



# Relatórios Orphanet

Coleção Medicamentos Órfãos

Abril 2016

## Lista de medicamentos para doenças raras na Europa\*

\* Autorização de comercialização da Comunidade Europeia no âmbito do procedimento centralizado

[www.orpha.net](http://www.orpha.net)

[www.orphadata.org](http://www.orphadata.org)



# Índice Geral

## PARTE 1:

<b>Lista de medicamentos órfãos na Europa, com designação órfã europeia e autorização europeia de introdução no mercado*</b>	<b>3</b>
<i>Índice</i>	3
<i>Metodologia</i>	3
<i>Classificação pelo nome comercial</i>	5
Anexo 1: Medicamentos órfãos retirados pelo Registo Comunitário Europeu de medicamentos órfãos	16
Anexo 2: Medicamentos órfãos retirados de utilização na União Europeia	21
<i>Classificação por data de AIM por ordem decrescente</i>	22
<i>Classificação por categoria ATC</i>	23
<i>Classificação pelo detentor da AIM</i>	24

## PARTE 2 :

<b>Lista de medicamentos destinados a doenças raras, na Europa, com autorização europeia de introdução no mercado* sem designação de medicamento órfão na Europa</b>	<b>25</b>
<i>Índice</i>	25
<i>Metodologia</i>	25
<i>Classificação pelo nome comercial</i>	26
<i>Classificação por data de AIM por ordem decrescente</i>	48
<i>Classificação por categoria ATC</i>	49
<i>Classificação pelo detentor da AIM</i>	51

Para quaisquer comentários ou perguntas, por favor contacte: [contact.orphanet@inserm.fr](mailto:contact.orphanet@inserm.fr)

## PARTE 1:

# Lista de medicamentos órfãos na Europa, com designação órfã europeia e autorização europeia de introdução no mercado\*

## Índice

<b>Lista de medicamentos órfãos na Europa, com designação órfã europeia e autorização europeia de introdução no mercado*</b>	<b>3</b>
<i>Metodologia</i>	3
<i>Classificação pelo nome comercial</i>	5
Anexo 1: Medicamentos órfãos retirados pelo Registo Comunitário Europeu de medicamentos órfãos	16
Anexo 2: Medicamentos órfãos retirados de utilização na União Europeia	21
<i>Classificação por data de AIM por ordem decrescente</i>	22
<i>Classificação por categoria ATC</i>	23
<i>Classificação pelo detentor da AIM</i>	24

## Metodologia

Este documento disponibiliza a lista de todos os medicamentos órfãos que receberam uma autorização de introdução no mercado (AIM) na Europa na data indicada no documento. Estes medicamentos podem agora estar acessíveis em alguns, mas não necessariamente em todos, os países europeus. Na realidade, a acessibilidade a certos medicamentos órfãos num determinado país depende da estratégia do laboratório e da decisão tomada pelas autoridades de saúde nacionais relativas ao reembolso.

Os medicamentos órfãos na Europa são aqueles que obtiveram uma designação europeia de medicamento órfão (de acordo com o Regulamento (EC) nº141/2000), que posteriormente lhes foi concedida uma autorização do mercado europeu e, se for o caso, uma avaliação positiva de benefício significativo.

Esta lista de medicamentos órfãos na Europa, com designação órfão e autorizações europeias de introdução no mercado, é determinada pela referência cruzada da lista de medicamentos que obteve uma designação de medicamento órfão (<http://ec.europa.eu/health/documents/community-register/html/alfregister.htm>)

com a lista de medicamentos que obteve uma autorização de introdução no mercado (<http://ec.europa.eu/health/documents/community-register/html/alfregister.htm>). Ambas as listas estão disponíveis na Direcção-Geral da Saúde e Segurança dos Alimentos (DG SANTE) da Comissão Europeia.

A primeira classificação por nome comercial fornece o nome da substância ativa, a indicação da autorização de introdução no mercado (AIM), a data da AIM e o seu detentor.

- Tabela de medicamentos órfãos removidos/retirados pelo Registo Comunitário dos medicamentos órfãos (ver anexo 1 - Medicamentos órfãos retirados pelo Registo Comunitário Europeu de medicamentos órfãos ; as suas indicações estão detalhadas na parte II, « Lista de medicamentos destinados a doenças raras na Europa com autorização de introdução no mercado europeu sem designação de medicamento órfão na Europa ».

- Tabela de medicamentos órfãos retirados de utilização na união europeia (ver anexo 2 - Medicamentos órfãos retirados de utilização na União

\*Autorização de comercialização da Comunidade Europeia no âmbito do procedimento centralizado

Europeia). Mais informações em [www.ema.europa.eu](http://www.ema.europa.eu).

Três listas adicionais propõem a classificação por:


- Data da AIM por ordem decrescente ;
- Categoria ATC;
- Detentor da AIM.

Para cada lista, os nomes comerciais são apresentados por ordem alfabética.

Pode encontrar informações adicionais sobre cada medicamento no separador « medicamentos órfãos »

no website da Orphanet: [www.orpha.net](http://www.orpha.net), ou no site EMA (Agência Europeia de Medicamentos) [www.ema.europa.eu](http://www.ema.europa.eu).

A lista da EMA abrange todos os medicamentos com autorização de introdução no mercado e não apenas medicamentos órfãos.

Os medicamentos órfãos aos quais foi concedida a designação europeia de medicamento órfão são indicados pelo logotipo 



Informações oficiais e atualizadas sobre medicamentos órfãos estão disponíveis no registo comunitário de medicamentos órfãos para uso humano:

<http://ec.europa.eu/health/documents/community-register/html/alforphreg.htm>

## Classificação pelo nome 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+ <b>Hodgkin lymphoma (HL)</b> : -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 relapsed or refractory systemic <b>anaplastic large cell lymphoma (sALCL)</b> .	25/10/2012	Takeda Pharma A/S
ADEMPAS	riociguat	Treatment of adult patients with WHO Functional Class (FC) II to III with inoperable <b>Chronic thromboembolic pulmonary hypertension (CTEPH)</b> , 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 <b>pulmonary arterial hypertension (PAH)</b> 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
ARZERRA	ofatumumab	In combination with chlorambucil or bendamustine, for the treatment of patients with <b>chronic lymphocytic leukaemia</b> who have not received prior therapy and who are not eligible for fludarabine-based therapy. Treatment of refractory <b>chronic lymphocytic leukaemia</b> in patients who are refractory to fludarabine and alemtuzumab.	19/04/2010	Novartis Europharm Ltd
ATRIANCE	nelarabine	Treatment of patients with <b>T-cell acute lymphoblastic leukaemia (T-ALL)</b> and <b>T-cell lymphoblastic lymphoma (T-LBL)</b> 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
BLINCYTO	blinatumomab	Treatment of adults with Philadelphia chromosome negative relapsed or refractory <b>B - precursor acute lymphoblastic leukaemia (ALL)</b> .	23/11/2015	Amgen Europe B.V.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
BOSULIF	bosutinib	Treatment of adult patients with chronic phase (CP), accelerated phase (AP), and blast phase (BP) <b>Philadelphia chromosome positive chronic myelogenous leukaemia (Ph+ CML)</b> 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
BRONCHITOL	mannitol	Treatment of <b>cystic fibrosis (CF)</b> 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 <b>hyperammonaemia</b> due to - <b>N-acetylglutamate synthase primary deficiency</b> , - <b>isovaleric acidaemia</b> , - <b>methymalonic acidaemia</b> , - <b>propionic acidaemia</b> .	01/06/2011	Orphan Europe S.a.r.l.
CAYSTON	aztreonam	Suppressive therapy of chronic pulmonary infections due to <i>Pseudomonas aeruginosa</i> in patients with <b>cystic fibrosis (CF)</b> aged 6 years and older.	21/09/2009	Gilead Sciences International Ltd
CEPLENE	histamine dihydrochloride	Maintenance therapy for adult patients with <b>acute myeloid leukaemia</b> 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 <b>Gaucher disease type 1 (GD1)</b> , who are CYP2D6 poor metabolisers (PMs), intermediate metabolisers (IMs) or extensive metabolisers (EMs)	19/01/2015	Genzyme Europe B.V.
COAGADEX	human coagulation factor X	Treatment and prophylaxis of bleeding episodes and for perioperative management in patients with <b>hereditary factor X deficiency</b> .	16/03/2016	Bio Products Laboratory Ltd
COMETRIQ	cabozantinib	Treatment of adult patients with progressive, unresectable locally advanced or metastatic <b>medullary thyroid carcinoma</b> . 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: - <b>invasive aspergillosis</b> - <b>mucormycosis</b> in patients for whom amphotericin B is inappropriate	15/10/2015	Basilea Medical Ltd
CYSTADANE	betaine anhydrous	Adjunctive treatment of <b>homocystinuria</b> , involving deficiencies or defects in cystathionine beta- synthase (CBS), 5,10-methylene-tetrahydrofolate reductase (MTHFR), cobalamin cofactor metabolism (cbf). 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.

NEW

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
DACOGEN	decitabine	Treatment of adult patients aged 65 years and above with newly diagnosed de novo or secondary <b>acute myeloid leukaemia (AML)</b> , according to the World Health Organization (WHO) classification, who are not candidates for standard induction chemotherapy.	20/09/2012	Janssen-Cilag International N.V.
DEFITELIO	defibrotide	Treatment of <b>severe hepatic veno-occlusive disease (VOD)</b> also known as <b>sinusoidal obstructive syndrome (SOS)</b> 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 <b>pulmonary multi-drugresistant tuberculosis (MDR-TB)</b> 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
DIACOMIT	stiripentol	Used in conjunction with clobazam and valproate as adjunctive therapy of refractory generalized tonic-clonic seizures in patients with <b>severe myoclonic epilepsy in infancy (SMEI, Dravet's syndrome)</b> whose seizures are not adequately controlled with clobazam and valproate.	04/01/2007	Biocodex
ELAPRASE	idursulfase	Long-term treatment of patients with <b>Hunter syndrome (Mucopolysaccharidosis II, MPS II)</b> . Heterozygous females were not studied in the clinical trials.	08/01/2007	Shire Human Genetic Therapies AB
ESBRIET	pirfenidone	In adults for the treatment of mild to moderate <b>Idiopathic Pulmonary Fibrosis (IPF)</b> .	28/02/2011	Roche Registration Ltd
EVOLTRA	clofarabine	Treatment of <b>acute lymphoblastic leukaemia(ALL)</b> 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.
FARYDAK	panobinostat lactate anhydrous	In combination with bortezomib and dexamethasone, for the treatment of adult patients with relapsed and/or refractory <b>multiple myeloma</b> 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 <b>hereditary angioedema (HAE)</b> in adults (with C1-esterase-inhibitor deficiency).	11/07/2008	Shire Orphan Therapies GmbH
FIRDAPSE (ex-ZENAS)	amifampridine	Symptomatic treatment of <b>Lambert-Eaton myasthenic syndrome (LEMS)</b> in adults.	23/12/2009	BioMarin Europe Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
GAZYVARO	obinutuzumab	In combination with chlorambucil, treatment of adult patients with previously untreated <b>chronic lymphocytic leukaemia (CLL)</b> and with comorbidities making them unsuitable for full-dose fludarabine based therapy.	23/07/2014	Roche Registration Ltd
GLIOLAN	5-aminolevulinic acid hydrochloride	In adult patients for visualisation of malignant tissue during surgery for <b>malignant glioma</b> (World Health Organization grade III and IV).	07/09/2007	Medac GmbH
GLYBERA	alipogene tiparovec	For adult patients diagnosed with <b>familial lipoprotein lipase deficiency (LPLD)</b> 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.
GRANUPAS (ex-PARA-AMINOSALICYLIC ACID LUCANE)	para-aminosalicylic acid	Indicated for use as part of an appropriate combination regimen for <b>multi-drug resistant tuberculosis</b> 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 <b>Non-24-Hour Sleep-Wake Disorder (Non-24)</b> 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 <b>limbal stem cell deficiency</b> (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 <sup>2</sup> 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 <b>chronic myeloid leukaemia (CML)</b> 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 <b>Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL)</b> 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



TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
IMBRUVICA	ibrutinib	Treatment of adult patients with relapsed or refractory <b>mantle cell lymphoma (MCL)</b> . Treatment of adult patients with <b>chronic lymphocytic leukaemia (CLL)</b> who have received at least one prior therapy, or in first line in the presence of 17p deletion or TP53 mutation in patients unsuitable for chemo-immunotherapy. Treatment of adult patients with <b>Waldenström's macroglobulinaemia (WM)</b> who have received at least one prior therapy, or in first line treatment for patients unsuitable for chemo-immunotherapy.	21/10/2014	Janssen-Cilag International N.V.
IMNOVID (ex POMALIDO MIDE CELGENE)	pomalidomide	In combination with dexamethasone, in the treatment of adult patients with relapsed and refractory <b>multiple myeloma</b> 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
INCRELEX	mecasermin	For the long-term treatment of <b>growth failure</b> in children and adolescents from 2 to 18 years with <b>severe primary insulin-like growth factor-1 deficiency (Primary IGFD)</b> . Severe Primary IGFD is defined by: - height standard deviation score $\leq$ -3.0 and - basal IGF-1 levels below the 2.5 <sup>th</sup> 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
INOVELON	rufinamide	Adjunctive therapy in the treatment of seizures associated with <b>Lennox-Gastaut syndrome</b> in patients aged 4 years and older.	16/01/2007	Eisai Ltd
KALYDECO	ivacaftor	Treatment of patients with <b>cystic fibrosis (CF)</b> aged 6 years and older and weighing 25kg or more who have one of the following gating (class III) mutations in the CFTR gene: <i>G551D</i> , <i>G1244E</i> , <i>G1349D</i> , <i>G178R</i> , <i>G551S</i> , <i>S1251N</i> , <i>S1255P</i> , <i>S549N</i> or <i>S549R</i> . Treatment of patients with cystic fibrosis (CF) aged 18 years and older who have an <i>R117H</i> 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 <b>lysosomal acid lipase (LAL) deficiency</b>	28/08/2015	Synageva BioPharma Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
KETOCONAZOLE HRA	ketoconazole	Treatment of <b>endogenous Cushing's syndrome</b> in adults and adolescents above the age of 12 years.	19/11/2014	Laboratoire HRA Pharma
KOLBAM (ex CHOLIC ACID FGK)	cholic acid	Treatment of <b>inborn errors in primary bile acid synthesis</b> due to <b>Sterol 27-hydroxylase</b> (presenting as cerebrotendinous xanthomatosis, CTX) <b>deficiency</b> , <b>2- (or <math>\alpha</math>-) methylacyl-CoA racemase (AMACR) deficiency</b> or <b>Cholesterol 7<math>\alpha</math>-hydroxylase (CYP7A1) deficiency</b> in infants, children and adolescents aged 1 month to 18 years and adults.	08/04/2014	Retrophin Europe Ltd
KUVAN	sapropterin dihydrochloride	Treatment of <b>hyperphenylalaninaemia (HPA)</b> in adult and paediatric patients of all ages with <b>phenylketonuria (PKU)</b> who have been shown to be responsive to such treatment. Treatment of <b>hyperphenylalaninaemia (HPA)</b> in adult and paediatric patients with <b>tetrahydrobiopterin (BH4) deficiency</b> who have been shown to be responsive to such treatment.	02/12/2008	Merck Serono Europe Ltd
KYPROLIS	carfilzomib	In combination with lenalidomide and dexamethasone is indicated for the treatment of adult patients with <b>multiple myeloma</b> who have received at least one prior therapy.	19/11/2015	Amgen Europe B.V.
LENVIMA	lenvatinib	Treatment of adult patients with progressive, locally advanced or metastatic, <b>differentiated</b> (papillary/follicular/Hürthle cell) <b>thyroid carcinoma (DTC)</b> refractory to radioactive iodine (RAI).	28/05/2015	Eisai Ltd
LYNPARZA	olaparib	Monotherapy for the maintenance treatment of adult patients with <b>platinum-sensitive relapsed BRCA-mutated</b> (germline and/or somatic) <b>high grade serous epithelial ovarian, fallopian tube, or primary peritoneal cancer</b> 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 <b>osteosarcoma</b> 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 <b>lymphoma</b> and <b>multiple myeloma</b> whose cells mobilise poorly.	31/07/2009	Genzyme Europe B.V.
NEOFORDEX	dexamethasone	Indicated in adults for the treatment of symptomatic <b>multiple myeloma</b> in combination with other medicinal products.	16/03/2016	Laboratoires CTRS



TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
NEXAVAR	sorafenib tosylate	Treatment of <b>hepatocellular carcinoma</b> Treatment of patients with advanced <b>renal cell carcinoma</b> who have failed prior interferon-alpha or interleukin-2 based therapy or are considered unsuitable for such therapy. Treatment of patients with progressive, locally advanced or metastatic, <b>differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma</b> , refractory to radioactive iodine.	19/07/2006	Bayer Pharma AG
NEXOBRID	concentrate of proteolytic enzymes enriched in bromelain	Removal of eschar in adults with <b>deep partial- and full-thickness thermal burns</b> .	18/12/2012	Mediwound Germany GmbH
NPLATE	romiplostim	Indicated for adult <b>chronic immune (idiopathic) thrombocytopenic purpura (ITP)</b> patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins)	04/02/2009	Amgen Europe B.V.
OFEV	nintedanib	Treatment in adults of <b>Idiopathic Pulmonary Fibrosis (IPF)</b> .	15/01/2015	Boehringer Ingelheim International GmbH
OPSUMIT	macitentan	Used as monotherapy or in combination, for the long-term treatment of <b>pulmonary arterial hypertension (PAH)</b> 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 <b>inborn errors in primary bile acid synthesis due to 3beta-Hydroxy-delta5-C27- steroid oxidoreductase deficiency or delta4-3-Oxosteroid-5beta-reductase deficiency</b> in infants, children and adolescents aged 1 month to 18 years and adults.	12/09/2013	Laboratoires CTRS
PEYONA (ex-NYMUSA)	caffeine citrate	Treatment of <b>primary apnea</b> of premature newborns.	02/07/2009	Chiesi Farmaceutici SpA
PLENADREN	hydrocortisone	Treatment of <b>adrenal insufficiency</b> in adults.	03/11/2011	ViroPharmaSPRL

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 $\geq 2$ months of age with <b>urea cycle disorders (UCDs)</b> including: <b>deficiencies of carbamoyl phosphate-synthase-I (CPS)</b> - <b>ornithine carbamoyltransferase (OTC)</b> - <b>argininosuccinate synthetase (ASS)</b> , - <b>argininosuccinate lyase (ASL)</b> - <b>arginase I (ARG)</b> - <b>ornithine translocase deficiency</b> <b>hyperornithinaemia</b> <b>-hyperammonaemia homocitrullinuria syndrome (HHH)</b> 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 adult patients with <b>Short Bowel Syndrome</b> . Patients should be stable following a period of intestinal adaptation after surgery.	30/08/2012	NPS Pharma Holdings Ltd
REVLIMID	lenalidomide	Treatment of adult patients with previously untreated <b>multiple myeloma</b> 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. Treatment of patients with transfusion-dependent anaemia due to low-or intermediate-1-risk <b>myelodysplastic syndromes</b> associated with an isolated deletion 5q cytogenetic abnormality when other therapeutic options are insufficient or inadequate.	14/06/2007	Celgene Europe Ltd
SAVENE	dexrazoxane	Treatment of <b>anthracycline extravasation</b> in adults.	28/07/2006	Clinigen Healthcare Ltd
SCENESSE	afamelanotide	Prevention of phototoxicity in adult patients with <b>erythropoietic protoporphyria (EPP)</b>	22/12/2014	Clinuvel UK Ltd
SIGNIFOR	pasireotide	Treatment of adult patients with <b>Cushing's disease</b> for whom surgery is not an option or for whom surgery has failed.	24/04/2012	Novartis Europharm Ltd
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 <b>Sickle Cell Syndrome</b> .	29/06/2007	Addmedica

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
SIRTURO	bedaquiline	Used as part of an appropriate combination regimen for <b>pulmonary multidrug-resistant tuberculosis (MDR-TB)</b> 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 : - <b>Paroxysmal nocturnal haemoglobinuria (PNH)</b> . Evidence of clinical benefit is demonstrated in patients with haemolysis with clinical symptom(s) indicative of high disease activity, regardless of transfusion history. - <b>atypical haemolytic uraemic syndrome (aHUS)</b> .	20/06/2007	Alexion Europe SAS
SPRYCEL	dasatinib	Treatment of adult patients with: - newly diagnosed <b>Philadelphia chromosome positive (Ph+) chronic myelogenous leukaemia (CML)</b> in the chronic phase. - chronic, accelerated or blast phase CML with resistance or intolerance to prior therapy including imatinib mesilate. - <b>Ph+ acute lymphoblastic leukaemia (ALL)</b> and lymphoid blast CML with resistance or intolerance to prior therapy.	20/11/2006	Bristol-Myers SquibbPharma EEIG
STRENSIQ	asfotase alfa	Long-term enzyme replacement therapy in patients with paediatric-onset <b>hypophosphatasia</b> to treat the bone manifestations of the disease.	28/08/2015	Alexion Europe SAS
SYLVANT	siltuximab	Treatment of adult patients with <b>multicentric Castleman's disease (MCD)</b> 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 <b>Philadelphia chromosome positive chronic myelogenous leukaemia (CML)</b> 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 <b>allogeneic or autologous haematopoietic progenitor cell transplantation (HPCT)</b> in <b>haematological diseases</b> 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 (ex THALIDOMIDE PHARMION)	thalidomide	In combination with melphalan and prednisone as first line treatment of patients with untreated <b>multiple myeloma</b> , 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

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
TOBI PODHALER	tobramycin	Suppressive therapy of chronic pulmonary infection due to <i>Pseudomonas aeruginosa</i> in adults and children aged 6 years and older with <b>cystic fibrosis</b> . 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 <b>renal cell carcinoma (RCC)</b> who have at least three of six prognostic risk factors. Treatment of adult patients with relapsed and / or refractory <b>mantle cell lymphoma (MCL)</b> .	19/11/2007	Pfizer Ltd
TRANSLARNA	ataluren	Treatment of <b>Duchenne muscular dystrophy</b> 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
UNTUXIN	dinutuximab	Treatment of high-risk <b>neuroblastoma</b> 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
VIDAZA	azacitidine	Treatment of adult patients who are not eligible for haematopoietic stem cell transplantation with: - intermediate-2 and high-risk <b>myelodysplastic syndromes (MDS)</b> according to the International Prognostic Scoring System (IPSS), - <b>chronic myelomonocytic leukaemia (CMML)</b> with 10-29% marrow blasts without myeloproliferative disorder, - <b>acute myeloid leukaemia (AML)</b> 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 <b>AML</b> with >30% marrow blasts according to the WHO classification.	17/12/2008	Celgene Europe Ltd
VIMIZIM	elosulfase alfa	Treatment of <b>mucopolysaccharidosis, type IVA (Morquio A Syndrome, MPS IVA)</b> in patients of all ages.	28/04/2014	BioMarin Europe Ltd
VOLIBRIS	ambrisentan	Treatment of <b>pulmonary arterial hypertension (PAH)</b> in adult patients of WHO Functional Class (FC) II to III, including use in combination treatment. Efficacy has been shown in <b>idiopathic PAH (IPAH)</b> and in PAH associated with connective tissue disease.	21/04/2008	Glaxo GroupLtd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
VOTUBIA	everolimus	Treatment of adult patients with <b>renal angiomyolipoma</b> associated with <b>tuberous sclerosis complex (TSC)</b> 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 <b>subependymal giant cell astrocytoma (SEGA)</b> 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 <b>type 1 Gaucher disease</b> .	26/08/2010	Shire Pharmaceuticals Ireland Ltd
VYENDAQEL	tafamidis	Treatment of <b>transthyretin amyloidosis</b> in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment.	16/11/2011	Pfizer Ltd
XAGRID	anagrelide hydrochloride	Reduction of elevated platelet counts in at-risk <b>essential-thrombocythaemia (ET)</b> 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 <sup>9</sup> /l or - a history of thrombo-haemorrhagic events.	16/11/2004	Shire Pharmaceutical Contracts Ltd
XALUPRINE (ex-MERCAPTOPURINE NOVA)	mercaptopurine	Treatment of <b>acute lymphoblastic leukaemia (ALL)</b> in adults, adolescents and children.	09/03/2012	Nova Laboratories Ltd
YONDELIS	trabectedin	Treatment of adult patients with advanced <b>soft tissue sarcoma</b> , after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents. Efficacy data are based mainly on <b>liposarcoma</b> and <b>leiomyosarcoma</b> patients. In combination with pegylated liposomal doxorubicin (PLD), treatment of patients with relapsed platinum-sensitive <b>ovarian cancer</b> .	17/09/2007	Pharma MarS.A.
ZAVESCA	miglustat	Treatment of progressive neurological manifestations in adult patients and paediatric patients with <b>Niemann-Pick type C disease</b> .	28/01/2009	Actelion Registration Ltd

## Anexo 1: Medicamentos orfãos retirados pelo Registo Comunitário Europeu de medicamentos orfãos

*Cf. Parte II “Lista de medicamentos destinados a doenças raras, na Europa, com autorização europeia de introdução no mercado sem designação de medicamento órfão na 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 <b>on request of the sponsor</b> . 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 <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine on 14 February 2001.	12/06/2003	12/06/2013
BUSILVEX	busulfan	This product was withdrawn from the Community register of orphan medicinal products <b>at the end of the 10-year period of market exclusivity</b> . 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 <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine for the treatment of n-acetylglutamate synthetase (NAGS) deficiency, on 18 October 2000.	28/01/2003	28/01/2013
CYRAMZA	ramucirumab	This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b> . It was originally designated an orphan medicine on 6 July 2012.	23/12/2014	27/01/2016
ELOCTA	efmoroctocog alfa	This product was withdrawn from the Community Register of designated Orphan Medicinal Products <b>on request of the sponsor</b> . It was originally designated an orphan medicine on 20 September 2010.	23/11/2015	23/11/2015
FABRAZYME	agalsidase beta	This product was withdrawn from the Community register of orphan medicinal products <b>at the end of the period of market exclusivity</b> . It was originally designated an orphan medicine on 8 August 2000.	07/08/2001	07/08/2011

