



# Les Cahiers d'Orphanet

*série Médicaments Orphelins*

Avril 2011

## 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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## Sommaire

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### Méthodologie 3

#### Liste des médicaments orphelins en Europe avec désignation orpheline et autorisation de mise sur le marché européennes 4

1- Par ordre alphabétique de nom de spécialité.....	4
2- Par date décroissante d'AMM.....	12
3- Par catégorie ATC.....	13
4- Par titulaire d'AMM.....	14

#### Liste des médicaments orphelins en Europe avec autorisation de mise sur le marché européenne sans désignation orpheline préalable 15

1- Par ordre alphabétique de nom de spécialité.....	15
2- Par date décroissante d'AMM.....	25
3- Par catégorie ATC.....	26
4- Par titulaire d'AMM.....	27

## 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/health/documents/community-register/html/alforphreg.htm>) avec la liste des produits ayant obtenu une autorisation de mise sur le marché (<http://ec.europa.eu/health/documents/community-register/html/alfregister.htm>).

Ces deux listes sont disponibles sur le site Internet de la Direction Générale de la santé et des consommateurs (DG Sanco) 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 Sanco : <http://ec.europa.eu/health/documents/community-register/html/alfregister.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.

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

- par date décroissante d'AMM
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Vous pouvez trouver des informations complémentaires sur chaque médicament dans l'onglet « Médicaments orphelins » du site [www.orphanet.fr](http://www.orphanet.fr) ou sur le site de l'EMA (Agence Européenne du Médicament) <http://www.ema.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](mailto: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 <b>renal cell carcinoma</b> , 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 <b>Mucopolysaccharidosis I (MPS I)</b> ; a [alpha]-L-iduronidase deficiency) to treat the non-neurological manifestations of the disease	10/06/2003	Genzyme Europe B.V.
ARZERRA	Ofatumumab (INN)	Treatment of <b>chronic lymphocytic leukaemia (CLL)</b> in patients who are refractory to fludarabine and alemtuzumab	19/04/2010	Glaxo Group Ltd
ATRIANCE	Nelarabine (INN)	Treatment of patients with <b>T-cell acute lymphoblastic leukaemia (T-ALL)</b> and <b>T-cell lymphoblastic lymphoma (T-LBL)</b> whose disease has not responded to or has relapsed following treatment with at least two chemotherapy regimens	22/08/2007	Glaxo Group Ltd
BUSILVEX	Busulfan (INN) (Intravenous use)	Followed by cyclophosphamide (BuCy2) is indicated as conditioning treatment prior to conventional <b>haematopoietic progenitor cell transplantation (HPCT)</b> 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 <b>hyperammonaemia due to N-acetylglutamate synthase deficiency</b>	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 <b>cystic fibrosis (CF)</b> aged 18 years and older	21/09/2009	Gilead Sciences International Limited
CEPLENE	Histamine dihydrochloride	Treatment of adult patients with <b>acute myeloid leukaemia</b> in first remission concomitantly treated with interleukin-2 (IL-2)	07/10/2008	EpiCept GmbH

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
CYSTADANE	Betaine anhydrous (INN)	Adjunctive treatment of <b>homocystinuria</b> , involving deficiencies or defects in cystathionine beta-synthase (CBS), 5,10-methylene-tetrahydrofolate reductase (MTHFR), cobalamin cofactor metabolism (cbl). Cystadane should be used as supplement to other therapies such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), folate and a specific diet	15/02/2007	Orphan Europe S.a.r.l.
DIACOMIT	Stiripentol (INN)	Use in conjunction with clobazam and valproate as adjunctive therapy of refractory generalized tonic-clonic seizures in patients with <b>severe myoclonic epilepsy in infancy</b> (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 <b>Hunter syndrome</b> (Mucopolysaccharidosis II, MPS II)	08/01/2007	Shire Human Genetic Therapies AB
ESBRIET	Pirfenidone	In adults for the treatment of mild to moderate <b>Idiopathic Pulmonary Fibrosis</b> (IPF)	28/02/2011	InterMune Europe Ltd.
EVOLTRA	Clofarabine (INN)	Treatment of <b>acute lymphoblastic leukaemia</b> (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 ( $\geq 7$ ml/kg/month of packed red blood cells) in patients with <b>beta thalassaemia major</b> aged 6 years and older. Treatment of <b>chronic iron overload</b> 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 <b>Fabry disease</b> (alpha-galactosidase A deficiency)	03/08/2001	Genzyme Europe B.V.
FIRAZYR	Icatibant acetate INN = Icatibant	Symptomatic treatment of acute attacks of <b>hereditary angioedema</b> (HAE) in adults (with C1-esterase-inhibitor deficiency)	11/07/2008	Jerini AG
FIRDAPSE (ex-ZENAS)	Amifampridine (INN)	Symptomatic treatment of <b>Lambert-Eaton myasthenic syndrome</b> (LEMS) in adults	23/12/2009	EUSA Pharma SAS
GLIOLAN	5-aminolevulinic acid hydrochloride (INN)	Visualisation of malignant tissue during surgery for <b>malignant glioma</b> (WHO grade III and IV)	07/09/2007	Medac GmbH

