014	Janssen-Cilag International N.V.
TASIGNA	nilotinib	Treatment of adult patients with newly diagnosed **Philadelphia chromosome positive chronic myelogenous leukaemia** (**CML**) in the chronic phase.	19/11/2007	Novartis Europharm Ltd
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.
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 Ltd
TOBI PODHALER	tobramycin	Suppressive therapy of chronic pulmonary infection due to *Pseudomonas aeruginosa* 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	First-line treatment of adult patients with advanced **renal cell carcinoma** (**RCC**) who have at least three of six prognostic risk factors. Treatment of adult patients with relapsed and / or refractory **mantle cell lymphoma** (**MCL**).	19/11/2007	Pfizer Ltd
TRANSLARNA	ataluren	Treatment of **Duchenne muscular dystrophy** resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 5 years and older. Efficacy has not been demonstrated in non-ambulatory patients. The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing.	31/07/2014	PTC Therapeutics Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
VENCLYXTO	venetoclax	As monotherapy for the treatment of **chronic lymphocytic leukaemia** (**CLL**) 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. As monotherapy for the treatment of CLL 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 Ltd
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 Ltd
VIMIZIM	elosulfase alfa	Treatment of **mucopolysaccharidosis, type IVA** (**Morquio A Syndrome, MPS IVA**) in patients of all ages.	28/04/2014	BioMarin Europe Ltd
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	Glaxo GroupLtd
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

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
VYNDAQEL	tafamidis	Treatment of **transthyretin amyloidosis** in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment.	16/11/2011	Pfizer Ltd
WAKIX	pitolisant	Treatment in adults of **narcolepsy with or without cataplexy**.	31/03/2016	Bioprojet Pharma
XALUPRINE (previously MERCAP-TOPURINE 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
YONDELIS	trabectedin	In combination with pegylated liposomal doxorubicin (PLD), treatment of patients with relapsed platinum-sensitive **ovarian cancer**.	28/10/2009	Pharma MarS.A.
ZALMOXIS	allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor (ΔLNGFR) 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	Actelion Registration Ltd

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

Cf. Parte II "Listado de medicamentos destinados a enfermedades raras en Europa con autorización de comercialización europea sin designación huérfana en Europa".

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
NEW 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
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 ***N-acetylglutamate synthetase (NAGS) deficiency***. It was originally designated an orphan medicine for this indication on 18 October 2000.	28/01/2003	28/01/2013
NEW 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
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
NEW 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

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
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
GLIOLAN	5-aminole-vulinic 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

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
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
NEW 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
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

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
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
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
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
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 condition: -Treatment of ***renal cell carcinoma*** (it was designated an orphan medicine on 29/07/2004)	19/07/2006	22/07/2016
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

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
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
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
NEW 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
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

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
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
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
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

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
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
NEW 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

Mas información: www.ema.europa.eu

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION (MA) INDICATION	MA DATE / MA HOLDER	MA WITHDRAWN DATE
BIOGRASTIM	filgrastim	In patients, children or adults, with **severe congenital, cyclic, or idiopathic neutropenia** with an absolute neutrophil count (ANC) of ≤ 0.5 x 10^9/l, and a history of severe or recurrent infections, long term administration of Biograstim was indicated to increase neutrophil counts and to reduce the incidence and duration of infection-related events.	15/09/2008 AbZ-Pharma GmbH	25/09/2015
NUEDEXTA	dextro methorphan hydrobromide / quinidine sulfate	Symptomatic treatment of pseudobulbar affect (PBA) in adults. Efficacy has been studied in patients with underlying **Amyotrophic Lateral Sclerosis**.	24/06/2013 Jenson Pharmaceutical Services Ltd	04/03/2016
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

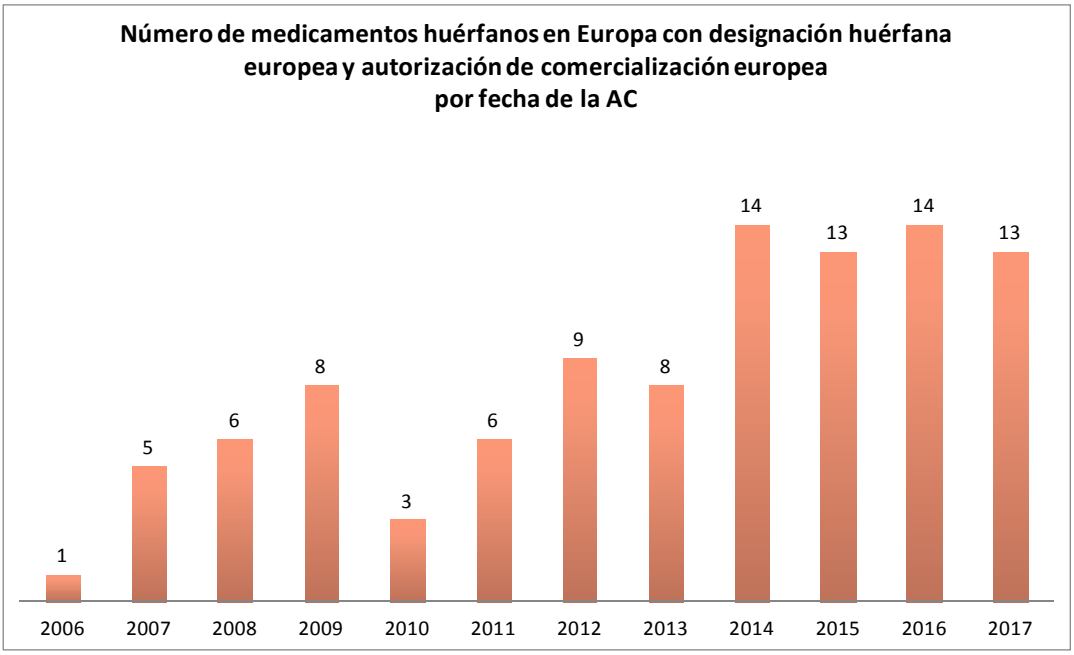
Clasificación por fecha de la AC en orden decreciente

2017
BAVENCIO
BESPONSA
BRINEURA
CHENODEOXYCHOLIC ACID LEADIANT
CYSTADROPS
DINUTUXIMAB BETA APEIRON
LEDAGA
LUTATHERA
NATPAR
OXERVATE
RYDAPT
SPINRAZA
XERMELO
2016
ALPROLIX
COAGADEX
DARZALEX
GALAFOLD
IDELVION
LARTRUVO
NINLARO
OCALIVA
ONIVYDE
SOMAKIT TOC
STRIMVELIS
VENCLYXTO
WAKIX
ZALMOXIS

2015
BLINCYTO
CERDELGA
CRESEMBA
FARYDAK
HETLIOZ
HOLOCLAR
KANUMA
KYPROLIS
LENVIMA
OFEV
RAVICTI
RAXONE
STRENSIQ
2014
ADEMPAS
COMETRIQ
DELTYBA
GAZYVARO
GRANUPAS
IMBRUVICA
KETOCONAZOLE HRA
KOLBAM
LYNPARZA
SCENESSE
SIRTURO
SYLVANT
TRANSLARNA
VIMIZIM
2013

