



Orphanet Report Series

Orphan Drugs collection

January 2012

Lists of Orphan Drugs in Europe

With European orphan designation and European market authorisation*

With European market authorisation* without prior orphan designation in Europe

**European Community marketing authorisation under the centralised procedure*

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Methodology

This document provides a list of all orphan drugs that have received a European marketing authorisation (MA) at the date stated in the document. These medicinal products may now be accessible in some, though not necessarily all, European countries. In reality, the availability of a certain orphan drug in a certain country depends on the strategy of the laboratory and the decision taken by national health authorities concerning reimbursement.

List of orphan drugs in Europe with European orphan designation and European marketing authorisation

Strictly speaking, orphan drugs in Europe are medicinal products that have been granted a European orphan designation (according to the Regulation (EC) No 141/2000), and then that have been granted a European market authorisation and a positive evaluation of significant benefit.

This list is determined by cross-referencing the list of medicinal products that have been granted an orphan designation (<http://ec.europa.eu/health/documents/community-register/html/alforphreg.htm>) with the list of medicinal products that have been granted a marketing authorisation (<http://ec.europa.eu/health/documents/community-register/html/alfregister.htm>).

Both lists are available on the DG health and consumers (DG Sanco) of the European Commission.

This list of drugs is arranged by tradename in alphabetical order.

The information provided here is the tradename, the name of active substance, the marketing authorisation (MA) indication, the date of MA and the MA holder.

In order to allow a multi-criteria search, three additional lists are proposed, sorted :

- by date of MA in descending order
- by ATC category
- by MA holder

List of orphan drugs in Europe with European market authorisation without prior orphan designation in Europe

By extension, the denomination "Orphan drugs" is used for medicinal products that have been granted a European marketing authorisation, but which have not been granted a European orphan designation or for which the designation was withdrawn.

These drugs may have been granted, or not, an orphan designation in another geographical area in the world.


In all cases, they have been granted a European marketing authorisation for one or more indication(s) of use for a rare disease and they appear in the DG Sanco list of medicinal products that have been granted a marketing authorisation : <http://ec.europa.eu/health/documents/community-register/html/alfregister.htm>

This list of drugs is arranged by tradenames in alphabetical order.

The information provided is the tradename, the name of active substance, the "rare disease" indication of marketing authorisation (MA), the date of MA and the MA holder.

In order to allow a multi-criteria search, three additional lists are proposed, sorted :

- by date of MA in descending order
- by ATC category
- by MA holder

You may find additional information on each product in the tab "Orphan drugs" on Orphanet website www.orpha.net or on the EMA website (European Medicines Agency) <http://www.ema.europa.eu>. The EMA listing covers all marketing authorised drugs, not just orphan drugs. Orphan drugs that have been granted a European orphan designation are indicated by the logo .

Information is available in up to 22 European languages.

For any questions or comments, please contact us: contact.orphanet@inserm.fr

List of orphan drugs in Europe with European orphan designation and European marketing authorisation

1- By tradename in alphabetical order

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
AFINITOR	Everolimus (INN)	This product is no longer an orphan medicine. It was originally designated an orphan medicine on 5 June 2007. Upon request of the marketing authorisation holder, Afinitor has now been removed from the Community Register of orphan medicinal products. Cf "List of orphan drugs in Europe with European market authorisation without prior orphan designation in Europe"		
ALDURAZYME	Laronidase (INN)	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis I (MPS I; a [alpha]-L-iduronidase deficiency) to treat the non-neurological manifestations of the disease	10/06/2003	Genzyme Europe B.V.
ARZERRA	Ofatumumab (INN)	Treatment of chronic lymphocytic leukaemia (CLL) in patients who are refractory to fludarabine and alemtuzumab	19/04/2010	Glaxo Group Ltd
ATRIANCE	Nelarabine (INN)	Treatment of patients with T-cell acute lymphoblastic leukaemia (T-ALL) and T-cell lymphoblastic lymphoma (T-LBL) whose disease has not responded to or has relapsed following treatment with at least two chemotherapy regimens	22/08/2007	Glaxo Group Ltd
BUSILVEX	Busulfan (INN) (Intravenous use)	Followed by cyclophosphamide (BuCy2) is indicated as conditioning treatment prior to conventional haematopoietic progenitor cell transplantation (HPCT) in adult patients when the combination is considered the best available option Followed by cyclophosphamide (BuCy4) or melphalan (BuMel) is indicated as conditioning treatment prior to conventional haematopoietic progenitor cell transplantation in paediatric patients	09/07/2003	Pierre Fabre Médicament
CARBAGLU	Carglumic acid (INN)	Treatment of hyperammonaemia due to N-acetylglutamate synthase primary deficiency, hyperammonaemia due to isovaleric acidaemia, hyperammonaemia due to methymalonic acidaemia, hyperammonaemia due to propionic acidaemia	24/01/2003	Orphan Europe S.a.r.l.
CAYSTON	Aztreonam (INN)	Suppressive therapy of chronic pulmonary infections due to <i>Pseudomonas aeruginosa</i> in patients with cystic fibrosis (CF) aged 18 years and older	21/09/2009	Gilead Sciences International Limited
CEPLENE	Histamine dihydrochloride	Treatment of adult patients with acute myeloid leukaemia in first remission concomitantly treated with interleukin-2 (IL-2)	07/10/2008	EpiCept GmbH

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
CYSTADANE	Betaine anhydrous (INN)	Adjunctive treatment of homocystinuria , involving deficiencies or defects in cystathionine beta-synthase (CBS), 5,10-methylene-tetrahydrofolate reductase (MTHFR), cobalamin cofactor metabolism (cbl). Cystadane should be used as supplement to other therapies such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), folate and a specific diet	15/02/2007	Orphan Europe S.a.r.l.
DIACOMIT	Stiripentol (INN)	Use in conjunction with clobazam and valproate as adjunctive therapy of refractory generalized tonic-clonic seizures in patients with severe myoclonic epilepsy in infancy (SMEI, Dravet's syndrome) whose seizures are not adequately controlled with clobazam and valproate	04/01/2007	Biocodex
ELAPRASE	Idursulfase (INN)	Long-term treatment of patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II)	08/01/2007	Shire Human Genetic Therapies AB
ESBRIET	Pirfenidone	In adults for the treatment of mild to moderate Idiopathic Pulmonary Fibrosis (IPF)	28/02/2011	InterMune UK Ltd.
EVOLTRA	Clofarabine (INN)	Treatment of acute lymphoblastic leukaemia (ALL) in paediatric patients who have relapsed or are refractory after receiving at least two prior regimens and where there is no other treatment option anticipated to result in a durable response	29/05/2006	Genzyme Europe B.V.
EXJADE	Deferasirox (INN)	Treatment of chronic iron overload due to frequent blood transfusions (≥ 7 ml/kg/month of packed red blood cells) in patients with beta thalassaemia major aged 6 years and older. Treatment of chronic iron overload due to blood transfusions when deferoxamine therapy is contraindicated or inadequate in the following patient groups : in patients with other anaemias, in patients aged 2 to 5 years, in patients with beta thalassaemia major with iron overload due to infrequent blood transfusions (<7 ml/kg/month of packed red blood cells)	28/08/2006	Novartis Europharm Ltd
FABRAZYME	Recombinant human alpha-galactosidase A INN = Agalsidase beta	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency)	03/08/2001	Genzyme Europe B.V.
FIRAZYR	Icatibant acetate INN = Icatibant	Symptomatic treatment of acute attacks of hereditary angioedema (HAE) in adults (with C1-esterase-inhibitor deficiency)	11/07/2008	Shire Orphan Therapies GmbH
FIRDAPSE (ex-ZENAS)	Amifampridine (INN)	Symptomatic treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults	23/12/2009	Biomarin Europe Ltd
GLIOLAN	5-aminolevulinic acid hydrochloride (INN)	Visualisation of malignant tissue during surgery for malignant glioma (WHO grade III and IV)	07/09/2007	Medac GmbH