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GLIVEC	Imatinib mesilate (INN)	<p>Treatment of :</p> <ul style="list-style-type: none"> <li>- adult and paediatric patients with newly diagnosed Philadelphia chromosome (bcr-abl) positive (Ph+) <b>chronic myeloid leukaemia</b> (CML) for whom bone marrow transplantation is not considered as the first line of treatment</li> <li>- adult and paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy, or in accelerated phase or blast crisis</li> <li>- adult patients with newly diagnosed Philadelphia chromosome positive <b>acute lymphoblastic leukaemia</b> (Ph+ ALL) integrated with chemotherapy</li> <li>- adult patients with relapsed or refractory Ph+ ALL as monotherapy</li> <li>- adult patients with <b>myelodysplastic/myeloproliferative diseases</b> (MDS/MPD) associated with platelet-derived growth factor receptor (PDGFR) gene re-arrangements</li> <li>- adult patients with advanced <b>hypereosinophilic syndrome</b> (HES) and/or <b>chronic eosinophilic leukaemia</b> (CEL) with FIP1L1-PDGFR<math>\alpha</math> rearrangement</li> <li>- adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant <b>gastrointestinal stromal tumours</b> (GIST)</li> <li>- adjuvant treatment of adult patients who are at significant risk of relapse following resection of Kit (CD117)-positive <b>GIST</b>. Patients who have a low or very low risk of recurrence should not receive adjuvant treatment</li> <li>- adult patients with unresectable <b>dermatofibrosarcoma protuberans</b> (DFSP) and adult patients with recurrent and/or metastatic DFSP who are not eligible for surgery</li> </ul>	07/11/2001	Novartis Europharm Ltd
ILARIS	Canakinumab (INN)	<p>Treatment of <b>Cryopyrin-Associated Periodic Syndromes</b> (CAPS) in adults, adolescents and children aged 4 years and older with body weight above 15 kg, including:</p> <ul style="list-style-type: none"> <li>- <b>Muckle-Wells Syndrome</b> (MWS),</li> <li>- <b>Neonatal-Onset Multisystem Inflammatory Disease</b> (NOMID) / <b>Chronic Infantile Neurological, Cutaneous, Articular Syndrome</b> (CINCA),</li> <li>- Severe forms of <b>Familial Cold Autoinflammatory Syndrome</b> (FCAS) / <b>Familial Cold Urticaria</b> (FCU) presenting with signs and symptoms beyond cold-induced urticarial skin rash</li> </ul>	23/10/2009	Novartis Europharm Ltd
INCRELEX	Mecasermin (INN)	Long-term treatment of <b>growth failure</b> in children and adolescents with severe <b>primary insulin-like growth factor 1 deficiency</b> (Primary IGF1)	03/08/2007	Ipsen Pharma
INOVELON	Rufinamide (INN)	Adjunctive therapy in the treatment of seizures associated with <b>Lennox Gastaut syndrome</b> in patients aged 4 years and older	16/01/2007	Eisai Ltd

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KUVAN	Sapropterin dihydrochloride INN = Sapropterin	Treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients of 4 years of age and over with <b>phenylketonuria</b> (PKU) who have been shown to be responsive to such treatment Treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients with <b>tetrahydrobiopterin (BH4) deficiency</b> who have been shown to be responsive to such treatment	02/12/2008	Merck KGaA
LITAK	Cladribine (INN) (subcutaneous use)	Treatment of <b>hairy cell leukaemia</b>	14/04/2004	Lipomed GmbH
LYSODREN	Mitotane (INN)	Symptomatic treatment of advanced (unresectable, metastatic or relapsed) <b>adrenal cortical carcinoma</b>	28/04/2004	Laboratoire HRA Pharma
MEPACT	Mifamurtide (INN)	In children, adolescents and young adults for the treatment of high-grade resectable non-metastatic <b>osteosarcoma</b> after macroscopically complete surgical resection. It is used in combination with post-operative multi-agent chemotherapy	06/03/2009	IDM Pharma S.A.
MOZOBIL	Plerixafor (INN)	In combination with G-CSF to enhance mobilisation of <b>haematopoietic stem cells</b> to the peripheral blood for collection and subsequent <b>autologous transplantation</b> in patients with <b>lymphoma</b> and <b>multiple myeloma</b> 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 <b>Pompe disease</b> (acid $\alpha$ -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 <b>Mucopolysaccharidosis VI (MPS VI)</b> ; N-acetylgalactosamine 4-sulfatase deficiency; Maroteaux-Lamy syndrome)	24/01/2006	BioMarin Europe Ltd
NEXAVAR	Sorafenib tosylate Sorafenib (INN)	Treatment of <b>hepatocellular carcinoma</b> Treatment of patients with advanced <b>renal cell carcinoma</b> who have failed prior interferon-alpha or interleukin-2 based therapy or are considered unsuitable for such therapy	19/07/2006	Bayer Schering Pharma AG
NPLATE	Romiplostim (INN)	Adult chronic <b>immune (idiopathic) thrombocytopenic purpura</b> (ITP) in splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Nplate may be considered as second line treatment for adult non-splenectomised patients where surgery is contra-indicated	04/02/2009	Amgen Europe B.V.
ONSENAL	Celecoxib (INN)	Reduction of the number of adenomatous intestinal polyps in <b>familial adenomatous polyposis (FAP)</b> , as an adjunct to surgery and further endoscopic surveillance	17/10/2003	Pfizer Ltd

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ORFADIN	Nitisinone (INN)	Treatment of patients with confirmed diagnosis of <b>hereditary tyrosinemia type 1</b> (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 <b>patent ductus arteriosus</b> in preterm newborn infants less than 34 weeks of gestational age	29/07/2004	Orphan Europe S.a.r.l.
PEYONA (ex-NYMUSA)	Caffeine citrate	Treatment of <b>primary apnea</b> of premature newborns	02/07/2009	Chiesi Farmaceutici SpA
PHOTOBARR	Porfimer sodium (INN) (for use with photodynamic therapy)	Ablation of high-grade dysplasia (HGD) in patients with <b>Barrett's Oesophagus</b>	25/03/2004	Axcan Pharma International BV
PRIALT	Ziconotide (INN) (intraspinal use)	Treatment of severe, <b>chronic pain</b> in patients who require <b>intrathecal (IT) analgesia</b>	21/02/2005	Eisai Ltd
REPLAGAL	Agalsidase alfa (INN)	Long-term enzyme replacement therapy in patients with a confirmed diagnosis of <b>Fabry Disease</b> (alpha-galactosidase A deficiency)	03/08/2001	Shire Human Genetic Therapies AB
REVATIO	Sildenafil citrate INN = Sildenafil	Revatio for oral use is indicated for treatment of patients with <b>pulmonary arterial hypertension</b> classified as WHO functional class II and III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and pulmonary hypertension associated with connective tissue disease. Revatio solution for injection is for the treatment of patients with pulmonary arterial hypertension who are currently prescribed oral Revatio and who are temporarily unable to take oral medicine, but are otherwise clinically and haemodynamically stable	28/10/2005	Pfizer Ltd
REVLIMID	Lenalidomide (INN)	In combination with dexamethasone, treatment of <b>multiple myeloma</b> patients who have received at least one prior therapy	14/06/2007	Celgene Europe Ltd
REVOLADE	Eltrombopag (INN)	For adult chronic <b>immune (idiopathic) thrombocytopenic purpura (ITP)</b> splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Revolade may be considered as second line treatment for adult non-splenectomised patients where surgery is contraindicated	11/03/2010	Glaxo-SmithKline Trading Services Limited
RILONACEPT REGENERON (ex-ARCALYST)	Rilonacept (INN)	Treatment of <b>Cryopyrin-Associated Periodic Syndromes</b> (CAPS) with severe symptoms, including <b>Familial Cold Autoinflammatory Syndrome</b> (FCAS) and <b>Muckle-Wells Syndrome</b> (MWS), in adults and children aged 12 years and older	23/10/2009	Regeneron UK Limited
SAVENE	Dexrazoxane (INN)	Treatment of <b>anthracycline extravasation</b>	28/07/2006	TopoTarget A/S