BOSULIF
DEFITELIO
ICLUSIG
IMNOVID
OPSUMIT
ORPHACOL
PROCYSBI
REVLIMID
2012
ADCETRIS
BRONCHITOL
DACOGEN
GLYBERA
KALYDECO
NEXOBRID
REVESTIVE
SIGNIFOR
XALUPRINE
2011
CARBAGLU
ESBRIET
PLENADREN
TOBI PODHALER
VOTUBIA
VYNDAQEL
2010
ARZERRA
TEPADINA
VPRIV

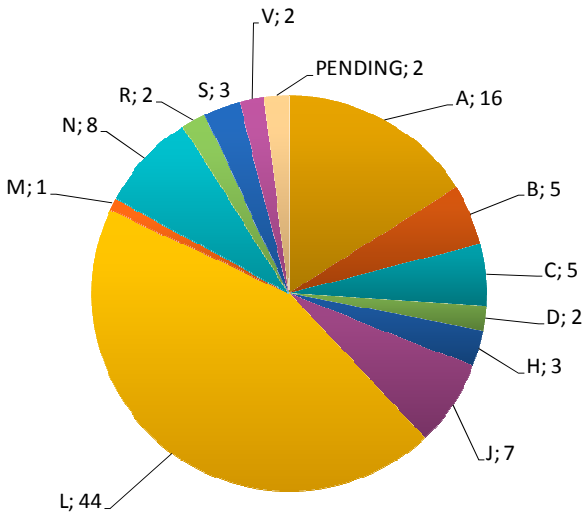
2009
CAYSTON
FIRDAPSE
MEPACT
MOZOBIL
NPLATE
PEYONA
YONDELIS
ZAVESCA
2008
CEPLENE
FIRAZYR
KUVAN
THALIDOMIDE CELGENE
VIDAZA
VOLIBRIS
2007
INOVELON
SIKLOS
SOLIRIS
TASIGNA
TORISEL
2006
NEXAVAR



Clasificación por categoría ATC

A- ALIMENTARY TRACT AND METABOLISM
BRINEURA
CARBAGLU
CERDELGA
CHENODEOXYCHOLIC ACID LEADIANT
KANUMA
KOLBAM
KUVAN
OCALIVA
ORPHACOL
PROCYSBI
RAVICTI
REVESTIVE
STRENSIQ
VIMIZIM
VPRIV
ZAVESCA
B- BLOOD AND BLOOD FORMING
ALPROLIX
COAGADEX
DEFITELIO
IDELVION
NPLATE
C-CARDIOVASCULAR SYSTEM
ADEMPAS
FIRAZYR
GLYBERA
OPSUMIT
VOLIBRIS
D- DERMATOLOGICALS
NEXOBRID
SCENESSE
H- SYSTEMIC HORMONAL PREPARATIONS, EXCL. SEX HORMONES AND INSULINS
NATPAR
PLENADREN
SIGNIFOR
J- GENERAL ANTIINFECTIVES FOR SYSTEMIC USE
CAYSTON
CRESEMBA
DELTYBA
GRANUPAS
KETOCONAZOLE
SIRTURO
TOBI PODHALER
L- ANTINEOPLASTIC AND IMMUNOMODULATING
ADCETRIS
ARZERRA
BAVENCIO
BESPONSA
BLINCYTO
BOSULIF
CEPLENE
COMETRIQ
DACOGEN
DARZALEX
DINUTUXIMAB BETA APEIRON
ESBRIET
FARYDAK
GAZYVARO
ICLUSIG
IMBRUVICA
IMNOVID
KYPROLIS
LARTRUVO
LEDAGA
LENVIMA
LYNPARZA
MEPACT
MOZOBIL
NEXAVAR
NINLARO
OFEV
NIVYDE
REVLIMID
RYDAPT
SIKLOS
SOLIRIS
STRIMVELIS
SYLVANT
TASIGNA
TEPADINA
THALIDOMIDE CELGENE
TORISEL
VENCLYXTO
VIDAZA
VOTUBIA
XALUPRINE
YONDELIS
ZALMOXIS
M- MUSCULO-SKELETAL SYSTEM
TRANSLARNA
N- NERVOUS SYSTEM
FIRDAPSE
HETLIOZ
INOVELON
PEYONA
RAXONE
SPINRAZA
VYNDAQEL
WAKIX
R- RESPIRATORY
BRONCHITOL
KALYDECO
S- SENSORY ORGANS
CYSTADROPS
HOLOCLAR
OXERVATE
V- VARIOUS
LUTATHERA
SOMAKIT TOC
PENDING
GALAFOLD
XERMELO

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



Clasificación por titular de la AC

ABBVIE LTD
VENCLYXTO
ACTELION REGISTRATION LTD
LEDAGA
OPSUMIT
ZAVESCA
ADDMEDICA
SIKLOS
ADIENNE SRL
TEPADINA
ADVANCED ACCELERATOR APPLICATIONS
LUTATHERA
SOMAKIT TOC
ALEXION EUROPE SAS
SOLIRIS
STRENSIQ
AMGEN EUROPE B.V.
BLINCYTO
KYPROLIS
NPLATE
AMICUS THERAPEUTICS UK LTD
GALAFOLD
APEIRON BIOLOGICS AG
DINUTUXIMAB BETA APEIRON
ARIAD PHARMA LTD
ICLUSIG
ASTRA ZENECA AB
LYNPARZA
BASILEA MEDICAL LTD
CRESEMBA
BAXALTA INNOVATIONS
ONIVYDE
BAYER PHARMA AG
ADEMPAS
NEXAVAR
BIO PRODUCTS LABORATORY LTD
COAGADEX
BIOGEN IDEC LTD
ALPROLIX
SPINRAZA
BIOMARIN EUROPE LTD
BRINEURA
FIRDAPSE
VIMIZIM
BIOPROJET PHARMA
WAKIX
BOEHRINGER INGELHEIM INTERNATIONAL GMBH
OFEV
CELGENE EUROPE LTD
IMNOVID
REVLIMID
THALIDOMIDE CELGENE
VIDAZA
CHIESI FARMACEUTICI SPA
HOLOCLAR
PEYONA
CLINUVEL UK LIMITED
SCENESSE
CSL BEHRING GMBH
IDELVION
DOMPE FARMACEUTICI S.P.A.
OXERVATE
ELI LILLY B.V.
LARTRUVO
ESAI LTD
INOVELON
LENVIMA
GENTIUM SPA
DEFITELIO
GENZYME EUROPE B.V.
CERDELGA
MOZOBIL
GILEAD SCIENCES INTERNATIONAL LTD
CAYSTON
GLAXO GROUP LTD
VOLIBRIS
GLAXOSMITHKLINE TRADING SERVICES LIMITED
STRIMVELIS
HORIZON THERAPEUTICS Ltd
RAVICTI
INTERCEPT PHARMA
OCALIVA
IPSEN PHARMA
XERMELO
JANSSEN-CILAG INTERNATIONAL NV
DACOGEN
DARZALEX
IMBRUVICA
SIRTURO
SYLVANT
LABORATOIRE HRA PHARMA
KETOCONAZOLE HRA
LABORATOIRES CTRS
ORPHACOL
LEADIANT GmbH
CHENODEOXYCHOLIC ACID LEADIANT
LUCANE PHARMA
GRANUPAS
MEDA AB
CEPLENE
MEDIWOUND GERMANY GMBH
NEXOBRID
MERCK SERONO EUROPE LTD
BAVENCIO
KUVAN
MolMed SpA
ZALMOXIS
NOVA LABORATORIES LTD
XALUPRINE
NOVARTIS EUROPHARM LTD
ARZERRA
FARYDAK
RYDAPT
SIGNIFOR
TASIGNA
TOBI PODHALER
VOTUBIA
NPS PHARMA HOLDINGS LIMITED
REVESTIVE
ORPHAN EUROPE SARL
CARBAGLU
CYSTADROPS
OTSUKA NOVEL PRODUCTS GMBH
DELTYBA
PFIZER LTD
BESPONSA
BOSULIF
TORISEL
VYNDAQEL
PHARMA MAR S.A.
YONDELIS
PHARMAXIS PHARMACEUTICALS LTD
BRONCHITOL
PTC THERAPEUTICS LTD
TRANSLARNA
RAPTOR PHARMACEUTICALS EUROPE B.V.
PROCYSBI
RETROPHIN EUROPE LTD
KOLBAM
ROCHE REGISTRATION LTD
ESBRIET
GAZYVARO
SANTHERA PHARMACEUTICALS (DEUTSCHLAND) GMBH
RAXONE
SHIRE ORPHAN THERAPIES GMBH
FIRAZYR
SHIRE PHARMACEUTICALS IRELAND LTD
NATPAR
VPRIV
SYNAGEVA BIOPHARMA LTD
KANUMA
TAKEDA FRANCE SAS
MEPACT
TAKEDA PHARMA A/S.
ADCETRIS
NINLARO
TMC PHARMA SERVICES LTD.
COMETRIQ
UNIQURE BIOPHARMA B.V.
GLYBERA
VANDA PHARMACEUTICALS LTD
HETLIOZ
VERTEX PHARMACEUTICALS (EUROPE) LTD
KALYDECO
VIROPHARMA SPRL
PLENADREN

