Orphanet Report Series

Orphan Drugs collection

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Lists of medicinal products for rare diseases in Europe

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Instituts thématiques Inserm
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leem Fondation
Sous l'égide de la Fondation de France

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PART 1:

List of orphan medicinal products in Europe with European orphan designation and European market authorisation*

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Methodology

This part of the document provides the list of all orphan medicinal products 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 accessibility of a certain orphan medicinal products in a certain country depends on the strategy of the laboratory and the decision taken by national health authorities concerning reimbursement.

Orphan medicinal products 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 - if applicable - a positive evaluation of significant benefit.

This orphan medicinal products list in Europe, with orphan désignation and european marketing authorisations, 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 authorization (http://ec.europa.eu/health/documents/community-register/html/alfregister.htm).

This list is available on the DG health and consumers (DG Sanco) of the European Commission.

A first classifying by tradename provides the name of active substance, the marketing authorisation (MA) indication, the date of MA and the MA holder.

Three additional lists propose another tradenames classifying by :

- Date of MA in descending order;
- ATC category;
- MA holder.

For each list, tradenames are presented in alphabetical order.

You may find additional information on each product in the tab “Orphan drugs” on the 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 medicinal products, not just orphan medicinal products. Orphan medicinal products that have been granted a European orphan designation are indicated by the logo O.

Official and up to date information about orphan medicinal products is available in the Community Register of orphan medicinal products for human use :
http://ec.europa.eu/health/documents/community-register/html/alforphreg.htm

**European Community marketing authorisation under the centralised procedure*

By tradename

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
ADCETRIS	Brentuximab vedotin	*Treatment of adult patients with relapsed or refractory CD30+ **Hodgkin lymphoma** (HL): 1. following autologous stem cell transplant (ASCT) or 2. 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 **anaplastic large cell lymphoma** (sALCL).	25/10/2012	Takeda Global Research and Development Centre (Europe) Ltd
AFINITOR	Everolimus	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 medicinal products intended for rare diseases in Europe with European market authorisation without orphan designation in Europe".		
ALDURAZYME	Laronidase	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of **Mucopolysaccharidosis I** (MPS I; a [alpha]-L-iduronidase deficiency) to treat the non-neurological manifestations of the disease.	10/06/2003	Genzyme Europe B.V.
ARZERRA	Ofatumumab	Treatment of **chronic lymphocytic leukaemia** (CLL) in patients who are refractory to fludarabine and alemtuzumab.	19/04/2010	Glaxo Group Ltd
ATRIANCE	Nelarabine	Treatment of patients with **T-cell acute lymphoblastic leukaemia** (T-ALL) and **T-cell lymphoblastic lymphoma** (T-LBL) whose disease has not responded to or has relapsed following treatment with at least two chemotherapy regimens. Due to the small patient populations in these disease settings, the information to support these indications is based on limited data.	22/08/2007	Glaxo Group Ltd
BRONCHITOL	Mannitol	For the treatment of **cystic fibrosis** (CF) in adults aged 18 years and above as an add-on therapy to best standard of care.	13/04/2012	Pharmaxis Pharmaceuti-cals Limited
BUSILVEX	Busulfan (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

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
CARBAGLU	Carglumic acid	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.** *This orphan designated product has completed its 10 years of "market exclusivity" **for its indication in hyperammonaemia due to N-acetylglutamate synthetase (NAGS) deficiency**.*	24/01/2003	Orphan Europe S.a.r.l.
CAYSTON	Aztreonam	Suppressive therapy of chronic pulmonary infections due to *Pseudomonas aeruginosa* in patients with **cystic fibrosis** (CF) aged 6 years and older.	21/09/2009	Gilead Sciences International Limited
CEPLENE	Histamine dihydrochloride	Maintainance therapy for adult patients with **acute myeloid leukaemia** in first remission concomitantly treated with interleukin-2 (IL-2). The efficacy of Ceplene has not been fully demonstrated in patients older than age 60.	07/10/2008	Meda AB
CYSTADANE	Betaine anhydrous	Adjunctive treatment of **homocystinuria**, involving deficiencies or defects in cystathionine beta-synthase (CBS), 5,10-methylene-tetrahydrofolate reductase (MTHFR), cobalamin cofactor metabolism (cbl). Cystadane should be used as supplement to other therapies such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), folate and a specific diet.	15/02/2007	Orphan Europe S.a.r.l.
DACOGEN	Decitabine	Treatment of adult patients aged 65 years and above with newly diagnosed de novo or secondary **acute myeloid leukaemia** (AML), according to the World Health Organization (WHO) classification, who are not candidates for standard induction chemotherapy.	20/09/2012	Janssen-Cilag International N V
DIACOMIT	Stiripentol	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	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	Treatment of **acute lymphoblastic leukaemia** (ALL) in paediatric patients who have relapsed or are refractory after receiving at least two prior regimens and where there is no other treatment option anticipated to result in a durable response. Safety and efficacy have been assessed in studies of patients ≤ 21 years old at initial diagnosis.	29/05/2006	Genzyme Europe B.V.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
EXJADE	Deferasirox	*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 beta thalassaemia major with iron overload due to frequent blood transfusions in (≥ 7 ml/kg/month of packed red blood cells) 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) aged 2 years and older, - in patients with other anaemias aged 2 years and older.	28/08/2006	Novartis Europharm Ltd
FABRAZYME	Recombinant human alpha-galactosidase A INN = Agalsidase beta	This product is no longer an orphan medicine. It was originally designated an orphan medicine on 8 August 2000. Fabrazyme was withdrawn from the Community register of orphan medicinal products in August 2011 at the end of the period of market exclusivity. Cf "List of medicinal products intended for rare diseases in Europe with European market authorisation without orphan designation in Europe".		
FIRAZYR	Icatibant acetate	Symptomatic treatment of acute attacks of **hereditary angioedema** (HAE) in adults (with C1-esterase-inhibitor deficiency).	11/07/2008	Shire Orphan Therapies GmbH
FIRDAPSE (ex-ZENAS)	Amifampridine	Symptomatic treatment of **Lambert-Eaton myasthenic syndrome** (LEMS) in adults.	23/12/2009	Biomarin Europe Ltd
GLIOLAN	5-aminolevulinic acid hydrochloride	In adult patients for visualisation of malignant tissue during surgery for **malignant glioma** (World Health Organization grade III and IV).	07/09/2007	Medac GmbH
GLIVEC	Imatinib mesilate	This product is no longer an orphan medicine. It was originally designated an orphan medicine for the following conditions: - treatment of chronic myeloid leukaemia (14/02/2001); - treatment of malignant gastrointestinal stromal tumours (20/11/2001); - treatment of dermatofibrosarcoma protuberans (26/08/2005); - treatment of acute lymphoblastic leukaemia (26/08/2005); - treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome (28/10/2005); - treatment of myelodysplastic / myeloproliferative diseases (23/12/2005). Upon request of the marketing-authorisation holder, Glivec has now been removed from the Community register of orphan medicinal products. Cf "List of medicinal products intended for rare diseases in Europe with European market authorisation without orphan designation in Europe".		
GLYBERA	Alipogene tiparvovec	For adult patients diagnosed with **familial lipoprotein lipase deficiency** (LPLD) and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. The diagnosis of LPLD has to be confirmed by genetic testing. The indication is restricted to patients with detectable levels of LPL protein.	29/10/2012	uniQure biopharma B.V.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
ILARIS	Canakinumab	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 medicinal products intended for rare diseases in Europe with European market authorisation without orphan designation in Europe".		
INCRELEX	Mecasermin	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	Adjunctive therapy in the treatment of seizures associated with **Lennox Gastaut syndrome** in patients aged 4 years and older.	16/01/2007	Eisai Ltd
JAKAVI	Ruxolitinib	Treatment of disease-related splenomegaly or symptoms in adult patients with **primary myelofibrosis** (also known as chronic idiopathic myelofibrosis), **post-polycythaemia-vera myelofibrosis** or **post-essential-thrombocythaemia myelofibrosis.**	23/08/2012	Novartis Europharm Ltd
KALYDECO	Ivacaftor	Kalydeco is indicated for the treatment of **cystic fibrosis** (CF) in patients age 6 years and older who have a *G551D* mutation in the *CFTR* gene.	23/07/2012	Vertex Pharmaceuticals (U.K.) Limited
KUVAN	Sapropterin dihydrochloride	*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 *Treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients with **tetrahydrobiopterin (BH4) deficiency** who have been shown to be responsive to such treatment.	02/12/2008	Merck Serono Europe Ltd
LITAK	Cladribine (subcutaneous use)	Treatment of **hairy cell leukaemia.**	14/04/2004	Lipomed GmbH
LYSODREN	Mitotane	Symptomatic treatment of advanced (unresectable, metastatic or relapsed) **adrenal cortical carcinoma.**	28/04/2004	Laboratoire HRA Pharma
MEPACT	Mifamurtide	In children, adolescents and young adults for the treatment of high-grade resectable non-metastatic **osteosarcoma** after macroscopically complete surgical resection. It is used in combination with post-operative multi-agent chemotherapy.	06/03/2009	IDM Pharma SAS
MOZOBIL	Plerixafor	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 = Algluco-sidase 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.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
NAGLAZYME	N-acetylgalac-tosamine-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	*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
NEXOBRID	Concentrate of proteolytic en-zymes enriched in bromelain	Removal of eschar in adults with **deep partial- and full-thickness thermal burns.**	18/12/2012	Teva Pharma GmbH
NOVOTHIR-TEEN	Catridecacog	This product is no longer an orphan medicine. It was originally designated an orphan medicine on 12 December 2003. Upon request of the marketing-authorisation holder, NovoThirteen has now been removed from the Community Register of orphan medicinal products. Cf "List of medicinal products intended for rare diseases in Europe with European market authorisation without orphan designation in Europe".		
NPLATE	Romiplostim	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	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	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 Biovi-trum Interna-tional AB
PEDEA	Ibuprofen	Treatment of a haemodynamically significant **patent *ductus arteriosus*** in preterm newborn infants less than 34 weeks of gestational age.	29/07/2004	Orphan Europe S.a.r.l.
PEYONA (ex-NYMUSA)	Caffeine citrate	Treatment of **primary apnea** of premature newborns.	02/07/2009	Chiesi Farma-ceutici SpA
PHOTOBARR	Porfimer sodium (for use with photodynamic therapy)	Ablation of high-grade dysplasia (HGD) in patients with **Barrett's Oesophagus.** ***This medicine is now withdrawn from use in the European Union, more information on:*** www.ema.europa.eu	25/03/2004	Pinnacle Bio-logics B.V.
PLENADREN	Hydrocortisone	Treatment of **adrenal insufficiency** in adults.	03/11/2011	ViroPharma SPRL