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
GLIVEC	Imatinib mesilate (INN)	<p>Treatment of :</p> <ul style="list-style-type: none"> - adult and paediatric patients with newly diagnosed Philadelphia chromosome (bcr-abl) positive (Ph+) chronic myeloid leukaemia (CML) for whom bone marrow transplantation is not considered as the first line of treatment - adult and paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy, or in accelerated phase or blast crisis - adult patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) integrated with chemotherapy - adult patients with relapsed or refractory Ph+ ALL as monotherapy - adult patients with myelodysplastic/myeloproliferative diseases (MDS/MPD) associated with platelet-derived growth factor receptor (PDGFR) gene re-arrangements - adult patients with advanced hypereosinophilic syndrome (HES) and/or chronic eosinophilic leukaemia (CEL) with FIP1L1-PDGFRα rearrangement - adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumours (GIST) - adjuvant treatment of adult patients who are at significant risk of relapse following resection of Kit (CD117)-positive GIST. Patients who have a low or very low risk of recurrence should not receive adjuvant treatment - adult patients with unresectable dermatofibrosarcoma protuberans (DFSP) and adult patients with recurrent and/or metastatic DFSP who are not eligible for surgery 	07/11/2001	Novartis Europharm Ltd
ILARIS	Canakinumab (INN)	This product is no longer an orphan medicine. It was originally designated an orphan medicine on 20 March 2007. Upon request of the marketing authorisation holder, Ilaris has now been removed from the Community Register of orphan medicinal products. Cf "List of orphan drugs in Europe with European market authorisation without prior orphan designation in Europe"		
INCRELEX	Mecasermin (INN)	Long-term treatment of growth failure in children and adolescents with severe primary insulin-like growth factor 1 deficiency (Primary IGFD)	03/08/2007	Ipsen Pharma
INOVELON	Rufinamide (INN)	Adjunctive therapy in the treatment of seizures associated with Lennox Gastaut syndrome in patients aged 4 years and older	16/01/2007	Eisai Ltd
KUVAN	Sapropterin dihydrochloride INN = Sapropterin	<p>Treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients of 4 years of age and over with phenylketonuria (PKU) who have been shown to be responsive to such treatment</p> <p>Treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients with tetrahydrobiopterin (BH4) deficiency who have been shown to be responsive to such treatment</p>	02/12/2008	Merck Serono Europe Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
LITAK	Cladribine (INN) (subcutaneous use)	Treatment of hairy cell leukaemia	14/04/2004	Lipomed GmbH
LYSODREN	Mitotane (INN)	Symptomatic treatment of advanced (unresectable, metastatic or relapsed) adrenal cortical carcinoma	28/04/2004	Laboratoire HRA Pharma
MEPACT	Mifamurtide (INN)	In children, adolescents and young adults for the treatment of high-grade resectable non-metastatic osteosarcoma after macroscopically complete surgical resection. It is used in combination with post-operative multi-agent chemotherapy	06/03/2009	IDM Pharma SAS
MOZOBIL	Plerixafor (INN)	In combination with G-CSF to enhance mobilisation of haematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in patients with lymphoma and multiple myeloma whose cells mobilise poorly	31/07/2009	Genzyme Europe B.V.
MYOZYME	Recombinant human acid alpha-glucosidase INN = Alglucosidase alpha	Long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of Pompe disease (acid α -glucosidase deficiency)	29/03/2006	Genzyme Europe B.V.
NAGLAZYME	N-acetylgalactosamine 4-sulfatase INN = Galsulfase	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis VI (MPS VI; N-acetylgalactosamine 4-sulfatase deficiency; Maroteaux-Lamy syndrome)	24/01/2006	BioMarin Europe Ltd
NEXAVAR	Sorafenib tosylate Sorafenib (INN)	Treatment of hepatocellular carcinoma Treatment of patients with advanced renal cell carcinoma who have failed prior interferon-alpha or interleukin-2 based therapy or are considered unsuitable for such therapy	19/07/2006	Bayer Pharma AG
NPLATE	Romiplostim (INN)	Adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) in splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Nplate may be considered as second line treatment for adult non-splenectomised patients where surgery is contra-indicated	04/02/2009	Amgen Europe B.V.
ONSENAL	Celecoxib (INN)	Reduction of the number of adenomatous intestinal polyps in familial adenomatous polyposis (FAP), as an adjunct to surgery and further endoscopic surveillance This medicine is now withdrawn from use in the European Union, more information on: www.ema.europa.eu	17/10/2003	Pfizer Ltd
ORFADIN	Nitisinone (INN)	Treatment of patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine	21/02/2005	Swedish Orphan Biovitrum International AB

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
PEDEA	Ibuprofen (INN)	Treatment of a haemodynamically significant patent ductus arteriosus in preterm newborn infants less than 34 weeks of gestational age	29/07/2004	Orphan Europe S.a.r.l.
PEYONA (ex-NYMUSA)	Caffeine citrate	Treatment of primary apnea of premature newborns	02/07/2009	Chiesi Farmaceutici SpA
PHOTOBARR	Porfimer sodium (INN) (for use with photodynamic therapy)	Ablation of high-grade dysplasia (HGD) in patients with Barrett's Oesophagus	25/03/2004	Pinnacle Biologics B.V.
PLENADREN	Hydrocortisone	Treatment of adrenal insufficiency in adults.	03/11/2011	DuoCort Pharma AB
PRIALT	Ziconotide (INN) (intraspinal use)	Treatment of severe, chronic pain in patients who require intrathecal (IT) analgesia	21/02/2005	Eisai Ltd
REPLAGAL	Agalsidase alfa (INN)	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry Disease (alpha-galactosidase A deficiency)	03/08/2001	Shire Human Genetic Therapies AB
REVATIO	Sildenafil citrate INN = Sildenafil	Treatment of adult patients with pulmonary arterial hypertension classified as WHO functional class II and III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and pulmonary hypertension associated with connective tissue disease. Treatment of paediatric patients aged 1 year to 17 years old with pulmonary arterial hypertension. Efficacy in terms of improvement of exercise capacity or pulmonary haemodynamics has been shown in primary pulmonary hypertension and pulmonary hypertension associated with congenital heart disease. Revatio solution for injection is for the treatment of adult patients with pulmonary arterial hypertension who are currently prescribed oral Revatio and who are temporarily unable to take oral therapy, but are otherwise clinically and haemodynamically stable. Revatio (oral) is indicated for treatment of adult patients with pulmonary arterial hypertension classified as WHO functional class II and III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and pulmonary hypertension associated with connective tissue disease	28/10/2005	Pfizer Ltd
REVLIMID	Lenalidomide (INN)	In combination with dexamethasone, treatment of multiple myeloma patients who have received at least one prior therapy	14/06/2007	Celgene Europe Ltd
REVOLADE	Eltrombopag (INN)	For adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) 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	11/03/2010	Glaxo-SmithKline Trading Services Limited

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
RILONACEPT REGENERON (ex-ARCALYST)	Rilonacept (INN)	Treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) with severe symptoms, including Familial Cold Autoinflammatory Syndrome (FCAS) and Muckle-Wells Syndrome (MWS), in adults and children aged 12 years and older	23/10/2009	Regeneron UK Limited
SAVENE	Dexrazoxane (INN)	Treatment of anthracycline extravasation	28/07/2006	SpePharm Holding BV
SIKLOS	Hydroxycarbamide (INN)	Prevention of recurrent painful vaso-occlusive crises including acute chest syndrome in paediatric and adult patients suffering from symptomatic Sickle Cell Syndrome	29/06/2007	Addmedica
SOLIRIS	Eculizumab (INN)	Treatment of patients with: - Paroxysmal nocturnal haemoglobinuria (PNH). Evidence of clinical benefit of Soliris in the treatment of patients with PNH is limited to patients with history of transfusions. - Atypical haemolytic uremic syndrome (aHUS).	20/06/2007	Alexion Europe SAS
SOMAVERT	Pegvisomant (INN)	Treatment of patients with acromegaly who have had an inadequate response to surgery and/or radiation therapy and in whom an appropriate medical treatment with somatostatin analogues did not normalize IGF-I concentrations or was not tolerated	13/11/2002	Pfizer Ltd
SPRYCEL	Dasatinib (INN)	Treatment of adult patients with: - newly diagnosed Philadelphia chromosome positive (Ph+) chronic myelogenous leukaemia (CML) in the chronic phase. - chronic, accelerated or blast phase CML with resistance or intolerance to prior therapy including imatinib mesilate. - Ph+ acute lymphoblastic leukaemia (ALL) and lymphoid blast CML with resistance or intolerance to prior therapy.	20/11/2006	Bristol-Myers Squibb Pharma EEIG
SUTENT	Sunitinib malate Sunitinib (INN)	This product was originally an orphan designated on 10 March 2005. Upon request by the MAH, Sutent has now been removed from the Community Register of Orphan Medicinal products. Cf "List of orphan drugs in Europe with European market authorisation without prior orphan designation in Europe"		
TASIGNA	Nilotinib (INN)	150 mg: Treatment of adult patients with newly diagnosed Philadelphia chromosome positive chronic myelogenous leukaemia (CML) in the chronic phase. 200 mg: Treatment of adult patients with: - newly diagnosed Philadelphia chromosome positive chronic myelogenous leukaemia (CML) in the chronic phase - chronic phase and accelerated phase Philadelphia chromosome positive chronic myelogenous leukaemia (CML) with resistance or intolerance to prior therapy including imatinib. Efficacy data in patients with CML in blast crisis are not available.	19/11/2007	Novartis Europharm Ltd