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SIKLOS	Hydroxycarbamide (INN)	Prevention of recurrent painful vaso-occlusive crises including acute chest syndrome in paediatric and adult patients suffering from symptomatic <b>Sickle Cell Syndrome</b>	29/06/2007	Addmedica SAS
SOLIRIS	Eculizumab (INN)	Treatment of patients with <b>paroxysmal nocturnal haemoglobinuria</b> (PNH)	20/06/2007	Alexion Europe SAS
SOMAVERT	Pegvisomant (INN)	Treatment of patients with <b>acromegaly</b> who have had an inadequate response to surgery and/or radiation therapy and in whom an appropriate medical treatment with somatostatin analogues did not normalize IGF-I concentrations or was not tolerated	13/11/2002	Pfizer Ltd
SPRYCEL	Dasatinib (INN)	Treatment of adult patients with: - newly diagnosed Philadelphia chromosome positive (Ph+) <b>chronic myelogenous leukaemia</b> (CML) in the chronic phase. - chronic, accelerated or blast phase CML with resistance or intolerance to prior therapy including imatinib mesilate. - Ph+ <b>acute lymphoblastic leukaemia</b> (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)	150 mg: Treatment of adult patients with newly diagnosed Philadelphia chromosome positive <b>chronic myelogenous leukaemia</b> (CML) in the chronic phase. 200 mg: Treatment of adult patients with: - newly diagnosed Philadelphia chromosome positive chronic myelogenous leukaemia (CML) in the chronic phase - chronic phase and accelerated phase Philadelphia chromosome positive chronic myelogenous leukaemia (CML) with resistance or intolerance to prior therapy including imatinib. Efficacy data in patients with CML in blast crisis are not available.	19/11/2007	Novartis Europharm Ltd
TEPADINA	Thiotepa (INN)	In combination with other chemotherapy medicinal products: 1) with or without total body irradiation (TBI), as conditioning treatment prior to <b>allogeneic or autologous haematopoietic progenitor cell transplantation</b> (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.

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THALIDOMIDE CELGENE	Thalidomide (INN)	In combination with melphalan and prednisone as first line treatment of patients with untreated <b>multiple myeloma</b> , aged $\geq$ 65 years or ineligible for high dose chemotherapy	16/04/2008	Celgene Europe Ltd
THELIN	Sitaxentan sodium (INN)	Treatment of patients with <b>pulmonary arterial hypertension</b> classified as WHO functional class III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and in pulmonary hypertension associated with connective tissue disease. <b><i>This medicine is now withdrawn from use in the European Union, more information on:</i></b> <a href="http://www.ema.europa.eu">www.ema.europa.eu</a>	10/08/2006	Pfizer Ltd
TORISEL	Temsirolimus (INN)	First-line treatment of patients with advanced <b>renal cell carcinoma</b> who have at least three of six prognostic risk factors Treatment of adult patients with relapsed and/or refractory <b>mantle cell lymphoma</b> (MCL)	19/11/2007	Wyeth Europa Ltd
TRACLEER	Bosentan mono- hydrate INN = Bosentan	Treatment of <b>pulmonary arterial hypertension</b> (PAH) to improve exercise capacity and symptoms in patients with WHO functional class III. Efficacy has been shown in: Primary (idiopathic and familial) PAH, PAH secondary to scleroderma without significant interstitial pulmonary disease, PAH associated with congenital systemic-to-pulmonary shunts and Eisenmenger's physiology. Some improvements have also been shown in patients with PAH WHO functional class II. To reduce the number of new digital ulcers in patients with <b>systemic sclerosis</b> and ongoing digital ulcer disease	15/05/2002	Actelion Registra- tion Ltd
TRISENOX	Arsenic Trioxide (INN)	Induction of remission and consolidation in adult patients with relapsed/refractory <b>acute promyelocytic leukaemia</b> (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 <b>primary pulmonary hypertension</b> , classified as NYHA functional class III, to improve exercise capacity and symptoms	16/09/2003	Bayer Schering Pharma AG

