

Liste des médicaments orphelins avec une autorisation de mise sur le marché européenne

*Les Cahiers
d'Orphanet*

Juillet 2008

Médicaments orphelins par ordre alphabétique de nom de spécialité

Médicaments orphelins par date décroissante d'autorisation de mise sur le marché

Médicaments orphelins par domaine thérapeutique

Médicaments orphelins par titulaires d'autorisation de mise sur le marché

Méthode

Ce document contient la liste de tous les médicaments orphelins qui ont reçu une autorisation de mise sur le marché (AMM) Européenne à la date indiquée dans le titre. Ces produits de santé peuvent n'être disponibles actuellement que dans certains pays Européens puisque des formalités administratives complémentaires peuvent être demandées par les autorités compétentes, par exemple, pour la définition du prix et du tarif de remboursement par l'Assurance Maladie.

Cette liste est établie par comparaison de la liste des produits de santé ayant obtenu une désignation orpheline avec la liste des produits ayant obtenu une AMM. 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 selon :

- 1- nom de spécialité
- 2- date d'AMM
- 3- domaine thérapeutique
- 4- titulaire d'autorisation de mise sur le marché

Les informations contenues dans le document sont le nom de la spécialité, le nom de la substance active, l'indication de la désignation orpheline et le promoteur de la désignation orpheline, le titulaire de l'AMM et la date d'AMM.

Vous pouvez trouver des informations complémentaires sur chaque médicament sur le site internet de l'EMA (Agence Européenne du médicament). Le registre de l'EMA liste tous les médicaments avec AMM, pas seulement les médicaments orphelins. Les informations sont proposées dans 21 langues de la communauté européenne.

Pour tous commentaires ou questions, s'adresser à : orphanet@orpha.net

Médicaments orphelins par ordre alphabétique de nom de spécialité

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
ALDURAZYME	Laronidase (INN)	Treatment of Mucopolysaccharidosis, type I	Genzyme Europe B.V.	10/06/2003
ATRIANCE	Nelarabine (INN)	Treatment of acute lymphoblastic leukaemia	GlaxoSmithKline Research & Development Limited	22/08/2007
BUSILVEX	Busulfan (INN) (Intravenous use)	Conditioning treatment prior to hematopoietic progenitor cell transplantation	Pierre Fabre Médicament	09/07/2003
CARBAGLU	N-carbamyl-L-glutamic acid INN=carglumic acid	Treatment of N-acetylglutamate synthetase (NAGS) deficiency	Orphan Europe	24/01/2003
CYSTADANE	Betaine anhydrous (INN)	Treatment of homocystinuria	Orphan Europe	15/02/2007
DIACOMIT	Stiripentol (INN)	Treatment of severe myoclonic epilepsy in infancy	Biocodex	04/01/2007
ELAPRASE	Idursulfase (INN)	Treatment of Mucopolysaccharidosis, type II (Hunter Syndrome)	TKT UK Ltd	08/01/2007
EVOLTRA	Clofarabine (INN)	Treatment of acute lymphoblastic leukaemia	Bioenvision Limited	29/05/2006
EXJADE	Deferasirox (INN)	Chronic iron overload requiring chelation therapy	Novartis Europharm Limited	28/08/2006
FABRAZYME	Alpha-Galactosidase A INN=agalsidase beta	Treatment of Fabry disease	Genzyme Europe B.V.	03/08/2001
GLIOLAN	5-aminolevulinic acid hydrochloride (INN)	Intra-operative photodynamic diagnosis of residual glioma	Medac Gesellschaft für klinische Spezialpräparate mbH	07/09/2007
GLIVEC	Imatinib mesilate (INN)	Treatment of chronic myeloid leukaemia Treatment of malignant gastrointestinal stromal tumours Treatment of acute lymphoblastic leukaemia Treatment of dermatofibrosarcoma protuberans Treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome Treatment of myelodysplastic / myeloproliferative diseases	Novartis Europharm Limited	07/11/2001
INCRELEX	Mecasermin (INN)	Treatment of primary insulin-like growth factor-1 deficiency due to molecular or genetic defects	Tercica Europe Limited	03/08/2007

