

Anexo 1: Comparación de listas de MH

APROVED ORPHAN DRUG			STATUS ORPHAN DRUG				
ATC	GENERIC NAME	INDICATION	REGION	DESIGNATED DATE	APPROVED DATE	TRADE NAME	SPONSOR
A16AB09	Idursulfase	is indicated for the long term treatment of patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II)	USA	28/11/2001	24/07/2006	ELAPRASE	Shire Human Genetic Therapies, Inc.
			EU	11/12/2001	08/01/2007	ELAPRASE	TKT Europe AB
A07AA11	Rifaximin	Reduction in the risk of overt hepatic encephalopathy (HE) recurrence in patients greater than or equal to 18 years of age	USA	02/10/1998	03-24-2010	Normix	Salix Pharmaceuticals, Inc.
A16AB	Galsulfase	Treatment of Mucopolysaccharidosis, type VI (Maroteaux-Lamy Syndrome)	USA	17/02/1999	31/05/2005	NAGLAZYME	BioMarin Pharmaceutical, Inc
			EU	14/02/2001	24/01/2006	NAGLAZYME	BioMarin Europe Ltd
A16AB05	Iaronidase	Treatment for patients with Hurler and Hurler-Scheie forms of Mucopolysaccharidosis I (MPS I) and for patients with the Scheie form	USA	24/09/1997	30/04/2003	Aldurazyme	BioMarin Pharmaceutical, Inc.
			EU	14/02/2001	10/06/2003	Aldurazyme	Genzyme Europe B.V.
A16AX06	Miglustat	for the treatment of mild to moderate type 1 Gaucher disease	USA	29/05/1998	31/07/2003	Zavesca	Actelion Pharmaceuticals Ltd
			EU	18/10/2000	20/11/2002	Zavesca	Oxford GlycoSciences (UK) Ltd
A16AX04	Nitisinone	Treatment of tyrosinaemia type I	EU	29/12/2000	21/02/2005	Orfadin	Swedish Orphan International AB
A16AX07	Sapropterin	Indicated to reduce blood phenylalanine (Phe) levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive Phenylketonuria (PKU). Kuvan is to be used in conjunction	USA	29/01/2004	13/12/2007	Kuvan	Biomarin Pharmaceutical Inc.
			EU	08/06/2004	02/12/2008	Kuvan	Merck KGaA Frankfurter Strasse
A16AA03	Glutamine	Treatment of short bowel syndrome in patients receiving specialized nutritional support when used in conjunction with a recombinant human growth hormone that is approved for this indication	USA	06/03/1995	10/06/2004	NutreStore	Nutritional Restart Pharmaceutical, L.P.
A16AB03	Agalsidase alfa	Treatment of Fabry disease	EU	08/08/2000	04/05/2001	Replagal	TKT Europe AB
A16AB04	Agalsidase beta	Treatment of Fabry disease	EU	08/08/2000	04/05/2001	Fabrazyme	Genzyme Europe B.V.
A16AA05	Carglumic acid	Treatment of N-acetylglutamate synthetase (NAGS) deficiency	EU	18/10/2000	24/01/2003	Carbaglu	Orphan Europe S.a.r.l.
A16AA06	Betaine	Treatment of homocystinuria	EU	09/07/2001	15/02/2007	Cystadane	Orphan Europe S.a.r.l.
A16AX05	Zinc acetate	Treatment of Wilson's disease	EU	31/07/2001	13/10/2004	Wilzin	Orphan Europe S.a.r.l.
A16AB07	Alglucosidase alfa	Treatment of Glycogen Storage Disease type II (Pompe's disease)	EU	14/02/2001	29/03/2006	MYOZYME	Genzyme Europe B.V.
A16AX06	Miglustat	Treatment of Niemann-Pick Disease, type C	EU	16/02/2006	26/01/2009	Zavesca	Actelion Registration Ltd
A07EC04	Balsalazide disodium	Treatment of mildly to moderately active ulcerative colitis in patients 5 years of age and older. Safety and Effectiveness of Colazal beyond 8 weeks in children (ages 5-17) and 12 weeks in adults have not been established.	USA	12/08/2005	20/12/2006	Colazal	Salix Pharmaceuticals, Inc.
B01AD12	Protein C concentrate	Prevention and treatment of venous thrombosis and purpura fulminans	USA	23/06/1992	30/03/2007	Ceprocin	Baxter Healthcare Corporation
B02BD05	Coagulation factor VIIa (recombinant)	Prevention of bleeding in surgical interventions or invasive procedures in patients with acquired hemophilia	USA	21/07/2004	13/10/2006	NovoSeven	Novo Nordisk, Inc.
B02BD05	Coagulation factor VIIa (recombinant)	Prevention of bleeding in surgical interventions or invasive procedures in patients with congenital F VII deficiency	USA	10/09/2004	11/07/2005	NovoSeven	Novo Nordisk, Inc.

