



Les Cahiers d'Orphanet

série Médicaments Orphelins

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Listes des médicaments orphelins en Europe

Avec désignation orpheline et autorisation de mise sur le marché européennes

Avec autorisation de mise sur le marché européenne
sans désignation orpheline préalable

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Méthodologie

Ce document contient la liste de tous les médicaments orphelins ayant reçu une autorisation de mise sur le marché (AMM) européenne à la date indiquée dans le document. Ces produits de santé peuvent n'être disponibles actuellement que dans certains pays européens. En effet, la diffusion dans les pays dépend de la stratégie du laboratoire et de la décision de remboursement prise par les autorités de santé nationales.

Liste des médicaments orphelins en Europe avec désignation orpheline et autorisation de mise sur le marché européennes

La définition « stricte » de médicaments orphelins en Europe concerne des produits de santé ayant obtenu une désignation orpheline européenne (établie selon la loi (EC) No 141/2000), suivie d'une autorisation de mise sur le marché européenne et d'une appréciation positive du service médical rendu.

Cette liste est donc établie par croisement de la liste des produits de santé ayant obtenu une désignation orpheline (<http://ec.europa.eu/enterprise/pharmaceuticals/register/alphreg.htm>) avec la liste des produits ayant obtenu une autorisation de mise sur le marché (<http://ec.europa.eu/enterprise/pharmaceuticals/register/index.htm>).

Ces deux listes sont disponibles sur le site Internet de la Direction Générale Entreprise (DG Entreprise) de la Commission Européenne.

La liste des médicaments orphelins est classée par ordre alphabétique de nom de spécialité.

Les informations proposées sont le nom de la spécialité, le nom de la substance active, l'indication de l'autorisation de mise sur le marché (AMM), la date d'AMM et le titulaire de l'AMM.

Pour permettre une recherche selon différents critères, trois listes annexes sont proposées :

- par date décroissante d'AMM
- par catégorie ATC
- par titulaire d'AMM

Liste des médicaments orphelins en Europe avec autorisation de mise sur le marché européenne sans désignation orpheline préalable


Par extension, l'appellation « médicaments orphelins » peut s'appliquer à des produits de santé ayant obtenu une autorisation de mise sur le marché européenne, mais pour lesquels il n'y a pas eu de désignation orpheline européenne ou pour lesquels elle a été retirée.

Ces médicaments peuvent avoir fait l'objet ou non d'une désignation orpheline dans une autre région du monde.

Dans tous les cas, ils ont obtenu une AMM européenne pour une ou plusieurs indication(s) rare(s) et sont présents dans la liste des produits ayant obtenu une autorisation de mise sur le marché de la DG Entreprise : <http://ec.europa.eu/enterprise/pharmaceuticals/register/index.htm>.

La liste proposée est classée par ordre alphabétique de nom de spécialité.

Les informations fournies sont le nom de la spécialité, le nom de la substance active, l'indication « rare » de l'autorisation de mise sur le marché (AMM), la date d'AMM et le titulaire de l'AMM.

Vous pouvez trouver des informations complémentaires sur chaque médicament dans l'onglet « Médicaments orphelins » du site www.orphanet.fr ou sur le site de l'EMA (Agence Européenne du médicament) <http://www.emea.europa.eu>. Le registre de l'EMA liste tous les médicaments avec AMM, pas seulement les médicaments orphelins. Les médicaments orphelins ayant obtenu une désignation orpheline européenne sont identifiables grâce au logo .

Les informations sont proposées dans 22 langues de la Communauté Européenne.

Pour tout commentaire ou question, s'adresser à : contact.orphanet@inserm.fr

Liste des médicaments orphelins en Europe avec désignation orpheline et autorisation de mise sur le marché européennes

1- Par ordre alphabétique de nom de spécialité

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
AFINITOR	Everolimus (INN)	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
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.
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 deficiency	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
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.