NEW



TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
GLIVEC	imatinib mesilate	<p>This product was withdrawn from the Community register of orphan medicinal products <b>at the end of the 10-year period of market exclusivity</b> for the following condition:</p> <ul style="list-style-type: none"> <li>- Treatment of chronic myeloid leukaemia (it was designated an orphan medicine on 14/02/2001).</li> </ul> <p>It was withdrawn from the Community register of orphan medicinal products on April 2012 <b>on request of the sponsor</b> for the following conditions:</p> <ul style="list-style-type: none"> <li>- Treatment of malignant gastrointestinal stromal tumours (it was designated an orphan medicine on 20/11/2001)</li> <li>- Treatment of dermatofibrosarcoma protuberans (it was designated an orphan medicine on 26/08/2005);</li> <li>- Treatment of acute lymphoblastic leukaemia (it was designated an orphan medicine on 26/08/2005);</li> <li>- Treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome (it was designated an orphan medicine on 28/10/2005)</li> <li>- Treatment of myelodysplastic / myeloproliferative diseases (it was designated an orphan medicine on 23/12/2005)</li> </ul>	<p>12/11/2001</p> <p>27/05/2002</p> <p>18/09/2006</p> <p>18/09/2006</p> <p>01/12/2006</p> <p>01/12/2006</p>	<p>12/11/2011</p> <p>16/04/2012</p>
ILARIS	canakinumab	<p>This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b>. It was originally designated an orphan medicine on 20 March 2007.</p>	27/10/2009	01/12/2010
IXIARO	Purified inactivated Japanese encephalitis SA14-4-2 virus vaccine	<p>This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b>. It was originally designated an orphan medicine on 26 January 2006.</p>	02/04/2009	12/03/2009
JAKAVI	ruxolitinib	<p>This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b>. It was originally designated an orphan medicine for the following conditions:</p> <ul style="list-style-type: none"> <li>-Treatment of polycythaemia vera (19/02/2014)</li> <li>-Treatment of chronic idiopathic myelofibrosis (07/11/2008)</li> <li>-Treatment of myelofibrosis secondary to polycythaemia vera or essential thrombocythaemia (03/04/2009).</li> </ul>	28/08/2012	20/02/2015
JINARC	tolvaptan	<p>This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b>. It was originally designated an orphan medicine on 5 august 2013</p>	29/05/2015	26/03/2015

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
LITAK	cladribine	This product was withdrawn from the Community register of orphan medicinal products <b>at the end of the 10-year period of market exclusivity</b> . 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 <b>at the end of the 10-year period of market exclusivity</b> . 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 <b>at the end of the 10-year period of market exclusivity</b> . 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 <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine on 22 February 2001.	26/01/2006	26/01/2016
NOVOTHIRTEEN	catridecacog	This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b> . 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 <b>on request of the sponsor</b> . 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 <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine on 29 December 2000.	24/02/2005	24/02/2015
ORKAMBI	Lumacaftor / ivacaftor	This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b> . 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 <b>at the end of the 10-year period of market exclusivity</b> . 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 <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine on 9 July 2001.	24/02/2005	24/02/2015

NEW

NEW

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE	
QUINSAIR	levofloxacin	This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b> . 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 <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine on 8 August 2000.	07/08/2001	07/08/2011	
NEW	REVATIO	Sildenafil citrate	This product was withdrawn from the Community register of orphan medicinal products <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine on 17 December 2003.	04/11/2005	04/11/2015
REVOLADE	eltrombopag	This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b> . It was originally designated an orphan medicine on 3 August 2007.	15/03/2010	01/01/2012	
SOMAVERT	pegvisomant	This product was withdrawn from the Community register of orphan medicinal products <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine on 14 February 2001.	15/11/2002	15/11/2012	
NEW	SPECTRILA	asparaginase	This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b> . It was originally designated an orphan medicine on 26 January 2005.	18/01/2016	18/01/2016
SUTENT	sunitinib malate	This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b> . 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 <b>on request of the sponsor</b> 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 <b>at the end of the 10-year period of market exclusivity</b> 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	

TRADENAME	ACTIVE SUBSTANCE	REGULAR STATUS	MARKETING AUTHORIZATION DATE	ORPHAN DESIGNATION WITHDRAWAL DATE
TRISENOX	arsenic trioxide	This product was withdrawn from the Community register of orphan medicinal products <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine on 18 October 2000.	07/03/2002	07/03/2012
VENTAVIS	iloprost	This product was withdrawn from the Community register of orphan medicinal products <b>at the end of the 10-year period of market exclusivity</b> . 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 <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine on 31 July 2001.	18/10/2004	18/10/2014
XYREM	sodium oxybate	This product was withdrawn from the Community Register of designated orphan medicinal products <b>on request of the sponsor</b> . It was originally designated an orphan medicine on 3 February 2003.	18/10/2005	11/01/2010
ZAVESCA	miglustat	This product was withdrawn from the Community register of orphan medicinal products <b>at the end of the 10-year period of market exclusivity</b> . It was originally designated an orphan medicine for the treatment of type 1 Gaucher disease on 18 October 2000.	21/11/2002	21/11/2012

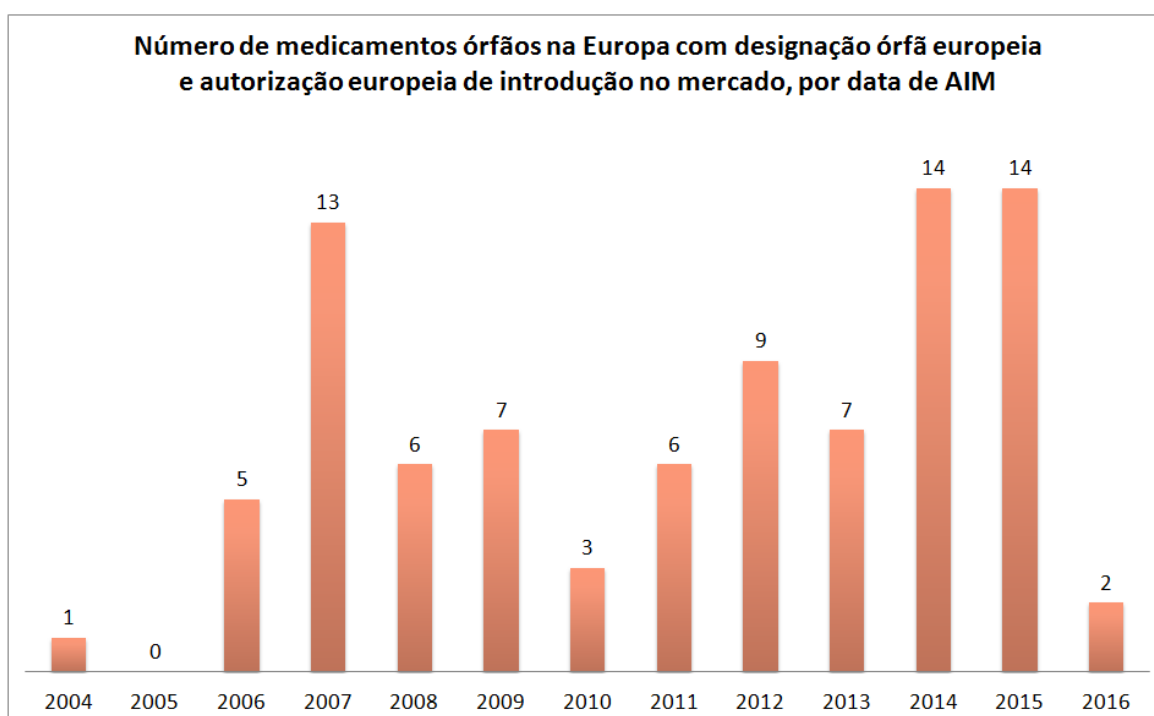
## Anexo 2: Medicamentos orfãos retirados de utilização na União Europeia

Informações adicionais: [www.ema.europa.eu](http://www.ema.europa.eu)

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION (MA) INDICATION	MA DATE / MA HOLDER	MA WITHDRAWN DATE
ONSENAL	celecoxib	Reduction of the number of adenomatous intestinal polyps in <b>familial adenomatous polyposis (FAP)</b> , 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 <b>Barrett's Oesophagus</b> .	25/03/2004 Pinnacle Biologics B.V.	20/04/2012
RILONACEPT REGENERON (ex-ARCALYST)	rilonacept	Treatment of <b>Cryopyrin-Associated Periodic Syndromes (CAPS)</b> with severe symptoms, including <b>Familial Cold Autoinflammatory Syndrome (FCAS)</b> and <b>Muckle-Wells Syndrome (MWS)</b> , 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 <b>pulmonary arterial hypertension</b> 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

## Classificação por data de AIM por ordem decrescente

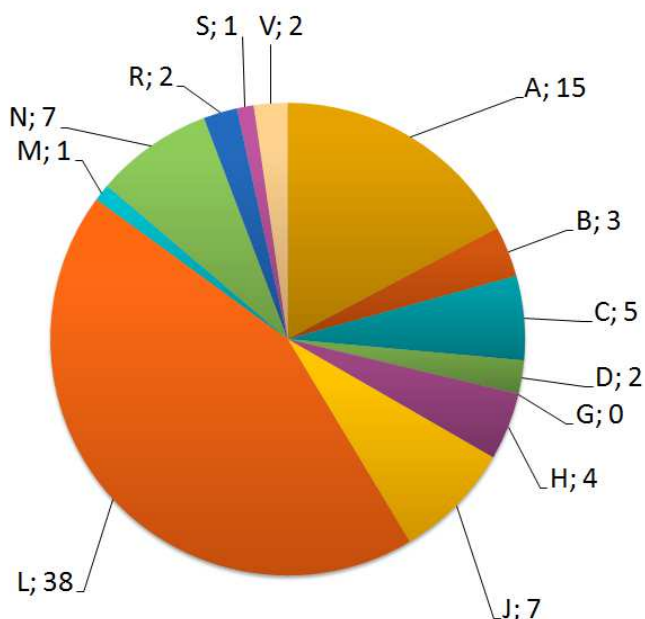
<b>2016</b>	LYNPARZA	ESBRIET	<b>2007</b>
COAGADDEX	SCENESSE	PLENADREN	ATRIANCE
NEOFORDEX	SIRTURO	TOBI PODHALER	CYSTADANE
<b>2015</b>	SYLVANT	VOTUBIA	DIACOMIT
BLINCYTO	TRANSLARNA	VYNDAQEL	ELAPRASE
CERDELGA	VIMIZIM	<b>2010</b>	GLIOLAN
CRESEMBA	<b>2013</b>	ARZERRA	INCRELEX
FARYDAK	BOSULIF	TEPADINA	INOVELON
HETLIOZ	DEFITELIO	VPRIV	REVLIMID
HOLOCLAR	ICLUSIG	<b>2009</b>	SIKLOS
KANUMA	IMNOVID	CAYSTON	SOLIRIS
KYPROLIS	OPSUMIT	FIRDAPSE	TASIGNA
LENVIMA	ORPHACOL	MEPACT	TORISEL
OFEV	PROCYSBI	MOZOBIL	YONDELIS
RAVICTI	<b>2012</b>	NPLATE	<b>2006</b>
RAXONE	ADCETRIS	PEYONA	EVOLTRA
STRENSIQ	BRONCHITOL	ZAVESCA	EXJADE
UNITUXIN	DACOGEN	<b>2008</b>	NEXAVAR
<b>2014</b>	GLYBERA	CEPLENE	SAVENE
ADEMPAS	KALYDECO	FIRAZYR	SPRYCEL
COMETRIQ	NEXOBRID	KUVAN	<b>2004</b>
DELTYBA	REVESTIVE	THALIDOMID E CELGENE	XAGRID
GAZYVARO	SIGNIFOR	VIDAZA	
GRANUPAS	XALUPRINE	VOLIBRIS	
IMBRUVICA	<b>2011</b>		
KETOCONAZOLE HRA	CARBAGLU		
KOLBAM			