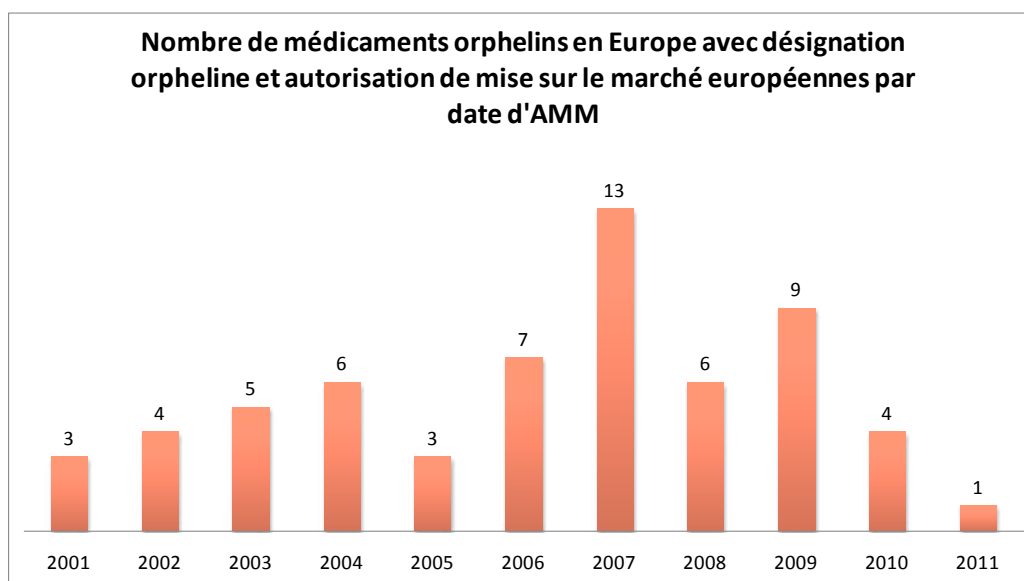
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NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
VIDAZA	Azacitidine (INN)	Treatment of adult patients who are not eligible for haematopoietic stem cell transplantation with: -intermediate 2 and high risk <b>myelodysplastic syndromes</b> (MDS) according to the International Prognostic Scoring System (IPSS) - <b>chronic myelomonocytic leukaemia</b> (CMML) with 10-29 % marrow blasts without myeloproliferative disorder - <b>acute myeloid leukaemia</b> (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 <b>pulmonary arterial hypertension</b> (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
VPRIV	Velaglucerase alfa	Long-term enzyme replacement therapy (ERT) in patients with <b>type 1 Gaucher disease</b>	26/08/2010	Shire Pharmaceuticals Ireland Ltd
WILZIN	Zinc acetate dihydrate (INN)	Treatment of <b>Wilson's disease</b>	13/10/2004	Orphan Europe S.a.r.l.
XAGRID	Anagrelide hydrochloride INN = Anagrelide	Reduction of elevated platelet counts in at risk <b>essential thrombocythaemia</b> patients who are intolerant to their current therapy or whose elevated platelet counts are not reduced to an acceptable level by their current therapy	16/11/2004	Shire Pharmaceutical Contracts Ltd
XYREM	Sodium oxybate (INN)	Ce produit avait reçu une désignation orpheline le 3 Février 2003. Suite à une requête du Titulaire de l'AMM, le Xyrem 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"		
YONDELIS	Trabectedin (INN)	Treatment of patients with advanced <b>soft tissue sarcoma</b> , after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents. Efficacy data are based mainly on <b>liposarcoma</b> and <b>leiomyosarcoma</b> patients In combination with pegylated liposomal doxorubicin (PLD), treatment of patients with relapsed platinum-sensitive <b>ovarian cancer</b>	17/09/2007	Pharma Mar S.A.
ZAVESCA	Miglustat (INN)	Oral treatment of mild to moderate <b>type 1 Gaucher disease</b> in patients for whom enzyme replacement therapy is unsuitable Treatment of progressive neurological manifestations in adult patients and paediatric patients with <b>Niemann-Pick type C disease</b>	20/11/2002	Actelion Registration Ltd

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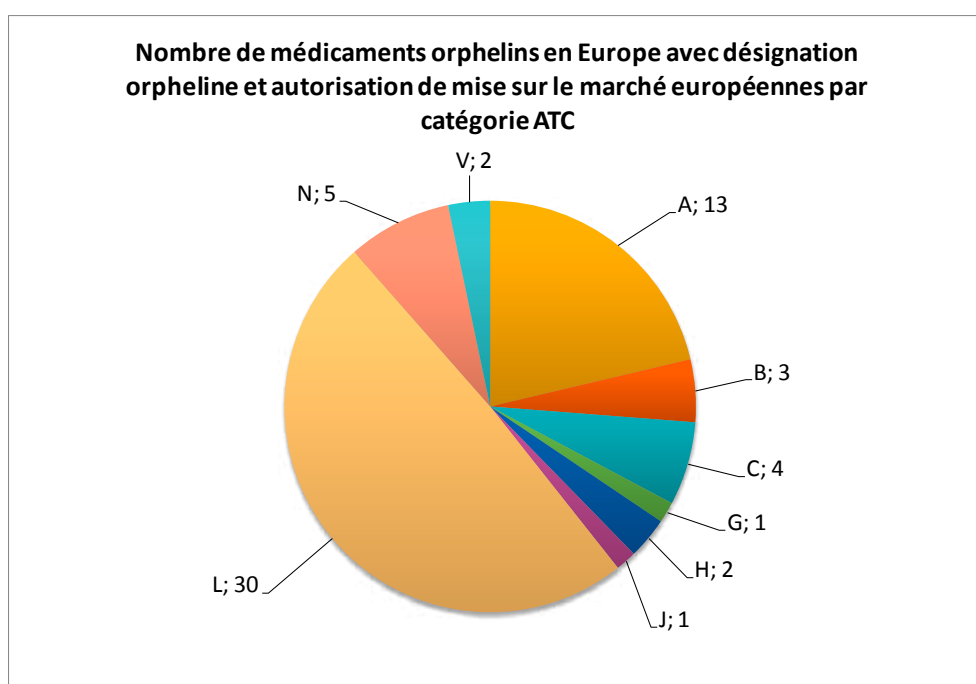
## 2- Par date décroissante d'AMM

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



### 3- Par catégorie ATC

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



## 4- Par titulaire d'AMM

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

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

### 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
ADCIRCA	Tadalafil (INN)	Treatment of <b>pulmonary arterial hypertension</b> (PAH) classified as WHO functional class II and III, to improve exercise capacity. Efficacy has been shown in idiopathic PAH (IPAH) and in PAH related to collagen vascular disease	30/11/2009	Eli Lilly Nederland B.V.
ADVATE	Octocog alpha (INN)	Treatment and prophylaxis of bleeding in patients with <b>haemophilia A</b> (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 <b>mesothelioma</b>	20/09/2004	Eli Lilly Nederland B.V.
AMMONAPS	Sodium phenylbutyrate (INN)	Adjunctive therapy in the chronic management of <b>urea cycle disorders</b> , 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 International AB
ATRYN	Antithrombin alpha (INN)	Prophylaxis of venous thromboembolism in surgery of patients with <b>congenital antithrombin deficiency</b> , normally given in association with heparin or low molecular weight heparin	28/07/2006	GTC Biotherapeutics UK Limited
AVASTIN	Bevacizumab (INN)	In combination with interferon alfa-2a, for first line treatment of patients with advanced and/or metastatic <b>renal cell cancer</b>	12/01/2005	Roche Registration Limited
BENEFIX	Recombinant coagulation Factor IX INN = Nonacog alpha	Treatment and prophylaxis of bleeding in patients with <b>haemophilia B</b> (congenital factor IX deficiency)	27/08/1997	Wyeth Europa Ltd
BIOGRASTIM	Filgrastim (INN)	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$ , and a history of severe or recurrent infections	15/09/2008	CT Arzneimittel GmbH