INN - International Nonproprietary Name = DCI - Dénomination Commune Internationale

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
INOVELON	Rufinamide (INN)	Treatment of Lennox-Gastaut syndrome	Eisai Limited	16/01/2007
LITAK	Cladribine (INN) (subcutaneous use)	Treatment of indolent non-Hodgkin's lymphoma	Lipomed GmbH	14/04/2004
LYSODREN	Mitotane (INN)	Treatment of adrenal cortical carcinoma	Laboratoire HRA Pharma	28/04/2004
MYOZYME	Recombinant human acid alpha-glucosidase INN=alglucosidase alfa	Treatment of Glycogen Storage Disease type II (Pompe's disease)	Genzyme Europe B.V.	29/03/2006
NAGLAZYME	N-acetylgalactosamine-4-sulfatase INN=galsulfase	Treatment of Mucopolysaccharidosis, type VI (Maroteaux-Lamy Syndrome)	BioMarin Europe Ltd	24/01/2006
NEXAVAR	Sorafenib (INN)	Treatment of renal cell carcinoma Treatment of hepatocellular carcinoma	Bayer Healthcare AG	19/07/2006
ONSENAL	Celecoxib (INN)	Treatment of Familial Adenomatous Polyposis	Pharmacia-Pfizer EEIG	17/10/2006
ORFADIN	Nitisinone (INN)	Treatment of tyrosinaemia type I	Swedish Orphan International AB	21/02/2005
PEDEA	Ibuprofen (INN)	Treatment of a haemodynamically significant patent ductus arteriosus in preterm newborn infants less than 34 weeks of gestational age	Orphan Europe	29/07/2004
PHOTOBARR	Porfimer sodium (INN) (for use with photodynamic therapy)	Treatment of high-grade dysplasia in Barrett's Esophagus	Axcan Pharma International BV	25/03/2004
PRIALT	Ziconotide (INN) (intraspinal use)	Treatment of chronic pain requiring intraspinal analgesia	Elan Pharma International Ltd	21/02/2005
REPLAGAL	Alpha-Galactosidase A INN=agalsidase alfa	Treatment of Fabry disease	TKT UK Ltd.	03/08/2001
REVATIO	Sildenafil citrate INN=sildenafil	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	Pfizer Limited	28/10/2005
REVLIMID	Lenalidomide (INN)	Treatment of Multiple Myeloma	Celgene Europe Limited	14/06/2007
SAVENE	Dexrazoxane (INN)	Treatment of anthracycline extravasations	TopoTarget A/S	28/07/2006
SIKLOS	Hydroxycarbamide (INN)	Treatment of sickle cell syndrome	Add Medica SAS	29/06/2007
SOLIRIS	Eculizumab (INN)	Treatment of paroxysmal nocturnal haemoglobinuria	Alexion Europe SAS	20/06/2007
SOMAVERT	Pegvisomant (INN)	Treatment of acromegaly	Pfizer Limited	13/11/2002
SPRYCEL	Dasatinib	Treatment of chronic myeloid leukaemia Treatment of acute lymphoblastic leukaemia	Bristol-Myers Squibb Pharma EEIG	20/11/2006