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B02BD05	Coagulation factor VIIa (recombinant)	Treatment of bleeding episodes in surgical interventions or invasive procedures in patients with acquired hemophilia	USA	16/07/2004	13/10/2006	NovoSeven	Novo Nordisk, Inc.
B02BD05	Coagulation factor VIIa (recombinant)	Prevention of bleeding in surgical interventions or invasive procedures in hemophilia A or B patients with inhibitors to Factor VIII or Factor IX	USA	18/06/2004	12/08/2005	NovoSeven	Novo Nordisk, Inc.
B02BD05	Coagulation factor VIIa (recombinant)	Treatment of bleeding episodes in patients with Factor VII Deficiency	USA	10/09/2004	11/07/2005	NovoSeven	Novo Nordisk, Inc.
B02BD02	Antihemophilic factor (human)	For surgical and/or invasive procedures in patients with von Willebrand Disease (VWD) in whom desmopressin is either ineffective or contraindicated. It is not indicated for patients with severe VWD (Type 3) undergoing major surgery	USA	05/01/1996	31/01/2007	Alphanate	Grifols Biologicals Inc.
B02BD06	Human Plasma Coagulation Factor VIII And Human Plasma Von Willebrand Factor	Treatment of spontaneous or trauma-induced bleeding episodes in patients with severe von Willebrand disease (VWD) as well as patients with mild or moderate VWD in whom the use of desmopressin is known or suspected to be ineffective or contraindicated	USA	04/18/2007	12/04/2009	Wilate(R)	Octapharma USA, Inc.
B03BA03	Hydroxocobalamin	Treatment on known or suspected cyanide poisoning	USA	25/11/2003	15/12/2006	Cyanokit	Merck Sante, s.a.a.
B01AC11	Iloprost	Treatment of pulmonary arterial hypertension (WHO Group I) in patients with NYHA Class III or IV symptoms	USA	17/08/2004	29/12/2004	Ventavis	CoTherix, Inc.
			EU	29/12/2000	16/09/2003	Ventavis	Schering AG
B01AC21	Treprostinil (Inhalational)	Treatment of pulmonary arterial hypertension (WHO Group I) in patients with NYHA Class III symptoms, to increase walk distance	USA	06/17/2010	07-30-2009	Tyvaso	LungRx, Inc.
B02BX05	Eltrombopagolamine	Treatment of idiopathic thrombocytopenic purpura	EU	03/08/2007	11/03/2010	Revolade	GlaxoSmithKline Trading Services Limited
B02BX04	Romiplostim	Treatment of idiopathic thrombocytopenic purpura	EU	27/05/2005	04/02/2009	Nplate	Amgen Europe B.V.
B01A	Recombinant Human Antithrombin	Prevention of peri-operative and peri-partum thromboembolic events, in hereditary antithrombin deficient patients.	USA	12/07/2007	02/06/2009	Atryn	GTC Biotherapeutics, Inc.
C02KX02	Ambrisentan	Treatment of pulmonary arterial hypertension (WHO group I) in patients with WHO class II or III symptoms to improve exercise capacity and delay clinical worsening	USA	16/07/2004	15/06/2007	Letairis	Gilead Colorado
			EU	11/04/2005	21/04/2008	Volibris	Glaxo Group Limited
C02KX01	Bosentan	Treatment of pulmonary arterial hypertension.	EU	14/02/2001	15/05/2002	Tracleer	Actelion Registration Limited
C01EB16	Ibuprofen	Treatment of patent ductus arteriosus	USA	29/10/1996	13/04/2006	NeoProfen	Farmacon-IL, LLC
			EU	14/02/2001	29/07/2004	Pedea	Orphan Europe S.a.r.l.
C07AA07	Sotalol (Iv)	For use as a substitute for oral sotalol in patients who are unable to take sotalol orally [oral sotalol is indicated for maintenance of normal sinus rhythm in patients with history of highly symptomatic atrial fibrillation /flutter, and for treatment of documented life-threatening ventricular arrhythmias]	USA	07/25/2008	07/02/2009	So-Aqueous	Academic Pharmaceuticals

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C07AA07	Tadalafil	Treatment of pulmonary arterial hypertension (WHO Group I) to improve exercise ability	USA	12/18/2006	05-22-2009	Adcirca	Eli Lilly and Company
C02KX03	Sitaxentan sodium	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	EU	21/10/2004	10/08/2006	Thelin	Encysive (UK) Limited
G03G	Recombinant human luteinizing hormone	Luveris (lutropin alfa for injection), concomitantly administered with Gonal-f (follitropin alfa for injection), is indicated for stimulation of follicular development in infertile hypogonadotropic hypogonadal women with profound LH deficiency (LH (less than) 1.2 IU/L).	USA	07/10/1994	08/10/2004	Luveris	EMD Serono, Inc.
G03XC01	Raloxifene	Reduction in risk of invasive breast cancer in postmenopausal women with osteoporosis and reduction in risk of invasive breast cancer in postmenopausal women at high risk for invasive breast cancer	USA	14/07/2005	13/09/2007	Evista	Eli Lilly and Company
G04BE03	Sildenafil citrate	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	EU	12/12/2003	28/10/2005	Revatio	Pfizer Ltd
H01AC01	Somatropin	Treatment of short stature in patients with Noonan syndrome	USA	09/08/2006	31/05/2007	Norditropin	Novo Nordisk Inc.
H01AC01	Somatropin	Treatment of short bowel syndrome in patients receiving specialized nutritional support	USA	06/03/1995	01/12/2003	Zorbtive	Serono Laboratories, Inc.
H01AC05	Mecasermin rinfabate	Treatment of growth failure in children with severe primary IGF-1 deficiency (Primary IGFD) or with growth hormone (GH) gene deletion who have developed neutralizing antibodies to growth hormone	USA	17/05/2002	12/12/2005	iPLEX	Insmed, Inc.
H01CA03	Histrelin	Treatment of central precocious puberty	USA	18/11/2005	03/05/2007	Supprelin LA	Indevus Pharmaceuticals (Formerly Valera Pharma.)
H01CB03	Lanreotide	Long-term treatment of acromegalic patients who have had an inadequate response to or cannot be treated with surgery and/or radiotherapy	USA	11/09/2000	30/08/2007	Somatuline Depot	IPSEN, Inc.
H05BX01	Cinacalcet	Treatment of hypercalcemia in patients with parathyroid carcinoma	USA	12/05/2003	08/03/2004	Sensipar	Amgen, Inc
H01AC03	Mecasermin	Long-term treatment of growth failure in children with severe primary IGF-1 deficiency (Primary IGFD) or with growth hormone (GH) gene deletion who have developed neutralizing antibodies to growth hormone.	EU	12/12/1995	30/08/2005	Increlex	Tercica, Inc.
			USA	26/08/2005	03/08/2007	Increlex	Ipsen Pharma
H01AX01	Pegvisomant	Treatment of acromegaly	EU	14/02/2001	13/11/2002	Somavert	
J05AB06	Ganciclovir	Treatment of acute herpetic keratitis (dendritic ulcers)	USA	03/22/2007	09-15-2009	Zirgan	Sirion Therapeutics, Inc.
J05AB04	Ribavirin	Treatment of chronic hepatitis C among previously untreated pediatric patients at least three years of age or older.	USA	04/04/2003	29/07/2003	REBETOL	Schering Corporation
J06BC	Botulism immune globulin	Indicated for treatment of infant botulism caused by type A or type B Clostridium botulinum.	USA	31/01/1989	23/10/2003	BabyBIG	California Department of Health Services
J06BB04	Hepatitis B immune globulin (human)	Prevention of hepatitis B recurrence following liver transplantation in HBsAG-positive liver transplant patients	USA	06/04/2007	24/03/2008	HepaGam	Cangene Corporation