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
CAELYX	Doxorubicin hydrochloride (pegylated liposomal)	For treatment of advanced <b>ovarian cancer</b> in women who have failed a first-line platinum-based chemotherapy regimen In combination with bortezomib for the treatment of progressive <b>multiple myeloma</b> in patients who have received at least one prior therapy and who have already undergone or are unsuitable for bone marrow transplant Treatment of AIDS-related <b>Kaposi's sarcoma</b> (KS) in patients with low CD4 counts (< 200 CD4 lymphocytes/mm <sup>3</sup> ) and extensive mucocutaneous or visceral disease	21/06/1996	SP Europe
CANCIDAS	Caspofungin	Treatment of invasive <b>aspergillosis</b> in adult or paediatric patients who are refractory to or intolerant of amphotericin B, lipid formulations of amphotericin B and/or itraconazole. Empirical therapy for presumed fungal infections (such as Candida or Aspergillus) in febrile, neutropaenic adult or paediatric patients	24/10/2001	Merck Sharp & Dohme Ltd
CEPROTIN	Human protein C (INN)	In purpura fulminans and coumarin-induced skin necrosis in patients with severe <b>congenital protein C deficiency</b> Short-term prophylaxis in patients with severe <b>congenital protein C deficiency</b> : if surgery or invasive therapy is imminent, while initiating coumarin therapy, when coumarin therapy alone is not sufficient, when coumarin therapy is not feasible	16/07/2001	Baxter AG
CEREZYME	Imiglucerase (INN)	Longterm enzyme replacement therapy in patients with a confirmed diagnosis of non-neuronopathic ( <b>Type 1</b> ) or chronic neuronopathic ( <b>Type 3</b> ) <b>Gaucher disease</b> 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.
CYSTAGON	Mercaptamine bitartrate (INN)	Treatment of proven nephropathic <b>cystinosis</b> Cysteamine reduces cystine accumulation in some cells (e.g. leukocytes, muscle and liver cells) of nephropathic cystinosis patients and, when treatment is started early, it delays the development of renal failure	23/06/1997	Orphan Europe S.A.R.L.
DUKORAL	Vibrio cholerae and recombinant cholera toxin B-subunit	Active immunisation against <b>disease caused by Vibrio cholerae</b> serogroup O1 in adults and children from 2 years of age who will be visiting endemic/epidemic areas	28/04/2004	Crucell Sweden AB
ENBREL	Etanercept (INN)	Treatment of active polyarticular <b>juvenile idiopathic arthritis</b> 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

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NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
ERBITUX	Cetuximab (INN)	Treatment of patients with <b>squamous cell cancer of the head and neck</b> , 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 <b>thalassaemia</b> 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 <b>hypogonadotrophic hypogonadism</b>	19/03/2009	Schering-Plough Europe
FILGRASTIM HEXAL	Filgrastim (INN)	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$ , and a history of severe or recurrent infections	06/02/2009	Hexal AG
FILGRASTIM RATIOPHARM	Filgrastim (INN)	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> 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
FLEBOGAMMA DIF	Human normal immunoglobulin	Replacement therapy in: <b>Primary immunodeficiency syndromes</b> such as: - congenital <b>agammaglobulinaemia</b> and <b>hypogammaglobulinaemia</b> - <b>common variable immunodeficiency</b> - <b>severe combined immunodeficiency</b> - <b>Wiskott Aldrich syndrome</b> <b>Myeloma</b> or <b>chronic lymphocytic leukaemia</b> with severe secondary hypogammaglobulinaemia and recurrent infections. Immunomodulation in: <b>Idiopathic thrombocytopenic purpura (ITP)</b> , in children or adults at high risk of bleeding or prior to surgery to correct the platelet count. <b>Guillain Barré syndrome</b> <b>Kawasaki disease</b>	23/07/2007	Instituto Grifols S.A.
GONAL-F	Recombinant human follicle stimulating hormone INN = Follitropin alpha	Stimulation of spermatogenesis in men who have <b>congenital</b> or acquired <b>hypogonadotrophic hypogonadism</b> 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 <b>haemophilia A</b> (congenital factor VIII deficiency)	04/08/2000	Bayer Schering Pharma AG

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NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
HERCEPTIN	Trastuzumab (INN)	In combination with capecitabine or 5-fluorouracil and cisplatin, for the treatment of patients with HER2 positive metastatic <b>adenocarcinoma of the stomach or gastro-esophageal junction</b> who have not received prior anti-cancer treatment for their metastatic disease. Herceptin should only be used in patients with metastatic gastric cancer whose tumours have HER2 overexpression as defined by IHC2+ and a confirmatory FISH+ result, or IHC 3+, as determined by an accurate and validated assay	28/08/2000	Roche Registration Limited
HUMIRA	Adalimumab (INN)	In combination with methotrexate for the treatment of active polyarticular <b>juvenile idiopathic arthritis</b> , 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
HYCANTIN	Topotecan (INN)	Treatment of patients with metastatic <b>carcinoma of the ovary</b> after failure of first-line or subsequent therapy Treatment of patients with relapsed <b>small cell lung cancer [SCLC]</b> for whom re-treatment with the first-line regimen is not considered appropriate In combination with cisplatin for patients with <b>carcinoma of the cervix</b> recurrent after radiotherapy and for patients with Stage IVB disease. Patients with prior exposure to cisplatin require a sustained treatment free interval to justify treatment with the combination HYCANTIN capsules are indicated as monotherapy for the treatment of adult patients with relapsed <b>small cell lung cancer (SCLC)</b> for whom re-treatment with the first-line regimen is not considered appropriate	12/11/1996	SmithKline Beecham Ltd
INOMAX	Nitric oxide (INN)	In conjunction with ventilatory support and other appropriate active substances: - for the treatment of newborn infants $\geq 34$ weeks gestation with hypoxic respiratory failure associated with clinical or echocardiographic evidence of <b>pulmonary hypertension</b> , in order to improve oxygenation and to reduce the need for extracorporeal membrane oxygenation. - as part of the treatment of peri- and post-operative pulmonary hypertension in adults and newborn infants, infants and toddlers, children and adolescents, ages 0-17 years in conjunction to heart surgery, in order to selectively decrease pulmonary arterial pressure and improve right ventricular function and oxygenation	01/08/2001	INO Therapeutics AB