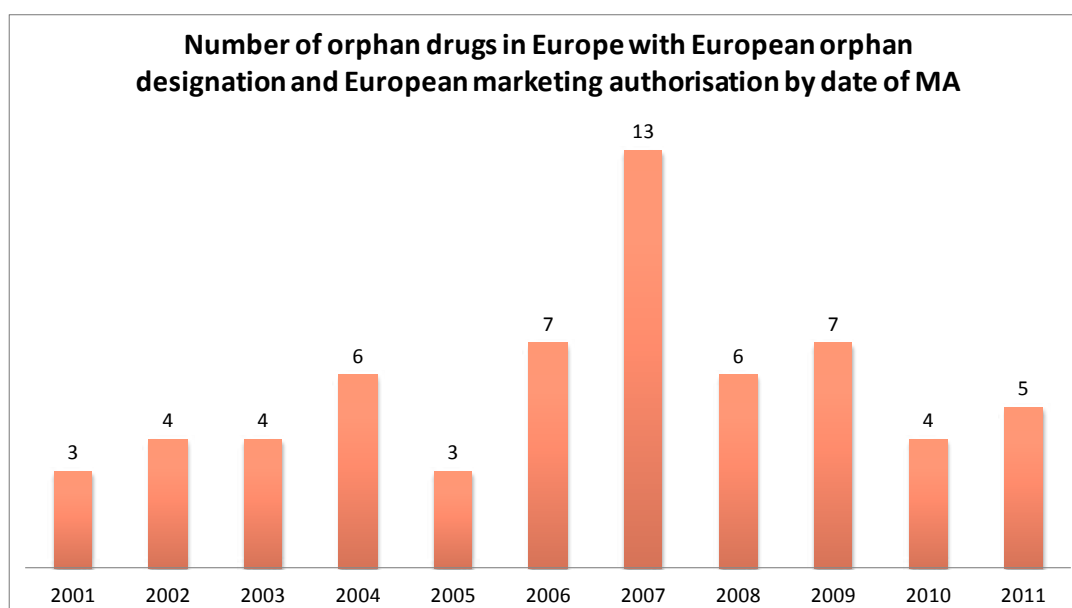
TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
TEPADINA	Thiotepa (INN)	In combination with other chemotherapy medicinal products: 1) with or without total body irradiation (TBI), as conditioning treatment prior to allogeneic or autologous haematopoietic progenitor cell transplantation (HPCT) in haematological diseases in adult and paediatric patients; 2) when high dose chemotherapy with HPCT support is appropriate for the treatment of solid tumours in adult and paediatric patients. It is proposed that Tepadina must be prescribed by physicians experienced in conditioning treatment prior to haematopoietic progenitor cell transplantation.	15/03/2010	Adienne S.r.l.
THALIDOMIDE CELGENE	Thalidomide (INN)	In combination with melphalan and prednisone as first line treatment of patients with untreated multiple myeloma , aged \geq 65 years or ineligible for high dose chemotherapy	16/04/2008	Celgene Europe Ltd
THELIN	Sitaxentan sodium (INN)	Treatment of patients with pulmonary arterial hypertension classified as WHO functional class III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and in pulmonary hypertension associated with connective tissue disease. <i>This medicine is now withdrawn from use in the European Union, more information on:</i> www.ema.europa.eu	10/08/2006	Pfizer Ltd
TOBI PODHALER	Tobramycin	Suppressive therapy of chronic pulmonary infection due to Pseudomonas aeruginosa in adults and children aged 6 years and older with cystic fibrosis	20/07/2011	Novartis Europharm Limited
TORISEL	Temsirolimus (INN)	First-line treatment of patients with advanced renal cell carcinoma who have at least three of six prognostic risk factors Treatment of adult patients with relapsed and/or refractory mantle cell lymphoma (MCL)	19/11/2007	Pfizer Limited
TRACLEER	Bosentan mono-hydrate INN = Bosentan	Treatment of pulmonary arterial hypertension (PAH) to improve exercise capacity and symptoms in patients with WHO functional class III. Efficacy has been shown in: Primary (idiopathic and familial) PAH, PAH secondary to scleroderma without significant interstitial pulmonary disease, PAH associated with congenital systemic-to-pulmonary shunts and Eisenmenger's physiology. Some improvements have also been shown in patients with PAH WHO functional class II. To reduce the number of new digital ulcers in patients with systemic sclerosis and ongoing digital ulcer disease	15/05/2002	Actelion Registration Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
TRISENOX	Arsenic Trioxide (INN)	Induction of remission and consolidation in adult patients with relapsed/refractory acute promyelocytic leukaemia (APL), characterised by the presence of the t(15;17) translocation and/or the presence of the Pro-Myelocytic Leukaemia/Retinoic-Acid Receptor-alpha (PML/RAR-alpha) gene. Previous treatment should have included a retinoid and chemotherapy	05/03/2002	Cephalon Europe
VENTAVIS	Iloprost (INN)	Treatment of patients with primary pulmonary hypertension , classified as NYHA functional class III, to improve exercise capacity and symptoms	16/09/2003	Bayer Schering Pharma AG
VIDAZA	Azacitidine (INN)	Treatment of adult patients who are not eligible for haematopoietic stem cell transplantation with: -intermediate 2 and high risk myelodysplastic syndromes (MDS) according to the International Prognostic Scoring System (IPSS) - chronic myelomonocytic leukaemia (CMML) with 10-29 % marrow blasts without myeloproliferative disorder - acute myeloid leukaemia (AML) with 20-30 % blasts and multi-lineage dysplasia, according to World Health Organisation (WHO) classification	17/12/2008	Celgene Europe Ltd
VOLIBRIS	Ambrisentan (INN)	Treatment of patients with pulmonary arterial hypertension (PAH) classified as WHO functional class II and III, to improve exercise capacity. Efficacy has been shown in idiopathic PAH (IPAH) and in PAH associated with connective tissue disease	21/04/2008	Glaxo Group Ltd
VOTUBIA	Everolimus	Treatment of patients aged 3 years and older with subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis complex (TSC) who require therapeutic intervention but are not amenable to surgery. The evidence is based on analysis of change in SEGA volume. Further clinical benefit, such as improvement in disease-related symptoms, has not been demonstrated.	02/09/2011	Novartis Europharm Ltd
VPRIV	Velaglucerase alfa	Long-term enzyme replacement therapy (ERT) in patients with type 1 Gaucher disease	26/08/2010	Shire Pharmaceuticals Ireland Ltd
VYNDAQEL	Tafamidis	Treatment of transthyretin amyloidosis in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment.	16/11/2011	Pfizer Specialty UK Ltd
WILZIN	Zinc acetate dihydrate (INN)	Treatment of Wilson's disease	13/10/2004	Orphan Europe S.a.r.l.
XAGRID	Anagrelide hydrochloride INN = Anagrelide	Reduction of elevated platelet counts in at risk essential thrombocythaemia patients who are intolerant to their current therapy or whose elevated platelet counts are not reduced to an acceptable level by their current therapy	16/11/2004	Shire Pharmaceutical Contracts Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
XYREM	Sodium oxybate (INN)	This product was originally an orphan designated on 3 February 2003. Upon request by the MAH, Xyrem has now been removed from the Community Register of Orphan Medicinal products. Cf "List of orphan drugs in Europe with European market authorisation without prior orphan designation in Europe"		
YONDELIS	Trabectedin (INN)	Treatment of patients with advanced soft tissue sarcoma , after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents. Efficacy data are based mainly on liposarcoma and leiomyosarcoma patients In combination with pegylated liposomal doxorubicin (PLD), treatment of patients with relapsed platinum-sensitive ovarian cancer	17/09/2007	Pharma Mar S.A.
ZAVESCA	Miglustat (INN)	Oral treatment of mild to moderate type 1 Gaucher disease in patients for whom enzyme replacement therapy is unsuitable Treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type C disease	20/11/2002	Actelion Registration Ltd