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NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
SUTENT	Sunitinib malate INN=sunitinib	Treatment of malignant gastro-intestinal stromal tumours Treatment of renal cell carcinoma	Pfizer Limited	19/07/2006
TASIGNA	Nilotinib (INN)	Treatment of chronic myeloid leukaemia	Novartis Euro-pharm Limited	19/11/2007
THALIDOMIDE PHARMION	Thalidomide (INN)	Treatment of Multiple Myeloma	Pharmion France	16/04/2008
THELIN	Sitaxentan sodium (INN)	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	Encysive (UK) Limited	10/08/2006
TORISEL	Temsirolimus (INN)	Treatment of renal cell carcinoma	Wyeth Europa Limited	19/11/2007
TRACLEER	Bosentan monohydrate INN=bosentan	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension Treatment of Systemic Sclerosis	Actelion Registration Limited	15/05/2002
TRISENOX	Arsenic Trioxide (INN)	Treatment of acute promyelocytic leukaemia	Cell Therapeutics (UK) Limited	05/03/2002
VENTAVIS	Iloprost (INN)	Treatment of primary and of the following forms of secondary pulmonary hypertension: connective tissue disease pulmonary hypertension, drug-induced pulmonary hypertension, portopulmonary hypertension, pulmonary hypertension associated with congenital heart disease, chronic thromboembolic pulmonary hypertension	Schering AG	16/09/2003
VOLIBRIS	Ambrisentan (INN)	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	Glaxo Group Limited	21/04/2008
WILZIN	Zinc acetate dihydrate (INN)	Treatment of Wilson's disease	Orphan Europe	13/10/2004
XAGRID	Anagrelide Hydrochloride INN=anagrelide	Treatment of essential thrombocythaemia	Shire Pharmaceutical Contracts Limited	16/11/2004
XYREM	Sodium oxybate (INN)	Treatment of narcolepsy	UCB Pharma Ltd.	13/10/2004
YONDELIS	Trabectedin (INN)	Treatment of soft tissue sarcoma	Pharma Mar SA	17/09/2007

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Médicaments orphelins par date décroissante d'autorisation de mise sur le marché

DATE D'AMM (JJ/MM/AAAA)	NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	TITULAIRE DE L'AMM
21/04/2008	VOLIBRIS	Ambrisentan (INN)	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	Glaxo Group Limited
16/04/2008	THALIDOMIDE PHARMION	Thalidomide (INN)	Treatment of Multiple Myeloma	Pharmion Ltd.
19/11/2007	TASIGNA	Nilotinib (INN)	Treatment of chronic myeloid leukaemia	Novartis Europharm Limited
19/11/2007	TORISEL	Temsirolimus (INN)	Treatment of renal cell carcinoma	Wyeth Europa Limited
17/09/2007	YONDELIS	Trabectedin (INN)	Treatment of soft tissue sarcoma	Pharma Mar SA
07/09/2007	GLIOLAN	5-aminolevulinic acid hydrochloride (INN)	Intra-operative photodynamic diagnosis of residual glioma	Medac GmbH
22/08/2007	ATRIANCE	Nelarabine (INN)	Treatment of acute lymphoblastic leukaemia	Glaxo Group Limited
03/08/2007	INCRELEX	Mecasermin (INN)	Treatment of primary insulin-like growth factor-1 deficiency due to molecular or genetic defects	Tercica Europe Limited
29/06/2007	SIKLOS	Hydroxycarbamide (INN)	Treatment of sickle cell syndrome	Addmedica SAS
20/06/2007	SOLIRIS	Eculizumab (INN)	Treatment of paroxysmal nocturnal haemoglobinuria	Alexion Europe SAS
14/06/2007	REVLIMID	Lenalidomide (INN)	Treatment of Multiple Myeloma	Celgene Europe Limited
15/02/2007	CYSTADANE	Betaine anhydrous (INN)	Treatment of homocystinuria	Orphan Europe
16/01/2007	INOVELON	Rufinamide (INN)	Treatment of Lennox-Gastaut syndrome	Eisai Limited
08/01/2007	ELAPRASE	Idursulfase (INN)	Treatment of Mucopolysaccharidosis, type II (Hunter Syndrome)	Shire Human Genetic Therapies AB
04/01/2007	DIACOMIT	Stiripentol (INN)	Treatment of severe myoclonic epilepsy in infancy	Biocodex
20/11/2006	SPRYCEL	Dasatinib	Treatment of chronic myeloid leukaemia Treatment of acute lymphoblastic leukaemia	Bristol-Myers Squibb Pharma EEIG
28/08/2006	EXJADE	Deferasirox (INN)	Chronic iron overload requiring chelation therapy	Novartis Europharm Limited
10/08/2006	THELIN	Sitaxentan sodium (INN)	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	Encysive (UK) Limited