## Classificação por categoria ATC

<b>A- ALIMENTARY TRACT AND METABOLISM</b>	OPSUMIT	BOSULIF	TORISEL
CARBAGLU	VOLIBRIS	CEPLENE	UNITUXIN
CERDELGA	<b>D- DERMATOLOGICALS</b>	COMETRIQ	VIDAZA
CYSTADANE	NEXOBRID	DACOGEN	VOTUBIA
ELAPRASE	SCENESSE	ESBRIET	XAGRID
KANUMA	<b>H- SYSTEMIC HORMONAL PREPARATIONS, EXCL,</b>	EVOLTRA	XALUPRINE
KOLBAM	INCRELEX	FARYDAK	YONDELIS
KUVAN	NEOFORDEX	GAZYVARO	<b>M- MUSCULO-SKELETAL SYSTEM</b>
ORPHACOL	PLENADREN	GLIOLAN	TRANSLARNA
PROCYSBI	SIGNIFOR	ICLUSIG	<b>N- NERVOUS SYSTEM</b>
RAVICTI	<b>J- GENERAL ANTIINFECTIVES FOR SYSTEMIC USE</b>	IMBRUVICA	DIACOMIT
REVESTIVE	CAYSTON	IMNOVID	FIRDAPSE
STRENSIQ	CRESEMBA	KYPROLIS	HETLIOZ
VIMIZIM	DELTYBA	LENVIMA	INOVELON
VPRIV	GRANUPAS	LYNPARZA	PEYONA
ZAVESCA	KETOCONAZOLE	MEPACT	RAXONE
<b>B- BLOOD AND BLOOD FORMING</b>	SIRTURO	MOZOBIL	VYNDAQEL
COAGADEX	TOBI PODHALER	NEXAVAR	<b>R- RESPIRATORY</b>
DEFITELIO	<b>L- ANTINEOPLASTIC AND IMMUNOMODULATING</b>	OFEV	BRONCHITOL
NPLATE	ADCETRIS	REVLIMID	KALYDECO
<b>C- CARDIOVASCULAR SYSTEM</b>	ARZERRA	SIKLOS	<b>S- SENSORY ORGANS</b>
ADEMPAS	ATRIANCE	SOLIRIS	HOLOCLAR
FIRAZYR	BLINCYTO	SPRYCEL	<b>V- VARIOUS</b>
GLYBERA		SYLVANT	EXJADE
		TASIGNA	SAVENE
		TEPADINA	
		THALIDOMIDE	
		CELGENE	

Número de medicamentos órfãos na Europa com designação órfã europeia e autorização europeia de introdução no mercado, por categoria ATC



## Classificação pelo detentor da AIM

<b>ACTELION REGISTRATION LTD</b>	<b>CHIESI FARMACEUTICI SPA</b>	<b>MEDIWOUND GERMANY GMBH</b>	<b>ROCHE REGISTRATION LTD</b>
OPSUMIT	HOLOCLAR	NEXOBRID	ESBRIET
ZAVESCA	PEYONA	<b>MERCK SERONO EUROPE LTD</b>	GAZYVARO
<b>ADDMEDICA</b>	<b>CLINIGEN HEALTHCARE LTD</b>	KUVAN	<b>SANTHERA PHARMACEUTICALS (DEUTSCHLAND) GMBH</b>
SIKLOS	SAVENE	<b>NOVA LABORATORIES LTD</b>	RAXONE
<b>ADIENNE SRL</b>	<b>CLINUVEL UK LIMITED</b>	XALUPRINE	<b>SHIRE HUMAN GENETIC THERAPIES AB</b>
TEPADINA	SCENESSE	<b>NOVARTIS EUROPHARM LTD</b>	ELAPRASE
<b>ALEXION EUROPE SAS</b>	<b>ESAI LTD</b>	ARZERRA	<b>SHIRE ORPHAN THERAPIES GMBH</b>
SOLIRIS	INOVELON	ATRIANCE	FIRAZYR
STRENSIQ	LENVIMA	EXJADE	<b>SHIRE PHARMACEUTICAL CONTRACTS LTD</b>
<b>AMGEN EUROPE B.V.</b>	<b>GENTIUM SPA</b>	FARYDAK	XAGRID
BLINCYTO	DEFITELIO	SIGNIFOR	<b>SHIRE PHARMACEUTICAL CONTRACTS LTD</b>
KYPROLIS	<b>GENZYME EUROPE B.V.</b>	TASIGNA	VPRIV
NPLATE	CERDELGA	TOBI PODHALER	<b>SYNAGEVA BIOPHARMA LTD</b>
<b>ARIAD PHARMA LTD</b>	EVOLTRA	VOTUBIA	KANUMA
ICLUSIG	MOZOBIL	<b>NPS PHARMA HOLDINGS LIMITED</b>	<b>TAKEDA FRANCE SAS</b>
<b>ASTRA ZENECA AB</b>	<b>GILEAD SCIENCES INTERNATIONAL LTD</b>	REVESTIVE	MEPACT
LYNPARZA	CAYSTON	<b>ORPHAN EUROPE SARL</b>	<b>TAKEDA PHARMA A/S.</b>
<b>BASILEA MEDICAL LTD</b>	<b>GLAXO GROUP LTD</b>	CARBAGLU	ADCETRIS
CRESEMBA	VOLIBRIS	CYSTADANE	<b>TMC PHARMA SERVICES LTD.</b>
<b>BAYER PHARMA AG</b>	<b>HORIZON THERAPEUTICS Ltd</b>	<b>OTSUKA NOVEL PRODUCTS GMBH</b>	COMETRIQ
ADEMPAS	RAVICTI	DELTYBA	<b>UNIQUIRE BIOPHARMA B.V.</b>
NEXAVAR	<b>IPSEN PHARMA</b>	<b>PFIZER LTD</b>	GLYBERA
<b>BIO PRODUCTS LABORATORY LTD</b>	INCRELEX	BOSULIF	<b>UNITED THERAPEUTICS EUROPE LTD</b>
COAGADEX	<b>JANSSEN-CILAG INTERNATIONAL NV</b>	TORISEL	UNITUXIN
<b>BIOCODEX</b>	DACOGEN	VYNDAQEL	<b>VANDA PHARMACEUTICALS LTD</b>
DIACOMIT	IMBRUVICA	<b>PHARMA MAR S.A.</b>	HETLIOZ
<b>BIOMARIN EUROPE LTD</b>	SIRTURO	YONDELIS	<b>VERTEX PHARMACEUTICALS (EUROPE) LTD</b>
FIRDAPSE	SYLVANT	<b>PHARMAXIS PHARMACEUTICALS LTD</b>	KALYDECO
VIMIZIM	<b>LABORATOIRE HRA PHARMA</b>	BRONCHITOL	<b>VIROPHARMA SPRL</b>
<b>BOEHRINGER INGELHEIM INTERNATIONAL GMBH</b>	KETOCONAZOLE HRA	<b>PTC THERAPEUTICS LTD</b>	PLENADREN
OFEV	<b>LABORATOIRES CTRS</b>	TRANSLARNA	
<b>BRISTOL MYERS SQUIBB EEIG</b>	NEOFORDEX	<b>RAPTOR PHARMACEUTICALS EUROPE B.V.</b>	
SPRYCEL	ORPHACOL	PROCYSBI	
<b>CELGENE EUROPE LTD</b>	<b>LUCANE PHARMA</b>	<b>RETROPHIN EUROPE LTD</b>	
IMNOVID	GRANUPAS	KOLBAM	
REVLIMID	<b>MEDA AB</b>		
THALIDOMIDE	CEPLENE		
CELGENE	<b>MEDAC GMBH</b>		
VIDAZA	GLIOLAN		



## PARTE 2 :

# Lista de medicamentos destinados a doenças raras, na Europa, com autorização europeia de introdução no mercado\* sem designação de medicamento órfão na Europa

## Índice

<b>Lista de medicamentos destinados a doenças raras, na Europa, com autorização europeia de introdução no mercado* sem designação de medicamento órfão na Europa</b>	<b>25</b>
<i>Metodologia</i>	25
<i>Classificação pelo nome comercial</i>	26
<i>Classificação por data de AIM por ordem decrescente</i>	48
<i>Classificação por categoria ATC</i>	49
<i>Classificação pelo detentor da AIM</i>	51

## Metodologia

Este documento disponibiliza uma lista de todos os medicamentos para doenças raras que receberam uma autorização de introdução no mercado (AIM) na Europa para uma ou mais indicações de uso para uma doença rara, mas que não lhes foi concedida uma designação europeia de medicamento órfão ou cuja designação foi removida/retirada.

Pode ter sido concedida, ou não, a estes medicamentos, uma designação de medicamento órfão em outra área geográfica no mundo. Eles aparecem na lista da DG SANTE de medicamentos aos quais foi concedida uma autorização de introdução no mercado:

<http://ec.europa.eu/health/documents/communitary-register/html/alfregister.htm>

A primeira classificação por nome comercial fornece o nome da substância ativa, a indicação da autorização de introdução no

mercado (AIM), a data da AIM e o seu detentor.

Três listas adicionais propõem outras classificações por:

- Data da AIM por ordem decrescente;
- Categoria ATC;
- Detentor da AIM.

Para cada lista, os nomes comerciais são apresentados por ordem alfabética.

Pode encontrar informações adicionais sobre cada medicamento no separador «medicamentos órfãos» no website da Orphanet: [www.orpha.net](http://www.orpha.net), ou no site EMA (Agência Europeia de Medicamentos) <http://www.ema.europa.eu>

\*Autorização de comercialização da Comunidade Europeia no âmbito do procedimento centralizado