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J07	Vaccinia Immune Globulin (Human) Intravenous	Treatment and modification of aberrant infections induced by vaccinia virus that include its accidental implantation in eyes (except in cases of isolated keratitis), mouth, or other areas where vaccinia infection would constitute a special hazard; eczema vaccinatum; progressive vaccinia; severe generalized vaccinia, and vaccinia infections in individuals who have skin conditions such as burns, impetigo, varicella-zoster, or poison ivy; or in individuals who have eczematous skin lesions because of either the activity	USA	18/06/2004	18/02/2005	no aclara	DynPort Vaccine Company LLC
J06BC	Botulism immune globulin	Indicated for treatment of infant botulism caused by type A or type B Clostridium botulinum.	USA	31/01/1989	23/10/2003	BabyBIG	California Department of Health Services
J01DF01	Aztreonam lysinate (inhalation use)	To improve respiratory symptoms in cystic fibrosis (CF) patients with Pseudomonas aeruginosa (gram negative)	USA	03/12/2002	02-22-2010	Cayston	Gilead Sciences (formerly Corus Pharma)
			EU	21/06/2004	21/09/2009	Cayston	Gilead Sciences International Limited
L01BB06	Clofarabine	Treatment of pediatric patients 1 to 21 years old with relapsed or refractory acute lymphoblastic leukemia after at least two prior regimens	USA	07/02/2002	28/12/2004	Clolar	Genzyme Corporation
			EU	05/02/2002	29/05/2006	Evoltra	Genzyme Europe BV
L01XE06	Dasatinib	Treatment of chronic myeloid leukaemia	USA	28/11/2005	28/06/2006	Sprycel	Bristol-Myers Squibb Company
			EU	23/12/2005	20/11/2006	Sprycel	Bristol-Myers Squibb Pharma EEIG
L04AA25	Eculizumab	Treatment of paroxysmal nocturnal haemoglobinuria	USA	20/08/2003	16/03/2007	Soliris	Alexion Pharmaceuticals, Inc.
			EU	17/10/2003	20/06/2007	Soliris	Alexion Europe SAS
L01XE01	Imatinib mesylate	Treatment of patients with Kit (CD117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumors (GIST)	EU	20/11/2001	24/05/2002	Glivec	Novartis Europharm Limited
L01XE01	Imatinib mesylate	Treatment of adult patients with aggressive mastocytosis (ASM) without the D816V c-Kit mutation or with c-Kit mutational status unknown	USA	09/09/2005	10/19/2006	Gleevec	Novartis Pharmaceuticals Corporation
L01XE01	Imatinib mesylate	Treatment of adult patients with myelodysplastic/myeloproliferative diseases (MDS/MPD) associated with PDGFR (platelet-derived growth factor receptor) gene re-arrangements	USA	05/10/2005	19/10/2006	Gleevec	Novartis Pharmaceuticals Corporation
			EU	23/12/2005	28/11/2006	Glivec	Novartis Europharm Limited
L01XE01	Imatinib mesylate	Treatment of adult patients with hypereosinophilic syndrome (HES) and/or chronic eosinophilic leukemia (CEL) who have the FIP1L1-PDGFR fusion kinase (mutational analysis or FISH demonstration of CHIC2 allele deletion) and for patients with HES and/or CEL who are FIP1L1-PDGFR fusion kinase negative or unknown	USA	25/08/2005	19/10/2006	Gleevec	Novartis Pharmaceuticals Corporation
			EU	28/10/2005	28/11/2006	Glivec	Novartis Europharm Limited
L01XE01	Imatinib mesylate	Treatment of adult patients with relapsed or refractory Philadelphia chromosome positive acute lymphoblastic leukemia (Ph+ALL)	USA	11/10/2005	19/10/2006	Gleevec	Novartis Pharmaceuticals Corporation
			EU	26/08/2005	13/09/2006	Glivec	Novartis Europharm Limited