PARTE 2 :

Listado de medicamentos destinados a enfermedades raras en Europa con autorización de commercialización europea* sin designación huérfana en Europa

Índice

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 Ltd
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
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.
AMGEVITA	adalimumab	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. Adalimumab has not been studied in patients aged less than 2 years. 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. 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.	22/03/2017	Amgen Europe B.V.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
AMMONAPS	sodium phenylbutyrate	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 *neonatal-onset* presentation (complete enzyme deficiencies, presenting within the first 28 days of life). It is also indicated in patients with *late-onset* disease (partial enzyme deficiencies, presenting after the first month of life) who have a history of hyperammonaemic encephalopathy.	08/12/1999	Swedish Orphan Biovitrum AB
ARMISARTE (previously PEMETREXED ACTAVIS)	pemetrexed	In combination with cisplatin is indicated for the treatment of chemotherapy naïve patients with unresectable malignant **pleural mesothelioma**.	18/01/2016	Actavis Group PTC ehf
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	GTC Biotherapeutics UK Ltd
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. In combination with paclitaxel and cisplatin or, alternatively, paclitaxel and topotecan in patients who cannot receive platinum therapy, is indicated for the treatment of adult patients with persistent, recurrent, or metastatic **carcinoma of the cervix**	12/01/2005	Roche Registration Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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	Finox BiotechAG
BENEFIX	nonacog alpha	Treatment and prophylaxis of bleeding in patients with **haemophilia B** (**congenital factor IX deficiency**).	27/08/1997	Pfizer Ltd
NEW BLITZIMA	rituximab	Treatment of previously untreated patients with stage III-IV **follicular lymphoma** in combination with chemotherapy. As maintenance therapy indicated for the treatment of follicular lymphoma patients responding to induction therapy. 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. 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 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.	13/07/2017	Celltrion Healthcare Hungary Kft.
BORTEZOMIB ACCORD	bortezomib	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. 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. 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. 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.	20/07/2015	Accord Healthcare Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
BORTEZOMIB HOSPIRA	bortezomib	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. 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. 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. 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.	22/07/2016	Hospira UK Limited
BORTEZOMIB SUN	bortezomib	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. 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. 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. 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.	22/07/2016	SUN Pharmaceutical Industries (Europe) B.V.
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	ViroPharma SPRL

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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 adults following prior vascular endothelial growth factor (VEGF)-targeted therapy.	09/09/2016	Ipsen Pharma
CAELYX	doxorubicin hydrochloride (pegylated liposomal)	Treatment of **advanced ovarian cancer** in women who have failed a first-line platinum-based chemotherapy regimen. 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. Treatment of AIDS-related **Kaposi's sarcoma** (**KS**) in patients with low CD4 counts (< 200 CD4 lymphocytes/mm3) and extensive mucocutaneous or visceral disease. 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).	21/06/1996	Janssen-Cilag International N.V.
CANCIDAS (previously CASPOFUNGIN MSD)	caspofungin	Treatment of **invasive candidiasis** in adult or paediatric patients. 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. Empirical therapy for presumed fungal infections (such as Candida or Aspergillus) in febrile, neutropaenic adult or paediatric patients.	24/10/2001	Merck Sharp & Dohme Ltd
CAPRELSA	vandetanib	Treatment of aggressive and symptomatic **medullary thyroid cancer** (**MTC**) in patients with unresectable locally advanced or metastatic disease. Caprelsa is indicated in adults, children and adolescents aged 5 years and older. 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.	17/02/2012	AstraZenecaAB
CARBAGLU	carglumic acid	Treatment of **hyperammonaemia due to N-acetylglutamate synthase (NAGS) primary deficiency**	28/01/2003	Orphan Europe S.A.R.L