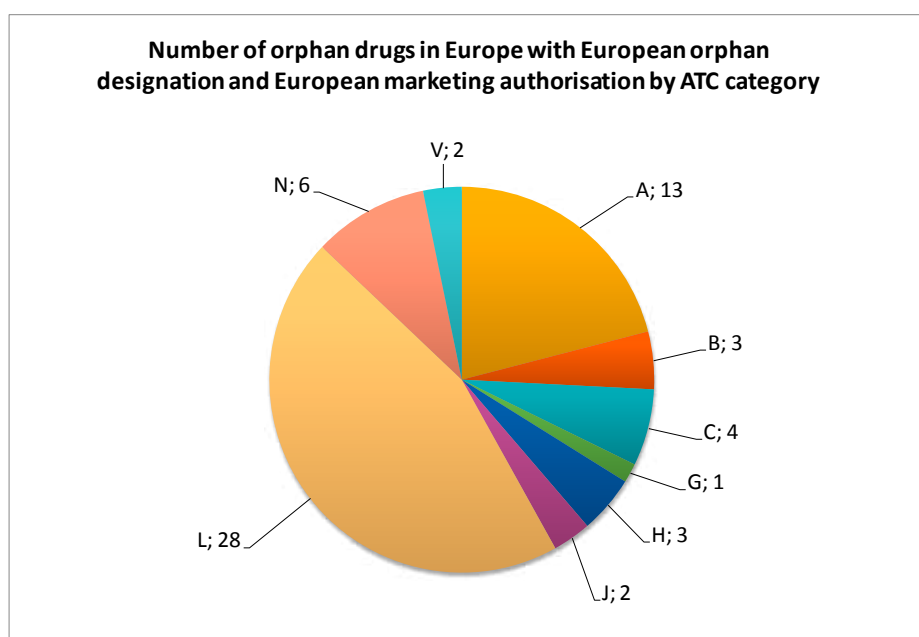
2- By date of MA in descending order

2011	2008	2006	2003
ESBRIET	CEPLENE	EVOLTRA	ALDURAZYME
PLENADREN	FIRAZYR	EXJADE	BUSILVEX
TOBI PODHALER	KUVAN	MYOZYME	CARBAGLU
VOTUBIA	THALIDOMIDE CELGENE	NAGLAZYME	VENTAVIS
VYNDAQEL	VIDAZA	NEXAVAR	2002
2010	VOLIBRIS	SAVENE	SOMAVERT
ARZERRA	2007	SPRYCEL	TRACLEER
REVOLADE	ATRIANCE	2005	TRISENOX
TEPADINA	CYSTADANE	ORFADIN	ZAVESCA
VPRIV	DIACOMIT	PRIALT	2001
2009	ELAPRASE	REVATIO	FABRAZYME
CAYSTON	GLIOLAN	2004	GLIVEC
FIRDAPSE (ex-ZENAS)	INCRELEX	LITAK	REPLAGAL
MEPACT	INOVELON	LYSODREN	
MOZOBIL	REVLIMID	PEDEA	
NPLATE	SIKLOS	PHOTOBARR	
PEYONA (ex-NYMUSA)	SOLIRIS	WILZIN	
RILONACEPT REGEN- ERON (ex-ARCALYST)	TASIGNA	XAGRID	
	TORISEL		
	YONDELIS		



3- By ATC category

A- ALIMENTARY TRACT AND METABOLISM	C- CARDIOVASCULAR SYSTEM	L- ANTINEOPLASTIC AND IMMUNOMODULATING AGENTS	SPRYCEL
ALDURAZYME	FIRAZYR	ARZERRA	TASIGNA
CARBAGLU	PEDEA	ATRIANCE	TEPADINA
CYSTADANE	TRACLEER	BUSILVEX	THALIDOMIDE CELGENE
ELAPRASE	VOLIBRIS	CEPLENE	TORISEL
FABRAZYME	G- GENITO URINARY SYSTEM AND SEX HORMONES	ESBRIET	TRISENOX
KUVAN	REVATIO	EVOLTRA	VIDAZA
MYOZYME	H- SYSTEMIC HORMONAL PREPARATIONS, EXCL, SEX HORMONES AND INSULINS	GLIOLAN	VOTUBIA
NAGLAZYME	INCRELEX	GLIVEC	XAGRID
ORFADIN	PLENADREN	LITAK	YONDELIS
REPLAGAL	SOMAVERT	LYSODREN	N- NERVOUS SYSTEM
VPRIV	J- GENERAL ANTIINFECTIVES FOR SYSTEMIC USE	MEPACT	DIACOMIT
WILZIN	CAYSTON	MOZOBIL	FIRDAPSE (ex-ZENAS)
ZAVESCA	TOBI PODHALER	NEXAVAR	INOVELON
B- BLOOD AND BLOOD FORMING ORGANS		PHOTOBARR	PEYONA (ex-NYMUSA)
NPLATE		REVLIMID	PRIALT
REVOLADE		RILONACEPT REGENERON (ex-ARCALYST)	VYNDAQEL
VENTAVIS		SIKLOS	V- VARIOUS
		SOLIRIS	EXJADE
			SAVENE



4- By MA holder

ACTELION REGISTRATION LTD	DUOCORT PHARMA AB	MEDAC GMBH	SHIRE HUMAN GENETIC THERAPIES AB
TRACLEER	PLENADREN	GLIOLAN	ELAPRASE
ZAVESCA	EISAI LTD	MERCK SERONO EUROPE LTD.	REPLAGAL
ADDMEDICA	INOVELON	KUVAN	SHIRE ORPHAN THERAPIES GMBH
SIKLOS	PRIALT	NOVARTIS EUROPHARM LTD	FIRAZYR
ADIENNE S.R.L.	EPICEPT GMBH	EXJADE	SHIRE PHARMACEUTICAL CONTRACTS LTD
TEPADINA	CEPLENE	GLIVEC	XAGRID
ALEXION EUROPE SAS	GENZYME EUROPE B.V.	TASIGNA	SHIRE PHARMACEUTICALS IRELAND LTD.
SOLIRIS	ALDURAZYME	TOBI PODHALER	VPRIV
AMGEN EUROPE B.V.	EVOLTRA	VOTUBIA	SPEPHARM HOLDING BV
NPLATE	FABRAZYME	ORPHAN EUROPE S.A.R.L.	SAVENE
BAYER PHARMA AG	MOZOBIL	CARBAGLU	SWEDISH ORPHAN BIOVITRUM INTERNATIONAL AB
NEXAVAR	MYOZYME	CYSTADANE	ORFADIN
BAYER SCHERING PHARMA AG	GILEAD SCIENCES INTERNATIONAL LIMITED	PEDEA	
VENTAVIS	CAYSTON	WILZIN	
BIOCODEX	GLAXO GROUP LTD	PFIZER LTD	
DIACOMIT	ARZERRA	REVATIO	
BIOMARIN EUROPE LTD	ATRIANCE	SOMAVERT	
FIRDAPSE	VOLIBRIS	TORISEL	
NAGLAZYME	GLAXOSMITHKLINE TRADING SERVICES LIMITED	PFIZER SPECIALTY UK LTD	
BRISTOL-MYERS SQUIBB PHARMA EEIG	REVOLADE	VYNDAQEL	
SPRYCEL	IDM PHARMA SAS	PINNACLE BIOLOGICS B.V.	
CELGENE EUROPE LTD	MEPACT	PHOTOBARR	
REVLIMID	IPSEN PHARMA	PHARMA MAR S.A.	
THALIDOMIDE CELGENE	INCRELEX	YONDELIS	
VIDAZA	INTERMUNE UK LTD.	PIERRE FABRE MÉDICAMENT	
CEPHALON EUROPE	ESBRIET	BUSILVEX	
TRISENOX	LABORATOIRE HRA PHARMA	REGENERON UK LIMITED	
CHIESI FARMACEUTICI SPA	LYSODREN	RILONACEPT REGENERON	
PEYONA	LIPOMED GMBH		
	LITAK		

List of orphan drugs in Europe with European market authorisation without prior orphan designation in Europe