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L01XE01	Imatinib mesylate	Treatment of adult patients with unresectable, recurrent and/or metastatic dermatofibrosarcoma protuberans (DFSP)	USA	19/12/2005	19/10/2006	Gleevec	Novartis Pharmaceuticals Corporation
			EU	26/08/2005	13/09/2006	Glivec	Novartis Europharm Limited
L01XE01	Imatinib mesylate	Treatment of chronic myeloid leukaemia	EU	14/02/2001	07/11/2001	Glivec	Novartis Europharm Limited
L01BB07	Nelarabine	Treatment of acute lymphoblastic leukaemia	USA	10/08/2004	28/10/2005	Arranon	GlaxoSmithKline
			EU	16/06/2005	22/08/2007	Atriance	Glaxo Group Limited
L01XE08	Nilotinib	For the use for chronic phase (CP) and accelerated phase (AP) Philadelphia chromosome positive chronic myelogenous leukemia (CML) in adult patients resistant to or intolerant to prior therapy that included Gleevec®(imatinib)	USA	27/04/2006	29/10/2007	Tasigna	Novartis Pharmaceutical Corporation
			EU	22/05/2006	19/11/2007	Tasigna	Novartis Europharm Limited
L01XC10	Ofatumumab	Treatment of chronic lymphocytic leukemia (CLL) refractory to alemtuzumab and fludarabine	USA	03/10/2009	10-26-2009	Arzerra	GlaxoSmithKline
			EU	07/11/2008	19/04/2010	Arzerra	Glaxo Group Limited
L01XD01	Porfimer	Treatment of high-grade dysplasia in Barrett's Esophagus	USA	19/10/2001	01/08/2003	Photofrin	Axcan Scandipharm Inc
			EU	06/03/2002	25/03/2004	PhotoBarr	Axcan Pharma International BV
L04AC	Rilonacept	Treatment of Cryopyrin-Assisted Periodic Syndromes (CAPS)	USA	20/12/2004	27/02/2008	Arcalyst	Regeneron Pharmaceuticals, Inc.
			EU	10/07/2007	23/10/2009	Rilonacept Regeneron (Arcalyst)	Regeneron UK Limited
L01XE05	Sorafenib	Treatment of renal cell carcinoma	USA	08/10/2004	20/12/2005	Nexavar	Bayer Pharmaceuticals Corporation
			EU	29/07/2004	19/07/2006	Nexavar	Bayer HealthCare AG
L01XE09	Temsirolimus	Treatment of renal cell carcinoma	USA	16/12/2004	30/05/2007	Torisel	Wyeth Pharmaceuticals, Inc.
			EU	06/04/2006	19/11/2007	Torisel	Wyeth Europa Limited
L04AX02	Thalidomide	Treatment of patients with newly diagnosed multiple myeloma	USA	14/10/1998	25/05/2006	Thalomid	Celgene Corporation
			EU	20/11/2001	16/04/2008	Thalomid	Celgene Europe Ltd
L01AB01	Busulfan	Conditioning treatment prior to hematopoietic progenitor cell transplantation	EU	29/12/2000	09/07/2003	Busilvex	Pierre Fabre Médicament
L01AD01	polifeprosan 20 with carmustine	Expanding the indication to include patients with malignant glioma undergoing primary surgical resection.	USA	13/12/1989	25/02/2003	Gliadel	Guilford Pharmaceuticals, Inc.
L01XC06	Cetuximab	For use in combination with radiation therapy, for the treatment of locally orregionally advanced squamous cell carcinoma of the head and neck (SCCHN) and for use as a single agent for the treatment of patients with recurrent or metastatic SCCHN for whom prior platinum-based therapy	USA	03/07/2000	01/03/2006	Erbix	ImClone Systems Incorporated
L01XE06	Dasatinib	Treatment of adults with Philadelphia chromosome-positive acute lymphoblastic leukemia with resistance or intolerance to prior therapy	USA	18/11/2005	28/06/2006	Sprycel	Bristol-Myers Squibb Company

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L01XE08	Nilotinib	Treatment of gastro intestinal stromal tumours	EU	13/04/2007	19/11/2007	Tasigna	Novartis Europharm Limited
L01XX05	Hydroxyurea	Treatment of sickle cell syndrome	EU	09/07/2003	29/06/2007	Siklos	Addmedica
L01XX23	Mitotane	Treatment of adrenal cortical carcinoma	EU	12/06/2002	28/04/2004	Lysodren	Laboratoire HRA Pharma
L01XX32	Bortezomib	Treatment of multiple myeloma patients who have received at least one prior therapy	USA	15/01/2003	25/03/2005	Velcade	Millennium Pharmaceuticals, Inc
L04AX04	lenalidomide	Treatment of patients with transfusion dependant anemia due to low or intermediate-1 risk myelodysplastic syndromes associated with a deletion 5 q cytogenetic abnormality with or without additional cytogenetic abnormalities	USA	29/01/2004	27/12/2005	Revlimid	Celgene Corporation
L01BC07	Azacitidine	Treatment of patients with the following myelodysplastic syndrome subtypes: refractory anemia or refractory anemia with ringed sideroblasts (if accompanied by neutropenia or thrombocytopenia and requiring transfusions), refractory anemia with excess blasts, refractory anemia with excess blasts in transformation, and chronic myelomonocytic leukemia	USA	03/12/2001	19/05/2004	Vidaza	Pharmion Corporation
L01AA09	Bendamustine hydrochloride	Treatment of patients with chronic lymphocytic leukemia	USA	17/08/2007	20/03/2008	Treanda	Cephalon, Inc.
L01AX03	Temozolomide	Treatment of adult patients with newly diagnosed glioblastoma multiforme concomitantly with radiotherapy and then as maintenance treatment	USA	05/10/1998	15/03/2005	Temodar	Schering-Plough Research Institute
L01BA04	Pemetrexed disodium	Treatment of patients with malignant pleural mesothelioma whose disease is either unresectable or who are otherwise not candidates for curative surgery	USA	28/08/2001	04/02/2004	Alimta	Eli Lilly and Company
L01BB04	Cladribine	Treatment of indolent non-Hodgkin's lymphoma	EU	18/09/2001	14/04/2004	Litak	Lipomed GmbH
L01BB06	Clofarabine	Treatment of pediatric patients 1 to 21 years old with relapsed or refractory acute lymphoblastic leukemia after at least two prior regimens	USA	07/02/2002	28/12/2004	Clolar	Genzyme Corporation
			EU	05/02/2002	29/05/2006	Evoltra	Genzyme Europe B.V.
L01BC08	Decitabine	for treatment of patients with myelodysplastic syndromes (MDS) including previously treated and untreated, de novo and secondary MDS of all French-American-British subtypes (refractory anemia, refractory anemia with ringed sideroblasts, refractory anemia with excess blasts, refractory anemia with excess blasts in transformation, and chronic myelomonocytic leukemia) and intermediate-1, intermediate-2, and high-risk International Prognostic	USA	08/03/1999	02/05/2006	Dacogen	MGI CP, Inc. d/b/a MGI Pharma
L01DB01	Doxorubicin HCL liposome injection	For use in combination with bortezomib for the treatment of patients with multiple myeloma who have not previously received bortezomib and have at least one prior therapy.	USA	29/12/2004	17/05/2007	Doxil	Johnson & Johnson Pharmaceutical Research & Dev.
L01CX01	Trabectedin	Treatment of soft tissue sarcoma	EU	30/05/2001	17/09/2007	Yondelis	PharmaMar S.A.
L01XC06	Cetuximab	For use in combination with radiation therapy, for the treatment of locally or regionally advanced squamous cell carcinoma of the head and neck (SCCHN) and for use as a single agent for the treatment of patients with recurrent or metastatic SCCHN for whom prior platinum-based therapy has	USA	03/07/2000	01/03/2006	Erbix	ImClone Systems Incorporated