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
PRIALT	Ziconotide (intraspinal use)	Treatment of severe, **chronic pain** in patients who require **intrathecal (IT) analgesia.**	21/02/2005	Eisai Ltd
REPLAGAL	Agalsidase alfa	This product is no longer an orphan medicine. It was originally designated an orphan medicine on 8 August 2000. Replagal was withdrawn from the Community register of orphan medicinal products in August 2011 at the end of the period of market exclusivity. Cf "List of medicinal products intended for rare diseases in Europe with European market authorisation without orphan designation in Europe".		
REVATIO	Sildenafil citrate	Treatment of adult patients with **pulmonary arterial hypertension** classified as WHO functional class II and III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and pulmonary hypertension associated with connective tissue disease. Treatment of paediatric patients aged 1 year to 17 years old with pulmonary arterial hypertension. Efficacy in terms of improvement of exercise capacity or pulmonary haemodynamics has been shown in primary pulmonary hypertension and pulmonary hypertension associated with congenital heart disease. 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
REVESTIVE	Teduglutide	Treatment of adult patients with **Short Bowel Syndrome**. Patients should be stable following a period of intestinal adaptation after surgery.	30/08/2012	Nycomed Danmark ApS
REVLIMID	Lenalidomide	In combination with dexamethasone, treatment of **multiple myeloma** in adult patients who have received at least one prior therapy.	14/06/2007	Celgene Europe Ltd
REVOLADE	Eltrombopag	This product is no longer an orphan medicine. It was originally designated an orphan medicine on 3 August 2007. Upon request of the marketing authorisation holder, Revolade has now been removed from the Community Register of orphan medicinal products. Cf "List of medicinal products intended for rare diseases in Europe with European market authorisation without orphan designation in Europe".		
RILONACEPT REGENERON (ex-ARCALYST)	Rilonacept	Treatment of **Cryopyrin-Associated Periodic Syndromes** (CAPS) with severe symptoms, including **Familial Cold Autoinflammatory Syndrome** (FCAS) and **Muckle-Wells Syndrome** (MWS), in adults and children aged 12 years and older. ***This medicine is now withdrawn from use in the European Union, more information on:*** www.ema.europa.eu	23/10/2009	Regeneron UK Limited
SAVENE	Dexrazoxane	In adults for the treatment of **anthracycline extravasation.**	28/07/2006	SpePharm Holding B.V.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
SIGNIFOR	Pasireotide	Treatment of adult patients with **Cushing's disease** for whom surgery is not an option or for whom surgery has failed.	24/04/12	Novartis Europharm Ltd
SIKLOS	Hydroxycarbamide	Prevention of recurrent painful vaso-occlusive crises including acute chest syndrome in adults, adolescents and children older than 2 years suffering from symptomatic **Sickle Cell Syndrome.**	29/06/2007	Addmedica
SOLIRIS	Eculizumab	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	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. *This orphan designated product has completed its 10 years of "market exclusivity".*	13/11/2002	Pfizer Ltd
SPRYCEL	Dasatinib	Treatment of adult patients with: - newly diagnosed Philadelphia chromosome positive (Ph+) **chronic myelogenous leukaemia** (CML) in the chronic phase. - chronic, accelerated or blast phase CML with resistance or intolerance to prior therapy including imatinib mesilate. - Ph+ **acute lymphoblastic leukaemia** (ALL) and lymphoid blast CML with resistance or intolerance to prior therapy.	20/11/2006	Bristol-Myers Squibb Pharma EEIG
SUTENT	Sunitinib malate Sunitinib	This product is no longer an orphan medicine. This product was originally an orphan designated on 10 March 2005. Upon request of the marketing authorisation holder, Sutent has now been removed from the Community register of orphan medicinal products. Cf "List of medicinal products intended for rare diseases in Europe with European market authorisation without orphan designation in Europe".		
TASIGNA	Nilotinib	* Tasigna 150 mg Treatment of adult patients with newly diagnosed Philadelphia-chromosome-positive **chronic myelogenous leukaemia (CML)** in the chronic phase. * Tasigna 200 mg Treatment of adult patients with : - newly diagnosed Philadelphia-chromosome-positive **CML** in the chronic phase; - chronic phase and accelerated phase Philadelphia-chromosome-positive **CML** with resistance or intolerance to prior therapy including imatinib. Efficacy data in patients with CML in blast crisis are not available.	19/11/2007	Novartis Europharm Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
TEPADINA	Thiotepa	In combination with other chemotherapy medicinal products: 1) with or without total body irradiation (TBI), as conditioning treatment prior to **allogeneic or autologous haematopoietic progenitor cell transplantation** (HPCT) in haematological diseases in adult and paediatric patients; 2) when high dose chemotherapy with HPCT support is appropriate for the treatment of solid tumours in adult and paediatric patients. 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	In combination with melphalan and prednisone as first line treatment of patients with untreated **multiple myeloma**, aged ≥ 65 years or ineligible for high dose chemotherapy.	16/04/2008	Celgene Europe Ltd
THELIN	Sitaxentan sodium	Treatment of patients with **pulmonary arterial hypertension** classified as WHO functional class III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and in pulmonary hypertension associated with connective tissue disease. ***This medicine is now withdrawn from use in the European Union, more information on:*** 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	*First-line treatment of adult patients with advanced **renal cell carcinoma** (RCC) who have at least three of six prognostic risk factors. *Treatment of adult patients with relapsed and / or refractory **mantle cell lymphoma** (MCL).	19/11/2007	Pfizer Limited
TRACLEER	Bosentan monohydrate	* Treatment of **pulmonary arterial hypertension** (PAH) to improve exercise capacity and symptoms in patients with WHO functional class III. Efficacy has been shown in : - primary (idiopathic and heritable) PAH, - PAH secondary to scleroderma without significant interstitial pulmonary disease, - PAH associated with congenital systemic-to-pulmonary shunts and Eisenmenger's physiology. Some improvements have also been shown in patients with PAH WHO functional class II. * To reduce the number of new digital ulcers in patients with **systemic sclerosis** and ongoing digital ulcer disease. *This orphan designated product has completed its 10 years of "market exclusivity" **for its indication in pulmonary arterial hypertension.***	15/05/2002	Actelion Registration Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
TRISENOX	Arsenic trioxide	This product is no longer an orphan medicine. It was originally designated an orphan medicine on 18 October 2000. Trisenox was withdrawn from the Community register of orphan medicinal products in March 2012 at the end of the period of market exclusivity. Cf "List of medicinal products intended for rare diseases in Europe with European market authorisation without orphan designation in Europe".		
VENTAVIS	Iloprost	Treatment of patients with **primary pulmonary hypertension**, classified as NYHA functional class III, to improve exercise capacity and symptoms.	16/09/2003	Bayer Pharma AG
VIDAZA	Azacitidine	Treatment of adult patients who are not eligible for haematopoietic stem cell transplantation with: - intermediate-2 and high-risk **myelodysplastic syndromes** (MDS) according to the International Prognostic Scoring System (IPSS), - **chronic myelomonocytic leukaemia** (CMML) with 10-29% marrow blasts without myeloproliferative disorder, - **acute myeloid leukaemia** (AML) with 20-30 % blasts and multi-lineage dysplasia, according to World Health Organisation (WHO) classification.	17/12/2008	Celgene Europe Ltd
VOLIBRIS	Ambrisentan	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 adult patients with **renal angiomyolipoma** associated with **tuberous sclerosis complex** (TSC) who are at risk of complications (based on factors such as tumour size or presence of aneurysm, or presence of multiple or bilateral tumours) but who do not require immediate surgery. The evidence is based on analysis of change in sum of angiomyolipoma volume. * Treatment of patients 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	Treatment of **Wilson's disease.**	13/10/2004	Orphan Europe S.a.r.l.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
XAGRID	Anagrelide hydrochloride	Reduction of elevated platelet counts in at-risk **essential-thrombocythaemia** (ET) patients who are intolerant to their current therapy or whose elevated platelet counts are not reduced to an acceptable level by their current therapy. **An at-risk patient** An at risk ET is defined by one or more of the following features: - > 60 years of age or - a platelet count > 1000 x 10^9/l or - a history of thrombo-haemorrhagic events.	16/11/2004	Shire Pharmaceutical Contracts Ltd
XALUPRINE (ex-MERCAPTOPURINE NOVA)	Mercaptopurine	Treatment of **acute lymphoblastic leukaemia** (ALL) in adults, adolescents and children.	09/03/2012	Nova Laboratories Ltd
XYREM	Sodium oxybate	This product is no longer an orphan medicine. This product was originally an orphan designated on 3 February 2003. Upon request of the marketing authorisation holder, Xyrem has now been removed from the Community register of orphan medicinal products. Cf "List of medicinal products intended for rare diseases in Europe with European market authorisation without orphan designation in Europe".		
YONDELIS	Trabectedin	*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	*Oral treatment of adult patients with mild to moderate **type 1 Gaucher disease**. Zavesca may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable *Treatment of progressive neurological manifestations in adult patients and paediatric patients with **Niemann-Pick type C disease.** *This orphan designated product has completed its 10 years of "market exclusivity" **for its indication in Gaucher Disease.***	20/11/2002	Actelion Registration Ltd