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NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
INTRONA	Interferon alpha-2b (INN)	Treatment of patients with <b>hairy cell leukaemia</b> Monotherapy treatment of adults with Philadelphia chromosome or bcr/abl translocation positive <b>chronic myelogenous leukaemia</b> 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 <b>multiple myeloma</b> , 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 <b>follicular lymphoma</b> 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	Schering-Plough Europe
IXIARO	Japanese Encephalitis Vaccine (inactivated, adsorbed)	For active immunization against <b>Japanese encephalitis</b> for adults	31/03/2009	Intercell AG
KEPPRA	Levetiracetam (INN)	As monotherapy in the treatment of partial onset seizures with or without secondary generalisation in patients from 16 years of age with newly diagnosed <b>epilepsy</b> . As adjunctive therapy in the treatment of partial onset seizures with or without secondary generalisation in adults, children and infants from <b>1 month</b> of age with epilepsy ; in the treatment of myoclonic seizures in adults and adolescents from 12 years of age with <b>Juvenile Myoclonic Epilepsy</b> ; 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 (INN)	Replacement therapy in <b>primary immunodeficiency syndromes</b> such as: - congenital <b>agammaglobulinaemia</b> and <b>hypogammaglobulinaemia</b> - <b>common variable immunodeficiency</b> - <b>severe combined immunodeficiency</b> - <b>Wiskott Aldrich syndrome</b> <b>Myeloma</b> or <b>chronic lymphocytic leukaemia (CLL)</b> with severe secondary hypogammaglobulinemia and recurrent infections. Immunomodulation : - <b>Idiopathic thrombocytopenic purpura (ITP)</b> , in children or adults at high risk of bleeding or prior to surgery to correct the platelet count. - <b>Guillain Barré syndrome</b> - <b>Kawasaki disease</b>	19/01/2006	Baxter AG

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KOGENATE BAYER	Octocog alpha (INN)	Treatment and prophylaxis of bleeding in patients with <b>haemophilia A</b> (congenital factor VIII deficiency)	04/08/2000	Bayer Schering Pharma AG
MABCAMPATH	Alemtuzumab (INN)	Treatment of patients with <b>B-cell chronic lymphocytic leukaemia</b> (B-CLL) for whom fludarabine combination chemotherapy is not appropriate	06/07/2001	Genzyme Europe BV
MABTHERA	Rituximab (INN)	Indicated in adults for: Treatment of previously untreated patients with stage III-IV <b>follicular lymphoma</b> 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 <b>diffuse large B cell non-Hodgkin's lymphoma</b> in combination with CHOP (cyclophosphamide, doxorubicin, vincristine, prednisolone) chemotherapy In combination with chemotherapy, treatment of patients with previously untreated and relapsed/refractory <b>chronic lymphocytic leukaemia</b> (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 (INN)	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$ , and a history of severe or recurrent infections	08/06/2010	Hospira UK Ltd
NOVOSEVEN	Human recombinant coagulation Factor VIIa INN = Eptacog alpha (activated)	Treatment of bleeding episodes and for the prevention of bleeding in those undergoing surgery or invasive procedures in the following patient groups : in patients with congenital <b>haemophilia</b> with inhibitors to coagulation factors VIII or IX > 5 BU; in patients with congenital <b>haemophilia</b> who are expected to have a high anamnestic response to factor VIII or factor IX administration; in patients with <b>acquired haemophilia</b> ; in patients with <b>congenital FVII deficiency</b> ; in patients with <b>Glanzmann's thrombasthenia</b> with antibodies to GP IIb - IIIa and/or HLA, and with past or present refractoriness to platelet transfusions	23/02/1996	Novo Nordisk A/S

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NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
NOXAFIL	Posaconazole (INN)	<p>Treatment of the fungal infections in adults:</p> <ul style="list-style-type: none"> <li>- Invasive <b>aspergillosis</b> in patients with disease that is refractory to amphotericin B or itraconazole or in patients who are intolerant of these medicinal products</li> <li>- <b>Fusariosis</b> in patients with disease that is refractory to amphotericin B or in patients who are intolerant of amphotericin B</li> <li>- <b>Chromoblastomycosis</b> and <b>mycetoma</b> in patients with disease that is refractory to itraconazole or in patients who are intolerant of itraconazole</li> <li>- <b>Coccidioidomycosis</b> in patients with disease that is refractory to amphotericin B, itraconazole or fluconazole or in patients who are intolerant of these medicinal products</li> </ul> <p>Prophylaxis of invasive fungal infections in :</p> <ul style="list-style-type: none"> <li>- 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</li> <li>- 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</li> </ul>	25/10/2005	Schering-Plough Europe
OMNITROPE	Somatropin (INN)	<p>Growth disturbance due to insufficient secretion of growth hormone (GH) and growth disturbance associated with <b>Turner syndrome</b> or chronic renal insufficiency.</p> <p><b>Prader-Willi syndrome (PWS)</b>, for improvement of growth and body composition.</p> <p>Replacement therapy in adults with pronounced <b>growth hormone deficiency</b> (patients with known hypothalamic pituitary pathology and at least one known deficiency of a pituitary hormone not being prolactin)</p>	12/04/2006	Sandoz GmbH
ORENCIA	Abatacept (INN)	In combination with methotrexate, for the treatment of moderate to severe active <b>polyarticular juvenile idiopathic arthritis (JIA)</b> in paediatric patients 6 years of age and older who have had an insufficient response to other DMARDs including at least one TNF inhibitor	21/05/2007	Bristol-Myers Squibb Pharma EEIG
PANRETIN	Alitretinoïn (INN)	Topical treatment of cutaneous lesions in patients with AIDS-related <b>Kaposi's sarcoma (KS)</b> : when lesions are not ulcerated or lymphoedematous, and treatment of visceral KS is not required, and when lesions are not responding to systemic antiretroviral therapy, and radiotherapy or chemotherapy are not appropriate	11/10/2000	Eisai Ltd