## Classificação pelo nome 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 <b>adenocarcinoma of the pancreas</b> .	11/01/2008	Celgene Europe Ltd
ADCIRCA	tadalafil	In adults for the treatment of <b>pulmonary arterial hypertension (PAH)</b> 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 <b>haemophilia A (congenital factor VIII deficiency)</b> . It is indicated in all age groups.	02/03/2004	Baxter AG
AFINITOR	everolimus	Treatment of unresectable or metastatic, well- or moderately-differentiated <b>neuroendocrine tumours of pancreatic origin</b> in adults with progressive disease. Treatment of patients with <b>advanced renal cell carcinoma</b> , whose disease has progressed on or after treatment with VEGF-targeted therapy.	03/08/2009	Novartis Europharm Ltd
ALDURAZYME	laronidase	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of <b>Mucopolysaccharidosis I (MPS I; a [alpha]-L-iduronidase deficiency)</b> 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 <b>pleural mesothelioma</b> .	20/09/2004	Eli Lilly Nederland B.V.
AMMONAPS	sodium phenylbutyrate	Adjunctive therapy in the chronic management of <b>urea cycle disorders</b> , involving <b>deficiencies of carbamyl phosphate synthetase, ornithine transcarbamylase, or argininosuccinate synthetase</b> . It is indicated in all patients with <i>neonatal-onset</i> presentation (complete enzyme deficiencies, presenting within the first 28 days of life). It is also indicated in patients with <i>late-onset</i> disease (partial enzyme deficiencies, presenting after the first month of life) who have a history of hyperammonaemic encephalopathy.	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 <b>pleural mesothelioma</b> .	18/01/2016	Actavis Group PTC ehf
ATRYN	antithrombin alpha	Prophylaxis of venous thromboembolism in surgery of adult patients with <b>congenital antithrombin deficiency</b> . It is normally given in association with heparin or low molecular weight heparin.	28/07/2006	GTC Biotherapeutics UK Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
AVASTIN	bevacizumab	<p>In combination with interferon alfa-2a it is indicated for first line treatment of adult patients with advanced and/or metastatic <b>renal cell cancer</b>.</p> <p>In combination with carboplatin and paclitaxel it is indicated for the front-line treatment of adult patients with <b>advanced</b> (International Federation of Gynecology and Obstetrics (FIGO) stages III B, III C and IV) <b>epithelial ovarian, fallopian tube, or primary peritoneal cancer</b>.</p> <p>In combination with carboplatin and gemcitabine, it is indicated for treatment of adult patients with first recurrence of <b>platinum-sensitive epithelial ovarian, fallopian tube or primary VEGF receptor-targeted agents</b>.</p> <p>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.</p> <p>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 <b>carcinoma of the cervix</b></p>	12/01/2005	Roche Registration Ltd
BEMFOLA	follitropin alfa	In adult men: stimulation of spermatogenesis in men who have <b>congenital or acquired hypogonadotropic hypogonadism</b> with concomitant human chorionic gonadotropin (hCG) therapy.	27/03/2014	Finox BiotechAG
BENEFIX	nonacog alpha	Treatment and prophylaxis of bleeding in patients with <b>haemophilia B (congenital factor IX deficiency)</b> .	27/08/1997	Pfizer Ltd
BIOGRASTIM	filgrastim	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/l$ , and a history of severe or recurrent infections, long term administration of Biograstim is indicated to increase neutrophil counts and to reduce the incidence and duration of infection-related events.	15/09/2008	AbZ-Pharma GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
BORTEZOMIB ACCORD	bortezomib	As monotherapy or in combination with pegylated liposomal doxorubicin or dexamethasone for the treatment of adult patients with progressive <b>multiple myeloma</b> 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 <b>mantle cell lymphoma</b> who are unsuitable for haematopoietic stem cell transplantation.	20/07/2015	Accord Healthcare Ltd
BUCCOLAM	midazolam	Treatment of prolonged, acute, convulsive <b>seizures in infants, toddlers, children and adolescents</b> (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
BUSILVEX	busulfan	Followed by cyclophosphamide (BuCy2), conditioning treatment prior to conventional <b>haematopoietic progenitor cell transplantation</b> 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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
CAELYX	doxorubicin hydrochloride (pegylated liposomal)	Treatment of <b>advanced ovarian cancer</b> in women who have failed a first-line platinum-based chemotherapy regimen. In combination with bortezomib for the treatment of progressive <b>multiple myeloma</b> 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 <b>Kaposi's sarcoma (KS)</b> in patients with low CD4 counts (< 200 CD4 lymphocytes/mm <sup>3</sup> ) 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 (ex CASPOFUNGIN MSD)	caspofungin	Treatment of <b>invasive candidiasis</b> in adult or paediatric patients. Treatment of <b>invasive aspergillosis</b> 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 <b>thyroid cancer (MTC)</b> in patients with unresectable locally advanced or metastatic disease. 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 <b>hyperammonaemia</b> due to <b>N-acetylglutamate synthase (NAGS) primary deficiency</b>	28/01/2003	Orphan Europe S.A.R.L
CEPROTIN	human protein c	In purpura fulminans and coumarin-induced skin necrosis in patients with severe <b>congenital protein C deficiency</b> . 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 ( <b>Type 1</b> ) or chronic neuronopathic ( <b>Type 3</b> ) <b>Gaucher disease</b> 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.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
CINRYZE	C1 inhibitor(human)	Treatment and pre-procedure prevention of angioedema attacks in adults and adolescents with <b>hereditary angioedema (HAE)</b> . 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 <i>Pseudomonas aeruginosa</i> in patients with <b>cystic fibrosis (CF)</b> aged 6 years and older. Consideration should be given to official guidance on the appropriate use of antibacterial agents.	13/02/2012	Forest Laboratories UK Ltd
CYSTAGON	mercaptamine bitartrate	Treatment of proven nephropathic <b>cystinosis</b> . 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 <b>lymphomatous meningitis</b> . In the majority of patients such treatment will be part of symptomatic palliation of the disease.	11/07/2001	Pacira Ltd
DOCETAXEL HOSPIRA UK LIMITED	docetaxel	In combination with cisplatin and 5-fluorouracil for the induction treatment of patients with locally advanced <b>squamous cell carcinoma of the head and neck</b> .	28/08/2015	Hospira UK Ltd
DUKORAL	vibrio cholerae and recombinant cholera toxinb-subunit	Indicated for active immunisation against disease caused by <b>Vibrio cholerae serogroup O1</b> 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
ELOCTA	efmoroctocog alfa	Treatment and prophylaxis of bleeding in patients with <b>haemophilia A</b> (congenital factor VIII deficiency). ELOCTA can be used for all age groups.	19/11/2015	Biogen Idec Ltd
ENBREL	etanercept	Treatment of <b>polyarthritis</b> (rheumatoid-factorpositive or -negative) and extended <b>oligoarthritis</b> 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 <b>psoriatic arthritis in adolescents</b> from the age of 12 years who have had an inadequate response to, or who have proved intolerant of, methotrexate. Treatment of <b>enthesitis-related arthritis in adolescents</b> 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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
ERBITUX	cetuximab	Treatment of patients with <b>squamous cell cancer of the head and neck</b> : - 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
ERIVEDGE	vismodegib	Treatment of adult patients with symptomatic metastatic <b>basal cell carcinoma</b> Treatment of adult patients with locally advanced basal cell carcinoma inappropriate for surgery or radiotherapy	12/07/2013	Roche Registration Ltd
EURARTESIM	piperazine tetraphosphate/dihydroartemisinin	Treatment of uncomplicated <b>Plasmodium falciparum malaria</b> 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 Farmaceutiche Riunite S.p.A
FABRAZYME	agalsidase beta	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of <b>Fabry disease (alphagalactosidase A deficiency)</b> .	03/08/2001	Genzyme Europe B.V.
FERRIPROX	deferiprone	Treatment of iron overload in patients with <b>thalassaemia major</b> when deferoxamine therapy is contraindicated or inadequate.	25/08/1999	Apotex Europe B.V.
FILGRASTIM HEXAL	filgrastim	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/l$ , and a history of severe or recurrent infections. Long term administration of filgrastim is indicated to increase neutrophil counts and to reduce the incidence and duration of infection-related events.	06/02/2009	Hexal AG
FLEBOGAMMA DIF	human normal immunoglobulin	Replacement therapy in adults, and children and adolescents (2-18 years) in: - <b>Primary immunodeficiency (PID)</b> syndromes with impaired antibody production. - <b>Hypogammaglobulinaemia</b> and recurrent bacterial infections in patients with chronic lymphocytic leukaemia, in whom prophylactic antibiotics have failed. - <b>Hypogammaglobulinaemia</b> and recurrent bacterial infections in plateau phase multiple myeloma patients who have failed to respond to pneumococcal immunisation. - <b>Hypogammaglobulinaemia</b> in patients after allogeneic haematopoietic stem cell transplantation (HSCT). Immunomodulation in adults, and children and adolescents (2-18 years) in - <b>Primary immune thrombocytopenia (ITP)</b> , in patients at high risk of bleeding or prior to surgery to correct the platelet count. - <b>Guillain-Barré syndrome</b> - <b>Kawasaki disease.</b>	23/07/2007	Instituto Grifols S.A.

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
GLIVEC	imatinib mesilate	<p>Treatment of adult and paediatric patients with newly diagnosed <b>Philadelphia chromosome (bcr-abl) positive (Ph+) chronic myeloid leukaemia (CML)</b> for whom bone marrow transplantation is not considered as the first line of treatment.</p> <p>Treatment of adult and paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy, or in accelerated phase or blast crisis.</p> <p>Treatment of adult and paediatric patients with newly diagnosed Philadelphia chromosome positive <b>acute lymphoblastic leukaemia (Ph+ ALL)</b> integrated with chemotherapy.</p> <p>Treatment of adult patients with relapsed or refractory Ph+ ALL as monotherapy.</p> <p>Treatment of adult patients with <b>myelodysplastic / myeloproliferative diseases (MDS/MPD)</b> associated with platelet-derived growth factor receptor (PDGFR) gene rearrangements.</p> <p>Treatment of adult patients with advanced <b>hypereosinophilic syndrome (HES)</b> and/or <b>chronic eosinophilic leukaemia (CEL)</b> with FIP1L1-PDGFR<math>\alpha</math> rearrangement.</p> <p>The effect of Glivec on the outcome of bone marrow transplantation has not been determined.</p> <p>Treatment of adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant <b>gastrointestinal stromal tumours (GIST)</b></p> <p>Adjuvant treatment of adult patients who are at significant risk of relapse following resection of Kit (CD117)-positive <b>GIST</b>. Patients who have a low or very low risk of recurrence should not receive adjuvant treatment.</p> <p>Treatment of adult patients with unresectable <b>dermatofibrosarcoma protuberans (DFSP)</b> and adult patients with recurrent and/or metastatic DFSP who are not eligible for surgery.</p>	07/11/2001	Novartis Europharm Ltd
GONAL-F	follitropin alpha	Stimulation of spermatogenesis in men who have <b>congenital or acquired hypogonadotropic hypogonadism</b> with concomitant human Chorionic Gonadotrophin (hCG) therapy.	20/10/1995	Merck Serono Europe Ltd
GRASTOFIL	filgrastim	In adult or children patients with <b>severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of <math>\leq 0.5 \times 10^9/L</math></b> , 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 <b>haemophilia A (congenital factor VIII deficiency)</b> . This preparation does not contain von Willebrand factor and is therefore not indicated in von Willebrand's disease.	04/08/2000	Bayer Pharma AG
HEMANGIOL	propranolol	<p>Treatment of <b>proliferating infantile haemangioma</b> requiring systemic therapy:</p> <ul style="list-style-type: none"> <li>- life- or function-threatening haemangioma,</li> <li>- ulcerated haemangioma with pain and/or lack of response to simple wound care measures,</li> <li>- haemangioma with a risk of permanent scars or disfigurement.</li> </ul> <p>It is to be initiated in infants aged 5 weeks to 5 months.</p>	23/04/2014	Pierre Fabre Dermatologie



Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
HERCEPTIN	trastuzumab	In combination with capecitabine or 5-fluorouracil and cisplatin, treatment of patients with HER2-positive metastatic <b>adenocarcinoma of the stomach or gastroesophageal junction</b> 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 <b>primary immunodeficiency syndromes</b> such as: - congenital <b>agammaglobulinaemia</b> and <b>hypogammaglobulinaemia</b> , - <b>common variable immunodeficiency</b> , - <b>severe combined immunodeficiency</b> , - <b>IgG subclass deficiencies</b> with recurrent infections. Replacement therapy in <b>myeloma</b> or <b>chronic lymphocytic leukaemia</b> 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 <b>polyarticular juvenile idiopathic arthritis</b> , 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 <b>enthesitis-related arthritis</b> in patients, 6 years of age and older, who have had an inadequate response to, or who are intolerant of, conventional therapy.	08/09/2003	Abbvie Ltd.
HYCAMTIN	topotecan	As monotherapy, treatment of: - patients with <b>metastatic carcinoma of the ovary</b> after failure of first-line or subsequent therapy. - patients with <b>relapsed small cell lung cancer (SCLC)</b> for whom retreatment with the first-line regimen is not considered appropriate. In combination with cisplatin, it is indicated for patients with <b>carcinoma of the cervix</b> 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
HYQVIA	human normal immunoglobulin	Replacement therapy in adults ( $\geq 18$ years) in primary immunodeficiency syndromes such as: - <b>congenital agammaglobulinaemia</b> and <b>hypogammaglobulinaemia</b> - <b>common variable immunodeficiency</b> - <b>severe combined immunodeficiency</b> - <b>IgG subclass deficiencies</b> with recurrent infections. Replacement therapy in adults ( $\geq 18$ years) in <b>myeloma</b> or <b>chronic lymphocytic leukaemia</b> with severe secondary hypogammaglobulinaemia and recurrent infections.	16/05/2013	Baxalta Innovations GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
IBLIAS	octocog alfa	Treatment and prophylaxis of bleeding in patients with <b>haemophilia A (congenital factor VIII deficiency)</b> . Iblias can be used for all age groups.	18/02/2016	Bayer Pharma AG
ILARIS	canakinumab	Treatment of <b>Cryopyrin-Associated Periodic Syndromes (CAPS)</b> in adults, adolescents and children aged 2 years and older with body weight of 7,5 kg or above, including: - <b>Muckle-Wells Syndrome (MWS)</b> , - <b>Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA)</b> , - Severe forms of <b>Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU)</b> presenting with signs and symptoms beyond cold- induced urticarial skin rash. Treatment of active <b>Systemic Juvenile Idiopathic Arthritis (SJIA)</b> 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
INLYTA	axitinib	Treatment of adult patients with advanced <b>renal cell carcinoma (RCC)</b> 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 $\geq 34$ weeks gestation with hypoxic respiratory failure associated with clinical or echo cardiographic evidence of <b>pulmonary hypertension</b> , 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 alfa-2b	<p>Treatment of patients with <b>hairy cell leukaemia</b>. As Monotherapy for the treatment of adult patients with <b>Philadelphia chromosome or bcr/abl translocation positive chronic myelogenous leukaemia</b>. 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 &lt; 34 % Ph+ leukaemic cells in the bone marrow, whereas a minor response is &gt; 34 %, but &lt; 90 % Ph+ cells in the marrow.</p> <p>In combination with interferon alfa-2b and cytarabine (Ara-C) during the first 12 months of treatment it has been demonstrated to significantly increase the rate of major cytogenetic responses and to significantly prolong the overall survival at three years when compared to interferon alfa-2b monotherapy.</p> <p>As maintenance therapy in patients with <b>multiple myeloma</b> who have achieved objective remission (more than 50 % reduction in myeloma protein) following initial induction chemotherapy. Current clinical experience indicates that maintenance therapy with interferon alfa-2b prolongs the plateau phase; however, effects on overall survival have not been conclusively demonstrated.</p> <p>Treatment of high tumour burden <b>follicular lymphoma</b> 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 (&gt; 7 cm), involvement of three or more nodal sites (each &gt; 3 cm), systemic symptoms (weight loss &gt; 10 %, pyrexia &gt; 38°C for more than 8 days, or nocturnal sweats), splenomegaly beyond the umbilicus, major organ obstruction or compression syndrome, orbital or epidural involvement, serous effusion, or leukaemia.</p> <p>Treatment of <b>carcinoid tumours</b> with lymph node or liver metastases and with "<b>carcinoid syndrome</b>".</p>	09/03/2000	Merck Sharp & Dohme Ltd
IXIARO	japanese encephalitis vaccine (inactivated, adsorbed)	<p>Active immunisation against <b>Japanese encephalitis</b> in adults, adolescents, children and infants aged 2 months and older.</p> <p>IXIARO should be considered for use in individuals at risk of exposure through travel or in the course of their occupation</p>	31/03/2009	Valneva Austria GmbH
JAKAVI	ruxolitinib	<p>Treatment of disease-related splenomegaly or symptoms in adult patients with <b>primary myelofibrosis</b> (also known as chronic idiopathic myelofibrosis), <b>post-polycythaemia-vera myelofibrosis</b> or <b>post-essential-thrombocythaemia myelofibrosis</b>.</p> <p>Treatment of adult patients with <b>polycythaemia vera</b> who are resistant to or intolerant of hydroxyurea.</p>	23/08/2012	Novartis Europharm Ltd
JINARC	tolvaptan	<p>Indicated to slow the progression of cyst development and renal insufficiency of <b>autosomal dominant polycystic kidney disease (ADPKD)</b> in adults with CKD stage 1 to 3 at initiation of treatment with evidence of rapidly progressing disease</p>	27/05/2015	Otsuka Pharmaceutical Europe 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 <b>epilepsy</b> . As adjunctive therapy in the treatment of partial onset seizures with or without secondary generalisation in adults, children and infants from 1 month of age with epilepsy; in the treatment of myoclonic seizures in adults and adolescents from 12 years of age with <b>Juvenile Myoclonic Epilepsy</b> Treatment of primary generalised tonic-clonic seizures in adults and adolescents from 12 years of age with <b>Idiopathic Generalised Epilepsy</b> .	29/09/2000	UCB Pharma SA
KINERET	anakinra	Treatment in adults, adolescents, children and infants aged 8 months and older with a body weight of 10 kg or above of <b>Cryopyrin-Associated Periodic Syndromes (CAPS)</b> , including:- <b>Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA)</b> ,- <b>Muckle-Wells Syndrome (MWS)</b> ,- <b>Familial Cold Autoinflammatory Syndrome (FCAS)</b> .	08/03/2002	Swedish Orphan Biovitrum AB
KIOVIG	human normal immunoglobulin	Replacement therapy in adults, and children and adolescents (0-18 years) in: - <b>Primary immunodeficiency syndromes</b> with impaired antibody production, - <b>Hypogammaglobulinaemia</b> and recurrent bacterial infections in patients with <b>chronic lymphocytic leukaemia</b> , in whom prophylactic antibiotics have failed - Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase <b>multiple myeloma</b> patients who have failed to respond to pneumococcal immunisation, - Hypogammaglobulinaemia in patients after allogeneic <b>haematopoietic stem cell transplantation (HSCT)</b> . - Congenital AIDS and recurrent bacterial infections. Immunomodulation in adults, and children and adolescents (0-18 years) in: - <b>Primary immune thrombocytopenia (ITP)</b> , in patients at high risk of bleeding or prior to surgery to correct the platelet count - <b>Guillain Barré syndrome</b> - <b>Kawasaki disease</b> - <b>Multifocal Motor Neuropathy (MMN)</b> .	19/01/2006	Baxter AG
KOGENATE BAYER	octocog alpha	Treatment and prophylaxis of bleeding in patients with <b>haemophilia A (congenital factor VIII deficiency)</b> . 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 <b>haemophilia A (congenital factor VIII deficiency)</b> . Kovaltry can be used for all age groups.	18/02/2016	Bayer Pharma AG
LITAK	cladribine	Treatment of <b>hairy cell leukaemia</b> .	14/04/2004	Lipomed GmbH