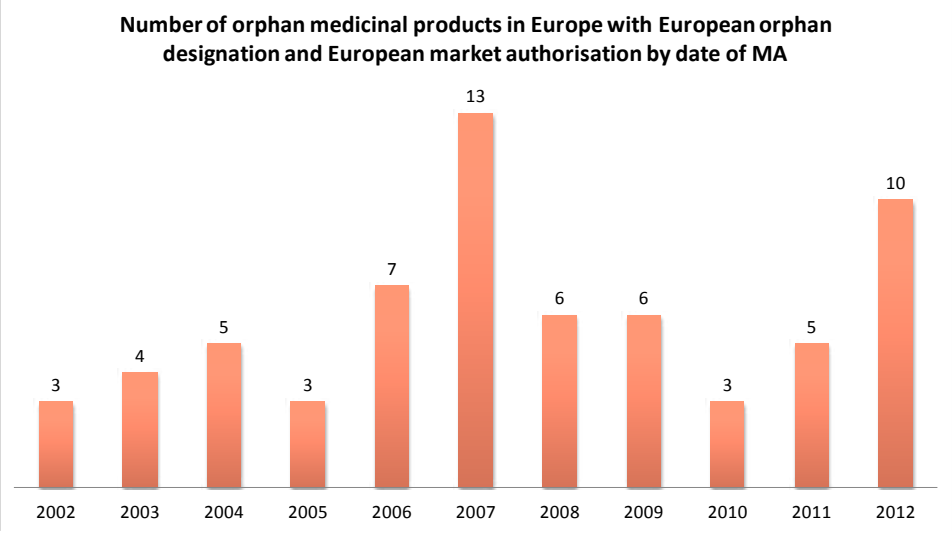
By date of MA in descending order

2012
ADCETRIS
BRONCHITOL
DACOGEN
GLYBERA
JAKAVI
KALYDECO
NEXOBRID
REVESTIVE
SIGNIFOR
XALUPRINE
2011
ESBRIET
PLENADREN
TOBI PODHALER
VOTUBIA
VYNDAQEL
2010
ARZERRA
TEPADINA
VPRIV