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NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
PRIVIGEN	Human normal immunoglobulin (IVIg)	Replacement therapy in : - <b>Primary immunodeficiency (PID)</b> syndromes such as: - congenital <b>agammaglobulinaemia</b> and <b>hypogammaglobulinaemia</b> - <b>common variable immunodeficiency</b> - <b>severe combined immunodeficiency</b> - <b>Wiskott Aldrich syndrome</b> - <b>Myeloma</b> or <b>chronic lymphocytic leukaemia</b> with severe secondary hypogammaglobulinaemia and recurrent infections. Immunomodulation in : - <b>Immune thrombocytopenic purpura (ITP)</b> , in children or adults at high risk of bleeding or prior to surgery to correct the platelet count - <b>Guillain-Barré syndrome</b> - <b>Kawasaki disease</b>	25/04/2008	CSL Behring GmbH
PUREGON	Follitropin beta (INN)	Treatment of deficient spermatogenesis due to <b>hypogonadotrophic hypogonadism</b>	03/05/1996	NV Organon
RATIOGRASTIM	Filgrastim (INN)	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> 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 <b>haemophilia A</b> (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 <b>heparin-induced thrombocytopenia (HIT)</b> type II and thromboembolic disease mandating parenteral antithrombotic therapy	13/03/1997	Celgene Europe Ltd
RILUTEK	Riluzole (INN)	To extend life or the time to mechanical ventilation for patients with <b>amyotrophic lateral sclerosis (ALS)</b>	10/06/1996	Aventis Pharma S.A.
RUCONEST	Conestat alfa	Treatment of acute angioedema attacks in adults with <b>hereditary angioedema (HAE) due to C1 esterase inhibitor deficiency</b>	28/10/2010	Pharming Group N.V.
SAMSCA	Tolvaptan (INN)	Treatment of adult patients with hyponatraemia secondary to <b>syndrome of inappropriate antidiuretic hormone secretion (SIADH)</b>	03/08/2009	Otsuka Pharmaceutical Europe Ltd
SUTENT	Sunitinib (INN)	Treatment of unresectable and/or metastatic malignant <b>gastrointestinal stromal tumour (GIST)</b> after failure of imatinib mesilate treatment due to resistance or intolerance Treatment of advanced/metastatic <b>renal cell carcinoma (MRCC)</b> in adults Treatment of unresectable or metastatic, well-differentiated <b>pancreatic neuroendocrine tumours (pNET)</b> with disease progression in adults Experience with SUTENT as first-line treatment is limited	19/07/2006	Pfizer Limited

INN - International Nonproprietary Name = DCI - Dénomination Commune Internationale

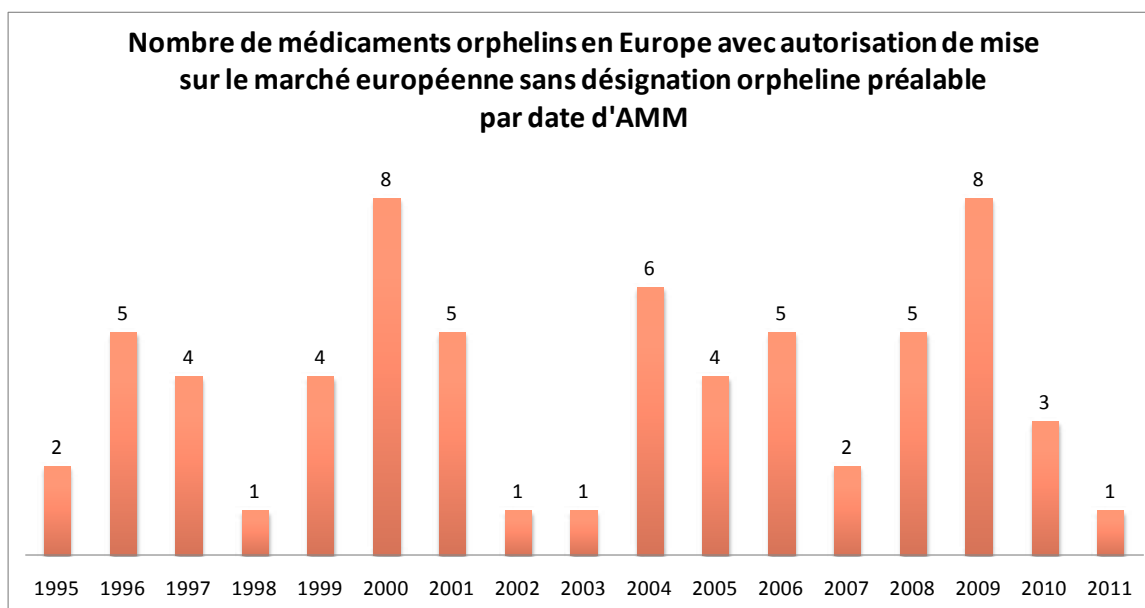
NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE L'AMM	DATE D'AMM (JJ/MM/AAAA)	TITULAIRE DE L'AMM
TARCEVA	Erlotinib (INN)	In combination with gemcitabine, for the treatment of patients with metastatic <b>pancreatic cancer</b> . When prescribing Tarceva, factors associated with prolonged survival should be taken into account . No survival advantage could be shown for patients with locally advanced disease	19/09/2005	Roche Registration Limited
TAXOTERE	Docetaxel (INN)	In combination with cisplatin and 5-fluorouracil for the treatment of patients with metastatic <b>gastric adenocarcinoma</b> , 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 <b>squamous cell carcinoma of the head and neck</b>	27/11/1995	Aventis Pharma S.A.
TEMODAL	Temozolomide (INN)	Treatment of adult patients with newly-diagnosed <b>glioblastoma</b> multiforme concomitantly with radiotherapy (RT) and subsequently as monotherapy treatment Treatment of children from the age of three years, adolescents and adult patients with <b>malignant glioma</b> , such as <b>glioblastoma</b> multiforme or anaplastic <b>astrocytoma</b> , showing recurrence or progression after standard therapy	26/01/1999	SP Europe
TEVAGRASTIM	Filgrastim (INN)	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$ , and a history of severe or recurrent infections	15/09/2008	Teva Generics GmbH
TEYSUNO	Tegafur/Gimeracil /Oteracil	In adults for the treatment of advanced <b>gastric cancer</b> when given in combination with cisplatin	14/03/2011	Taiho Pharma Europe Ltd.
THYROGEN	Thyrotropin alfa	For use with serum thyroglobulin (Tg) testing with or without radioiodine imaging for the detection of thyroid remnants and <b>well-differentiated thyroid cancer</b> in postthyroidectomy patients maintained on hormone suppression therapy (THST) For pre-therapeutic stimulation in combination with 100 mCi (3.7 GBq) radioiodine for ablation of thyroid tissue remnants in patients who have undergone a near-total or total thyroidectomy for <b>well-differentiated thyroid cancer</b> and who do not have evidence of distant metastatic thyroid cancer	09/03/2000	Genzyme Europe B.V.
VALTROPIN	Somatropin (INN)	Long-term treatment of children with growth failure due to an inadequate secretion of normal endogenous growth hormone Treatment of short stature in children with <b>Turner syndrome</b> , confirmed by chromosome analysis Replacement therapy in adults with pronounced <b>growth hormone deficiency</b> 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