1- By tradename in alphabetical order

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
ADCIRCA	Tadalafil (INN)	Treatment of pulmonary arterial hypertension (PAH) classified as WHO functional class II and III, to improve exercise capacity. Efficacy has been shown in idiopathic PAH (IPAH) and in PAH related to collagen vascular disease	30/11/2009	Eli Lilly Nederland B.V.
ADVATE	Octocog alpha (INN)	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency)	02/03/2004	Baxter AG
AFINITOR	Everolimus (INN)	Treatment of unresectable or metastatic, well- or moderately-differentiated neuroendocrine tumours of pancreatic origin in adults with progressive disease. Treatment of patients with advanced renal cell carcinoma , whose disease has progressed on or after treatment with VEGF-targeted therapy.	03/08/2009	Novartis Europharm Ltd
ALIMTA	Pemetrexed (INN)	In combination with cisplatin for the treatment of chemotherapy naïve patients with unresectable malignant pleural mesothelioma	20/09/2004	Eli Lilly Nederland B.V.
AMMONAPS	Sodium phenylbutyrate (INN)	Adjunctive therapy in the chronic management of urea cycle disorders , involving deficiencies of carbamyl phosphate synthetase, ornithine transcarbamylase, or argininosuccinate synthetase. It is indicated in all patients with neonatal-onset presentation (complete enzyme deficiencies, presenting within the first 28 days of life). It is also indicated in patients with late-onset disease (partial enzyme deficiencies, presenting after the first month of life) who have a history of hyperammonaemic encephalopathy	08/12/1999	Swedish Orphan Biovitrum International AB
ATRYN	Antithrombin alpha (INN)	Prophylaxis of venous thromboembolism in surgery of patients with congenital antithrombin deficiency , normally given in association with heparin or low molecular weight heparin	28/07/2006	GTC Biotherapeutics UK Limited
AVASTIN	Bevacizumab (INN)	In combination with interferon alfa-2a, for first line treatment of patients with advanced and/or metastatic renal cell cancer	12/01/2005	Roche Registration Limited
BENEFIX	Recombinant coagulation Factor IX INN = Nonacog alpha	Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency)	27/08/1997	Wyeth Europa Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
BIOGRASTIM	Filgrastim (INN)	In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$, and a history of severe or recurrent infections	15/09/2008	CT Arzneimittel GmbH
BUCCOLAM	Midazolam	Treatment of prolonged, acute, convulsive seizures in infants, toddlers, children and adolescents (from 3 months to < 18 years)	05/09/2011	ViroPharma SPRL
CAELYX	Doxorubicin hydrochloride (pegylated liposomal)	For treatment of advanced ovarian cancer in women who have failed a first-line platinum-based chemotherapy regimen In combination with bortezomib for the treatment of progressive multiple myeloma in patients who have received at least one prior therapy and who have already undergone or are unsuitable for bone marrow transplant Treatment of AIDS-related Kaposi's sarcoma (KS) in patients with low CD4 counts (< 200 CD4 lymphocytes/mm ³) and extensive mucocutaneous or visceral disease	21/06/1996	Janssen-Cilag International N.V.
CANCIDAS	Caspofungin	Treatment of invasive aspergillosis in adult or paediatric patients who are refractory to or intolerant of amphotericin B, lipid formulations of amphotericin B and/or itraconazole. Empirical therapy for presumed fungal infections (such as Candida or Aspergillus) in febrile, neutropaenic adult or paediatric patients	24/10/2001	Merck Sharp & Dohme Ltd
CEPROTIN	Human protein C (INN)	In purpura fulminans and coumarin-induced skin necrosis in patients with severe congenital protein C deficiency Short-term prophylaxis in patients with severe congenital protein C deficiency : if surgery or invasive therapy is imminent, while initiating coumarin therapy, when coumarin therapy alone is not sufficient, when coumarin therapy is not feasible	16/07/2001	Baxter AG
CEREZYME	Imiglucerase (INN)	Longterm enzyme replacement therapy in patients with a confirmed diagnosis of non-neuronopathic (Type 1) or chronic neuronopathic (Type 3) Gaucher disease and who exhibit clinically significant non-neurological manifestations of the disease, including one or more of the following conditions : anaemia after exclusion of other causes, such as iron deficiency; thrombocytopenia; bone disease after exclusion of other causes such as Vitamin D deficiency; hepatomegaly or splenomegaly	17/11/1997	Genzyme Europe B.V.
CINRYZE	C1 inhibitor (human)	Treatment and pre-procedure prevention of angioedema attacks in adults and adolescents with hereditary angioedema (HAE) . Routine prevention of angioedema attacks in adults and adolescents with severe and recurrent attacks of hereditary angioedema (HAE), who are intolerant to or insufficiently protected by oral prevention treatments, or patients who are inadequately managed with repeated acute treatment	15/06/2011	ViroPharma SPRL

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
CYSTAGON	Mercaptamine bitartrate (INN)	Treatment of proven nephropathic cystinosis Cysteamine reduces cystine accumulation in some cells (e.g. leukocytes, muscle and liver cells) of nephropathic cystinosis patients and, when treatment is started early, it delays the development of renal failure	23/06/1997	Orphan Europe S.A.R.L.
DUKORAL	Vibrio cholerae and recombinant cholera toxin B-subunit	Active immunisation against disease caused by Vibrio cholerae serogroup O1 in adults and children from 2 years of age who will be visiting endemic/epidemic areas	28/04/2004	Crucell Sweden AB
ENBREL	Etanercept (INN)	Treatment of active polyarticular juvenile idiopathic arthritis in children and adolescents from the age of 2 years who have had an inadequate response to, or who have proved intolerant of, methotrexate. Enbrel has not been studied in children aged less than 2 years	03/02/2000	Pfizer Ltd
ERBITUX	Cetuximab (INN)	Treatment of patients with squamous cell cancer of the head and neck , in combination with radiation therapy for locally advanced disease and in combination with platinum-based chemotherapy for recurrent and/or metastatic disease	29/06/2004	Merck KGaA
EURARTESIM	Piperaquine tetraphosphate / dihydroartemisinin	Treatment of uncomplicated Plasmodium falciparum malaria in adults, children and infants 6 months and over and weighing 5 kg or more.	27/10/2011	Sigma-Tau Industrie Farmaceutiche Riunite S.p.A
FERRIPROX	Deferiprone (INN)	Treatment of iron overload in patients with thalassaemia major when deferoxamine therapy is contraindicated or inadequate	25/08/1999	Apotex Europe B.V.
FILGRASTIM HEXAL	Filgrastim (INN)	In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$, and a history of severe or recurrent infections	06/02/2009	Hexal AG
FLEBOGAMMA DIF	Human normal immunoglobulin	Replacement therapy in: Primary immunodeficiency syndromes such as: - congenital agammaglobulinaemia and hypogammaglobulinaemia - common variable immunodeficiency - severe combined immunodeficiency - Wiskott Aldrich syndrome Myeloma or chronic lymphocytic leukaemia with severe secondary hypogammaglobulinaemia and recurrent infections. Immunomodulation in: Idiopathic thrombocytopenic purpura (ITP) , in children or adults at high risk of bleeding or prior to surgery to correct the platelet count. Guillain Barré syndrome Kawasaki disease	23/07/2007	Instituto Grifols S.A.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
GONAL-F	Recombinant human follicle stimulating hormone INN = Follitropin alpha	Stimulation of spermatogenesis in men who have congenital or acquired hypogonadotropic hypogonadism with concomitant human Chorionic Gonadotrophin (hCG) therapy	20/10/1995	Merck Serono Europe Ltd
HELIXATE NEXGEN	Octocog alpha (INN)	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency)	04/08/2000	Bayer Pharma AG
HERCEPTIN	Trastuzumab (INN)	In combination with capecitabine or 5-fluorouracil and cisplatin is indicated for the treatment of patients with HER2 positive metastatic adenocarcinoma of the stomach or gastro-esophageal junction who have not received prior anti-cancer 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 IHC 3+ result. Accurate and validated assay methods should be used.	28/08/2000	Roche Registration Limited
HIZENTRA	Human normal immunoglobulin (SCIg)	Replacement therapy in adults and children in primary immunodeficiency syndromes such as: - congenital agammaglobulinaemia and hypogammaglobulinaemia - common variable immunodeficiency - severe combined immunodeficiency - IgG subclass deficiencies with recurrent infections - Replacement therapy in myeloma or chronic lymphocytic leukaemia with severe secondary hypogammaglobulinaemia and recurrent infections	14/04/2011	CSL Behring GmbH
HUMIRA	Adalimumab (INN)	In combination with methotrexate for the treatment of active polyarticular juvenile idiopathic arthritis , in adolescents aged 4 to 17 years who have had an inadequate response to one or more disease-modifying anti-rheumatic drugs (DMARDs) As monotherapy in case of intolerance to methotrexate or when continued treatment with methotrexate is inappropriate Humira has not been studied in children aged less than 4 years	08/09/2003	Abbott Laboratories Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
HYCANTIN	Topotecan (INN)	<p>Treatment of patients with metastatic carcinoma of the ovary after failure of first-line or subsequent therapy</p> <p>Treatment of patients with relapsed small cell lung cancer [SCLC] for whom re-treatment with the first-line regimen is not considered appropriate</p> <p>In combination with cisplatin for patients with carcinoma of the cervix recurrent after radiotherapy and for patients with Stage IVB disease. Patients with prior exposure to cisplatin require a sustained treatment free interval to justify treatment with the combination</p> <p>HYCANTIN capsules are indicated as monotherapy for the treatment of adult patients with relapsed small cell lung cancer (SCLC) for whom re-treatment with the first-line regimen is not considered appropriate</p>	12/11/1996	SmithKline Beecham Ltd
ILARIS	Canakinumab (INN)	<p>Treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) in adults, adolescents and children aged 4 years and older with body weight above 15 kg, including:</p> <ul style="list-style-type: none"> - Muckle-Wells Syndrome (MWS), - Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA), - Severe forms of Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU) presenting with signs and symptoms beyond cold-induced urticarial skin rash 	23/10/2009	Novartis Europharm Ltd
INOMAX	Nitric oxide (INN)	<p>In conjunction with ventilatory support and other appropriate active substances:</p> <ul style="list-style-type: none"> - for the treatment of newborn infants ≥ 34 weeks gestation with hypoxic respiratory failure associated with clinical or echocardiographic evidence of pulmonary hypertension, in order to improve oxygenation and to reduce the need for extracorporeal membrane oxygenation. - as part of the treatment of peri- and post-operative pulmonary hypertension in adults and newborn infants, infants and toddlers, children and adolescents, ages 0-17 years in conjunction to heart surgery, in order to selectively decrease pulmonary arterial pressure and improve right ventricular function and oxygenation 	01/08/2001	INO Therapeutics AB