2009
CAYSTON
FIRDAPSE
MEPACT
MOZOBIL
NPLATE
PEYONA
2008
CEPLENE
FIRAZYR
KUVAN
THALIDOMIDE CELGENE
VIDAZA
VOLIBRIS
2007
ATRIANCE
CYSTADANE
DIACOMIT
ELAPRASE
GLIOLAN

INCRELEX
INOVELON
REVLIMID
SIKLOS
SOLIRIS
TASIGNA
TORISEL
YONDELIS
2006
EVOLTRA
EXJADE
MYOZYME
NAGLAZYME
NEXAVAR
SAVENE
SPRYCEL
2005
ORFADIN
PRIALT
REVATIO

2004
LITAK
LYSODREN
PEDEA
WILZIN
XAGRID
2003
ALDURAZYME
BUSILVEX
CARBAGLU
VENTAVIS
2002
SOMAVERT
TRACLEER
ZAVESCA



By ATC category

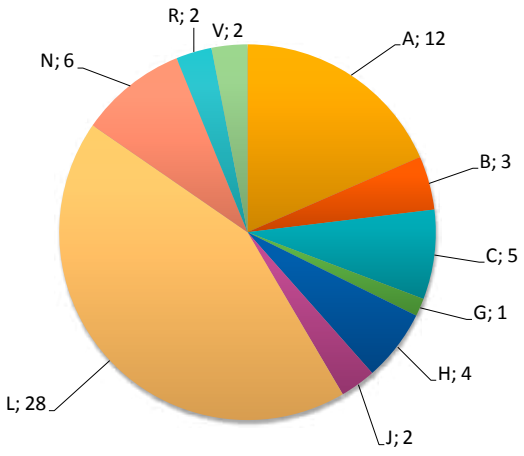
A- ALIMENTARY TRACT AND METABOLISM
ALDURAZYME
CARBAGLU
CYSTADANE
ELAPRASE
KUVAN
MYOZYME
NAGLAZYME
ORFADIN
REVESTIVE
VPRIV
WILZIN
ZAVESCA
B- BLOOD AND BLOOD FORMING ORGANS
NPLATE
VENTAVIS
C- CARDIOVASCULAR SYSTEM
FIRAZYR
GLYBERA
PEDEA
TRACLEER
VOLIBRIS

G- GENITO URINARY SYSTEM AND SEX HORMONES
REVATIO
H- SYSTEMIC HORMONAL PREPARATIONS, EXCL, SEX HORMONES AND INSULINS
INCRELEX
PLENADREN
SIGNIFOR
SOMAVERT
J- GENERAL ANTIINFECTIVES FOR SYSTEMIC USE
CAYSTON
TOBI PODHALER
L- ANTINEOPLASTIC AND IMMUNOMODULATING AGENTS
ADCETRIS
ARZERRA
ATRIANCE
BUSILVEX
CEPLENE
DACOGEN
ESBRIET
EVOLTRA

GLIOLAN
JAKAVI
LITAK
LYSODREN
MEPACT
MOZOBIL
NEXAVAR
REVLIMID
SIKLOS
SOLIRIS
SPRYCEL
TASIGNA
TEPADINA
THALIDOMIDE CELGENE
TORISEL
VIDAZA
VOTUBIA
XAGRID
XALUPRINE
YONDELIS

N- NERVOUS SYSTEM
DIACOMIT
FIRDAPSE (ex-ZENAS)
INOVELON
PEYONA (ex-NYMUSA)
PRIALT
VYNDAQEL
R- RESPIRATORY SYSTEM
BRONCHITOL
KALYDECO
V- VARIOUS
EXJADE
SAVENE
ATC CODE NOT YET ASSIGNED
NEXOBRID

Number of orphan medicinal products in Europe with European orphan designation and European market authorisation by ATC category



By MA holder

ACTELION REGISTRATION LTD
TRACLEER
ZAVESCA
ADDMEDICA
SIKLOS
ADIENNE SRL
TEPADINA
ALEXION EUROPE SAS
SOLIRIS
AMGEN EUROPE B.V.
NPLATE
BAYER PHARMA AG
NEXAVAR
VENTAVIS
BIOCODEX
DIACOMIT
BIOMARIN EUROPE LTD
FIRDAPSE
NAGLAZYME
BRISTOL MYERS SQUIBB EEIG
SPRYCEL
CELGENE EUROPE LTD
REVLIMID
THALIDOMIDE CELGEN
VIDAZA
CHIESI FARMACEUTICI SPA
PEYONA
ESAI LTD
INOVELON
PRIALT

GENZYME EUROPE B.V.
ALDURAZYME
EVOLTRA
MOZOBIL
MYOZYME
GILEAD SCIENCES INTERNATIONAL LTD
CAYSTON
GLAXO GROUP LTD
ARZERRA
ATRIANCE
VOLIBRIS
IDM PHARMA SAS
MEPACT
INTERMUNE UK LTD
ESBRIET
IPSEN PHARMA
INCRELEX
JANSSEN-CILAG INTERNATIONAL NV
DACOGEN
LABORATOIRE HRA PHARMA
LYSODREN
LIPOMED GMBH
LITAK
MEDA AB
CEPLENE
MEDAC GMBH
GLIOLAN
MERCK SERONO EUROPE LTD
KUVAN
NOVA LABORATORIES LTD
XALUPRINE

NOVARTIS EUROPHARM LTD
EXJADE
JAKAVI
SIGNIFOR
TASIGNA
TOBI PODHALER
VOTUBIA
NYCOMED DANMARK APS
REVESTIVE
ORPHAN EUROPE S.A.R.L
CARBAGLU
CYSTADANE
PEDEA
WILZIN
PFIZER LTD
REVATIO
SOMAVERT
TORISEL
PFIZER SPECIALTY UK LTD
VYNDAQEL
PHARMA MAR S.A.
YONDELIS
PHARMAXIS PHARMACEUTICALS LTD
BRONCHITOL
PIERRE FABRE MEDICAMENT
BUSILVEX
SHIRE HUMAN GENETIC THERAPIES AB
ELAPRASE
SHIRE ORPHAN THERAPIES GMBH
FIRAZYR
XAGRID

SHIRE PHARMACEUTICALS IRELAND LTD
VPRIV
SPEPHARM HOLDING BV
SAVENE
SWEDISH ORPHAN BIOVITRUM INTERNATIONAL AB
ORFADIN
TAKEDA GLOBAL RESEARCH AND DEVELOPMENT CENTRE (EUROPE) LTD
ADCETRIS
TEVA PHARMA GMBH
NEXOBRID
UNIQURE BIOPHARMA B.V.
GLYBERA
VERTEX PHARMACEUTICALS (U.K.) LTD
KALYDECO
VIROPHARMA SPRL
PLENADREN

PART 2:

List of medicinal products intended for rare diseases in Europe with European market authorisation* (without orphan designation in Europe)

Table of content

Methodology

This part of the document provides a list of all medicinal products for rare diseases that have received a European marketing autorisation (MA) for one or more indication(s) of use for a rare disease, but which have not been granted a European orphan designation or for which the designation was withdrawn.

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

A first classification by tradename provides the name of active substance, the marketing authorisation (MA) indication, the date of MA and the MA holder.

Three additional lists propose another tradenames classification by :

- Date of MA in descending order;
- ATC category;
- MA holder.