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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	ViroPharma SPRL
COLOBREATHE	colistimethate sodium	Management of chronic pulmonary infections due to *Pseudomonas aeruginosa* in patients with **cystic fibrosis** (**CF**) aged 6 years and older. Consideration should be given to official guidance on the appropriate use of antibacterial agents.	13/02/2012	Forest Labo- ratories UK Ltd
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
CYSTADANE	betaine anhydrous	Adjunctive treatment of **homocystinuria**, involving **deficiencies** or **defects in cystathionine beta- synthase** (**CBS**), **5,10-methylene-tetrahydrofolate reductase** (**MTHFR**), **cobalamin cofactor metabolism (cbl)**. Cystadane should be used as supplement to other therapies such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), folate and a specific diet.	15/02/2007	Orphan Europe S.a.r.l.
CYSTAGON	mercaptamine bitartrate	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.	23/06/1997	Orphan Europe S.A.R.L
DEPOCYTE	cytarabine	Intrathecal treatment of **lymphomatous meningitis**. In the majority of patients such treatment will be part of symptomatic palliation of the disease.	11/07/2001	Pacira Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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
DOCETAXEL HOSPIRA UK LIMITED	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.**	28/08/2015	Hospira UK Ltd
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	Crucell 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	Biogen Idec Ltd
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 Ltd
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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/ Yyyy)	Marketing Authorisation Holder
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 Ltd
EURARTESIM	piperaquine tetraphosphate/ dihydroartemi-sinin	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	Sigma-Tau Industrie Far- maceutiche Riunite 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.
EXJADE	deferasirox	Treatment of chronic iron overload due to frequent blood transfusions (≥7ml/kg/month of packed red blood cells) in patients with **beta thalassaemia major** aged 6 years and older. Treatment of chronic iron overload due to blood transfusions when deferoxamine therapy s contraindicated or inadequate in the following patient groups: -in paediatric patients with beta thalassaemia major with iron overload due to frequent blood transfusions (≥7ml/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 (<7ml/kg/month of packed red blood cells)aged 2years and older, -in adult and paediatric patients with other anaemias aged 2 years and older. Treatment of chronic iron overload requiring chelation therapy when deferoxamine therapy is contraindicated or inadequate in patients with **non-transfusion-dependent thalassaemia syndromes** aged 10years and older.	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.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/ Yyyy)	Marketing Authorisation Holder
FILGRASTIM HEXAL	filgrastim	In patients, children or adults, with **severe congenital, cyclic,** or **idiopathic neutropenia** with an absolute neutrophil count (ANC) of ≤ 0.5 x 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	Hexal AG
FLEBOGAMMA DIF	human normal immunoglobulin	Replacement therapy in adults, and children and adolescents (2-18 years) in: **- 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). Immunomodulation in adults, and children and adolescents (2-18 years) in - **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.
GLIOLAN	5-aminole-vulinic 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 GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
GLIVEC	imatinib mesilate	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. Tratment of adult and paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy, or in accelerated phase or blast crisis. Treatment of adult and paediatric patients with newly diagnosed Philadelphia chromosome positive **acute lymphoblastic leukaemia** (**Ph+ ALL**) integrated with chemotherapy. Treatment of adult patients with relapsed or refractory Ph+ ALL as monotherapy. Treatment of adult patients with **myelodysplastic / myeloproliferative diseases** (**MDS/MPD**) associated with platelet-derived growth factor receptor (PDGFR) gene rearrangements. Treatment of adult patients with advanced **hypereosinophilic syndrome** (**HES**) and/or **chronic eosinophilic leukaemia** (**CEL**) with FIP1L1-PDGFRα rearrangement. The effect of Glivec on the outcome of bone marrow transplantation has not been determined. Treatment of adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant **gastrointestinal stromal tumours** (**GIST**) 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. Treatment of adult patients with unresectable **dermatofibrosarcoma protuberans** (**DFSP**) and adult patients with recurrent and/or metastatic DFSP who are not eligible for surgery.	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 Serono Europe Ltd
GRASTOFIL	filgrastim	In adult or children patients with **severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil** count (ANC) of ≤ 0.5 x 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.
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 Pharma AG

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/ Yyyy)	Marketing Authorisation Holder
HERCEPTIN	trastuzumab	In combination with capecitabine or 5-fluorouraciland 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 Ltd
HIZENTRA	human normal immunoglobulin (scig)	Replacement therapy in adults and children in **primary immunodeficiency syndromes** such as: - congenital **agammaglobulinaemia** and **hypogammaglobulinaemia,** - **common variable immunodeficiency,** - **severe combined immunodeficiency,** - **IgG subclass deficiencies** with recurrent infections. Replacement therapy in **myeloma** or **chronic lymphocytic leukaemia** with severe secondary hypogammaglobulinaemia and recurrent infections.	14/04/2011	CSL Behring GmbH
HUMIRA	adalimumab	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). As monotherapy in case of intolerance to methotrexate or when continued treatment with methotrexate is inappropriate. It has not been studied in children aged less than 2 years. 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. 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. 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.	08/09/2003	Abbvie Ltd.
HYCAMTIN	topotecan	As monotherapy, treatment of: -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. In combination with cisplatin, it is indicated for patients with **carcinoma of the cervix** recurrent after radiotherapy and for patients with Stage IVB disease. Patients with prior exposure to cisplatin require a sustained treatment free interval to justify treatment with the combination.	12/11/1996	Novartis Europharm Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
HYQVIA	human normal immunoglobulin	Replacement therapy in adults (≥ 18 years) in primary immunodeficiency syndromes such as: - **congenital agammaglobulinaemia** and **hypogammaglobulinaemia** - **common variable immunodeficiency** - **severe combined immunodeficiency** - **IgG subclass deficiencies** with recurrent infections. Replacement therapy in adults (**≥** 18 years) in **myeloma or chronic lymphocytic leukaemia** with severe secondary hypogammaglobulinaemia and recurrent infections.	16/05/2013	Baxalta Innovations 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
ILARIS	canakinumab	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: -**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. 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.	23/10/2009	Novartis Europharm Ltd
IMRALDI	adalimumab	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. 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. 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.	24/08/2017	Samsung Bioepis UK Limited (SBUK)