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DATE D'AMM (JJ/MM/AAAA)	NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	TITULAIRE DE L'AMM
28/07/2006	SAVENE	Dexrazoxane (INN)	Treatment of anthracycline extravasations	TopoTarget A/S
19/07/2006	NEXAVAR	Sorafenib (INN)	Treatment of renal cell carcinoma Treatment of hepatocellular carcinoma	Bayer Healthcare AG
19/07/2006	SUTENT	Sunitinib malate INN=sunitinib	Treatment of malignant gastrointestinal stromal tumours Treatment of renal cell carcinoma	Pfizer Limited
29/05/2006	EVOLTRA	Clofarabine (INN)	Treatment of acute lymphoblastic leukaemia	Bioenvision Limited
29/03/2006	MYOZYME	Recombinant human acid alpha-glucosidase INN=alglucosidase alfa	Treatment of Glycogen Storage Disease type II (Pompe's disease)	Genzyme Europe B.V.
24/01/2006	NAGLAZYME	N-acetylgalactosamine-4-sulfatase INN=galsulfase	Treatment of Mucopolysaccharidosis, type VI (Maroteaux-Lamy Syndrome)	BioMarin Europe Ltd
28/10/2005	REVATIO	Sildenafil citrate INN=sildenafil	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	Pfizer Limited
13/10/2004	XYREM	Sodium oxybate (INN)	Treatment of narcolepsy	UCB Pharma Ltd.
21/02/2005	ORFADIN	Nitisinone (INN)	Treatment of tyrosinaemia type I	Swedish Orphan International AB
21/02/2005	PRIALT	Ziconotide (INN) (intraspinal use)	Treatment of chronic pain requiring intraspinal analgesia	EISAI Limited
16/11/2004	XAGRID	Anagrelide Hydrochloride INN=anagrelide	Treatment of essential thrombocythaemia	Shire Pharmaceutical Contracts Limited
13/10/2004	WILZIN	Zinc acetate dihydrate (INN)	Treatment of Wilson's disease	Orphan Europe
29/07/2004	PEDEA	Ibuprofen (INN)	Treatment of a haemodynamically significant patent ductus arteriosus in preterm newborn infants less than 34 weeks of gestational age	Orphan Europe
28/04/2004	LYSODREN	Mitotane (INN)	Treatment of adrenal cortical carcinoma	Laboratoire HRA Pharma
14/04/2004	LITAK	Cladribine (INN) (subcutaneous use)	Treatment of indolent non-Hodgkin's lymphoma	Lipomed GmbH
25/03/2004	PHOTOBARR	Porfimer sodium (INN) (for use with photodynamic therapy)	Treatment of high-grade dysplasia in Barrett's Esophagus	Axcan Pharma International BV
17/10/2006	ONSENAL	Celecoxib (INN)	Treatment of Familial Adenomatous Polyposis	Pharmacia-Pfizer EEIG

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DATE D'AMM (JJ/MM/AAAA)	NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	TITULAIRE DE L'AMM
16/09/2003	VENTAVIS	Iloprost (INN)	Treatment of primary and of the following forms of secondary pulmonary hypertension: connective tissue disease pulmonary hypertension, drug-induced pulmonary hypertension, portopulmonary hypertension, pulmonary hypertension associated with congenital heart disease, chronic thromboembolic pulmonary hypertension	Bayer Schering Pharma AG
09/07/2003	BUSILVEX	Busulfan (INN) (Intravenous use)	Conditioning treatment prior to hematopoietic progenitor cell transplantation	Pierre Fabre Médicament
10/06/2003	ALDURAZYME	Laronidase (INN)	Treatment of Mucopolysaccharidosis, type I	Genzyme Europe B.V.
24/01/2003	CARBAGLU	N-carbamyl-L-glutamic acid INN=carglumic acid	Treatment of N-acetylglutamate synthetase (NAGS) deficiency	Orphan Europe
20/11/2002	ZAVESCA	Miglustat (INN)	Treatment of Gaucher Disease	Actelion Registration Limited
13/11/2002	SOMAVERT	Pegvisomant (INN)	Treatment of acromegaly	Pfizer Limited
15/05/2002	TRACLEER	Bosentan monohydrate INN=bosentan	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension Treatment of Systemic Sclerosis	Actelion Registration Limited
05/03/2002	TRISENOX	Arsenic Trioxide (INN)	Treatment of acute promyelocytic leukaemia	Cephalon Europe
07/11/2001	GLIVEC	Imatinib mesilate	Treatment of myelodysplastic / myeloproliferative diseases Treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome Treatment of dermatofibrosarcoma protuberans Treatment of acute lymphoblastic leukaemia Treatment of chronic myeloid leukaemia Treatment of malignant gastrointestinal stromal tumours	Novartis Europharm Limited
03/08/2001	FABRAZYME	Alpha-Galactosidase A INN=agalsidase beta	Treatment of Fabry disease	Genzyme Europe B.V.
03/08/2001	REPLAGAL	Alpha-Galactosidase A INN=agalsidase alfa	Treatment of Fabry disease	Shire Human Genetic Therapies AB