For each list, tradenames are presented in alphabetical order.

You may find additional information on each medicinal product in the tab “Orphan drugs” on the Orphanet website www.orpha.net or on the EMA website (European Medicines Agency) http://www.ema.europa.eu.

**European Community marketing authorisation under the centralised procedure*

By tradename

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
ADCIRCA	Tadalafil	In adults for the treatment of **pulmonary arterial hypertension** (PAH) classified as WHO functional class II and III, to improve exercise capacity. Efficacy has been shown in idiopathic PAH (IPAH) and in PAH related to collagen vascular disease.	01/10/2008	Eli Lilly Nederland B.V.
ADVATE	Octocog alpha	Treatment and prophylaxis of bleeding in patients with **haemophilia A** (congenital factor VIII deficiency).	02/03/2004	Baxter AG
AFINITOR	Everolimus	*Treatment of unresectable or metastatic, well- or moderately-differentiated **neuroendocrine tumours of pancreatic origin** in adults with progressive disease. *Treatment of 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	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	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	Prophylaxis of venous thromboembolism in surgery of adult patients with **congenital antithrombin deficiency**. Atryn is normally given in association with heparin or low molecular weight heparin.	28/07/2006	GTC Biotherapeutics UK Limited
AVASTIN	Bevacizumab	*In combination with interferon alfa-2a, for first line treatment of patients with advanced and/or metastatic **renal cell cancer.** *In combination with carboplatin and paclitaxel, for the front-line treatment of advanced (FIGO stages III B, III C and IV) **epithelial ovarian, fallopian tube, or primary peritoneal 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	Pfizer Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
BIOGRASTIM	Filgrastim	In patients, children or adults, with severe congenital, cyclic, or idiopathic neutropenia with an absolute neutrophil count (ANC) of ≤ 0.5 x 10^9/l, and a history of severe or recurrent infections, long term administration of Biograstim is indicated to increase neutrophil counts and to reduce the incidence and duration of infection-related events.	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). 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
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
CAPRELSA	Vandetanib	Treatment of aggressive and symptomatic medullary **thyroid cancer** (MTC) 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	AstraZeneca AB
CEPROTIN	Human protein C	*In purpura fulminans and coumarin-induced skin necrosis in patients with severe **congenital protein C deficiency.** *Short-term prophylaxis in patients with severe **congenital protein C deficiency** : if surgery or invasive therapy is imminent, while initiating coumarin therapy, when coumarin therapy alone is not sufficient, when coumarin therapy is not feasible.	16/07/2001	Baxter AG

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
CEREZYME	Imiglucerase	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of non-neuronopathic (**Type 1**) or chronic neuronopathic (**Type 3**) **Gaucher disease** and who exhibit clinically significant 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
COLOBREATHE	Colistimethate sodium	Management of chronic pulmonary infections due to *Pseudomonas aeruginosa* in patients with **cystic fibrosis** (CF) aged 6 years and older.	13/02/2012	Forest Laboratories UK Ltd
CYSTAGON	Mercaptamine bitartrate	Treatment of proven nephropathic **cystinosis** Cysteamine reduces cystine accumulation in some cells (e.g. leukocytes, muscle and liver cells) of nephropathic cystinosis patients and, when treatment is started early, it delays the development of renal failure.	23/06/1997	Orphan Europe S.A.R.L.
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	*Treatment of **polyarthritis** (rheumatoid-factor-positive or -negative) and extended **oligoarthritis** in children and adolescents from the age of **2 years** who have had an inadequate response to, or who have proved intolerant of, methotrexate. *Treatment of **psoriatic arthritis** in adolescents from the age of 12 years who have had an inadequate response to, or who have proved intolerant of, methotrexate. *Treatment of **enthesitis-related arthritis** in adolescents from the age of 12 years who have had an inadequate response to, or who have proved intolerant of, conventional therapy. 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 **squamous cell cancer of the head and neck** : - in combination with radiation therapy for locally advanced disease, - in combination with platinum-based chemotherapy for recurrent and/or metastatic disease.	29/06/2004	Merck KGaA
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. 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	Recombinant human alphagalactosidase INN = Agalsidase beta	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of **Fabry disease** (alphagalactosidase A deficiency).	03/08/2001	Genzyme Europe B.V.
FERRIPROX	Deferiprone	Treatment of iron overload in patients with **thalassaemia** major when deferoxamine therapy is contraindicated or inadequate.	25/08/1999	Apotex Europe B.V.
FILGRASTIM HEXAL	Filgrastim	In patients, children or adults, with **severe congenital, cyclic,** or **idiopathic neutropenia** with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9$/l, and a history of severe or recurrent infections.	06/02/2009	Hexal AG
FLEBOGAMMA DIF	Human normal immunoglobulin	* Replacement therapy in adults, children and adolescents (2-18 years) in : - **Primary immunodeficiency** syndromes with impaired antibody production. - Hypogammaglobulinaemia and recurrent bacterial infections in patients with chronic lymphocytic leukaemia, in whom prophylactic antibiotics have failed. - Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase multiple myeloma patients who failed to respond to pneumococcal immunisation. - Hypogammaglobulinaemia in patients after allogenic haematopoietic stem cell transplantation (HSCT). - Congenital AIDS with recurrent bacterial infections. * Immunomodulation in adults, children and adolescents (2-18 years) in: **- Primary immune thrombocytopenia** (ITP), in patients at high risk of bleeding or prior to surgery to correct the platelet count, - **Guillain Barré syndrome,** - **Kawasaki disease.**	23/07/2007	Instituto Grifols S.A.

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
GLIVEC	Imatinibmesilate	* Treatment of : - adult and paediatric patients with newly diagnosed Philadelphia chromosome (bcr-abl) positive (Ph+) **chronic myeloid leukaemia** (CML) for whom bone marrow transplantation is not considered as the first line of treatment; - 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
GONAL-F	Recombinant human follicle stimulating hormone INN = Follitropin alpha	Stimulation of spermatogenesis in men who have **congenital** or acquired **hypogonadotrophic hypogonadism** with concomitant human Chorionic Gonadotrophin (hCG) therapy.	20/10/1995	Merck Serono Europe Ltd
HELIXATE NEXGEN	Octocog alpha	Treatment and prophylaxis of bleeding in patients with **haemophilia A** (congenital factor VIII deficiency).	04/08/2000	Bayer Pharma AG
HERCEPTIN	Trastuzumab	*In combination with capecitabine or 5-fluorouracil and cisplatin, treatment of patients with HER2-positive metastatic **adenocarcinoma of the stomach or gastroesophageal junction** who have not received prior anticancer treatment for their metastatic disease. *Herceptin should only be used in patients with metastatic **gastric cancer** whose tumours have HER2 overexpression as defined by IHC2+ and a confirmatory SISH or FISH result, or by an IHC3+ result. Accurate and validated assay methods should be used.	28/08/2000	Roche Registration Limited