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
VEDROP	Tocofersolan (INN)	Indicated in vitamin E deficiency due to digestive malabsorption in paediatric patients suffering from <b>congenital chronic cholestasis</b> or <b>hereditary chronic cholestasis</b> , from birth (in term newborns) to 16 or 18 years of age, depending on the region	24/07/2009	Orphan Europe S.A.R.L
VELCADE	Bortezomib (INN)	In combination with melphalan and prednisone for the treatment of patients with previously untreated <b>multiple myeloma</b> who are not eligible for high-dose chemotherapy with bone marrow transplant As mono-therapy for the treatment of progressive multiple myeloma in patients who have received at least 1 prior therapy and who have already undergone or are unsuitable for bone marrow transplantation	26/04/2004	Janssen-Cilag International NV
VFEND	Voriconazole	For treatment of invasive <b>aspergillosis</b> For treatment of serious fungal infections caused by <i>Scedosporium</i> spp. and <i>Fusarium</i> spp. ( <b>Fusariosis</b> ) VFEND should be administered primarily to patients with progressive, possibly life-threatening infections	19/03/2002	Pfizer Limited
VOTRIENT	Pazopanib (INN)	For the first line treatment of advanced <b>Renal Cell Carcinoma (RCC)</b> and for patients who have received prior cytokine therapy for advanced disease	14/06/2010	Glaxo Group Ltd
XELODA	Capecitabine (INN)	First-line treatment of advanced <b>gastric cancer</b> in combination with a platinum-based regimen	02/02/2001	Roche Registration Limited
XYREM	Sodium oxybate (INN)	Treatment of <b>narcolepsy with cataplexy</b> in adult patients	13/10/2005	UCB Pharma Ltd
ZARZIO	Filgrastim (INN)	In patients, children or adults, with <b>severe congenital, cyclic, or idiopathic neutropenia</b> with an absolute neutrophil count (ANC) of $0.5 \times 10^9/L$ , and a history of severe or recurrent infections	06/02/2009	Sandoz GmbH
ZEVALIN	Ibritumomab tiuxetan (INN)	Consolidation therapy after remission induction in previously untreated patients with <b>follicular lymphoma</b> Treatment of adult patients with rituximab relapsed or refractory CD20+ <b>follicular B-cell non-Hodgkin's lymphoma (NHL)</b>	16/01/2004	Bayer Schering Pharma AG
ZUTECTRA	Human Hepatitis B Immunoglobulin	Prevention of <b>hepatitis B virus (HBV) re-infection</b> in HBV-DNA negative patients over 6 months <b>after liver transplantation for hepatitis B induced liver failure</b> . 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

## 2- Par date décroissante d'AMM

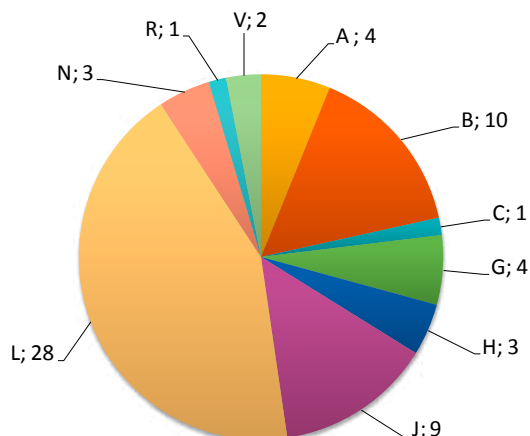
<b>2011</b>	<b>2007</b>	<b>2002</b>	<b>1998</b>
TEYSUNO	FLEBOGAMMA DIF	VFEND	MABTHERA
<b>2010</b>	ORENCIA	<b>2001</b>	<b>1997</b>
NIVESTIM	<b>2006</b>	CANCIDAS	BENEFIX
RUCONEST	ATRYN	CEPROTIN	CEREZYME
VOTRIENT	KIOVIG	INOMAX	CYSTAGON
<b>2009</b>	OMNITROPE	MABCAMPATH	REFLUDAN
ADCIRCA	SUTENT	XELODA	<b>1996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



### 3- Par catégorie ATC

<b>A- SYSTÈME DIGESTIF ET MÉTABOLISME</b>	<b>G- SYSTÈME GÉNITO-URINAIRE ET HORMONES SEXUELLES</b>	<b>L- ANTINÉOPLASIQUES ET AGENTS IMMUNOMODULANTS</b>	TEMODAL
AMMONAPS	ADCIRCA	ALIMTA	TEVAGRASTIM
CEREZYME	FERTAVID	AVASTIN	TEYSUNO
CYSTAGON	GONAL-F	BIOGRASTIM	VELCADE
VEDROP	PUREGON	CAELYX	VOTRIENT
<b>B- SANG ET ORGANES HÉMATOPOIÉTIQUES</b>	<b>H- PRÉPARATIONS SYSTÉMIQUES HORMONALES, À L'EXCLUSION DES HORMONES SEXUELLES ET DES INSULINES</b>	ENBREL	XELODA
ADVATE	OMNITROPE	ERBITUX	ZARZIO
ATRYN	THYROGEN	FILGRASTIM HEXAL	<b>N- SYSTÈME NERVEUX</b>
BENEFIX	VALTROPIN	FILGRASTIM RATIOPHARM	KEPPRA
CEPROTIN	<b>J- ANTI-INFECTIEUX GÉNÉRAUX À USAGE SYSTÉMIQUE</b>	HERCEPTIN	RILUTEK
HELIXATE NEXGEN	CANCIDAS	HUMIRA	XYREM
KOGENATE BAYER	DUKORAL	HYCAMTIN	<b>R- SYSTEME RESPIRATOIRE</b>
NOVOSEVEN	FLEBOGAMMA DIF	INTRONA	INOMAX
REFACTO AF	IXIARO	MABCAMPATH	<b>V- DIVERS</b>
REFLUDAN	KIOVIG	MABTHERA	FERRIPROX
RUCONEST	NOXAFIL	NIVESTIM	ZEVALIN
<b>C- SYSTÈME CARDIO-VASCULAIRE</b>	PRIVIGEN	ORENCIA	
SAMSCA	VFEND	PANRETIN	
	ZUTECTRA	RATIOGRASTIM	
		SUTENT	
		TARCEVA	
		TAXOTERE	

Nombre de médicaments orphelins en Europe avec autorisation de mise sur le marché européenne sans désignation orpheline préalable par catégorie ATC



## 4- Par titulaire d'AMM

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

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

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