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L01XD04	5-aminolevulinic acid hydrochloride	Intra-operative photodynamic diagnosis of residual glioma	EU	13/11/2002	07/09/2007	Gliolan	Medac Gesellschaft für klinische Spezialpräparate mbH
L01XE01	Imatinib mesylate	Treatment of adult patients with aggressive mastocytosis (ASM) without the D816V c-Kit mutation or with c-Kit mutational status unknown	USA	09/09/2005	19/10/2006	Gleevec	Novartis Pharmaceuticals Corporation
L01XE04	Sunitinib	Treatment of malignant gastrointestinal stromal tumours	EU	10/03/2005	19/07/2006	Sutent	Pfizer Ltd
L01XE05	Sorafenib	Treatment of unresectable hepatocellular carcinoma	USA	20/04/2006	24/03/2008	Nexavar	Bayer Pharmaceuticals Corporation
L01XE08	Nilotinib	Treatment of gastro intestinal stromal tumours	EU	13/04/2007	19/11/2007	Tasigna	Novartis Europharm Limited
L01XX05	Hydroxyurea	Treatment of sickle cell syndrome	EU	09/07/2003	29/06/2007	Siklos	Addmedica
L01XX23	Mitotane	Treatment of adrenal cortical carcinoma	EU	12/06/2002	28/04/2004	Lysodren	Laboratoire HRA Pharma
L01XX35	Anagrelide	Treatment of essential thrombocythaemia	EU	29/12/2000	16/11/2004	Xagrid	Shire Pharmaceutical Development Ltd
L01XX27	Arsenic trioxide	Treatment of acute promyelocytic leukaemia	EU	18/10/2000	05/03/2002	Trisenox	Cephalon Europe
L01XX33	celecoxib	Treatment of Familial Adenomatous Polyposis	EU	20/11/2001	17/10/2003	Onsenal	Pfizer Limited
L01XX38	Vorinostat	Treatment of cutaneous manifestations in patients with cutaneous T-cell lymphoma (CTCL) who have progressive, persistent or recurrent disease on or following two systemic therapies	USA	16/03/2004	06/10/2006	Zolinza	Merck & Co., Inc.
L04AB02	Infliximab	For reducing signs and symptoms and inducing and maintaining clinical remission in pediatric patients with moderately to severely active Crohn's disease who have had an inadequate response to conventional therapy	USA	12/11/2003	19/05/2006	REMICADE	Centocor, Inc.
L04AB04	Adalimumab	Treatment of Juvenile Idiopathic Arthritis	USA	21/03/2005	21/02/2008	Humira	Abbott Laboratories
L01XE10	Everolimus	Treatment of renal cell carcinoma	EU	05/06/2007	03/08/2009	Afinitor	Novartis Europharm Limited
L03AX16	Plerixafor	Treatment to mobilize progenitor cells prior to stem cell transplantation	EU	20/10/2004	31/07/2009	Mozobil	Genzyme Europe B.V.
L01BA05	Pralatrexate	Treatment of patients with relapsed or refractory peripheral T-cell lymphoma	USA	07/20/2006	09-25-2009	Folotyn	Allos Therapeutics, Inc.
L01XX39	Romidepsin	Treatment of cutaneous T-cell lymphoma (CTCL) in patients who have received at least one prior systemic therapy	USA	09/30/2004	11/05/2009	Istodax	Celgene Corporation
L03AX15	Mifamurtide	Treatment of osteosarcoma	EU	21/06/2004	06/03/2009	Mepact	IDM Pharma S.A.
L04AC04	Rilonacept	Treatment of cryopyrin-associated periodic syndromes (Familial Cold Urticaria Syndrome (FCUS), Muckle-Wells Syndrome (MWS), and Neonatal Onset Multisystem Inflammatory Disease (NOMID), also known as Chronic Infantile Neurological Cutaneous Articular Syndrome (CINCA)	EU	10/07/2007	23/10/2009	Rilonacept Regeneron (Arcalyst)	Regeneron UK Limited
L01XE09	Temsirolimus	Treatment of mantle cell lymphoma	EU	06/11/2006	21/08/2009	Torisel	Wyeth Europa Limited
L01AC01	Thiotepa	Conditioning treatment prior to haematopoietic progenitor cell transplantation	EU	29/01/2007	15/03/2010	Tepadina	Adienne S.r.l.