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
INCRELEX	mecasermin	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**). Severe Primary IGFD is defined by: - height standard deviation score ≤ -3.0 and - basal IGF-1 levels below the 2.5th percentile for age and gender and - GH sufficiency - exclusion of secondary forms of IGF-1 deficiency, such as malnutrition, hypothyroidism, or chronic treatment with pharmacologic doses of anti-inflammatory steroids. Severe Primary IGFD includes patients with mutations in the GH receptor (GHR), post-GHR 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.	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 Ltd
INOMAX	nitric oxide	In conjunction with ventilatory support and other appropriate active substances: - for the treatment of newborn infants ≥ 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	Treatment of patients with **hairy cell leukaemia**. 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. 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. 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. 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. Treatment of **carcinoid tumours** with lymph node or liver metastases and with "**carcinoid syndrome**".	09/03/2000	Merck Sharp & Dohme Ltd
IXIARO	japanese encephalitis vaccine (inacti-vated, adsorbed)	Active immunisation against **Japanese encephalitis** in adults, adolescents, children and infants aged 2 months and older. IXIARO should be considered for use in individuals at risk of exposure through travel or in the course of their occupation	31/03/2009	Valneva Austria GmbH
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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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 from12 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 Limited
KINERET	anakinra	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**).	08/03/2002	Swedish Orphan Biovitrum AB
KIOVIG	human normal immunoglobulin	Replacement therapy in adults, and children and adolescents (0-18 years) in: - **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. Immunomodulation in adults, and children and adolescents (0-18 years) in: - **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
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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/ Yyyy)	Marketing Authorisation Holder
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 Pharma 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 Pharma AG
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
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 Ltd
MYOZYME	alglucosidase alpha	Long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of **Pompe disease** (**acid α-glucosidase deficiency**). Myozyme is indicated in adults and paediatric patients of all ages	29/03/2006	Genzyme Europe B.V.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
MYSILDECARD	sildenafil	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.	15/09/2016	MYLAN S.A.S.
NAGLAZYME	galsulfase	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of **Mucopolysaccharidosis VI** (**MPS VI**; **N-acetylgalactosamine 4-sulfatase deficiency**; **Maroteaux-Lamy syndrome**) 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.	24/01/2006	BioMarin Europe Ltd
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 Pharma 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
NIVESTIM	filgrastim	In patients, children or adults, with **severe congenital, cyclic,** or **idiopathic neutropenia** with an absolute neutrophil count (ANC) of ≤ 0.5 x 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
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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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 antibodies toGP IIb - IIIa and/or HLA, and with past or present refractoriness to platelet transfusions.	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
NOXAFIL	posaconazole	Treatment of the fungal infections in adults: - 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. 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. Prophylaxis of invasive fungal infections in : - 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 Ltd
OBIZUR	susoctocog alfa	Treatment of bleeding episodes in patients with **acquired haemophilia** caused by antibodies to Factor VIII.	11/11/2015	Baxalta Innovations GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
OMNITROPE	somatropin	Infants, children and adolescents: - 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. Adults - Replacement therapy in adults with pronounced **growth hormone deficiency**. - *Adult onset:* 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. - *Childhood onset:* 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. All other patients will require IGF-I assay and one growth hormone stimulation test.	12/04/2006	Sandoz GmbH
ONCASPAR	pegaspargase	Indicated as a component of antineoplastic combination therapy in **acute lymphoblastic leukaemia** (**ALL**) in paediatric patients from birth to 18 years, and adult patients.	14/01/2016	Baxalta Innovations GmbH
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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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 12 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 Pharmaceuticals Europe 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	In combination with cisplatin is indicated for the treatment of chemotherapy naïve patients with unresectable malignant **pleural mesothelioma**.	18/01/2016	Accord Healthcare Ltd
PEMETREXED FRESENIUS KABI	pemetrexed	In combination with cisplatin is indicated for the treatment of chemotherapy naïve patients with unresectable malignant **pleural mesothelioma**.	22/07/2016	Fresenius Kabi Oncology Plc
PEMETREXED HOSPIRA	pemetrexed	In combination with cisplatin indicated for the treatment of chemotherapy naïve patients with unresectable malignant **pleural mesothelioma.**	20/11/2015	Hospira UK Ltd
PEMETREXED LILLY	pemetrexed	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	In combination with cisplatin indicated for the treatment of chemotherapy naïve patients with unresectable malignant **pleural mesothelioma**.	27/11/2015	Medac GmbH
PEMETREXED SANDOZ	pemetrexed	In combination with cisplatin for the treatment of chemotherapy naive 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)	Replacement therapy in adults, and children and adolescents (0-18 years) in: - **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. Immunomodulation in adults, and children and adolescents (0-18 years) in: - **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**). Only limited experience is available of use of intravenous immunoglobulins in children with CIDP.	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 Ltd
QUINSAIR	levofloxacin	Management of chronic pulmonary infections due to *Pseudomonas aeruginosa* in adult patients with **cystic fibrosis**	26/03/2015	Aptalis Pharma SAS
RATIOGRASTIM	filgrastim	In patients, children or adults, with **severe congenital, cyclic,** or **idiopathic neutropenia** with an absolute neutrophil count (ANC) of ≤ 0.5 x 10^9/l, and a history of severe or recurrent infections.	15/09/2008	Ratiopharm GmbH
REFACTO AF	moroctocog alpha	Treatment and prophylaxis of bleeding in patients with **haemophilia A** (**congenital factor VIII deficiency**) in adults and children of all ages, including newborns. ReFacto AF is appropriate for use in adults and children of all ages, including newborns. ReFacto AF does not contain von Willebrand factor, and hence is not indicated in von Willebrand's disease.	13/04/1999	Pfizer Ltd
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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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 Ltd
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 Ltd
REVOLADE	eltrombopag	Indicated for **chronic immune (idiopathic) thrombocytopenic purpura** (**ITP**) patients aged 1 year and above who are refractory to other treatments. 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.	11/03/2010	Novartis Europharm Ltd
RILUTEK	riluzole	To extend life or the time to mechanical ventilation for patients with **amyotrophic lateral sclerosis** (**ALS**). 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. 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.	10/06/1996	Aventis Pharma S.A.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
NEW RITEMVIA	rituximab	Treatment of previously untreated patients with stage III, IV **follicular lymphoma** in combination with chemotherapy. As maintenance therapy for the treatment of follicular lymphoma patients responding to induction therapy. 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. 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 glucocorticoids, is indicated for the induction of remission in adult patients with severe, active **granulomatosis with polyangiitis** (**Wegener's**) (**GPA**) and **microscopic polyangiitis** (**MPA**).	13/07/2017	Celltrion Healthcare Hungary Kft
NEW RITUZENA (previously TUXELLA)	rituximab	Treatment of previously untreated patients with stage III IV **follicular lymphoma** in combination with chemotherapy. 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. 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 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.	13/07/2017	Celltrion Healthcare Hungary Kft