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Médicaments orphelins par domaine thérapeutique

Cardiovasculaire

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
PEDEA	Ibuprofen (INN)	Treatment of a haemodynamically significant patent ductus arteriosus in preterm newborn infants less than 34 weeks of gestational age	Orphan Europe	29/07/2004
REVATIO	Sildenafil citrate INN=sildenafil	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	Pfizer Limited	28/10/2005
THELIN	Sitaxentan sodium (INN)	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	Encysive (UK) Limited	10/08/2006
TRACLEER	Bosentan monohydrate INN=bosentan	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension Treatment of Systemic Sclerosis	Actelion Registration Limited	15/05/2002
VENTAVIS	Iloprost (INN)	Treatment of primary and of the following forms of secondary pulmonary hypertension: connective tissue disease pulmonary hypertension, drug-induced pulmonary hypertension, portopulmonary hypertension, pulmonary hypertension associated with congenital heart disease, chronic thromboembolic pulmonary hypertension	Schering AG	16/09/2003
VOLIBRIS	Ambrisentan (INN)	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	Glaxo Group Limited	21/04/2008

Endocrinologie

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
INCRELEX	Mecasermin (INN)	Treatment of primary insulin-like growth factor-1 deficiency due to molecular or genetic defects	Tercica Europe Limited	03/08/2007
SOMAVERT	Pegvisomant (INN)	Treatment of acromegaly	Pfizer Limited	13/11/2002

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Gastro-entérologie

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
ONSENAL	Celecoxib (INN)	Treatment of Familial Adenomatous Polyposis	Pharmacia-Pfizer EEIG	17/10/2006
PHOTOBARR	Porfimer sodium (INN) (for use with photodynamic therapy)	Treatment of high-grade dysplasia in Barrett's Esophagus	Axcan Pharma International BV	25/03/2004

Hématologie

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
SIKLOS	Hydroxycarbamide (INN)	Treatment of sickle cell syndrome	Add Medica SAS	29/06/2007
SOLIRIS	Eculizumab (INN)	Treatment of paroxysmal nocturnal haemoglobinuria	Alexion Europe SAS	20/06/2007
XAGRID	Anagrelide Hydrochloride INN=anagrelide	Treatment of essential thrombocythaemia	Shire Pharmaceutical Development Ltd	16/11/2004

Immunologie

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
BUSILVEX	Busulfan (INN) (Intravenous use)	Conditioning treatment prior to hematopoietic progenitor cell transplantation	Pierre Fabre Médicament	09/07/2003