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
INTRONA	Interferon alpha-2b (INN)	Treatment of patients with hairy cell leukaemia Monotherapy treatment of adults with Philadelphia chromosome or bcr/abl translocation positive chronic myelogenous leukaemia Combination therapy with cytarabine administered during the first 12 months of treatment 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 Treatment of patients with multiple myeloma , as maintenance therapy in patients who have achieved objective remission (more than 50 % reduction in myeloma protein) following initial induction chemotherapy Treatment of high tumour burden follicular lymphoma as adjunct to appropriate combination induction chemotherapy such as a CHOP-like regimen Treatment of carcinoid tumours with lymph node or liver metastases and with "carcinoid syndrome"	09/03/2000	Merck Sharp & Dohme Limited
IXIARO	Japanese Encephalitis Vaccine (inactivated, adsorbed)	For active immunization against Japanese encephalitis for adults	31/03/2009	Intercell AG
KEPPRA	Levetiracetam (INN)	As monotherapy in the treatment of partial onset seizures with or without secondary generalisation in patients from 16 years of age with newly diagnosed epilepsy . As adjunctive therapy in the treatment of partial onset seizures with or without secondary generalisation in adults, children and infants from 1 month of age with epilepsy ; in the treatment of myoclonic seizures in adults and adolescents from 12 years of age with Juvenile Myoclonic Epilepsy ; in the treatment of primary generalised tonic-clonic seizures in adults and adolescents from 12 years of age with Idiopathic Generalised Epilepsy	29/09/2000	UCB Pharma SA

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
KIOVIG	Human normal immunoglobulin (INN)	<p>Replacement therapy in adults, and children and adolescents (0-18 years) in:</p> <ul style="list-style-type: none"> - Primary immunodeficiency syndromes with impaired antibody production - Hypogammaglobulinaemia and recurrent bacterial infections in patients with chronic lymphocytic leukaemia, in whom prophylactic antibiotics have failed. - Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase multiple myeloma patients who have failed to respond to pneumococcal immunisation. - Hypogammaglobulinaemia in patients after allogeneic haematopoietic stem cell transplantation (HSCT). <p>Immunomodulation in adults, and children and adolescents (0-18 years) in:</p> <ul style="list-style-type: none"> - Primary immune thrombocytopenia (ITP), in patients at high risk of bleeding or prior to surgery to correct the platelet count. - Guillain Barré syndrome. - Kawasaki disease. - Multifocal Motor Neuropathy (MMN). 	19/01/2006	Baxter AG
KOGENATE BAYER	Octocog alpha (INN)	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency)	04/08/2000	Bayer Pharma AG
MABCAMPATH	Alemtuzumab (INN)	Treatment of patients with B-cell chronic lymphocytic leukaemia (B-CLL) for whom fludarabine combination chemotherapy is not appropriate	06/07/2001	Genzyme Europe BV
MABTHERA	Rituximab (INN)	<p>Indicated in adults for:</p> <p>Treatment of previously untreated patients with stage III-IV follicular lymphoma in combination with chemotherapy</p> <p>Maintenance therapy is indicated for the treatment of follicular lymphoma patients responding to induction therapy</p> <p>Monotherapy is indicated for treatment of patients with stage III-IV follicular lymphoma who are chemoresistant or are in their second or subsequent relapse after chemotherapy</p> <p>Treatment of patients with CD20 positive diffuse large B cell non-Hodgkin's lymphoma in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy</p> <p>In combination with chemotherapy, treatment of patients with previously untreated and relapsed/refractory chronic lymphocytic leukaemia (CLL).</p> <p>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</p>	02/06/1998	Roche Registration Limited

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
NIVESTIM	Filgrastim (INN)	In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$, and a history of severe or recurrent infections	08/06/2010	Hospira UK Ltd
NOVOSEVEN	Human recombinant coagulation Factor VIIa INN = 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 : in patients with congenital haemophilia with inhibitors to coagulation factors VIII or IX > 5 BU; in patients with congenital haemophilia who are expected to have a high anamnestic response to factor VIII or factor IX administration; in patients with acquired haemophilia ; in patients with congenital FVII deficiency ; in patients with Glanzmann's thrombasthenia 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
NOXAFIL	Posaconazole (INN)	Treatment of the fungal infections in adults: - Invasive aspergillosis in patients with disease that is refractory to amphotericin B or itraconazole or in patients who are intolerant of these medicinal products - Fusariosis in patients with disease that is refractory to amphotericin B or in patients who are intolerant of amphotericin B - Chromoblastomycosis and mycetoma in patients with disease that is refractory to itraconazole or in patients who are intolerant of itraconazole - Coccidioidomycosis in patients with disease that is refractory to amphotericin B, itraconazole or fluconazole or in patients who are intolerant of these medicinal products Prophylaxis of invasive fungal infections in : - Patients receiving remission-induction chemotherapy for acute myelogenous leukemia (AML) or myelodysplastic syndromes (MDS) expected to result in prolonged neutropenia and who are at high risk of developing invasive fungal infections - Hematopoietic stem cell transplant (HSCT) recipients who are undergoing high-dose immunosuppressive therapy for graft versus host disease and who are at high risk of developing invasive fungal infections	25/10/2005	Merck Sharp & Dohme Ltd.
OMNITROPE	Somatropin (INN)	Growth disturbance due to insufficient secretion of growth hormone (GH) and growth disturbance associated with Turner syndrome or chronic renal insufficiency. Prader-Willi syndrome (PWS) , for improvement of growth and body composition. Replacement therapy in adults with pronounced growth hormone deficiency (patients with known hypothalamic pituitary pathology and at least one known deficiency of a pituitary hormone not being prolactin)	12/04/2006	Sandoz GmbH

INN - International Nonproprietary Name

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
ORENCIA	Abatacept (INN)	In combination with methotrexate, for the treatment of moderate to severe active polyarticular juvenile idiopathic arthritis (JIA) in paediatric patients 6 years of age and older who have had an insufficient response to other DMARDs including at least one TNF inhibitor	21/05/2007	Bristol-Myers Squibb Pharma EEIG
OZURDEX	Dexamethasone	For the treatment of adult patients with inflammation of the posterior segment of the eye presenting as non-infectious uveitis	27/07/2010	Allergan Pharmaceuticals Ireland
PANRETIN	Alitretinoin (INN)	Topical treatment of cutaneous lesions in patients with AIDS-related Kaposi's sarcoma (KS) : when lesions are not ulcerated or lymphoedematous, and treatment of visceral KS is not required, and when lesions are not responding to systemic antiretroviral therapy, and radiotherapy or chemotherapy are not appropriate	11/10/2000	Eisai Ltd
PRIVIGEN	Human normal immunoglobulin (IVIg)	Replacement therapy in : <ul style="list-style-type: none"> - Primary immunodeficiency (PID) syndromes such as: <ul style="list-style-type: none"> - congenital agammaglobulinaemia and hypogammaglobulinaemia - common variable immunodeficiency - severe combined immunodeficiency - Wiskott Aldrich syndrome - Myeloma or chronic lymphocytic leukaemia with severe secondary hypogammaglobulinaemia and recurrent infections. Immunomodulation in : <ul style="list-style-type: none"> - Immune thrombocytopenic purpura (ITP), in children or adults at high risk of bleeding or prior to surgery to correct the platelet count - Guillain-Barré syndrome - Kawasaki disease 	25/04/2008	CSL Behring GmbH
PUREGON	Follitropin beta (INN)	Treatment of deficient spermatogenesis due to hypogonadotropic hypogonadism	03/05/1996	NV Organon
RATIOGRASTIM	Filgrastim (INN)	In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$, and a history of severe or recurrent infections	15/09/2008	Ratiopharm GmbH
REFACTO AF	Moroctocog alpha (INN)	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency) in adults and children of all ages, including newborns	13/04/1999	Pfizer Ltd
REFLUDAN	Lepirudin (INN)	Anticoagulation in adult patients with heparin-induced thrombocytopenia (HIT) type II and thromboembolic disease mandating parenteral antithrombotic therapy	13/03/1997	Celgene Europe Ltd
RILUTEK	Riluzole (INN)	To extend life or the time to mechanical ventilation for patients with amyotrophic lateral sclerosis (ALS)	10/06/1996	Aventis Pharma S.A.