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
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 <b>homozygous familial hypercholesterolaemia (HoFH)</b> . 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) <b>adrenal cortical carcinoma</b> . The effect of Lysodren on non functional adrenal cortical carcinoma is not established.	28/04/2004	Laboratoire HRA Pharma
MABTHERA	rituximab	<b>Non-Hodgkin's lymphoma (NHL)</b> - 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 <b>diffuse large B cell non- Hodgkin's lymphoma</b> in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy. In combination with chemotherapy, treatment of patients with previously untreated and relapsed/refractory <b>chronic lymphocytic leukaemia</b> . 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. <b>Granulomatosis with polyangiitis and Microscopic polyangiitis</b> 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 <b>Pompe disease (acid <math>\alpha</math>-glucosidase deficiency)</b> . Myozyme is indicated in adults and paediatric patients of all ages	29/03/2006	Genzyme Europe B.V.
NAGLAZYME	galsulfase	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of <b>Mucopolysaccharidosis VI (MPS VI; N-acetylgalactosamine 4-sulfatase deficiency; Maroteaux-Lamy syndrome)</b> 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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
NIVESTIM	filgrastim	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/l$ , and a history of severe or recurrent infections.	08/06/2010	Hospira UK Ltd
NONAFACT	human coagulation factor IX	Treatment and prophylaxis of bleeding in patients with <b>haemophilia B (congenital factor IX deficiency)</b> .	03/07/2001	Sanquin
NOVOEIGHT	turoctocog alpha	Treatment and prophylaxis of bleeding in patients with <b>haemophilia A (congenital factor VIII deficiency)</b> . NovoEight can be used for all age groups.	13/11/2013	Novo Nordisk A/S
NOVOSEVEN	eptacog alpha (activated)	Treatment of bleeding episodes and for the prevention of bleeding in those undergoing surgery or invasive procedures in the following patient groups : - patients with <b>congenital haemophilia</b> with inhibitors to coagulation factors <b>VIII</b> or <b>IX</b> > 5 BU - patients with congenital haemophilia who are expected to have a high anamnestic response to factor VIII or factor IX administration - patients with <b>acquired haemophilia</b> - patients with <b>congenital FVII deficiency</b> ; - patients with <b>Glanzmann's thrombasthenia</b> with antibodies to GP 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 <b>congenital factor XIII A-subunit deficiency</b>	03/09/2012	Novo Nordisk A/S
NOXAFIL	posaconazole	Treatment of the fungal infections in adults: - Invasive <b>aspergillosis</b> in patients with disease that is refractory to amphotericin B or itraconazole or in patients who are intolerant of these medicinal products - <b>Fusariosis</b> in patients with disease that is refractory to amphotericin B or in patients who are intolerant of amphotericin B. - <b>Chromoblastomycosis</b> and <b>mycetoma</b> in patients with disease that is refractory to itraconazole or in patients who are intolerant of itraconazole - <b>Coccidioidomycosis</b> 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 <b>acute myelogenous leukemia (AML)</b> or <b>myelodysplastic syndromes (MDS)</b> expected to result in prolonged neutropenia and who are at high risk of developing invasive fungal infections - <b>Hematopoietic stem cell transplant (HSCT)</b> 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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
NUEDEXTA	dextro methorphan hydrobromide / quinidine sulfate	Symptomatic treatment of pseudobulbar affect (PBA) in adults. Efficacy has been studied in patients with underlying <b>Amyotrophic Lateral Sclerosis</b> .	24/06/2013	Jenson Pharmaceutical Services Ltd
OBIZUR	susoctocog alfa	Treatment of bleeding episodes in patients with <b>acquired haemophilia</b> caused by antibodies to Factor VIII.	11/11/2015	Baxalta Innovations GmbH
OMNITROPE	somatropin	<p>Infants, children and adolescents:</p> <ul style="list-style-type: none"> <li>- Growth disturbance due to insufficient secretion of growth hormone (<b>growth hormone deficiency, GHD</b>).</li> <li>- Growth disturbance associated with <b>Turner syndrome</b>.</li> <li>- Growth disturbance associated with chronic renal insufficiency.</li> <li>- Growth disturbance (current height standard deviation score (SDS) &lt; -2.5 and parental adjusted height SDS &lt; -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 &lt; 0 during the last year) by 4 years of age or later.</li> <li>- <b>Prader-Willi syndrome (PWS)</b>, for improvement of growth and body composition. The diagnosis of PWS should be confirmed by appropriate genetic testing.</li> </ul> <p>Adults</p> <ul style="list-style-type: none"> <li>- Replacement therapy in adults with pronounced <b>growth hormone deficiency</b>.</li> <li>- <i>Adult onset</i>: Patients who have severe growth hormone deficiency associated with multiple hormone deficiencies as a result of known hypothalamic or pituitary pathology, and who have at least one known deficiency of a pituitary hormone not being prolactin. These patients should undergo an appropriate dynamic test in order to diagnose or exclude a growth hormone deficiency.</li> <li>- <i>Childhood onset</i>: Patients who were growth hormone deficient during childhood as a result of congenital, genetic, acquired, or idiopathic causes. Patients with childhood onset GHD should be re-evaluated for growth hormone secretory capacity after completion of longitudinal growth. In patients with a high likelihood for persistent GHD, i.e. a congenital cause or GHD secondary to a hypothalamic-pituitary disease or insult, an insulin-like growth factor-I (IGF-I) SDS &lt; -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.</li> </ul>	12/04/2006	Sandoz GmbH
ONCASPAR	pegaspargase	Indicated as a component of antineoplastic combination therapy in <b>acute lymphoblastic leukaemia (ALL)</b> in paediatric patients from birth to 18 years, and adult patients.	14/01/2016	Baxalta Innovations GmbH
ORENCIA	abatacept	In combination with methotrexate, for the treatment of moderate to severe active <b>polyarticular juvenile idiopathic arthritis (JIA)</b> 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

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
ORFADIN	nitisinone	Treatment of adult and paediatric (in any age range) patients with confirmed diagnosis of <b>hereditary tyrosinemia type 1 (HT-1)</b> in combination with dietary restriction of tyrosine and phenylalanine.	21/02/2005	Swedish Orphan Biovitrum AB
ORKAMBI	lumacaftor / ivacaftor	Treatment of <b>cystic fibrosis (CF)</b> 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 <b>congenital</b> or <b>acquired hypogonadotropic hypogonadism</b> 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 <b>non-infectious uveitis</b> .	27/07/2010	Allergan Pharmaceuticals Ireland
PANRETIN	alitretinoin	Topical treatment of cutaneous lesions in patients with AIDS-related <b>Kaposi's sarcoma (KS)</b> : - 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 <b>patent ductus arteriosus</b> 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 <b>pleural mesothelioma</b> .	18/01/2016	Accord Healthcare Ltd
PEMETREXED HOSPIRA	pemetrexed	In combination with cisplatin indicated for the treatment of chemotherapy naïve patients with unresectable malignant <b>pleural mesothelioma</b> .	20/11/2015	Hospira UK Ltd
PEMETREXED LILLY	pemetrexed	In combination with cisplatin for the treatment of chemotherapy naïve patients with unresectable malignant <b>pleural mesothelioma</b> .	14/09/2015	Eli Lilly Netherlands
PEMETREXED MEDAC	pemetrexed	In combination with cisplatin indicated for the treatment of chemotherapy naïve patients with unresectable malignant <b>pleural mesothelioma</b> .	27/11/2015	Medac GmbH
PEMETREXED SANDOZ	pemetrexed	In combination with cisplatin for the treatment of chemotherapy naïve patients with unresectable malignant <b>pleural mesothelioma</b> .	18/09/2015	Sandoz GmbH
PIXUVRI	pixantrone dimaleate	As monotherapy for the treatment of adult patients with multiply relapsed or refractory aggressive <b>Non-Hodgkin B-cell Lymphomas (NHL)</b> . 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
PRIALT	ziconotide	Treatment of severe, chronic pain in patients who require <b>intrathecal (IT) analgesia</b> .	21/02/2005	Eisai Ltd