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Métabolisme

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
ALDURAZYME	Laronidase (INN)	Treatment of Mucopolysaccharidosis, type I	Genzyme Europe B.V.	10/06/2003
CARBAGLU	N-carbamyl-L-glutamic acid INN=carglumic acid	Treatment of N-acetylglutamate synthetase (NAGS) deficiency	Orphan Europe	24/01/2003
CYSTADANE	Betaine anhydrous (INN)	Treatment of homocystinuria	Orphan Europe	15/02/2007
ELAPRASE	Idursulfase (INN)	Treatment of Mucopolysaccharidosis, type II (Hunter Syndrome)	TKT UK Ltd	08/01/2007
FABRAZYME	Alpha-Galactosidase A INN=agalsidase beta	Treatment of Fabry disease	Genzyme Europe B.V.	03/08/2001
MYOZYME	Recombinant human acid alpha-glucosidase INN=alglucosidase alfa	Treatment of Glycogen Storage Disease type II (Pompe's disease)	Genzyme Europe B.V.	29/03/2006
NAGLAZYME	N-acetylgalactosamine-4-sulfatase INN=galsulfase	Treatment of Mucopolysaccharidosis, type VI (Maroteaux-Lamy Syndrome)	BioMarin Europe Ltd	24/01/2006
ORFADIN	Nitisinone (INN)	Treatment of tyrosinaemia type I	Swedish Orphan International AB	21/02/2005
REPLAGAL	Alpha-Galactosidase A INN=agalsidase alfa	Treatment of Fabry disease	TKT UK Ltd.	03/08/2001
WILZIN	Zinc acetate dihydrate (INN)	Treatment of Wilson's disease	Orphan Europe	13/10/2004
ZAVESCA	Miglustat (INN)	Treatment of Gaucher Disease	Actelion Registration Limited	20/11/2002

Neurologie

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
DIACOMIT	Stiripentol (INN)	Treatment of severe myoclonic epilepsy in infancy	Biocodex	04/01/2007
INOVELON	Rufinamide (INN)	Treatment of Lennox-Gastaut syndrome	EISAI Limited	16/01/2007
PRIALT	Ziconotide (INN) (intraspinal use)	Treatment of chronic pain requiring intraspinal analgesia	Elan Pharma International Ltd.	21/02/2005
XYREM	Sodium oxybate (INN)	Treatment of narcolepsy	UCB Pharma Ltd.	13/10/2004

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NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
ATRIANCE	Nelarabine (INN)	Treatment of acute lymphoblastic leukaemia	GlaxoSmithKline Research & Development Limited	22/08/2007
EVOLTRA	Clofarabine (INN)	Treatment of acute lymphoblastic leukaemia	Bioenvision Limited	29/05/2006
GLIOLAN	5-aminolevulinic acid hydrochloride (INN)	Intra-operative photodynamic diagnosis of residual glioma	Medac Gesellschaft für klinische Spezialpräparate mbH	07/09/2007
GLIVEC	Imatinib mesilate (INN)	Treatment of chronic myeloid leukaemia Treatment of myelodysplastic / myeloproliferative diseases Treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome Treatment of dermatofibrosarcoma protuberans Treatment of acute lymphoblastic leukaemia Treatment of malignant gastrointestinal stromal tumours	Novartis Europharm Limited	07/11/2001
LITAK	Cladribine (INN) (subcutaneous use)	Treatment of indolent non-Hodgkin's lymphoma	Lipomed GmbH	14/04/2004
LYSODREN	Mitotane (INN)	Treatment of adrenal cortical carcinoma	Laboratoire HRA Pharma	28/04/2004
NEXAVAR	Sorafenib (INN)	Treatment of renal cell carcinoma Treatment of hepatocellular carcinoma	Bayer Healthcare AG	19/07/2006
REVLIMID	Lenalidomide (INN)	Treatment of Multiple Myeloma	Celgene Europe Limited	14/06/2007
SPRYCEL	Dasatinib	Treatment of chronic myeloid leukaemia Treatment of acute lymphoblastic leukaemia	Bristol-Myers Squibb Pharma EEIG	20/11/2006
SUTENT	Sunitinib malate INN=sunitinib	Treatment of malignant gastrointestinal stromal tumours Treatment of renal cell carcinoma	Pfizer Limited	19/07/2006
TASIGNA	Nilotinib (INN)	Treatment of chronic myeloid leukaemia	Novartis Europharm Limited	19/11/2007
THALIDOMIDE PHARMION	Thalidomide (INN)	Treatment of Multiple Myeloma	Pharmion France	16/04/2008
TORISEL	Temsirolimus (INN)	Treatment of renal cell carcinoma	Wyeth Europa Limited	19/11/2007
TRISENOX	Arsenic Trioxide (INN)	Treatment of acute promyelocytic leukaemia	Cell Therapeutics (UK) Limited	05/03/2002
YONDELIS	Trabectedin (INN)	Treatment of soft tissue sarcoma	Pharma Mar SA	17/09/2007