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ATC	GENERIC NAME	INDICATION	REGION	DESIGNATED DATE	APPROVED DATE	TRADE NAME	SPONSOR
L01CX01	Trabectedin	Treatment of ovarian cancer	EU	17/10/2003	28/10/2009	Yondelis	Pharmamar S.A. Sociedad Unipersonal
L04AC	Rilonacept	Treatment of Cryopyrin-Assisted Periodic Syndromes (CAPS)	USA	20/12/2004	27/02/2008	Arcalyst	Regeneron Pharmaceuticals, Inc.
L04AD02	Tacrolimus	Prophylaxis of organ rejection in patients receiving allogenic heart transplants	USA	06/06/2005	29/03/2006	Prograf	Astellas Pharma US, Inc.
L04AX04	Lenalidomide	Treatment of multiple myeloma	EU	12/12/2003	14/06/2007	Revlimid	Celgene Europe Limited
L01XC07	Bevacizumab	Treatment of glioblastoma with progressive disease following prior therapy	USA	05/26/2006	05/05/2009	Avastin	Genentech, Inc.
L04AC08	Canakinumab	Treatment of cryopyrin-associated periodic syndromes (CAPS), in adults and children 4 years of age and older.	USA	12/18/2007	06-17-2009	Ilaris	Novartis Pharmaceuticals Corporation
L01XC07	Bevacizumab	Treatment of renal cell carcinoma in combination with interferon alfa	USA	11/06/2003	07-31-2009	Avastin	Genentech, Inc.
M01AC06	Meloxicam	For relief of the signs and symptoms of pauciarticular or polyarticular course Juvenile Rheumatoid Arthritis in patients 2 years of age or older	USA	22/11/2002	11/08/2005	Mobic	Boehringer Ingelheim Pharmaceuticals, Inc.
M02AB01	Capsaicin	Management of neuropathic pain associated with postherpetic neuralgia	USA	05/22/2009	11-16-2009	Qutenza	NeurogesX, Inc.
M04AC01	Colchicine	Treatment of familial Mediterranean fever	USA	09/25/2007	07-29-2009	Colcrys	AR Holding Company, Inc.
N02BG08	Ziconotide (intraspinial use)	Treatment of chronic pain requiring intraspinal analgesia	EU	09/07/2001	21/02/2005	Prialt	Eisai Limited
N03AF03	Rufinamide	Treatment of Lennox-Gastaut syndrome	EU	20/10/2004	16/01/2007	Inovelon	Eisai Limited
N03AX17	Stiripentol	Treatment of severe myoclonic epilepsy in infancy	EU	05/12/2001	04/01/2007	Diacomit	Biocodex
N04BC07	Apomorphine HCl	For the acute, intermittent treatment of hypomobility, "off" episodes ("end-of-dose-wearing-off" and unpredictable "on/off" episodes) associated with advanced Parkinson's disease	USA	22/04/1993	20/04/2004	Apokyn	Vernalis R & D Ltd
N03AG04	Vigabatrin	For infantile spasms (IS) - 1 month to 2 years of age.	USA	06/12/2000	08-21-2009	Sabril	H. Lundbeck A/S
N07XX05	Amifampridine	Treatment of Lambert-Eaton myasthenic syndrome	EU	18/12/2002	23/12/2009	Firdapse	BioMarin Europe Limited
N07XX04	Oxybate	Treatment of narcolepsy	USA	07/11/1994	18/11/2005	Xyrem	Jazz Pharmaceuticals
			EU	03/02/2003	13/10/2005	Xyrem	UCB Pharma Ltd.
N06BC01	Caffeine citrate	Treatment of primary apnoea of premature newborns	EU	17/02/2003	02/07/2009	Nymusa	Chiesi Farmaceutici S.P.A.
P01AB02	Tinidazole	Treatment of giardiasis caused by G. duodenalis (also termed G. lamblia) in both adults and pediatric patients older than three years of age	USA	18/04/2002	17/05/2004	Tindamax	Presutti Laboratories, Inc.
P01AB02	Tinidazole	Treatment of intestinal amebiasis and amebic liver abscess caused by E. histolytica in both adults and pediatric patients older than three years of age. It is not indicated for the treatment of asymptomatic cyst passage.	USA	20/08/2003	17/05/2004	Tindamax	Presutti Laboratories, Inc.
P01BC01	Quinine Sulfate	Treatment of uncomplicated Plasmodium falciparum malaria	USA	03/06/2004	12/08/2005	QUALAQUIN	AR Holding Company, Inc.
P01BE52	Artemether/Lumefantrine	Treatment of malaria in patients of 5 kg bodyweight or above with acute, uncomplicated malaria due to Plasmodium falciparum or mixed infections including P. falciparum	USA	08/31/2007	04/07/2009	Coartem	Novartis Pharmaceuticals
S01BA15	Fluocinolone	Treatment of chronic non-infectious uveitis affecting the posterior segment of the eye	USA	31/07/2000	08/04/2005	Retisert	Bausch & Lomb Pharmaceuticals, Inc.

Anexo 1: Comparación de listas de MH

APROVED ORPHAN DRUG			STATUS ORPHAN DRUG				
ATC	GENERIC NAME	INDICATION	REGION	DESIGNATED DATE	APPROVED DATE	TRADE NAME	SPONSOR
V03AC03	Deferasirox	Conditioning treatment prior to hematopoietic progenitor cell transplantation for the treatment of chronic iron overload in patients with transfusion-dependent anaemias	USA	21/11/2002	02/11/2005	Exjade	Novartis Pharmaceuticals Corporation
			EU	13/03/2002	28/08/2006	Exjade	Novartis Europharm Limited
V03AF02	Dexrazoxane	Treatment of anthracycline extravasations	USA	25/03/2004	06/09/2007	Totect	Topo Target A/S
			EU	19/09/2001	28/07/2006	Savene	TopoTarget A/S
V04CJ01	Thyrotropin alfa	For use as an adjunctive treatment for radioiodine ablation of thyroid tissue remnants in patients who have undergone thyroidectomy for well-differentiated thyroid cancer and who do not have evidence of metastatic thyroid cancer	USA	03/08/2001	14/12/2007	Thyrogen	Genzyme Corporation
V03AB23	Acetylcysteine	For the use of Acetadote(registered TM) Injection, administered intravenously within 8 to 10 hours after ingestion of a potentially hepatotoxic quantity of acetaminophen, to prevent or lessen hepatic injury	USA	19/10/2001	23/01/2004	Acetadote	Cumberland Pharmaceuticals, Inc.
V03AF04	Levoleucovorin calcium O LEVOFOLINATO CALCICO	Levoleucovorin rescue is indicated after high-dose methotrexate therapy in osteosarcoma. Levoleucovorin is also indicated to diminish the toxicity and counteract the effects of impaired methotrexate elimination and of inadvertent overdosage of folic acid antagonists	USA	01/08/1991	07/03/2008	Levoleucovorin	Spectrum Pharmaceuticals, Inc.
V10XA53	Tositumomab and iodine I 131 tositumomab	Treatment of patients with CD20 positive, follicular, non-Hodgkin's lymphoma, with and without transformation, whose disease is refractory to Rituximab and has relapsed following chemotherapy	USA	16/05/1994	27/06/2003	Bexxar	GlaxoSmithKline
sin código	Collagenase Clostridium Histolyticum	Treatment of adult patients with Dupuytren's contracture with a palpable cord	USA	05/23/1996	02/02/2010	Xiaflex	Auxilium Pharmaceuticals, Inc.
sin código	Dalfampridine	Treatment to improve walking in patients with multiple sclerosis (MS)	USA	06/02/1987	01-22-2010	Ampyra	Acorda Therapeutics
sin código	Ecallantide	Treatment of acute attacks of hereditary angioedema in patients 16 years of age and older	USA	02/04/2003	12/01/2009	Kalbitor	Dyax Corp
sin código	Human Fibrinogen Concentrate, Pasteurized	Treatment of acute bleeding episodes in patients with congenital fibrinogen deficiency (afibrinogenemia and hypofibrinogenemia)	USA	03/13/2008	01-16-2009	Riastap	CSL Behring, LLC
sin código	Diethylenetriamin epentaacetic acid (DTPA)	Treatment of internal contamination with plutonium, americium or curium	USA	28/04/2004	11/08/2004	no aclara	HamelN Pharmaceuticals gmbh
sin código	Iron(III)-hexacyanoferrate (II)	Treatment of patients with known or suspected internal contamination with radioactive cesium and/or radioactive or non-radioactive thallium to increase their rates of elimination	USA	01/05/2003	02/10/2003	Radiogardase	Heyl Chemisch-Pharmzeitische Fabrik GMBH & Co, KG
sin código	Poractant Alfa	Treatment (rescue) of respiratory distress syndrome in premature	USA	02/08/1993	01/01/9999	Curosurf	Dey Laboratories
sin código	Poractant Alfa	Treatment (rescue) of respiratory distress syndrome in premature infants.	USA	02/08/1993	01/01/9999	Curosurf	Dey Laboratories