NEW



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: - <b>Primary immunodeficiency (PID)</b> syndromes with impaired antibody production - Hypogammaglobulinaemia and recurrent bacterial infections in patients with <b>chronic lymphocytic leukaemia</b> , in whom prophylactic antibiotics have failed. - Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase <b>multiple myeloma</b> patients who have failed to respond to pneumococcal immunisation. - Hypogammaglobulinaemia in patients after <b>allogeneic haematopoietic stem cell transplantation (HSCT)</b> . - <b>Congenital AIDS</b> with recurrent bacterial infections. Immunomodulation in adults, and children and adolescents (0-18 years) in: - <b>Primary immune thrombocytopenia (ITP)</b> , in patients at high risk of bleeding or prior to surgery to correct the platelet count. - <b>Guillain-Barré syndrome</b> . - <b>Kawasaki disease</b> . - <b>Chronic inflammatory demyelinating polyneuropathy (CIDP)</b> . 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 <b>hypogonadotrophic hypogonadism</b> .	03/05/1996	Merck Sharp & Dohme Ltd
QUINSAIR	levofloxacin	Management of chronic pulmonary infections due to <i>Pseudomonas aeruginosa</i> in adult patients with <b>cystic fibrosis</b>	26/03/2015	Aptalis Pharma SAS
RATIOGRASTIM	filgrastim	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/l$ , and a history of severe or recurrent infections.	15/09/2008	Ratiopharm GmbH
REFACTO AF	moroctocog alpha	Treatment and prophylaxis of bleeding in patients with <b>haemophilia A (congenital factor VIII deficiency)</b> 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
REPATHA	evolocumab	Indicated in adults and adolescents aged 12 years and over with <b>homozygous familial hypercholesterolaemia</b> 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 <b>Fabry Disease (alpha- galactosidase A deficiency)</b>	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 <b>alpha1-proteinase inhibitor deficiency</b> (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 <b>pulmonary arterial hypertension</b> 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
REVOLADE	eltrombopag	For adult chronic <b>immune (idiopathic) thrombocytopenic purpura (ITP)</b> splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Revolade may be considered as second line treatment for adult non-splenectomised patients where surgery is contraindicated. Indicated in adult patients with <b>acquired severe aplastic anaemia (SAA)</b> 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 <b>amyotrophic lateral sclerosis (ALS)</b> . 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.
RIXUBIS	nonacog gamma	Treatment and prophylaxis of bleeding in patients with <b>haemophilia B (congenital factor IX deficiency)</b> . 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 <b>systemic juvenile idiopathic arthritis (sJIA)</b> 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 <b>juvenile idiopathic polyarthritis (pJIA; rheumatoid factor positive or negative</b> 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 with <b>hereditary angioedema (HAE) due to C1 esterase inhibitor deficiency.</b>	28/10/2010	Pharming Group N.V.
SOMAVERT	pegvisomant	Treatment of adult patients with <b>acromegaly</b> 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 <b>acute lymphoblastic leukaemia (ALL)</b> in paediatric patients from birth to 18 years and adults.	14/01/2016	Medac Gesellschaft fuer klinische Spezialpraeparate mbH
STAYVEER	bosentan monohydrate	Treatment of <b>pulmonary arterial hypertension (PAH)</b> to improve exercise capacity and symptoms in patients with WHO functional class III. Efficacy has been shown in: <ul style="list-style-type: none"> <li>• <b>Primary</b> (idiopathic and heritable) <b>pulmonary arterial hypertension</b></li> <li>• <b>Pulmonary arterial hypertension secondary to scleroderma</b> without significant interstitial pulmonary disease</li> <li>• <b>Pulmonary arterial hypertension associated with congenital systemic-to-pulmonary shunts and Eisenmenger's physiology.</b></li> </ul> 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 <b>systemic sclerosis</b> and ongoing digital ulcer disease	24/06/2013	Marklas Nederland BV
SUTENT	sunitinib	Treatment of unresectable and/or metastatic <b>malignant gastrointestinal stromal tumour (GIST)</b> in adults after failure of imatinib treatment due to resistance or intolerance. Treatment of <b>advanced/metastatic renal cell carcinoma (MRCC)</b> in adults. Treatment of unresectable or metastatic, well-differentiated <b>pancreatic neuroendocrine tumours (pNET)</b> with disease progression in adults. Experience with SUTENT as first-line treatment is limited	19/07/2006	Pfizer Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
TARCEVA	erlotinib	In combination with gemcitabine, for the treatment of patients with metastatic <b>pancreatic cancer</b> . 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 <b>cutaneous T-cell lymphoma (CTCL)</b> 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 <b>squamous cell carcinoma of the head and neck</b> .	27/11/1995	Aventis Pharma S.A.
TEMODAL	temozolomide	Treatment of adult patients with newly-diagnosed <b>glioblastoma multiforme</b> concomitantly with radiotherapy (RT) and subsequently as monotherapy treatment. Treatment of children from the age of three years, adolescents and adult patients with <b>malignant glioma</b> , such as glioblastoma multiforme or <b>anaplastic astrocytoma</b> , showing recurrence or progression after standard therapy.	26/01/1999	Merck Sharp & Dohme Ltd
TEVAGRASTIM	filgrastim	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/l$ , and a history of severe or recurrent infections.	15/09/2008	Teva GmbH
TEYSUNO	tegafur/gimeracil/o teracil	In adults for the treatment of advanced <b>gastric cancer</b> 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 <b>well-differentiated thyroid cancer</b> in post-thyroidectomy patients maintained on hormone suppression therapy (THST). Low-risk patients with well-differentiated thyroid carcinoma who have undetectable serum Tg levels on THST and no rh (recombinant human) TSH- stimulated increase of Tg levels may be followed-up by assaying rh TSH-stimulated Tg levels. For pre-therapeutic stimulation in combination with a range of 30 mCi (1.1 GBq) to 100 mCi (3.7 GBq) radioiodine for ablation of thyroid tissue remnants in patients who have undergone a near-total or total thyroidectomy for <b>well-differentiated thyroid cancer</b> and who do not have evidence of distant metastatic thyroid cancer.	09/03/2000	Genzyme Europe B.V.

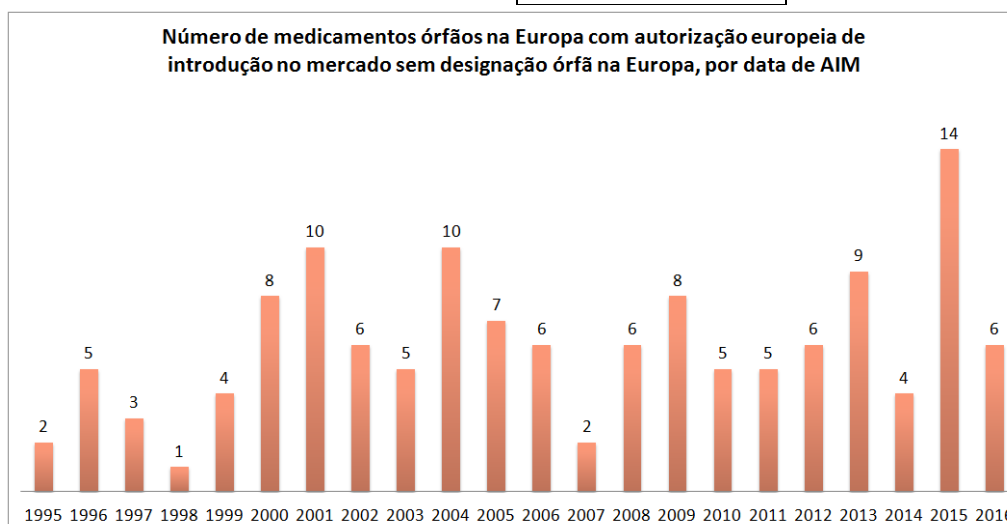
Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
TRACLEER	bosentan monohydrate	Treatment of <b>pulmonary arterial hypertension (PAH)</b> to improve exercise capacity and symptoms in patients with WHO functional class III. Efficacy has been shown in : - <b>primary (idiopathic and heritable) PAH,</b> - <b>PAH secondary to scleroderma</b> without significant interstitial pulmonary disease, - <b>PAH associated with congenital systemic-to- pulmonary shunts</b> and <b>Eisenmenger's physiology</b> . 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 <b>systemic sclerosis</b> and ongoing digital ulcer disease.	15/05/2002	Actelion Registration Ltd
TRISENOX	arsenic trioxide	Induction of remission and consolidation in adult patients with relapsed/refractory <b>acute promyelocytic leukaemia (APL)</b> , characterised by the presence of the t(15;17) translocation and/or the presence of the Pro-Myelocytic Leukaemia/ Retinoic-Acid Receptor-alpha (PML/RAR-alpha) gene. Previous treatment should have included a retinoid and chemotherapy. The response rate of other acute myelogenous leukaemia subtypes to TRISENOX has not been examined.	05/03/2002	Teva Pharma B.V.
VEDROP	tocofersolan	Indicated in vitamin E deficiency due to digestive malabsorption in paediatric patients with <b>congenital chronic cholestasis</b> or <b>hereditary chronic cholestasis</b> , from birth (full term newborns) up to 18 years of age.	24/07/2009	Orphan Europe S.A.R.L
VELCADE	bortezomib	As monotherapy or in combination with pegylated liposomal doxorubicin or dexamethasone is indicated for the treatment of adult patients with progressive <b>multiple myeloma</b> 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 <b>mantle cell lymphoma</b> who are unsuitable for haematopoietic stem cell transplantation.	26/04/2004	Janssen-Cilag International N.V.
VENTAVIS	iloprost	Treatment of patients with <b>primary pulmonary hypertension</b> , classified as NYHA functional class III, to improve exercise capacity and symptoms.	16/09/2003	Bayer Pharma AG

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
VFEND	voriconazole	In adults and children aged 2 years and above as follows: - treatment of <b>invasive aspergillosis</b> . - treatment of <b>serious fungal infections</b> caused by <b><i>Scedosporium spp.</i></b> and <b><i>Fusarium spp.</i></b> . 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 <b>von Willebrand disease (VWD)</b> , when desmopressin (DDAVP) treatment alone is ineffective or contraindicated. Prophylaxis and treatment of bleeding in patients with <b>haemophilia A (congenital FVIII deficiency)</b> .	12/08/2013	CSL Behring GmbH
VORICONAZOLE HOSPIRA	voriconazole	In adults and children aged 2 years and above as follows: - treatment of <b>invasive aspergillosis</b> . - treatment of serious fungal infections caused by <b><i>Scedosporium spp.</i></b> and <b><i>Fusarium spp.</i></b> . Voriconazole should be administered primarily to patients with progressive, possibly life – threatening infections.	27/05/2015	Hospira UK Ltd
VOTRIENT	pazopanib	In adults for the first-line treatment of advanced <b>renal cell carcinoma (RCC)</b> and for patients who have received prior cytokine therapy for advanced disease. For the treatment of adult patients with selective subtypes of advanced <b>soft-tissue sarcoma (STS)</b> 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 <b>Wilson's disease</b> .	13/10/2004	Orphan Europe S.A.R.L
XELODA	capecitabine	First-line treatment of advanced <b>gastric cancer</b> in combination with a platinum-based regimen	02/02/2001	Roche Registration Ltd
XYREM	sodium oxybate	Treatment of <b>narcolepsy with cataplexy</b> in adult patients.	13/10/2005	UCB Pharma Ltd
ZARZIO	filgrastim	In children and adults with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9/l$ , and a history of severe or recurrent infections, long term administration of filgrastim is indicated to increase neutrophil counts and to reduce the incidence and duration of infection-related events.	06/02/2009	Sandoz GmbH
ZAVESCA	miglustat	Treatment of adult patients with mild to moderate <b>type 1 Gaucher disease</b> . Zavesca may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable.	21/11/2002	Actelion Registration Ltd

Tradename	Active Substance	Marketing Authorisation Indication	Marketing Authorisation Date (Dd/Mm/Yyyy)	Marketing Authorisation Holder
ZEVALIN	ibritumomab tiuxetan	Consolidation therapy after remission induction in previously untreated patients with <b>follicular lymphoma</b> . Treatment of adult patients with rituximab relapsed or refractory CD20+ follicular <b>B-cell non-Hodgkin's lymphoma (NHL)</b> .	16/01/2004	Spectrum Pharmaceuticals B.V.
ZUTECTRA	human hepatitis b immunoglobulin	Prevention of <b>hepatitis B virus (HBV) re-infection</b> in HBV-DNA negative patients over 6 months <b>after liver transplantation for hepatitis B induced liver failure</b> . Zuteetra 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 <b>chronic lymphocytic leukaemia (CLL)</b> : - 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 <b>follicular lymphoma (FL)</b> that is refractory to two prior lines of treatment.	18/09/2014	Gilead Sciences International Ltd.

## Classificação por data de AIM por ordem decrescente

<b>2016</b>	STAYVEER	<b>2006</b>	DEPOCYTE
ARMISARTE	VONCENTO	ATRYN	FABRAZYME
IBLIAS	<b>2012</b>	KIOVIG	GLIVEC
KOVALTRY	CAPRELSA	OMNITROPE	INOMAX
ONCOSPARG	COLOBREATHA	MYOZYME	NONAFACT
PEMETREXED	INLYTA	NAGLAZYME	REPLAGAL
ACCORD	JAKAVI	SUTENT	TARGRETIN
SPECTRILA	NOVOTHIRTEEN	<b>2005</b>	XELODA
<b>2015</b>	PIXUVRI	AVASTIN	<b>2000</b>
BORTEZOMIB	<b>2011</b>	NOXAFIL	ENBREL
ACCORD	BUCCOLAM	ORFADIN	HELIXATE NEXGEN
DOCETAXEL	CINRYZE	PRIALT	HERCEPTIN
HOSPIRA UK LTD	EURARTESIM	REVATIO	INTRONA
ELOCTA	HIZENTRA	TARCEVA	KEPPRA
JINARC	TEYSUNO	XYREM	KOGENATE BAYER
OBIZUR	<b>2010</b>	<b>2004</b>	PANRETIN
ORKAMBI	NIVESTIM	ADVATE	THYROGEN
PEMETREXED	OZURDEX	ALIMTA	<b>1999</b>
HOSPIRA	REVOLADE	DUKORAL	AMMONAPS
PEMETREXED LILLY	RUCONEST	ERBITUX	FERRIPROX
PEMETREXED	VOTRIENT	LITAK	REFACTO AF
MEDAC	<b>2009</b>	LYSODREN	TEMODAL
PEMETREXED	AFINITOR	PEDEA	<b>1998</b>
SANDOZ	FILGRASTIM	VELCADE	MABTHERA
QUINSAIR	HEXAL	WILZIN	<b>1997</b>
REPATHA	ILARIS	ZEVALIN	BENEFIX
RESPREEZA	IXIARO	<b>2003</b>	CEREZYME
VORICONAZOLE	ROACTEMRA	ALDURAZYME	CYSTAGON
HOSPIRA	VEDROP	BUSILVEX	<b>1996</b>
<b>2014</b>	ZARZIO	CARBAGLU	CAELYX
BEMFOLA	ZUTECTRA	HUMIRA	HYCANTIN
HEMANGIOL	<b>2008</b>	VENTAVIS	NOVOSEVEN
RIXUBIS	ABRAXANE	<b>2002</b>	PUREGON
ZYDELIG	ADCIRCA	KINERET	RILUTEK
<b>2013</b>	BIOGRASTIM	SOMAVERT	<b>1995</b>
ERIVEDGE	PRIVIGEN	TRACLEER	GONAL-F
GRASTOFIL	RATIOGRASTIM	TRISENOX	TAXOTERE
HYQVIA	TEVAGRASTIM	VFEND	
LOJUXTA	<b>2007</b>	ZAVESCA	
NOVOEIGHT	FLEBOGAMMA DIF	<b>2001</b>	
NUEDEXTA	ORENCIA	CANCIDAS	
OVALEAP		CEPROTIN	