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Toxicologie

NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	PROMOTEUR DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
EXJADE	Deferasirox (INN)	Chronic iron overload requiring chelation therapy	Novartis Euro-pharm Limited	28/08/2006
SAVENE	Dexrazoxane (INN)	Treatment of anthracycline extravasations	TopoTarget A/S	28/07/2006

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Médicaments orphelins par titulaires d'autorisation de mise sur le marché

TITULAIRE DE L'AMM	NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
Actelion Registration Limited	TRACLEER	Bosentan monohydrate INN=bosentan	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension Treatment of Systemic Sclerosis	15/05/2002
Actelion Registration Limited	ZAVESCA	Miglustat (INN)	Treatment of Gaucher Disease	20/11/2002
Addmedica SAS	SIKLOS	Hydroxycarbamide (INN)	Treatment of sickle cell syndrome	29/06/2007
Alexion Europe SAS	SOLIRIS	Eculizumab (INN)	Treatment of paroxysmal nocturnal haemoglobinuria	20/06/2007
Axcan Pharma International BV	PHOTOBARR	Porfimer sodium (INN) (for use with photodynamic therapy)	Treatment of high-grade dysplasia in Barrett's Esophagus	25/03/2004
Bayer Healthcare AG	NEXAVAR	Sorafenib (INN)	Treatment of renal cell carcinoma Treatment of hepatocellular carcinoma	19/07/2006
Bayer Schering Pharma AG	VENTAVIS	Iloprost (INN)	Treatment of primary and of the following forms of secondary pulmonary hypertension: connective tissue disease pulmonary hypertension, drug-induced pulmonary hypertension, portopulmonary hypertension, pulmonary hypertension associated with congenital heart disease, chronic thromboembolic pulmonary hypertension	16/09/2003
Biocodex	DIACOMIT	Stiripentol (INN)	Treatment of severe myoclonic epilepsy in infancy	04/01/2007
Bioenvision Limited	EVOLTRA	Clofarabine (INN)	Treatment of acute lymphoblastic leukaemia	29/05/2006
BioMarin Europe Ltd	NAGLAZYME	N-acetylgalactosamine-4-sulfatase INN=galsulfase	Treatment of Mucopolysaccharidosis, type VI (Maroteaux-Lamy Syndrome)	24/01/2006
Bristol-Myers Squibb Pharma EEIG	SPRYCEL	Dasatinib	Treatment of chronic myeloid leukaemia Treatment of acute lymphoblastic leukaemia	20/11/2006
Celgene Europe Limited	REVLIMID	Lenalidomide (INN)	Treatment of Multiple Myeloma	14/06/2007
Cephalon Europe	TRISENOX	Arsenic Trioxide (INN)	Treatment of acute promyelocytic leukaemia	05/03/2002