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
RIXATHON	rituximab	Treatment of previously untreated patients with stage III-IV **follicular lymphoma** in combination with chemotherapy. As maintenance therapy for the treatment of follicular lymphoma patients responding to induction therapy. 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. 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. 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. 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**).	15/06/2017	Sandoz GmbH
RIXIMYO	rituximab	Treatment of previously untreated patients with stage III-IV **follicular lymphoma** in combination with chemotherapy. As maintenance therapy for the treatment of follicular lymphoma patients responding to induction therapy. 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. 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. 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**).	15/06/2017	Sandoz GmbH
RIXUBIS	nonacog gamma	Treatment and prophylaxis of bleeding in patients with **haemophilia B** (**congenital factor IX deficiency**). RIXUBIS is indicated in patients of all age groups.	19/12/2014	Baxalta Innovations GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
ROACTEMRA	tocilizumab	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. 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.	16/01/2009	Roche Registration Ltd
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
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.
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 Ltd
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.	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	Treatment of **pulmonary arterial hypertension** (**PAH**) to improve exercise capacity and symptoms in patients with WHO functional class III. Efficacy has been shown in: • **Primary** (idiopathic and 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**. Some improvements have also been shown in patients with pulmonary arterial hypertension WHO functional class II. Indicated to reduce the number of new digital ulcers in patients with **systemic sclerosis** and ongoing digital ulcer disease	24/06/2013	Marklas Nederland BV
SUTENT	sunitinib	Treatment of unresectable and/or metastatic **malignant gastrointestinal stromal tumour** (**GIST**) in adults after failure of imatinib treatment due to resistance or intolerance. Treatment of **advanced/metastatic renal cell carcinoma** (**MRCC**) in adults. Treatment of unresectable or metastatic, well-differentiated **pancreatic neuroendocrine tumours** (**pNET**) with disease progression in adults. Experience with SUTENT as first-line treatment is limited	19/07/2006	Pfizer Ltd
TADALAFIL GENERICS	tadalafil	Indicated 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.	09/01/2017	MYLAN S.A.S
TARCEVA	erlotinib	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.	19/09/2005	Roche Registration Ltd
TARGRETIN	bexarotene	Treatment of skin manifestations of advanced stage **cutaneous T-cell lymphoma** (**CTCL**) patients refractory to at least one systemic treatment.	29/03/2001	Eisai Ltd
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 Ltd
TEVAGRASTIM	filgrastim	In patients, children or adults, with **severe congenital, cyclic,** or **idiopathic neutropenia** with an absolute neutrophil count (ANC) of ≤ 0.5 x 10^9/l, and a history of severe or recurrent infections.	15/09/2008	Teva GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/ Yyyy)	Marketing Authorisation Holder
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
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 remnantsin patients who have undergone a near-total ortotal thyroidectomy for well-differentiated thyroid cancer and who do not have evidence of distantmetastatic thyroid cancer.	09/03/2000	Genzyme Europe B.V.
TRACLEER	bosentan monohydrate	Treatment of **pulmonary arterial hypertension** (**PAH**) to improve exercise capacity and symptoms in patients with WHO functional class III. Efficacy has been shown in : - **primary (idiopathic and 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. To reduce the number of new digital ulcers in patients with **systemic sclerosis** and ongoing digital ulcer disease.	15/05/2002	Actelion Registration Ltd
TRISENOX	arsenic trioxide	Indicated for induction of remission, and consolidation in adult patients with: • Newly diagnosed low-to-intermediate risk **acute promyelocytic leukaemia** (**APL**) (white blood cell count, ≤ 10 x 103/µl) 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. The response rate of other acute myelogenous leukaemia subtypes to arsenic trioxide has not been examined.	05/03/2002	Teva Pharma B.V.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
TRUXIMA	rituximab	Treatment of previously untreated patients with stage III-IV **follicular lymphoma** in combination with chemotherapy. Truxima maintenance therapy is indicated for the treatment of follicular lymphoma patients responding to induction therapy. 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. 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 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. 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**).	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	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. 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.	12/05/2016	Actelion Registration Ltd
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	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. 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. 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. 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.	26/04/2004	Janssen-Cilag International N.V.
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 Pharma AG
VFEND	voriconazole	In adults and children aged 2 years and above as follows: - treatment of **invasive aspergillosis**. - treatment of **serious fungal infections** caused by ***Scedosporium spp.*** and ***Fusarium spp.*** 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 Ltd
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 ***Scedosporium spp.*** and ***Fusarium spp.*** Voriconazole should be administered primarily to patients with progressive, possibly life – threatening infections.	27/05/2015	Hospira UK Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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
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 Pharmaceutical Contracts Ltd
XELODA	capecitabine	First-line treatment of advanced **gastric cancer** in combination with a platinum-based regimen	02/02/2001	Roche Registration Ltd
XYREM	sodium oxybate	Treatment of **narcolepsy with cataplexy** in adult patients.	13/10/2005	UCB Pharma Ltd
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 ≤ 0.5 x 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	Actelion Registration Ltd
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.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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
ZYDELIG	idelalisib	In combination with rituximab, treatment of adult patients with **chronic lymphocytic leukaemia** (**CLL**): - 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. As monotherapy, treatment of adult patients with **follicular lymphoma (FL)** that is refractory to two prior lines of treatment.	18/09/2014	Gilead Sciences International Ltd.

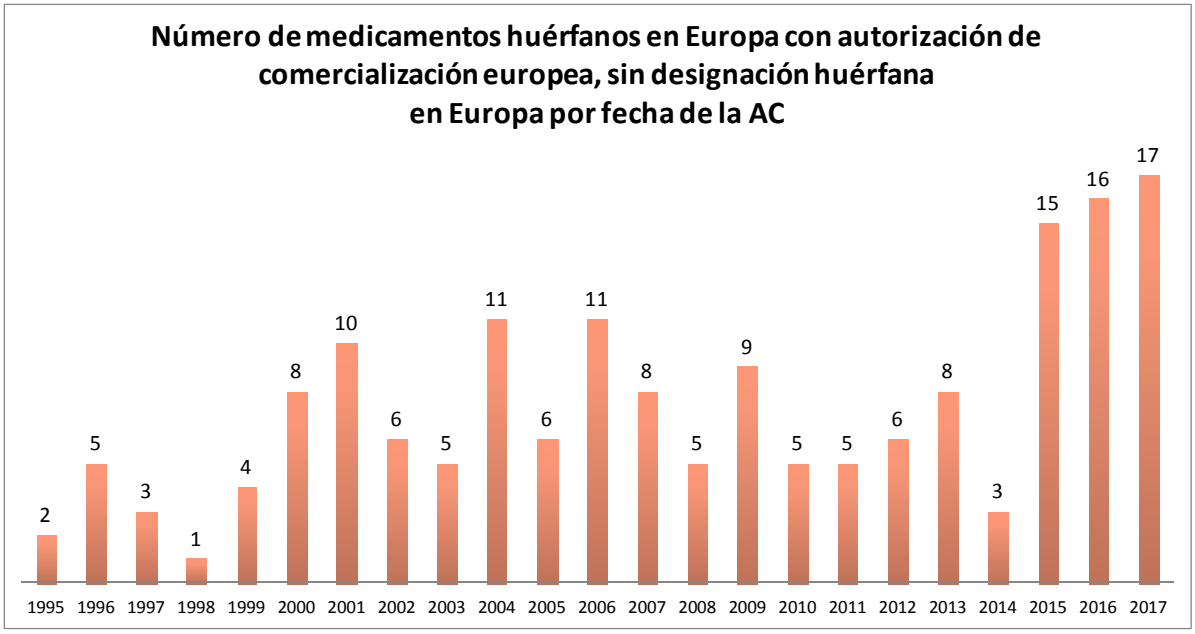
Clasificación por fecha de la AC en orden decreciente

2017
AFSTYLA
AMGEVITA
BLITZIMA
CUPRIOR
ELMIRON
ERELZI
IMRALDI
NITISINONE MDK
REFIXIA
REVLIMID
RITEMVIA
RITUZENA
RIXATHON
RIXIMYO
TADALAFIL GENERICS
TRUXIMA
UCEDANE
YARGESA
2016
ARMISARTE
BORTEZOMIB HOSPIRA
BORTEZOMIB SUN
CABOMETYX
EMPLICITI
IBLIAS
KISPLYX
KOVALTRY
MYSILDECARD
NEOFORDEX
NORDIMET
ONCASPAR
PEMETREXED ACCORD
PEMETREXED FRESENIUS KABI
SPECTRILA
UPTRAVI
2015
BORTEZOMIB ACCORD
DOCETAXEL HOSPIRA
ELOCTA
KEYTRUDA
OBIZUR
OPDIVO
ORKAMBI
PEMETREXED HOSPIRA
PEMETREXED LILLY
PEMETREXED MEDAC
PEMETREXED SANDOZ
QUINSAIR
REPATHA

RESPREEZA
VORICONAZOLE HOSPIRA
2014
BEMFOLA
RIXUBIS
ZYDELIG
2013
ERIVEDGE
GRASTOFIL
HYQVIA
LOJUXTA
NOVOEIGHT
OVALEAP
STAYVEER
VONCENTO
2012
CAPRELSA
COLOBREATHE
INLYTA
JAKAVI
NOVOTHIRTEEN
PIXUVRI
2011
BUCCOLAM
CINRYZE
EURARTESIM
HIZENTRA
TEYSUNO
2010
NIVESTIM
OZURDEX
REVOLADE
RUCONEST
VOTRIENT
2009
AFINITOR
FILGRASTIM HEXAL
ILARIS
IXIARO
ROACTEMRA
SIMPONI
VEDROP
ZARZIO
ZUTECTRA
2008
ABRAXANE
ADCIRCA
PRIVIGEN
RATIOGRASTIM
TEVAGRASTIM