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
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
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	Jerini AG
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

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
GLIVEC	Imatinib mesilate (INN)	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-PDGFR α rearrangement - adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumours (GIST) - 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
INCRELEX	Mecasermin (INN)	Long-term treatment of growth failure in children and adolescents with severe primary insulin-like growth factor 1 deficiency (Primary IGF1D)	03/08/2007	Tercica Europe Ltd
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	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 KGaA
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

INN - International Nonproprietary Name = DCI - Dénomination Commune Internationale

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
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 HealthCare 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.
NYMUSA	Caffeine citrate	Treatment of primary apnea of premature newborns	02/07/2009	Chiesi Farmaceutici SpA
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	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 International AB
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.
PHOTOBARR	Porfimer sodium (INN) (for use with photodynamic therapy)	Ablation of high-grade dysplasia (HGD) in patients with Barrett's Oesophagus	25/03/2004	Axcan Pharma International BV
PRIALT	Ziconotide (INN) (intraspinal use)	Treatment of severe, chronic pain in patients who require intrathecal (IT) analgesia	21/02/2005	Eisai Ltd

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NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
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 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
SAVENE	Dexrazoxane (INN)	Treatment of anthracycline extravasation	28/07/2006	TopoTarget A/S
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 SAS
SOLIRIS	Eculizumab (INN)	Treatment of patients with paroxysmal nocturnal haemoglobinuria (PNH)	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 adults with chronic, accelerated or blast phase chronic myeloid leukaemia (CML) with resistance or intolerance to prior therapy including imatinib mesilate Treatment of adults with Philadelphia chromosome positive (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)	Ce produit avait reçu une désignation orpheline le 10 Mars 2005. Suite à une requête du Titulaire de l'AMM, le Sutent a été exclu du Registre Communautaire des médicaments orphelins. Cf "Liste des médicaments orphelins en Europe avec autorisation de mise sur le marché européenne sans désignation orpheline préalable"		
TASIGNA	Nilotinib (INN)	Treatment of adults with chronic phase and accelerated phase Philadelphia chromosome positive chronic myelogenous leukaemia (CML) with resistance or intolerance to prior therapy including imatinib	19/11/2007	Novartis Europharm Ltd
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

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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.	10/08/2006	Encysive (UK) Ltd
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	Wyeth Europa Ltd
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 To reduce the number of new digital ulcers in patients with systemic sclerosis and ongoing digital ulcer disease	15/05/2002	Actelion Registration Ltd
TRISENOX	Arsenic Trioxide (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	21/04/2008	Glaxo Group Ltd
WILZIN	Zinc acetate dihydrate (INN)	Treatment of Wilson's disease	13/10/2004	Orphan Europe S.a.r.l.

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NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
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
XYREM	Sodium oxybate (INN)	Treatment of narcolepsy with cataplexy in adult patients	13/10/2005	UCB Pharma Ltd
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.	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

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2- Par date décroissante d'AMM

2009
AFINITOR
CAYSTON
MEPACT
MOZOBIL
NPLATE
NYMUSA
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
THELIN

2005
ORFADIN
PRIALT
REVATIO
XYREM
2004
LITAK
LYSODREN
PEDEA
PHOTOBARR
WILZIN
XAGRID
2003
ALDURAZYME
BUSILVEX
CARBAGLU
ONSENAL
VENTAVIS
2002
SOMAVERT
TRACLEER
TRISENOX
ZAVESCA
2001
FABRAZYME
GLIVEC
REPLAGAL