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APROVED ORPHAN DRUG			STATUS ORPHAN DRUG				
ATC	GENERIC NAME	INDICATION	REGION	DESIGNATED DATE	APPROVED DATE	TRADE NAME	SPONSOR
sin código	Trypan Blue	For use as an aid in ophthalmic surgery by staining the epiretinal membranes during ophthalmic surgical vitrectomy procedures, facilitating removal of the tissue	USA	08/02/2006	02-20-2009	Membraneblue	Dutch Ophthalmic Research Center Int'l BV
sin código	Benzoate/phenyl acetate	Adjunctive therapy in the treatment of acute hyperammonemia and associated encephalopathy in patients with deficiencies in enzymes of the urea cycle.	USA	22/11/1993	17/02/2005	Ammonul	Medicis Pharmaceutical Corp
sin código	Multi-vitiam infusion without vitamin K	Prevention of vitamin deficiency and thrombolytic complications in people receiving home parenteral nutrition who also receive warfarin-type anticoagulant therapy	USA	08/03/2004	09/09/2004	M.V.I.-12	Mayne Pharma (USA) Inc
sin código	C1-Esterase-Inhibitor, Human, Pasteurized	Treatment of acute attacks of hereditary angioedema.	USA	10/16/1992	10/08/2009	Beriner P	CSL Behring L.L.C.
sin código	Biocarbonate infusate	Use as a replacement solution in Continuous Renal Replacement Therapy (CRRT) to replace water and to correct electrolytes and acid-base imbalances in adults and children	USA	09/08/2005	26/07/2006	Normocarb HF	Dialysis Solutions, Inc.
sin código	Velaglucerase alfa	Treatment of Gaucher disease	EU	09/06/2010	26/08/2010	VPRIV	Shire Pharmaceuticals Ireland Limited

Anexo 1: Comparación de listas de MH

APROVED ORPHAN DRUG			USA	EU	SPAIN		ITALY	ARGENTINA		CANADA		PERU	BRAZIL
ATC	GENERIC NAME	INDICATION	R	R	R	C (a)	R	R	C (b)	R	C (a)	R	R
A16AB09	Idursulfase	is indicated for the long term treatment of patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II)	si	si	si	si	no	no	-	si	si	no	si
A07AA11	Rifaximin	Reduction in the risk of overt hepatic encephalopathy (HE) recurrence in patients greater than or equal to 18 years of age	si	no	si	si	si	si	no	no	-	si	no
A16AB	Galsulfase	Treatment of Mucopolysaccharidosis, type VI (Maroteaux-Lamy Syndrome)	si	si	si	si	si	no	-	no	-	no	si
A16AB05	laronidase	Treatment for patients with Hurler and Hurler-Scheie forms of Mucopolysaccharidosis I (MPS I) and for patients with the Scheie form	si	si	si	si	si	si	no	si	si	si	si
A16AX06	Miglustat	for the treatment of mild to moderate type 1 Gaucher disease	si	si	si	si	si	no	-	si	si	no	si
A16AX04	Nitisinone	Treatment of tyrosinaemia type I	si	si	si	si	no	no	-	no	-	no	no
A16AX07	Sapropterin	Indicated to reduce blood phenylalanine (Phe) levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive Phenylketonuria (PKU). Kuvan is to be used in conjunction	si	si	si	si	si	no	-	no	-	no	no
A16AA03	Glutamine	Treatment of short bowel syndrome in patients receiving specialized nutritional support when used in conjunction with a recombinant human growth hormone that is approved for this indication	si	no	no	-	no	no	-	no	-	si	no
A16AB03	Agalsidase alfa	Treatment of Fabry disease	no	si	si	si	si	si	no	si	si	no	si
A16AB04	Agalsidase beta	Treatment of Fabry disease	si	si	si	si (*)	si	si	no	si	si	no	si
A16AA05	Carglumic acid	Treatment of N-acetylglutamate synthetase (NAGS) deficiency	no	si	si	si	si	no	-	no	-	no	no
A16AA06	Betaine	Treatment of homocystinuria	si	si	si	si	si	si	si	si	si	no	no
A16AX05	Zinc acetate	Treatment of Wilson's disease	si	si	si	si	si	si	si	si	si	si	si
A16AB07	Alglucosidase alfa	Treatment of Glycogen Storage Disease type II (Pompe's disease)	si	no	si	si	si	si	si	no	-	no	no
A16AX06	Miglustat	Treatment of Niemann-Pick Disease, type C	si	si	si	si	si	no	-	si	si	no	si
A07EC04	Balsalazide disodium	Treatment of mildly to moderately active ulcerative colitis in patients 5 years of age and older. Safety and Effectiveness of Colazal beyond 8 weeks in children (ages 5-17) and 12 weeks in adults have not been established.	si	no	no	-	si	si	no	no	-	no	no
B01AD12	Protein C concentrate	Prevention and treatment of venous thrombosis and purpura fulminans	si	si	si	si	si	no	-	no	-	no	no
B02BD05	Coagulation factor VIIa (recombinant)	Prevention of bleeding in surgical interventions or invasive procedures in patients with acquired hemophilia	si	si	no	-	si	si	si	no	-	no	no
B02BD05	Coagulation factor VIIa (recombinant)	Prevention of bleeding in surgical interventions or invasive procedures in patients with congenital F VII deficiency	si	si	no	-	si	si	si	no	-	no	no