2007
ATRIANCE
CYSTADANE
DIACOMIT
ELAPRASE
FLEBOGAMMA DIF
GLIOLAN
INCRELEX
ORENCIA
YONDELIS
2006
ATRYN
KIOVIG
EVOLTRA
EXJADE
NEXAVAR
OMNITROPE
MYOZYME
NAGLAZYME
SAVENE
SPRYCEL
SUTENT
2005
AVASTIN
NOXAFIL
ORFADIN
REVATIO
TARCEVA
XYREM
2004
ADVATE
ALIMTA
DUKORAL
ERBITUX
LITAK
LYSODREN
PEDEA
VELCADE
WILZIN
XAGRID
ZEVALIN
2003
ALDURAZYME
BUSILVEX
CARBAGLU
HUMIRA
VENTAVIS
2002
KINERET
SOMAVERT

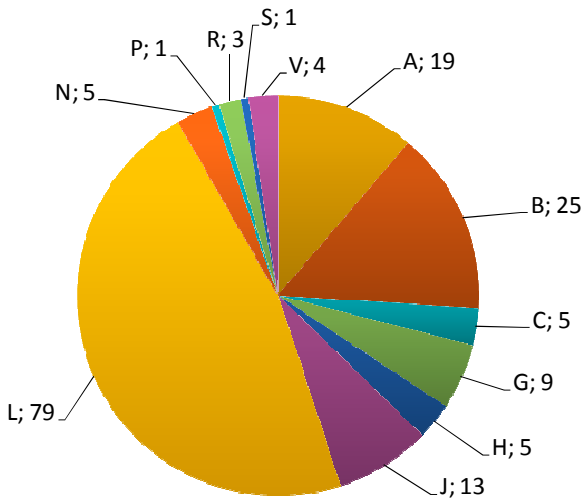
TRACLEER
TRISENOX
VFEND
ZAVESCA
2001
CANCIDAS
CEPROTIN
DEPOCYTE
FABRAZYME
GLIVEC
INOMAX
NONAFACT
REPLAGAL
TARGRETIN
XELODA
2000
ENBREL
HELIXATE NEXGEN
HERCEPTIN
INTRONA
KEPPRA
KOGENATE BAYER
PANRETIN
THYROGEN
1999
AMMONAPS
FERRIPROX
REFACTO AF
TEMODAL
1998
MABTHERA
1997
BENEFIX
CEREZYME
CYSTAGON
1996
CAELYX
HYCAMTIN
NOVOSEVEN
PUREGON
RILUTEK
1995
GONAL-F
TAXOTERE


Número de medicamentos huérfanos en Europa con autorización de comercialización europea, sin designación huérfana en Europa por fecha de la AC
2
5
3
1
4
8
10
6
5
11
6
11
8
5
9
5
5
6
8
3
15
16
17
1995
1996
1997
1998
1999
2000
2001
2002
2003
2004
2005
2006
2007
2008
2009
2010
2011
2012
2013
2014
2015
2016
2017

Clasificación por categoría ATC

A- ALIMENTARY TRACT AND METABOLISM
ALDURAZYME
AMMONAPS
CARBAGLU
CEREZYME
CUPRIOR
CYSTADANE
CYSTAGON
ELAPRASE
FABRAZYME
MYOZYME
NAGLAZYME
NITISINONE MDK
ORFADIN
REPLAGAL
UCEDANE
VEDROP
WILZIN
YARGESA
ZAVESCA
B- BLOOD AND BLOOD FORMING ORGANS
ADVATE
AFSTYLA
ATRYN
BENEFIX
CEPROTIN
CINRYZE
ELOCTA
HELIXATE NEXGEN
IBLIAS
KOGENATE BAYER
KOVALTRY
NONAFACT
NOVOEIGHT
NOVOSEVEN
NOVOTHIRTEEN
OBIZUR
REFACTO AF
REFIXIA
RESPREEZA
REVOLADE
RIXUBIS
RUCONEST
UPTRAVI
VENTAVIS
VONCENTO
C- CARDIOVASCULAR SYSTEM
LOJUXTA
PEDEA
REPATHA
STAYVEER
TRACLEER
G- GENITO URINARY SYSTEM AND SEX HORMONES
ADCIRCA
BEMFOLA
ELMIRON
GONAL-F
MYSILDECARD
OVALEAP
PUREGON
REVATIO
TADALAFIL GENERICS
H- SYSTEMIC HORMONAL PREPARATIONS, EXCL, SEX HORMONES AND INSULINS
INCRELEX
NEOFORDEX
OMNITROPE
SOMAVERT
THYROGEN
J- GENERAL ANTIINFECTIVES FOR SYSTEMIC USE
CANCIDAS
DUKORAL
FLEBOGAMMA DIF
HIZENTRA
HYQVIA
IXIARO
KIOVIG
NOXAFIL
PRIVIGEN
QUINSAIR
VFEND
VORICONAZOLE HOSPIRA
ZUTECTRA
L- ANTINEOPLASTIC AND IMMUNOMODULATING AGENTS
ABRAXANE
AFINITOR
ALIMTA
AMGEVITA
ARMISARTE
ATRIANCE
AVASTIN
BLITZIMA
BORTEZOMIB ACCORD
BORTEZOMIB HOSPIRA
BORTEZOMIB SUN
BUSILVEX
CABOMETYX
CAELYX
CAPRELSA
DEPOCYTE
DOCETAXEL HOSPIRA UK LTD
EMPLICITI
ENBREL
ERBITUX
ERELZI
ERIVEDGE
EVOLTRA
FILGRASTIM HEXAL
GLIOLAN
GLIVEC
GRASTOFIL
HERCEPTIN
HUMIRA
HYCAMTIN
ILARIS
IMRALDI
INLYTA
INTRONA
JAKAVI
KEYTRUDA
KINERET
KISPLYX
LITAK
LYSODREN
MABTHERA
NEXAVAR
NIVESTIM
NORDIMET
ONCASPAR
OPDIVO
ORENCIA
PANRETIN
PEMETREXED ACCORD
PEMETREXED FRESENIUS KABI
PEMETREXED HOSPIRA
PEMETREXED LILLY
PEMETREXED MEDAC
PEMETREXED SANDOZ
PIXUVRI
RATIOGRASTIM
REVLIMID
RITEMVIA
RITUZENA
RIXATHON
RIXIMYO
ROACTEMRA
SIMPONI
SPECTRILA
SPRYCEL
SUTENT
TARCEVA
TARGRETIN
TAXOTERE
TEMODAL
TEVAGRASTIM
TEYSUNO
TRISENOX
TRUXIMA
VELCADE
VOTRIENT
XAGRID
XELODA
YONDELIS
ZARZIO
ZYDELIG
N- NERVOUS SYSTEM
BUCCOLAM
DIACOMIT
KEPPRA
RILUTEK
XYREM
P- ANTIPARASITIC PRODUCTS, INSECTICIDES AND REPELLENTS
EURARTESIM
R- RESPIRATORY SYSTEM
COLOBREATHE
INOMAX
ORKAMBI
S- SENSORY ORGANS
OZURDEX
V- VARIOUS
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 LTD
HUMIRA
ACCORD HEALTHCARE LTD
BORTEZOMIB ACCORD
PEMETREXED ACCORD
ACTAVIS GROUP PTC EHF
ARMISARTE
ACTELION REGISTRATION LTD
TRACLEER
UPTRAVI
ZAVESCA
AEGERION PHARMACEUTICALS SAS
LOJUXTA
ALLERGAN PHARMACEUTICALS IRELAND
OZURDEX
AMGEN EUROPE BV
AMGEVITA
REPATHA
APOTEX EUROPE B.V.
FERRIPROX
GRASTOFIL
APTALIS PHARMA SAS
QUINSAIR
ASTRAZENECA AB
CAPRELSA
AVENTIS PHARMA S.A.
RILUTEK
TAXOTERE
BAXALTA INNOVATIONS GMBH
HYQVIA
OBIZUR
ONCASPAR
RIXUBIS
BAXTER AG
ADVATE
CEPROTIN
KIOVIG
BAYER PHARMA AG
HELIXATE NEXGEN
IBLIAS
KOGENATE BAYER
KOVALTRY
NEXAVAR
VENTAVIS
BENE- ARZNEIMITTEL GMBH
ELMIRON
BIOCODEX
DIACOMIT
BIOGEN IDEC LTD
ELOCTA
BIOMARIN EUROPE LTD
NAGLAZYME
BIOTEST PHARMA GMBH
ZUTECTRA
BRISTOL-MYERS SQUIBB PHARMA EEIG
EMPLICITI
OPDIVO
ORENCIA
SPRYCEL