3- Par catégorie ATC

A- SYSTÈME DIGESTIF ET MÉTABOLISME	L- ANTINÉOPLASIQUES ET AGENTS IMMUNOMODULANTS	V- DIVERS
ALDURAZYME	AFINITOR	EXJADE
CARBAGLU	ATRIANCE	SAVENE
CYSTADANE	BUSILVEX	
ELAPRASE	CEPLENE	
FABRAZYME	EVOLTRA	
KUVAN	GLIOLAN	
MYOZYME	GLIVEC	
NAGLAZYME	LITAK	
ORFADIN	LYSODREN	
REPLAGAL	MEPACT	
WILZIN	MOZOBIL	
ZAVESCA	NEXAVAR	
B- SANG ET ORGANES HÉMATOPOIÉTIQUES	ONSENAL	
NPLATE	PHOTOBARR	
VENTAVIS	REVLIMID	
C- SYSTÈME CARDIO-VASCULAIRE	SIKLOS	
FIRAZYR	SOLIRIS	
PEDEA	SPRYCEL	
THELIN	TASIGNA	
TRACLEER	THALIDOMIDE CELGENE	
VOLIBRIS	TORISEL	
G- SYSTÈME GÉNITO-URINAIRE ET HORMONES SEXUELLES	TRISENOX	
REVATIO	VIDAZA	
H- PRÉPARATIONS SYSTÉMIQUES HORMONALES, À L'EXCLUSION DES HORMONES SEXUELLES ET DES INSULINES	XAGRID	
INCRELEX	YONDELIS	
SOMAVERT	N- SYSTÈME NERVEUX	
J- ANTI-INFECTIEUX GÉNÉRAUX À USAGE SYSTÉMIQUE	DIACOMIT	
CAYSTON	INOVELON	
	NYMUSA	
	PRIALT	
	XYREM	

4- Par titulaire d'AMM

ACTELION REGISTRATION LTD	EPICEPT GMBH	PFIZER LTD
TRACLEER	CEPLENE	ONSENAL
ZAVESCA	GENZYME EUROPE B.V.	REVATIO
ADDMEDICA SAS	ALDURAZYME	SOMAVERT
SIKLOS	EVOLTRA	PHARMA MAR S.A.
ALEXION EUROPE SAS	FABRAZYME	YONDELIS
SOLIRIS	MOZOBIL	PIERRE FABRE MÉDICAMENT
AMGEN EUROPE B.V.	MYOZYME	BUSILVEX
NPLATE	GILEAD SCIENCES INTERNATIONAL LIMITED	SHIRE HUMAN GENETIC THERAPIES AB
AXCAN PHARMA INTERNATIONAL BV	CAYSTON	ELAPRASE
PHOTOBARR	GLAXO GROUP LTD	REPLAGAL
BAYER HEALTHCARE AG	ATRIANCE	SHIRE PHARMACEUTICAL CONTRACTS LTD
NEXAVAR	VOLIBRIS	XAGRID
BAYER SCHERING PHARMA AG	IDM PHARMA	SWEDISH ORPHAN INTERNATIONAL AB
VENTAVIS	MEPACT	ORFADIN
BIOCODEX	JERINI AG	TERCICA EUROPE LTD
DIACOMIT	FIRAZYR	INCRELEX
BIOMARIN EUROPE LTD	LABORATOIRE HRA PHARMA	TOPOTARGET A/S
NAGLAZYME	LYSODREN	SAVENE
BRISTOL-MYERS SQUIBB PHARMA EEIG	LIPOMED GMBH	UCB PHARMA LTD
SPRYCEL	LITAK	XYREM
CELGENE EUROPE LTD	MEDAC GMBH	WYETH EUROPA LTD
REVLIMID	GLIOLAN	TORISEL
THALIDOMIDE CELGENE	MERCK KGAA	
VIDAZA	KUVAN	
CEPHALON EUROPE	NOVARTIS EUROPHARM LTD	
TRISENOX	AFINITOR	
CHIESI FARMACEUTICI SPA	EXJADE	
NYMUSA	GLIVEC	
EISAI LTD	TASIGNA	
INOVELON	ORPHAN EUROPE S.A.R.L.	
PRIALT	CARBAGLU	
ENCYSIVE (UK) LTD	CYSTADANE	
THELIN	PEDEA	
	WILZIN	

Liste des médicaments orphelins en Europe avec autorisation de mise sur le marché européenne sans désignation orpheline préalable

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
ADVATE	Octocog alpha (INN)	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency)	02/03/2004	Baxter AG
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	08/12/1999	Swedish Orphan 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	LEO Pharma A/S
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
BIOGRASTIM	Filgrastim (INN)	In patients, children or adults, with severe congenital, cyclic , or idiopathic neutropenia with an absolute neutrophil count (ANC) of 0.5 x 10 ⁹ /L, and a history of severe or recurrent infections	15/09/2008	CT Arzneimittel GmbH
CAELYX	Doxorubicin hydrochloride (pegylated liposomal)	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	SP Europe
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