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
HIZENTRA	Human normal immunoglobulin (SCIg)	* Replacement therapy in adults and children in **primary immunodeficiency syndromes** such as: - congenital **agammaglobulinaemia** and **hypogammaglobulinaemia,** - **common variable immunodeficiency,** - **severe combined immunodeficiency,** - **IgG subclass deficiencies** with recurrent infections. * Replacement therapy in **myeloma** or **chronic lymphocytic leukaemia** with severe secondary hypogammaglobulinaemia and recurrent infections.	14/04/2011	CSL Behring GmbH
HUMIRA	Adalimumab	*In combination with methotrexate is indicated for the treatment of active polyarticular **juvenile idiopathic arthritis**, in children and adolescents aged 4 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 Humira has not been studied in children aged less than 4 years.	08/09/2003	Abbott Laboratories Ltd
HYCAMTIN	Topotecan	**HYCAMTIN powder for concentrate for solution for infusion:** *Monotherapy for the treatment of: - patients with metastatic **carcinoma of the ovary** after failure of first-line or subsequent therapy. - patients with relapsed **small cell lung cancer** (SCLC) for whom re-treatment with the first-line regimen is not considered appropriate. *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. **HYCAMTIN capsules:** 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.	12/11/1996	SmithKline Beecham Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
ILARIS	Canakinumab	Treatment of **Cryopyrin-Associated Periodic Syndromes** (CAPS) in adults, adolescents and children aged 4 years and older with body weight above 15 kg, including: - **Muckle-Wells Syndrome** (MWS), - **Neonatal-Onset Multisystem Inflammatory Disease** (NOMID) / **Chronic Infantile Neurological, Cutaneous, Articular Syndrome** (CINCA), - Severe forms of **Familial Cold Autoinflammatory Syndrome** (FCAS) / **Familial Cold Urticaria** (FCU) presenting with signs and symptoms beyond cold-induced urticarial skin rash.	23/10/2009	Novartis Europharm Ltd
INLYTA	Axitinib	For the treatment of adult patients with advanced **renal cell carcinoma** (RCC) after failure of prior treatment with sunitinib or a cytokine.	03/09/2012	Pfizer Ltd.
INOMAX	Nitric oxide	In conjunction with ventilatory support and other appropriate active substances: - for the treatment of newborn infants ≥ 34 weeks gestation with hypoxic respiratory failure associated with clinical or 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
INTRONA	Interferon alpha-2b	*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

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
IXIARO	Japanese Encephalitis Vaccine (inactivated, adsorbed)	For active immunization against **Japanese encephalitis** for adults.	31/03/2009	Intercell AG
KEPPRA	Levetiracetam	*As monotherapy in the treatment of partial onset seizures with or without secondary generalisation in patients from 16 years of age with newly diagnosed **epilepsy**. *As adjunctive therapy in the treatment of partial onset seizures with or without secondary generalisation in adults, children and infants from **1 month** of age with epilepsy ; in the treatment of myoclonic seizures in adults and adolescents from 12 years of age with **Juvenile Myoclonic Epilepsy** ; 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
KIOVIG	Human normal immunoglobulin	*Replacement therapy in adults, and children and adolescents (0-18 years) in: - **Primary immunodeficiency syndromes** with impaired antibody production, - **Hypogammaglobulinaemia** and recurrent bacterial infections in patients with chronic lymphocytic leukaemia, in whom prophylactic antibiotics have failed, - Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase multiple myeloma patients who have failed to respond to pneumococcal immunisation, - Hypogammaglobulinaemia in patients after allogeneic haematopoietic stem cell transplantation (HSCT). *Immunomodulation in adults, and children and adolescents (0-18 years) in: - **Primary immune thrombocytopenia** (ITP), in patients at high risk of bleeding or prior to surgery to correct the platelet count, - **Guillain Barré syndrome,** - **Kawasaki disease**, - **Multifocal Motor Neuropathy** (MMN).	19/01/2006	Baxter AG
KOGENATE BAYER	Octocog alpha	Treatment and prophylaxis of bleeding in patients with **haemophilia A** (congenital factor VIII deficiency).	04/08/2000	Bayer Pharma AG

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
MABTHERA	Rituximab	Indicated in adults for: *Treatment of previously untreated patients with stage III-IV **follicular lymphoma** in combination with chemotherapy. Maintenance therapy is indicated for the treatment of follicular lymphoma patients responding to induction therapy. 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. Treatment of patients with CD20 positive **diffuse large B cell non-Hodgkin's lymphoma** in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy. *In combination with chemotherapy, treatment of patients with previously untreated and relapsed/refractory **chronic lymphocytic leukaemia** (CLL). 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.	02/06/1998	Roche Registration Limited
NIVESTIM	Filgrastim	In patients, children or adults, with **severe congenital, cyclic,** or **idiopathic neutropenia** with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9$/l, and a history of severe or recurrent infections.	08/06/2010	Hospira UK Ltd
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
NOVOTHIRTEEN	Catridecacog	Long term prophylactic treatment of bleeding in patients 6 years and above with **congenital factor XIII A-subunit deficiency.**	03/09/2012	Novo Nordisk A/S

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
NOXAFIL	Posaconazole	*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	*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
ORENCIA	Abatacept	In combination with methotrexate, for the treatment of moderate to severe active **polyarticular juvenile idiopathic arthritis** (JIA) in paediatric patients 6 years of age and older who have had an insufficient response to other DMARDs including at least one TNF inhibitor.	21/05/2007	Bristol-Myers 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	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

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
PIXUVRI	Pixantrone dimaleate	As monotherapy for the treatment of adult patients with multiply relapsed or refractory aggressive **non-Hodgkin B cell lymphomas** (NHL). The benefit of pixantrone treatment has not been established in patients when used as fifth line or greater chemotherapy in patients who are refractory to last therapy.	10/05/2012	CTI Life Sciences Ltd
PRIVIGEN	Human normal immunoglobulin (IVIg)	*Replacement therapy in : - **Primary immunodeficiency** (PID) 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 : - **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	Treatment of deficient spermatogenesis due to **hypogonadotrophic hypogonadism.**	03/05/1996	NV Organon
RATIOGRASTIM	Filgrastim	In patients, children or adults, with **severe congenital, cyclic,** or **idiopathic neutropenia** with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9$/l, and a history of severe or recurrent infections.	15/09/2008	Ratiopharm GmbH
REFACTO AF	Moroctocog alpha	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
REPLAGAL	Agalsidase alfa	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of **Fabry Disease** (alpha-galactosidase A deficiency)	03/08/2001	Shire Human Genetic Therapies AB
REVOLADE	Eltrombopag	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
RILUTEK	Riluzole	To extend life or the time to mechanical ventilation for patients with **amyotrophic lateral sclerosis** (ALS).	10/06/1996	Aventis Pharma S.A.
ROACTEMRA	Tocilizumab	Treatment of active **systemic juvenile idiopathic arthritis** (sJIA) in patients 2 years of age and older, who have responded inadequately to previous therapy with NSAIDs and systemic corticosteroids. RoActemra can be given as monotherapy (in case of intolerance to MTX or where treatment with MTX is inappropriate) or in combination with MTX.	16/01/2009	Roche Registration Ltd