CELGENE EUROPE LTD
ABRAXANE
CELLTRION HEALTHCARE HUNGARY KFT.
BLITZIMA
RITEMVIA
RITUZENA
TRUXIMA
CLINIGEN HEALTHCARE LTD
SAVENE
CRUCELL SWEDEN AB
DUKORAL
CSL BEHRING GMBH
AFSTYLA
HIZENTRA
PRIVIGEN
RESPREEZA
VONCENTO
CTI LIFE SCIENCES LTD
PIXUVRI
EISAI LTD
KISPLYX
PANRETIN
TARGRETIN
ELI LILLY NEDERLAND B.V.
ADCIRCA
ALIMTA
PEMETREXED LILLY
FINOX BIOTECH AG
BEMFOLA
FOREST LABORATORIES UK LTD
COLOBREATHE
FRESENIUS KABI ONCOLOGY PLC
PEMETREXED FRESENIUS
GENZYME EUROPE B.V.
ALDURAZYME
CEREZYME
EVOLTRA
FABRAZYME
MYOZYME
THYROGEN
GILEAD SCIENCES INTERNATIONAL LTD
ZYDELIG
GMP-ORPHAN SA
CUPRIOR
GTC BIOTHERAPEUTICS UK LIMITED
ATRYN
HEXAL AG
FILGRASTIM HEXAL
HOSPIRA UK LTD
BORTEZOMIB HOSPIRA
DOCETAXEL HOSPIRA UK LTD
PEMETREXED HOSPIRA
NIVESTIM
VORICONAZOLE HOSPIRA
INSTITUTO GRIFOLS S.A.
FLEBOGAMMA DIF
IPSEN PHARMA
CABOMETYX
INCRELEX

JANSSEN-CILAG INTERNATIONAL NV
CAELYX
SIMPONI
VELCADE
JENSON PHARMACEUTICALS SERVICES LIMITED
YARGESA
LABORATOIRES CTRS
NEOFORDEX
LABORATOIRE HRA PHARMA
LYSODREN
LINDE HEALTHCARE AB
INOMAX
LIPOMED GMBH
LITAK
LUCANE PHARMA
UCEDANE
MARKLAS NEDERLAND BV
STAYVEER
MEDAC GMBH
GLIOLAN
PEMETREXED MEDAC
SPECTRILA
MENDELIKABS EUROPE LTD
NITISINONE MDK
MERCK KGAA
ERBITUX
MERCK SERONO EUROPE LTD
GONAL-F
MERCK SHARP & DOHME LTD
CANCIDAS
INTRONA
KEYTRUDA
NOXAFIL
PUREGON
TEMODAL
MYLAN SAS
MYSILDECARD
TADALAFIL GENERICS
NORDIC GROUP BV
NORDIMET
TEYSUNO
NOVARTIS EUROPHARM LTD
AFINITOR
ATRIANCE
EXJADE
GLIVEC
HYCAMTIN
ILARIS
JAKAVI
REVOLADE
VOTRIENT
NOVO NORDISK A/S
NOVOEIGHT
NOVOSEVEN
NOVOTHIRTEEN
REFIXIA
ORPHAN EUROPE S.A.R.L.
CARBAGLU
CYSTADANE

CYSTAGON
PEDEA
VEDROP
WILZIN
PACIRA LIMITED
DEPOCYTE
PFIZER LTD
BENEFIX
ENBREL
INLYTA
REFACTO AF
REVATIO
MAVERT
SUTENT
VFEND
PHARMA MARS.A.
YONDELIS
PHARMING GROUP N.V.
RUCONEST
PIERRE FABRE MÉDICAMENTS
BUSILVEX
RATIOPHARM GMBH
RATIOGRASTIM
ROCHE REGISTRATION LTD
AVASTIN
ERIVEDGE
HERCEPTIN
MABTHERA
ROACTEMRA
TARCEVA
XELODA
SAMSUNG BIOEPIS UK LIMITED (SBUK)
IMRALDI
SANDOZ GMBH
ERELZI
OMNITROPE
PEMETREXED SANDOZ
RIXATHON
RIXIMYO
ZARZIO
SANQUIN
NONAFACT
SHIRE PHARMACEUTICAL CONTRACTS LTD
XAGRID
SHIRE HUMAN GENETIC THERAPIES AB
ELAPRASE
REPLAGAL
SIGMA-TAU INDUSTRIE FARMACEUTICHE RIUNITE S.P.A
EURARTESIM
SPECTRUM PHARMACEUTICALS B.V.
ZEVALIN
SUN Pharmaceutical Industries (Europe) B.V.
BORTEZOMIB SUN
SWEDISH ORPHAN BIOVITRUM INTERNATIONAL AB
AMMONAPS
KINERET
ORFADIN

TEVA GMBH
TEVAGRASTIM
TEVA PHARMA BV
OVALEAP
TRISENOX

UCB PHARMA LTD
XYREM
UCB PHARMA SA
KEPPRA
VALNEVA AUSTRIA GMBH

IXIARO
VERTEX PHARMACEUTICALS (EUROPE) LTD
ORKAMBI

VIROPHARMA SPRL
BUCCOLAM
CINRYZE

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

La forma adecuada par citar este documento es la siguiente :
« Listados de medicamentos para enfermedades raras en Europa », Informes Periódicos de Orphanet, Serie *Medicamentos Huérfanos*, Octubre 2017

Los informes de Orphanet forman parte de la 677024 RD-ACTION Joint Action, que ha recibido financiación del Programa de Salud de la Unión Europea.