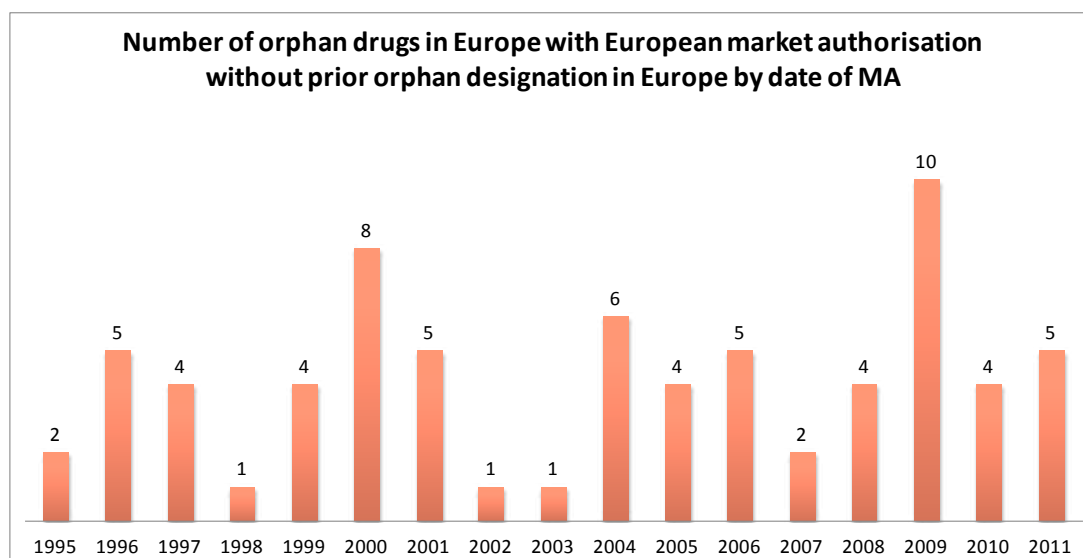
TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
ROACTEMRA	Tocilizumab (INN)	Treatment of active systemic juvenile idiopathic arthritis (sJIA) in patients 2 years of age and older, who have responded inadequately to previous therapy with NSAIDs and systemic corticosteroids. RoActemra can be given as monotherapy (in case of intolerance to MTX or where treatment with MTX is inappropriate) or in combination with MTX.	16/01/2009	Roche Registration Ltd
RUCONEST	Conestat alfa	Treatment of acute angioedema attacks in adults with hereditary angioedema (HAE) due to C1 esterase inhibitor deficiency	28/10/2010	Pharming Group N.V.
SAMSCA	Tolvaptan (INN)	Treatment of adult patients with hyponatraemia secondary to syndrome of inappropriate antidiuretic hormone secretion (SIADH)	03/08/2009	Otsuka Pharmaceutical Europe Ltd
SUTENT	Sunitinib (INN)	Treatment of unresectable and/or metastatic malignant gastrointestinal stromal tumour (GIST) after failure of imatinib mesilate treatment due to resistance or intolerance Treatment of advanced/metastatic renal cell carcinoma (MRCC) in adults Treatment of unresectable or metastatic, well-differentiated pancreatic neuroendocrine tumours (pNET) with disease progression in adults Experience with SUTENT as first-line treatment is limited	19/07/2006	Pfizer Limited
TARCEVA	Erlotinib (INN)	In combination with gemcitabine, for the treatment of patients with metastatic pancreatic cancer . When prescribing Tarceva, factors associated with prolonged survival should be taken into account . No survival advantage could be shown for patients with locally advanced disease	19/09/2005	Roche Registration Limited
TAXOTERE	Docetaxel (INN)	In combination with cisplatin and 5-fluorouracil for the treatment of patients with metastatic gastric adenocarcinoma , including adenocarcinoma of the gastroesophageal junction, who have not received prior chemotherapy for metastatic disease In combination with cisplatin and 5-fluorouracil for the induction treatment of patients with locally advanced squamous cell carcinoma of the head and neck	27/11/1995	Aventis Pharma S.A.
TEMODAL	Temozolomide (INN)	Treatment of adult patients with newly-diagnosed glioblastoma multiforme concomitantly with radiotherapy (RT) and subsequently as monotherapy treatment Treatment of children from the age of three years, adolescents and adult patients with malignant glioma , such as glioblastoma multiforme or anaplastic astrocytoma , showing recurrence or progression after standard therapy	26/01/1999	Schering-Plough Europe

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
TEVAGRASTIM	Filgrastim (INN)	In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$, and a history of severe or recurrent infections	15/09/2008	Teva Generics GmbH
TEYSUNO	Tegafur/Gimeracil /Oteracil	In adults for the treatment of advanced gastric cancer when given in combination with cisplatin	14/03/2011	Nordic Group BV
THYROGEN	Thyrotropin alfa	For use with serum thyroglobulin (Tg) testing with or without radioiodine imaging for the detection of thyroid remnants and well-differentiated thyroid cancer in postthyroidectomy patients maintained on hormone suppression therapy (THST) For pre-therapeutic stimulation in combination with 100 mCi (3.7 GBq) radioiodine for ablation of thyroid tissue remnants in patients who have undergone a near-total or total thyroidectomy for well-differentiated thyroid cancer and who do not have evidence of distant metastatic thyroid cancer	09/03/2000	Genzyme Europe B.V.
VALTROPIN	Somatropin (INN)	Long-term treatment of children with growth failure due to an inadequate secretion of normal endogenous growth hormone Treatment of short stature in children with Turner syndrome , confirmed by chromosome analysis Replacement therapy in adults with pronounced growth hormone deficiency of either childhood- or adult-onset aetiology (patients with known hypothalamic-pituitary pathology and at least one additional known deficiency of a pituitary hormone not being prolactin)	24/04/2006	BioPartners GmbH
VEDROP	Tocofersolan (INN)	Indicated in vitamin E deficiency due to digestive malabsorption in paediatric patients suffering from congenital chronic cholestasis or hereditary chronic cholestasis , from birth (in term newborns) to 16 or 18 years of age, depending on the region	24/07/2009	Orphan Europe S.A.R.L
VELCADE	Bortezomib (INN)	In combination with melphalan and prednisone for the treatment of patients with previously untreated multiple myeloma who are not eligible for high-dose chemotherapy with bone marrow transplant As mono-therapy for the treatment of progressive multiple myeloma in patients who have received at least 1 prior therapy and who have already undergone or are unsuitable for bone marrow transplantation	26/04/2004	Janssen-Cilag International NV
VFEND	Voriconazole	For treatment of invasive aspergillosis For treatment of serious fungal infections caused by <i>Scedosporium</i> spp. and <i>Fusarium</i> spp. (Fusariosis) VFEND should be administered primarily to patients with progressive, possibly life-threatening infections	19/03/2002	Pfizer Limited
VOTRIENT	Pazopanib (INN)	For the first line treatment of advanced Renal Cell Carcinoma (RCC) and for patients who have received prior cytokine therapy for advanced disease	14/06/2010	Glaxo Group Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
XELODA	Capecitabine (INN)	First-line treatment of advanced gastric cancer in combination with a platinum-based regimen	02/02/2001	Roche Registration Limited
XYREM	Sodium oxybate (INN)	Treatment of narcolepsy with cataplexy in adult patients	13/10/2005	UCB Pharma Ltd
ZARZIO	Filgrastim (INN)	In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$, and a history of severe or recurrent infections	06/02/2009	Sandoz GmbH
ZEVALIN	Ibritumomab tiuxetan (INN)	Consolidation therapy after remission induction in previously untreated patients with follicular lymphoma Treatment of adult patients with rituximab relapsed or refractory CD20+ follicular B-cell non-Hodgkin's lymphoma (NHL)	16/01/2004	Bayer Pharma AG
ZUTECTRA	Human Hepatitis B Immunoglobulin	Prevention of hepatitis B virus (HBV) re-infection in HBV-DNA negative patients over 6 months after liver transplantation for hepatitis B induced liver failure . 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