## Classificação por categoria ATC

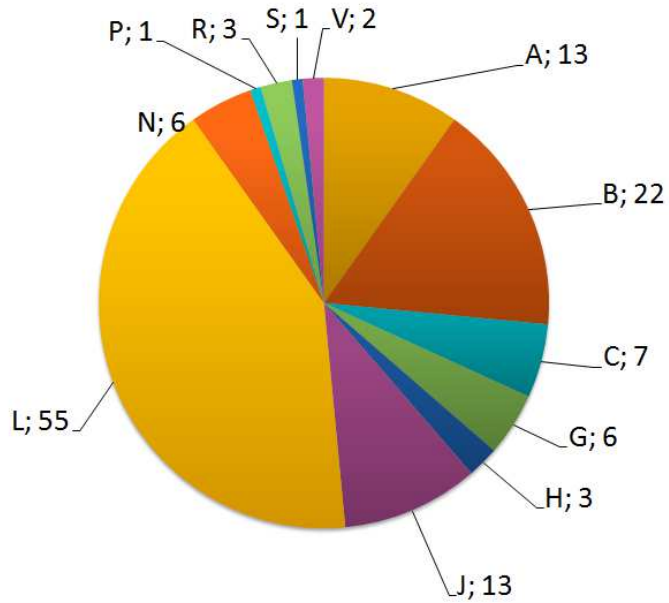
<b>A- ALIMENTARY TRACT AND METABOLISM</b>	<b>G- GENITO URINARY SYSTEM AND SEX HORMONES</b>	GRASTOFIL
ALDURAZYME	ADCIRCA	HERCEPTIN
AMMONAPS	BEMFOLA	HUMIRA
CARBAGLU	GONAL-F	HYCAMTIN
CEREZYME	OVALEAP	ILARIS
CYSTAGON	PUREGON	INLYTA
FABRAZYME	REVATIO	INTRONA
MYOZYME	<b>H- SYSTEMIC HORMONAL PREPARATIONS, EXCL, SEX HORMONES AND INSULINS</b>	JAKAVI
NAGLAZYME	OMNITROPE	KINERET
ORFADIN	SOMAVERT	LITAK
REPLAGAL	THYROGEN	LYSODREN
VEDROP	<b>J- GENERAL ANTIINFECTIVES FOR SYSTEMIC USE</b>	MABTHERA
WILZIN	CANCIDAS	NIVESTIM
ZAVESCA	DUKORAL	ONCASPAR
<b>B- BLOOD AND BLOOD FORMING ORGANS</b>	FLEBOGAMMA DIF	ORENCIA
ADVATE	HIZENTRA	PANRETIN
ATRYN	HYQVIA	PEMETREXED ACCORD
BENEFIX	IXIARO	PEMETREXED HOSPIRA
CEPROTIN	KIOVIG	PEMETREXED LILLY
CINRYZE	NOXAFIL	PEMETREXED MEDAC
ELOCTA	PRIVIGEN	PEMETREXED SANDOZ
HELIXATE NEXGEN	QUINSAIR	PIXUVRI
IBLIAS	VFEND	RATIOGRASTIM
KOGENATE BAYER	VORICONAZOLE HOSPIRA	ROACTEMRA
KOVALTRY	ZUTECTRA	SPECTRILA
NONAFAC	<b>L- ANTINEOPLASTIC AND IMMUNOMODULATING AGENTS</b>	SUTENT
NOVOEIGHT	ABRAXANE	TARCEVA
NOVOSEVEN	AFINITOR	TARGRETIN
NOVOTHIRTEEN	ALIMTA	TAXOTERE
OBIZUR	ARMISARTE	TEMODAL
REFACTO AF	AVASTIN	TEVAGRASTIM
RESPREEZA	BIOGRASTIM	TEYSUNO
REVOLADE	BORTEZOMIB ACCORD	TRISENOX
RIXUBIS	BUSILVEX	VELCADE
RUCONEST	CAELYX	VOTRIENT
VENTAVIS	CAPRELSA	XELODA
VONCENTO	DEPOCYTE	ZARZIO
<b>C- CARDIOVASCULAR SYSTEM</b>	DOCETAXEL HOSPIRA UK LTD	ZYDELIG
HEMANGIOL	ENBREL	<b>N- NERVOUS SYSTEM</b>
JINARC	ERBITUX	BUCCOLAM
LOJUXTA	ERIVEDGE	KEPPRA
PEDEA	FILGRASTIM HEXAL	NUDEXTA
REPATHA	GLIVEC	PRIALT
STAYVEER		RILUTEK
TRACLEER		XYREM
		<b>P- ANTIPARASITIC PRODUCTS, INSECTICIDES AND REPELLENTS</b>
		EURARTESIM

<b>R- RESPIRATORY SYSTEM</b>
COLOBREATHE
INOMAX

ORKAMBI
<b>S- SENSORY ORGANS</b>
OZURDEX

<b>V- VARIOUS</b>
FERRIPROX
ZEVALIN

**Número de medicamentos órfãos na Europa com autorização europeia de introdução no mercado sem designação órfã prévia na Europa, por categoria ATC**



## Classificação pelo detentor da AIM

<b>ABBVIE LTD</b>	RESPREEZA	<b>MERCK SHARP &amp; DOHME LTD</b>
HUMIRA	VONCENTO	CANCIDAS
<b>ABZ-PHARMA GMBH</b>	<b>CTI LIFE SCIENCES LTD</b>	INTRONA
BIOGRASTIM	PIXUVRI	NOXAFIL
<b>ACCORD HEALTHCARE LTD</b>	<b>EISAI LTD</b>	PUREGON
BORTEZOMIB ACCORD	PANRETIN	TEMODAL
PEMETREXED ACCORD	PRIALT	<b>NORDIC GROUP BV</b>
<b>ACTAVIS GROUP PTC EHF</b>	TARGRETIN	TEYSUNO
ARMISARTE	<b>ELI LILLY NEDERLAND B.V.</b>	<b>NOVARTIS EUROPHARM LTD</b>
<b>ACTELION REGISTRATION LTD</b>	ADCIRCA	AFINITOR
TRACLEER	ALIMTA	GLIVEC
ZAVESCA	PEMETREXED LILLY	HYCANTIN
<b>AEGERION PHARMACEUTICALS SAS</b>	<b>FINOX BIOTECH AG</b>	ILARIS
LOJUXTA	BEMFOLA	JAKAVI
<b>ALLERGAN PHARMACEUTICALS IRELAND</b>	<b>FOREST LABORATORIES UK LTD</b>	REVLADE
OZURDEX	COLOBRETHE	VOTRIENT
<b>AMGEN EUROPE BV</b>	<b>GENZYME EUROPE B.V.</b>	<b>NOVO NORDISK A/S</b>
REPATHA	ALDURAZYME	NOVOEIGHT
<b>APOTEX EUROPE B.V.</b>	CEREZYME	NOVOSEVEN
FERRIPROX	FABRAZYME	NOVOTHIRTEEN
GRASTOFIL	MYOZYME	<b>ORPHAN EUROPE S.A.R.L.</b>
<b>APTALIS PHARMA SAS</b>	THYROGEN	CARBAGLU
QUINSAIR	<b>GILEAD SCIENCES INTERNATIONAL LTD</b>	CYSTAGON
<b>ASTRAZENECA AB</b>	ZYDELIG	PEDEA
CAPRELSA	<b>GTC BIOTHERAPEUTICS UK LIMITED</b>	VEDROP
<b>AVENTIS PHARMA S.A.</b>	ATRYN	WILZIN
RILUTEK	<b>HEXAL AG</b>	<b>OTSUKA PHARMACEUTICAL EUROPE LTD</b>
TAXOTERE	FILGRASTIM HEXAL	JINARC
<b>BAXALTA INNOVATIONS GMBH</b>	<b>HOSPIRA UK LTD</b>	<b>PACIRA LIMITED</b>
HYQVIA	DOCETAXEL HOSPIRA UK LTD	DEPOCYTE
OBIZUR	PEMETREXED HOSPIRA	<b>PFIZER LTD</b>
ONCASPAR	NIVESTIM	BENEFIX
RIXUBIS	VORICONAZOLE HOSPIRA	ENBREL
<b>BAXTER AG</b>	<b>INSTITUTO GRIFOLS S.A.</b>	INLYTA
ADVATE	FLEBOGAMMA DIF	REFACTO AF
CEPROTIN	<b>JANSSEN-CILAG INTERNATIONAL NV</b>	REVATIO
KIOVIG	CAELYX	SOMAVERT
<b>BAYER PHARMA AG</b>	VELCADE	SUTENT
HELIXATE NEXGEN	<b>JENSON PHARMACEUTICALS SERVICES LIMITED</b>	VFEND
IBLIAS	NUEDEXTA	<b>PHARMING GROUP N.V.</b>
KOGENATE BAYER	<b>LABORATOIRE HRA PHARMA</b>	RUCONEST
KOVALTRY	LYSODREN	<b>PIERRE FABRE DERMATOLOGIE</b>
VENTAVIS	<b>LINDE HEALTHCARE AB</b>	HEMANGIOL
<b>BIOGEN IDEC LTD</b>	INOMAX	<b>PIERRE FABRE MÉDICAMENTS</b>
ELOCTA	<b>LIPOMED GMBH</b>	BUSILVEX
<b>BIOMARIN EUROPE LTD</b>	LITAK	<b>RATIOPHARM GMBH</b>
NAGLAZYME	<b>MARKLAS NEDERLAND BV</b>	RATIOGRASTIM
<b>BIOTEST PHARMA GMBH</b>	STAYVEER	<b>ROCHE REGISTRATION LTD</b>
ZUTECTRA	<b>MEDAC GMBH</b>	AVASTIN
<b>BRISTOL-MYERS SQUIBB PHARMA EEIG</b>	PEMETREXED MEDAC	ERIVEDGE
ORENCIA	SPECTRILA	HERCEPTIN
<b>CELGENE EUROPE LTD</b>	<b>MERCK KGAA</b>	MABTHERA
ABRAXANE	ERBITUX	ROACTEMRA
<b>CRUCCELL SWEDEN AB</b>	<b>MERCK SERONO EUROPE LTD</b>	TARCEVA
DUKORAL	GONAL-F	XELODA
<b>CSL BEHRING GMBH</b>		<b>SANDOZ GMBH</b>
HIZENTRA		OMNITROPE
PRIVIGEN		PEMETREXED SANDOZ
		ZARZIO

<b>SANQUIN</b>	<b>SWEDISH ORPHAN BIOVITRUM INTERNATIONAL AB</b>	<b>UCB PHARMA SA</b>
NONAFACT	AMMONAPS	KEPPRA
<b>SHIRE HUMAN GENETIC THERAPIES AB</b>	KINERET	<b>VALNEVA AUSTRIA GMBH</b>
REPLAGAL	ORFADIN	IXIARO
<b>SIGMA-TAU INDUSTRIE FARMACEUTICHE RIUNITE S.P.A</b>	<b>TEVA GMBH</b>	<b>VERTEX PHARMACEUTICALS (EUROPE) LTD</b>
EURARTESIM	TEVAGRASTIM	ORKAMBI
<b>SPECTRUM PHARMACEUTICALS B.V.</b>	<b>TEVA PHARMA BV</b>	<b>VIROPHARMA SPRL</b>
ZEVALIN	OVALEAP	BUCCOLAM
	TRISENOX	CINRYZE
	<b>UCB PHARMA LTD</b>	
	XYREM	

---

Por favor, note que todos os dados apresentados neste relatório estão disponíveis para download em [Orphadata](#)

---

Editores: Ana Rath & Valérie Salamon • Fotografia: M. Depardieu/Inserm

*A forma correta de citar este documento é:*

« Lista de medicamentos para doenças raras na Europa », Relatórios Orphanet, *Coleção Medicamentos Órfãos*, abril 2016, [http://www.orpha.net/orphacom/cahiers/docs/PT/Lista\\_de\\_medicamentos\\_orfaos\\_na\\_europa.pdf](http://www.orpha.net/orphacom/cahiers/docs/PT/Lista_de_medicamentos_orfaos_na_europa.pdf)

Esta Série de Relatórios da Orphanet é parte da acção conjunta 677024 RD-Action, que recebeu um financiamento do Programa de Saúde da União Europeia (2014-2020).

O conteúdo destes relatórios representa o ponto de vista do autor e são da sua responsabilidade; não podendo ser considerados como a opinião da Comissão Europeia e/ou da Agência de Execução para os Consumidores, a Saúde, a Agricultura e a Alimentação ou qualquer outro organismo da União Europeia. A comissão Europeia e a Agência não aceitam qualquer responsabilidade pelo uso que possa ser feito das informações contidas nestes relatórios