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TITULAIRE DE L'AMM	NOM DE LA SPÉCIALITÉ	SUBSTANCE ACTIVE	INDICATION DE LA DÉSIGNATION ORPHELINE	DATE D'AMM (JJ/MM/AAAA)
Eisai Limited	INOVELON	Rufinamide (INN)	Treatment of Lennox-Gastaut syndrome	16/01/2007
Eisai Limited	PRIALT	Ziconotide (INN) (in-traspinal use)	Treatment of chronic pain requiring intraspinal analgesia	21/02/2005
Encysive (UK) Limited	THELIN	Sitaxentan sodium (INN)	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	10/08/2006
Genzyme Europe B.V.	ALDURAZYME	Laronidase (INN)	Treatment of Mucopolysaccharidosis, type I	10/06/2003
Genzyme Europe B.V.	FABRAZYME	Alpha-Galactosidase A INN=agalsidase beta	Treatment of Fabry disease	03/08/2001
Genzyme Europe B.V.	MYOZYME	Recombinant human acid alpha-glucosidase INN=alglucosidase alfa	Treatment of Glycogen Storage Disease type II (Pompe's disease)	29/03/2006
Glaxo Group Limited	ATRIANCE	Nelarabine (INN)	Treatment of acute lymphoblastic leukaemia	22/08/2007
Glaxo Group Limited	VOLIBRIS	Ambrisentan (INN)	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	21/04/2008
Laboratoire HRA Pharma	LYSODREN	Mitotane (INN)	Treatment of adrenal cortical carcinoma	28/04/2004
Lipomed GmbH	LITAK	Cladribine (INN) (subcutaneous use)	Treatment of indolent non-Hodgkin's lymphoma	14/04/2004
Medac GmbH	GLIOLAN	5-aminolevulinic acid hydrochloride (INN)	Intra-operative photodynamic diagnosis of residual glioma	07/09/2007
Novartis Europharm Limited	EXJADE	Deferasirox (INN)	Chronic iron overload requiring chelation therapy	28/08/2006
Novartis Europharm Limited	GLIVEC	Imatinib mesilate (INN)	Treatment of malignant gastrointestinal stromal tumours Treatment of myelodysplastic / myeloproliferative diseases Treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome Treatment of dermatofibrosarcoma protuberans Treatment of acute lymphoblastic leukaemia Treatment of chronic myeloid leukaemia	07/11/2001
Novartis Europharm Limited	TASIGNA	Nilotinib (INN)	Treatment of chronic myeloid leukaemia	19/11/2007
Orphan Europe	CARBAGLU	N-carbamyl-L-glutamic acid INN=carglumic acid	Treatment of N-acetylglutamate synthetase (NAGS) deficiency	24/01/2003
Orphan Europe	CYSTADANE	Betaine anhydrous (INN)	Treatment of homocystinuria	15/02/2007

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Orphan Europe	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	WILZIN	Zinc acetate dihydrate (INN)	Treatment of Wilson's disease	13/10/2004
Pfizer Limited	REVATIO	Sildenafil citrate INN=sildenafil	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	28/10/2005
Pfizer Limited	SOMAVERT	Pegvisomant (INN)	Treatment of acromegaly	13/11/2002
Pfizer Limited	SUTENT	Sunitinib malate INN=sunitinib	Treatment of malignant gastrointestinal stromal tumours	19/07/2006
Pharmacia-Pfizer EEIG	ONSENAL	Celecoxib (INN)	Treatment of Familial Adenomatous Polyposis	17/10/2006
Pharma Mar SA	YONDELIS	Trabectedin (INN)	Treatment of soft tissue sarcoma	17/09/2007
Pharmion Ltd.	THALIDOMIDE PHARMION	Thalidomide (INN)	Treatment of Multiple Myeloma	16/04/2008
Pierre Fabre Médicament	BUSILVEX	Busulfan (INN) (Intravenous use)	Conditioning treatment prior to hematopoietic progenitor cell transplantation	09/07/2003
Shire Human Genetic Therapies AB	ELAPRASE	Idursulfase (INN)	Treatment of Mucopolysaccharidosis, type II (Hunter Syndrome)	08/01/2007
Shire Human Genetic Therapies AB	REPLAGAL	Alpha-Galactosidase A INN=agalsidase alfa	Treatment of Fabry disease	03/08/2001
Shire Pharmaceutical Contracts Limited	XAGRID	Anagrelide Hydrochloride INN=anagrelide	Treatment of essential thrombocythaemia	16/11/2004
Swedish Orphan International AB	ORFADIN	Nitisinone (INN)	Treatment of tyrosinaemia type I	21/02/2005
Tercica Europe Limited	INCRELEX	Mecasermin (INN)	Treatment of primary insulin-like growth factor-1 deficiency due to molecular or genetic defects	03/08/2007
TopoTarget A/S	SAVENE	Dexrazoxane (INN)	Treatment of anthracycline extravasations	28/07/2006
UCB Pharma Ltd.	XYREM	Sodium oxybate (INN)	Treatment of narcolepsy	13/10/2004
Wyeth Europa Limited	TORISEL	Temsirolimus (INN)	Treatment of renal cell carcinoma	19/11/2007

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