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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	17/11/1997	Genzyme Europe B.V.
CYSTAGON	Mercaptamine bitartrate (INN)	Treatment of proven nephropathic cystinosis	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	SBL Vaccin AB
ENBREL	Etanercept (INN)	Treatment of active polyarticular juvenile idiopathic arthritis in children and adolescents from the age of 4 years who have had an inadequate response to, or who have proved intolerant of, methotrexate	03/02/2000	Wyeth Europa 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
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.
FERTAVID	Follitropin beta (INN)	Treatment of deficient spermatogenesis due to hypogonadotropic hypogonadism	19/03/2009	Schering-Plough Europe
FILGRASTIM RATIOPHARM	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
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	Serono Europe Limited
HELIXATE NEXGEN	Octocog alpha (INN)	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency)	04/08/2000	Bayer HealthCare AG
HUMIRA	Adalimumab (INN)	In combination with methotrexate for the treatment of active polyarticular juvenile idiopathic arthritis , in adolescents aged 13 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	08/09/2003	Abbott Laboratories Ltd.

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HYCAMTIN	Topotecan (INN)	Treatment of patients with metastatic carcinoma of the ovary after failure of first-line or subsequent therapy Treatment of 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
INOMAX	Nitric oxide (INN)	In conjunction with ventilatory support and other appropriate agents, for the treatment of newborns \geq 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	01/08/2001	INO Therapeutics AB
INTRONA	Interferon alfa-2b (INN)	Treatment of patients with hairy cell leukaemia Treatment of adult patients with Philadelphia chromosome or bcr/abl translocation positive chronic myelogenous leukaemia 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	SP Europe
IXIARO	Japanese Encephalitis Vaccine (inactivated, adsorbed)	For active immunization against Japanese encephalitis for adults	31/03/2009	Intercell AG
KEPPRA	Levetiracetam (INN)	As adjunctive therapy in the treatment of myoclonic seizures in adults and adolescents from 12 years of age with Juvenile Myoclonic Epilepsy . In the treatment of partial onset seizures with or without secondary generalisation in infants from 1 month of age with epilepsy	29/09/2000	UCB Pharma SA
KOGENATE BAYER	Octocog alpha (INN)	Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency)	04/08/2000	Bayer HealthCare 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

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MABTHERA	Rituximab (INN)	Treatment of previously untreated patients with stage III-IV follicular lymphoma in combination with chemotherapy. Maintenance therapy is indicated for patients with relapsed/refractory follicular lymphoma responding to induction therapy with chemotherapy with or without MabThera. 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 First-line treatment of patients with chronic lymphocytic leukaemia (CLL) in combination with chemotherapy	02/06/1998	Roche Registration Limited
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 - Chromoblastomycosis and mycetoma in patients with disease that is refractory to itraconazole or in patients who are intolerant of itraconazole 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	Schering-Plough Europe

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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
PANRETIN	Alitretinoïn (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 : - 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 (INN)	Treatment of deficient spermatogenesis due to hypogonadotrophic 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	Wyeth Europa 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	Pharmion Ltd.

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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.
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 malate (INN)	Treatment of unresectable and/or metastatic malignant gastrointestinal stromal tumour (GIST) after failure of imatinib mesylate treatment due to resistance or intolerance	19/07/2006	Pfizer Limited
TAXOTERE	Docetaxel trihydrate (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 patients with newly diagnosed glioblastoma multiforme concomitantly with radiotherapy (RT) and subsequently as monotherapy treatment Treatment of patients with malignant glioma, such as glioblastoma multiforme or anaplastic astrocytoma , showing recurrence or progression after standard therapy	26/01/1999	SP Europe
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
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

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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
XELODA	Capecitabine (INN)	First-line treatment of advanced gastric cancer in combination with a platinum-based regimen	02/02/2001	Roche Registration Limited
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 Schering Pharma

Pour toute question ou suggestion, n'hésitez pas à nous contacter: contact.orphanet@inserm.fr

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