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
RUCONEST	Conestat alfa	Treatment of acute angioedema attacks in adults with **hereditary angioedema (HAE) due to C1 esterase inhibitor deficiency.**	28/10/2010	Pharming Group N.V.
SAMSCA	Tolvaptan	Treatment of adult patients with hyponatraemia secondary to **syndrome of inappropriate antidiuretic hormone secretion** (SIADH).	03/08/2009	Otsuka Pharmaceutical Europe Ltd
SUTENT	Sunitinib	*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	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
TARGRETIN	Bexarotene	Treatment of skin manifestations of advanced stage **cutaneous T-cell lymphoma** (CTCL) patients refractory to at least one systemic treatment.	29/03/2001	Eisai Ltd
TAXOTERE	Docetaxel	*In combination with cisplatin and 5-fluorouracil for the 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	*Treatment of adult patients with newly-diagnosed **glioblastoma** multiforme concomitantly with radiotherapy (RT) and subsequently as monotherapy treatment. *Treatment of children from the age of three years, adolescents and adult patients with **malignant glioma**, such as **glioblastoma** multiforme or anaplastic **astrocytoma**, showing recurrence or progression after standard therapy.	26/01/1999	Merck Sharp & Dohme Ltd.
TEVAGRASTIM	Filgrastim	In patients, children or adults, with **severe congenital, cyclic,** or **idiopathic neutropenia** with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9$/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

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
THYROGEN	Thyrotropin alfa	For use with serum thyroglobulin (Tg) testing with or without radioiodine imaging for the detection of thyroid remnants and **well-differentiated thyroid cancer** in post-thyroidectomy patients maintained on hormone suppression therapy (THST). Low-risk patients with well-differentiated thyroid carcinoma who have undetectable serum Tg levels on THST and no rh (recombinant human) TSH-stimulated increase of Tg levels may be followed-up by assaying rh TSH-stimulated Tg levels. For pre-therapeutic stimulation in combination with a range of 30 mCi (1.1 GBq) to 100 mCi (3.7 GBq) radioiodine for ablation of thyroid tissue remnants in patients who have undergone a near-total or total thyroidectomy for **well-differentiated thyroid cancer** and who do not have evidence of distant metastatic thyroid cancer.	09/03/2000	Genzyme Europe B.V.
TRISENOX	Arsenic trioxide	Induction of remission and consolidation in adult patients with relapsed/refactory **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
VEDROP	Tocofersolan	Indicated in vitamin E deficiency due to digestive malabsorption in paediatric patients with **congenital chronic cholestasis** or **hereditary chronic cholestasis**, from birth (full term newborns) up to 18 years of age.	24/07/2009	Orphan Europe S.A.R.L
VELCADE	Bortezomib	*In combination with melphalan and prednisone, treatment of adult patients with previously untreated **multiple myeloma** who are not eligible for high-dose chemotherapy with bone marrow transplant. *As monotherapy for the treatment of progressive **multiple myeloma** in adult patients who have received at least one prior therapy and who have already undergone or are unsuitable for bone marrow transplantation.	26/04/2004	Janssen-Cilag International NV
VFEND	Voriconazole	In adults and children aged 2 years and above as follows: - treatment of invasive **aspergillosis**. - treatment of **serious fungal infections** caused by *Scedosporium spp.* and *Fusarium spp.* Vfend should be administered primarily to patients with progressive, possibly life-threatening infections.	19/03/2002	Pfizer Limited

TRADENAME	ACTIVE SUBSTANCE	MARKETING AUTHORISATION INDICATION	MARKETING AUTHORISATION DATE (DD/MM/YYYY)	MARKETING AUTHORISATION HOLDER
VOTRIENT	Pazopanib	*In adults for the first-line treatment of advanced **renal cell carcinoma** (RCC) and for patients who have received prior cytokine therapy for advanced disease. *For the treatment of adult patients with selective subtypes of advanced **soft-tissue sarcoma** (STS) who have received prior chemotherapy for metastatic disease or who have progressed within 12 months after (neo)-adjuvant therapy. Efficacy and safety have only been established in certain STS histological tumour subtypes.	14/06/2010	Glaxo Group Ltd
XELODA	Capecitabine	First-line treatment of advanced **gastric cancer** in combination with a platinum-based regimen	02/02/2001	Roche Registration Limited
XYREM	Sodium oxybate	Treatment of **narcolepsy with cataplexy** in adult patients.	13/10/2005	UCB Pharma Ltd
ZARZIO	Filgrastim	In children and adults with **severe congenital, cyclic, or idiopathic neutropenia** with an absolute neutrophil count (ANC) of $\leq 0.5 \times 10^9$/l, and a history of severe or recurrent infections, long term administration of filgrastim is indicated to increase neutrophil counts and to reduce the incidence and duration of infection-related events.	06/02/2009	Sandoz GmbH
ZEVALIN	Ibritumomab tiuxetan	*Consolidation therapy after remission induction in previously untreated patients with **follicular lymphoma.** *Treatment of adult patients with rituximab relapsed or refractory CD20+ **follicular B-cell non-Hodgkin's lymphoma** (NHL).	16/01/2004	Spectrum Pharmaceuti-cals B.V.
ZUTECTRA	Human Hepatitis B Immunoglobulin	Prevention of **hepatitis B virus** (HBV) **re-infection** in HBV-DNA negative patients over 6 months **after liver transplantation for hepatitis B induced liver failure**. Zutectra is indicated in adults only. The concomitant use of adequate virostatic agents should be considered, if appropriate, as standard of hepatitis B re-infection prophylaxis.	30/11/2009	Biotest Pharma GmbH

By date of MA in descending order

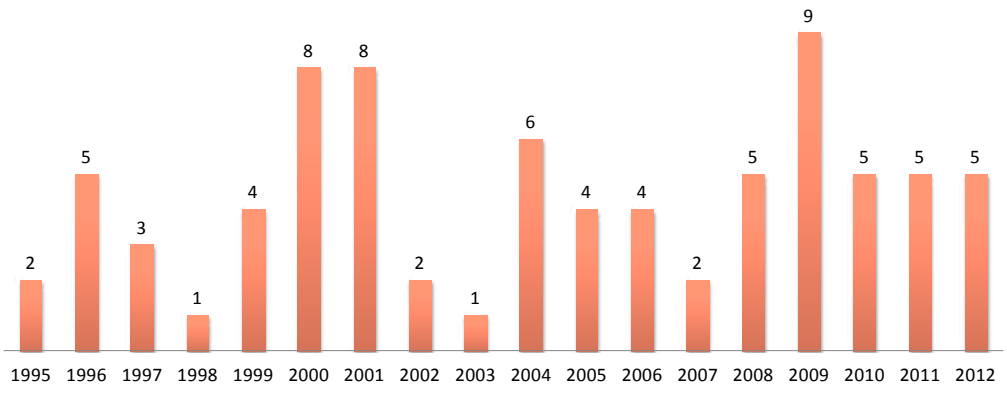
2012
CAPRELSA
COLOBREATHE
INLYTA
NOVOTHIRTEEN
PIXUVRI
2011
BUCCOLAM
CINRYZE
EURARTESIM
HIZENTRA
TEYSUNO
2010
NIVESTIM
OZURDEX
REVOLADE
RUCONEST
VOTRIENT
2009
AFINITOR
FILGRASTIM HEXAL
ILARIS
IXIARO
ROACTEMRA
SAMSCA

VEDROP
ZARZIO
ZUTECTRA
2008
ADCIRCA
BIOGRASTIM
PRIVIGEN
RATIOGRASTIM
TEVAGRASTIM
2007
FLEBOGAMMA DIF
ORENCIA
2006
ATRYN
KIOVIG
OMNITROPE
SUTENT
2005
AVASTIN
NOXAFIL
TARCEVA
XYREM
2004
ADVATE
ALIMTA
DUKORAL