2- By date of MA in descending order

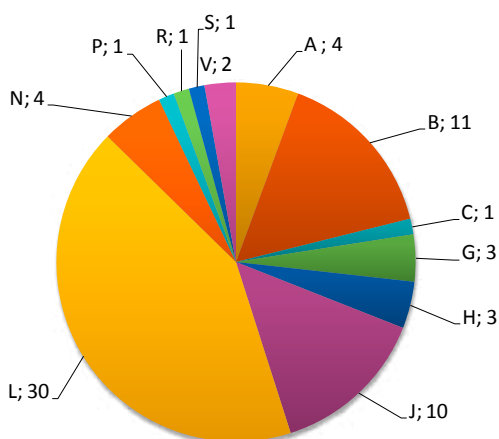
2011	2008	2003	1997
BUCCOLAM	BIOGRASTIM	HUMIRA	BENEFIX
CINRYZE	PRIVIGEN	2002	CEREZYME
EURARTESIM	RATIOGRASTIM	VFEND	CYSTAGON
HIZENTRA	TEVAGRASTIM	2001	REFLUDAN
TEYSUNO	2007	CANCIDAS	1996
2010	FLEBOGAMMA DIF	CEPROTIN	CAELYX
NIVESTIM	ORENCIA	INOMAX	HYCAMTIN
OZURDEX	2006	MABCAMPATH	NOVOSEVEN
RUCONEST	ATRYN	XELODA	PUREGON
VOTRIENT	KIOVIG	2000	RILUTEK
2009	OMNITROPE	ENBREL	1995
ADCIRCA	SUTENT	HELIXATE NEXGEN	GONAL-F
AFINITOR	VALTROPIN	HERCEPTIN	TAXOTERE
FILGRASTIM HEXAL	2005	INTRONA	
ILARIS	AVASTIN	KEPPRA	
IXIARO	NOXAFIL	KOGENATE BAYER	
ROACTEMRA	TARCEVA	PANRETIN	
SAMSCA	XYREM	THYROGEN	
VEDROP	2004	1999	
ZARZIO	ADVATE	AMMONAPS	
ZUTECTRA	ALIMTA	FERRIPROX	
	DUKORAL	REFACTO AF	
	ERBITUX	TEMODAL	
	VELCADE	1998	
	ZEVALIN	MABTHERA	



3- By ATC category

A- ALIMENTARY TRACT AND METABOLISM	G- GENITO URINARY SYSTEM AND SEX HORMONES	L- ANTINEOPLASTIC AND IMMUNOMODULATING AGENTS	TAXOTERE
AMMONAPS	ADCIRCA	AFINITOR	TEMODAL
CEREZYME	GONAL-F	ALIMTA	TEVAGRASTIM
CYSTAGON	PUREGON	AVASTIN	TEYSUNO
VEDROP	H- SYSTEMIC HORMONAL PREPARATIONS, EXCL, SEX HORMONES AND INSULINS	BIOGRASTIM	VELCADE
B- BLOOD AND BLOOD FORMING ORGANS	OMNITROPE	CAELYX	VOTRIENT
ADVATE	THYROGEN	ENBREL	XELODA
ATRYN	VALTROPIN	ERBITUX	ZARZIO
BENEFIX	J- GENERAL ANTIINFECTIVES FOR SYSTEMIC USE	FILGRASTIM HEXAL	N- NERVOUS SYSTEM
CEPROTIN	CANCIDAS	HERCEPTIN	BUCCOLAM
CINRYZE	DUKORAL	HUMIRA	KEPPRA
HELIXATE NEXGEN	FLEBOGAMMA DIF	HYCANTIN	RILUTEK
KOGENATE BAYER	HIZENTRA	ILARIS	XYREM
NOVOSEVEN	IXIARO	INTRONA	P- ANTIPARASITIC PRODUCTS, INSECTICIDES AND REPELLENTS
REFACTO AF	KIOVIG	MABCAMPATH	EURARTESIM
REFLUDAN	NOXAFIL	MABTHERA	R- RESPIRATORY SYSTEM
RUCONEST	PRIVIGEN	NIVESTIM	INOMAX
C- CARDIOVASCULAR SYSTEM	VFEND	ORENCIA	S- SENSORY ORGANS
SAMSCA	ZUTECTRA	PANRETIN	V- VARIOUS
		RATIOGRASTIM	FERRIPROX
		ROACTEMRA	ZEVALIN
		SUTENT	
		TARCEVA	

Number of orphan drugs in Europe with European market authorisation without prior orphan designation in Europe by ATC category



4- By MA holder

ABBOTT LABORATORIES LTD	EISAI LTD	MERCK SHARP & DOHME LTD	ROCHE REGISTRATION LTD
HUMIRA	PANRETIN	CANCIDAS	AVASTIN
ALLERGAN PHARMACEUTICALS IRELAND	ELI LILLY NEDERLAND B.V.	INTRONA	HERCEPTIN
OZURDEX	ADCIRCA	NOXAFIL	MABTHERA
APOTEX EUROPE B.V.	ALIMTA	NORDIC GROUP BV	ROACTEMRA
FERRIPROX	GENZYME EUROPE B.V.	TEYSUNO	TARCEVA
AVENTIS PHARMA S.A.	CEREZYME	NOVARTIS EUROPHARM LTD	XELODA
RILUTEK	MABCAMPATH	AFINITOR	SANDOZ GMBH
TAXOTERE	THYROGEN	ILARIS	OMNITROPE
BAXTER AG	GLAXO GROUP LTD	NOVO NORDISK A/S	ZARZIO
ADVATE	VOTRIENT	NOVOSEVEN	SCHERING-PLOUGH EUROPE
CEPROTIN	GTC BIOTHERAPEUTICS UK LIMITED	NV ORGANON	TEMODAL
KIOVIG	ATRYN	PUREGON	SMITHKLINE BEECHAM LTD
BAYER PHARMA AG	HEXAL AG	ORPHAN EUROPE S.A.R.L.	HYCANTIN
HELIXATE NEXGEN	FILGRASTIM HEXAL	CYSTAGON	SIGMA-TAU INDUSTRIE FARMACEUTICHE RIUNITE S.P.A
KOGENATE BAYER	HOSPIRA UK LTD	VEDROP	EURARTESIM
ZEVALIN	NIVESTIM	OTSUKA PHARMACEUTICAL EUROPE LTD	SWEDISH ORPHAN BIOVITRUM INTERNATIONAL AB
BIOPARTNERS GMBH	INO THERAPEUTICS AB	SAMSCA	AMMONAPS
VALTROPIN	INOMAX	PFIZER LTD	TEVA GENERICS GMBH
BIOTEST PHARMA GMBH	INSTITUTO GRIFOLS S.A.	ENBREL	TEVAGRASTIM
ZUTECTRA	FLEBOGAMMA DIF	REFACTO AF	UCB PHARMA LTD
BRISTOL-MYERS SQUIBB PHARMA EEIG	INTERCELL AG	SUTENT	XYREM
ORENCIA	IXIARO	VFEND	UCB PHARMA SA
CELGENE EUROPE LTD	JANSSEN-CILAG INTERNATIONAL NV	PHARMING GROUP N.V.	KEPPRA
REFLUDAN	CAELYX	RUCONEST	VIROPHARMA SPRL
CRUCELL SWEDEN AB	VELCADE	RATIOPHARM GMBH	BUCCOLAM
DUKORAL	MERCK KGAA	RATIOGRASTIM	CINRYZE
CSL BEHRING GMBH	ERBITUX		WYETH EUROPA LTD
HIZENTRA	MERCK SERONO EUROPE LTD		BENEFIX
PRIVIGEN	GONAL-F		
CT ARZNEIMITTEL GMBH			
BIOGRASTIM			

For any questions or comments, please contact us: contact.orphanet@inserm.fr

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The correct form when quoting this document is:

« Lists of Orphan Drugs in Europe », Orphanet Report Series, *Orphan Drugs collection*, January 2012,
http://www.orpha.net/orphacom/cahiers/docs/GB/list_of_orphan_drugs_in_europe.pdf