ERBITUX
VELCADE
ZEVALIN
2003
HUMIRA
2002
TRISENOX
VFEND
2001
CANCIDAS
CEPROTIN
FABRAZYME
GLIVEC
INOMAX
REPLAGAL
TARGRETIN
XELODA
2000
ENBREL
HELIXATE NEXGEN
HERCEPTIN
INTRONA
KEPPRA
KOGENATE BAYER
PANRETIN
THYROGEN

1999
AMMONAPS
FERRIPROX
REFACTO AF
TEMODAL
1998
MABTHERA
1997
BENEFIX
CEREZYME
CYSTAGON
1996
CAELYX
HYCAMTIN
NOVOSEVEN
PUREGON
RILUTEK
1995
GONAL-F
TAXOTERE

Number of medicinal products intended for rare diseases in Europe with European market authorisation without prior orphan designation in Europe by date of MA



By ATC category

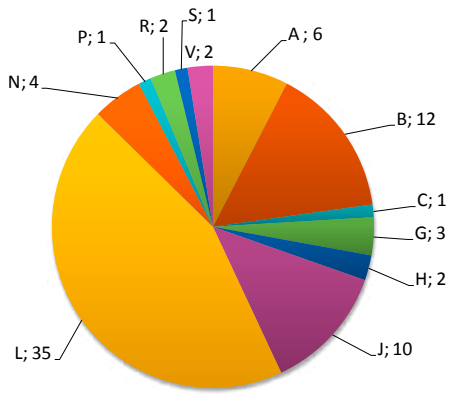
A- ALIMENTARY TRACT AND METABOLISM
AMMONAPS
CEREZYME
CYSTAGON
FABRAZYME
REPLAGAL
VEDROP
B- BLOOD AND BLOOD FORMING ORGANS
ADVATE
ATRYN
BENEFIX
CEPROTIN
CINRYZE
HELIXATE NEXGEN
KOGENATE BAYER
NOVOSEVEN
NOVOTHIRTEEN
REFACTO AF
REVOLADE
RUCONEST
C- CARDIOVASCULAR SYSTEM
SAMSCA

G- GENITO URINARY SYSTEM AND SEX HORMONES
ADCIRCA
GONAL-F
PUREGON
H- SYSTEMIC HORMONAL PREPARATIONS, EXCL, SEX HORMONES AND INSULINS
OMNITROPE
THYROGEN
J- GENERAL ANTIINFECTIVES FOR SYSTEMIC USE
CANCIDAS
DUKORAL
FLEBOGAMMA DIF
HIZENTRA
IXIARO
KIOVIG
NOXAFIL
PRIVIGEN
VFEND
ZUTECTRA
L- ANTINEOPLASTIC AND IMMUNOMODULATING AGENTS
AFINITOR

ALIMTA
AVASTIN
BIOGRASTIM
CAELYX
CAPRELSA
ENBREL
ERBITUX
FILGRASTIM HEXAL
GLIVEC
HERCEPTIN
HUMIRA
HYCAMTIN
ILARIS
INLYTA
INTRONA
MABTHERA
NIVESTIM
ORENCIA
PANRETIN
PIXUVRI
RATIOGRASTIM
ROACTEMRA
SUTENT
TARCEVA
TARGRETIN
TAXOTERE

TEMODAL
TEVAGRASTIM
TEYSUNO
TRISENOX
VELCADE
VOTRIENT
XELODA
ZARZIO
N- NERVOUS SYSTEM
BUCCOLAM
KEPPRA
RILUTEK
XYREM
P- ANTIPARASITIC PRODUCTS, INSECTICIDES AND REPELLENTS
EURARTESIM
R- RESPIRATORY SYSTEM
COLOBREATHE
INOMAX
S- SENSORY ORGANS
OZURDEX
V- VARIOUS
FERRIPROX
ZEVALIN

Number of medicinal products intended for rare diseases in Europe with European market authorisation without prior orphan designation in Europe by ATC category



By MA holder

ABBOTT LABORATORIES LTD
HUMIRA
ALLERGAN PHARMACEUTICALS IRELAND
OZURDEX
APOTEX EUROPE B.V.
FERRIPROX
ASTRAZENECA AB
CAPRELSA
AVENTIS PHARMA S.A.
RILUTEK
TAXOTERE
BAXTER AG
ADVATE
CEPROTIN
KIOVIG
BAYER PHARMA AG
HELIXATE NEXGEN
KOGENATE BAYER
BIOTEST PHARMA GMBH
ZUTECTRA
BRISTOL-MYERS SQUIBB PHARMA EEIG
ORENCIA
CEPHALON EUROPE
TRISENOX
CRUCELL SWEDEN AB
DUKORAL
CSL BEHRING GMBH
HIZENTRA
PRIVIGEN
CT ARZNEIMITTEL GMBH
BIOGRASTIM
CTI LIFE SCIENCES LTD
PIXUVRI
EISAI LTD
PANRETIN
TARGRETIN

ELI LILLY NEDERLAND B.V.
ADCIRCA
ALIMTA
FOREST LABORATORIES UK LTD
COLOBREATHE
GENZYME EUROPE B.V.
CEREZYME
FABRAZYME
THYROGEN
GLAXO GROUP LTD
VOTRIENT
GLAXOSMITHKLINE TRADING SERVICES LIMITED
REVOLADE
GTC BIOTHERAPEUTICS UK LIMITED
ATRYN
HEXAL AG
FILGRASTIM HEXAL
HOSPIRA UK LTD
NIVESTIM
INO THERAPEUTICS AB
INOMAX
INSTITUTO GRIFOLS S.A.
FLEBOGAMMA DIF
INTERCELL AG
IXIARO
JANSSEN-CILAG INTERNATIONAL NV
CAELYX
VELCADE
MERCK KGAA
ERBITUX

MERCK SERONO EUROPE LTD
GONAL-F
MERCK SHARP & DOHME LTD
CANCIDAS
INTRONA
NOXAFIL
TEMODAL
NORDIC GROUP BV
TEYSUNO
NOVARTIS EUROPHARM LTD
AFINITOR
GLIVEC
ILARIS
NOVO NORDISK A/S
NOVOSEVEN
NOVOTHIRTEEN
NV ORGANON
PUREGON
ORPHAN EUROPE S.A.R.L.
CYSTAGON
VEDROP
OTSUKA PHARMACEUTICAL EUROPE LTD
SAMSCA
PFIZER LTD
BENEFIX
ENBREL
INLYTA
REFACTO AF
SUTENT
VFEND
PHARMING GROUP N.V.
RUCONEST
RATIOPHARM GMBH
RATIOGRASTIM

ROCHE REGISTRATION LTD
AVASTIN
HERCEPTIN
MABTHERA
ROACTEMRA
TARCEVA
XELODA
SANDOZ GMBH
OMNITROPE
ZARZIO
SHIRE HUMAN GENETIC THERAPIES AB
REPLAGAL
SIGMA-TAU INDUSTRIE FARMACEUTICHE RIUNITE S.P.A
EURARTESIM
SMITHKLINE BEECHAM LTD
HYCAMTIN
SPECTRUM PHARMACEUTICALS B.V.
ZEVALIN
SWEDISH ORPHAN BIOVITRUM INTERNATIONAL AB
AMMONAPS
TEVA GENERICS GMBH
TEVAGRASTIM
UCB PHARMA LTD
XYREM
UCB PHARMA SA
KEPPRA
VIROPHARMA SPRL
BUCCOLAM
CINRYZE

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