R=registrado

C=comercializ

(*) con problemas de suministro

Anexo 1: Comparación de listas de MH

APROVED ORPHAN DRUG			USA	EU	SPAIN		ITALY	ARGENTINA		CANADA		PERU	BRAZIL
ATC	GENERIC NAME	INDICATION	R	R	R	C (a)	R	R	C (b)	R	C (a)	R	R
B02BD05	Coagulation factor VIIa (recombinant)	Treatment of bleeding episodes in surgical interventions or invasive procedures in patients with acquired hemophilia	si	si	no	-	si	si	si	no	-	no	no
B02BD05	Coagulation factor VIIa (recombinant)	Prevention of bleeding in surgical interventions or invasive procedures in hemophilia A or B patients with inhibitors to Factor VIII or Factor IX	si	si	no	-	si	si	si	no	-	no	no
B02BD05	Coagulation factor VIIa (recombinant)	Treatment of bleeding episodes in patients with Factor VII Deficiency	si	si	no	-	si	si	si	no	-	no	no
B02BD02	Antihemophilic factor (human)	For surgical and/or invasive procedures in patients with von Willebrand Disease (VWD) in whom desmopressin is either ineffective or contraindicated. It is not indicated for patients with severe VWD (Type 3) undergoing major surgery	si	no	no	-	si	si	si	si	si	no	no
B02BD06	Human Plasma Coagulation Factor VIII And Human Plasma Von Willebrand Factor	Treatment of spontaneous or trauma-induced bleeding episodes in patients with severe von Willebrand disease (VWD) as well as patients with mild or moderate VWD in whom the use of desmopressin is known or suspected to be ineffective or contraindicated	no	no	si	no	si	si	si	si	si	si	si
B03BA03	Hydroxocobalamin	Treatment on known or suspected cyanide poisoning	si	si	si	si	si	no	-	si	si	no	no
B01AC11	Iloprost	Treatment of pulmonary arterial hypertension (WHO Group I) in patients with NYHA Class III or IV symptoms	si	si	si	si	si	si	si	no	-	si	si
B01AC21	Treprostinil (Inhalational)	Treatment of pulmonary arterial hypertension (WHO Group I) in patients with NYHA Class III symptoms, to increase walk distance	si	no	no	-	no	no	-	no	-	no	no
B02BX05	Eltrombopagolamine	Treatment of idiopathic thrombocytopenic purpura	si	si	si	si	no	si	si	no	-	no	si
B02BX04	Romiplostim	Treatment of idiopathic thrombocytopenic purpura	no	si	si	si	si	no	-	si	si	no	no
B01A	Recombinant Human Antithrombin	Prevention of peri-operative and peri-partum thromboembolic events, in hereditary antithrombin deficient patients.	no	si	si	no	si	si	si	si	si	no	si
C02KX02	Ambrisentan	Treatment of pulmonary arterial hypertension (WHO group I) in patients with WHO class II or III symptoms to improve exercise capacity and delay clinical worsening	si	si	si	si	si	no	-	si	si	no	no
C02KX01	Bosentan	Treatment of pulmonary arterial hypertension.	si	si	si	si	si	si	no	si	si	si	si
C01EB16	Ibuprofen	Treatment of patent ductus arteriosus	si	si	si	si	si	si	si	si	si	no	no
C07AA07	Sotalol (Iv)	For use as a substitute for oral sotalol in patients who are unable to take sotalol orally [oral sotalol is indicated for maintenance of normal sinus rhythm in patients with history of highly symptomatic atrial fibrillation /flutter, and for treatment of documented life-threatening ventricular arrhythmias]	no	no	si	si	si	si	si	si	si	no	no

R=registrado

C=comercializ

viene ev

(a) según web
(b) según lab

Anexo 1: Comparación de listas de MH

APROVED ORPHAN DRUG			USA	EU	SPAIN		ITALY	ARGENTINA		CANADA		PERU	BRAZIL
ATC	GENERIC NAME	INDICATION	R	R	R	C (a)	R	R	C (b)	R	C (a)	R	R
C07AA07	Tadalafil	Treatment of pulmonary arterial hypertension (WHO Group I) to improve exercise ability	si	si	si	si	si	si	si	si	si	si	si
C02KX03	Sitaxentan sodium	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	no	si	si	si	si	no	-	no	-	no	no
G03G	Recombinant human luteinizing hormone	Luveris (lutropin alfa for injection), concomitantly administered with Gonal-f (follitropin alfa for injection), is indicated for stimulation of follicular development in infertile hypogonadotropic hypogonadal women with profound LH deficiency (LH (less than) 1.2 IU/L).	si	si	si	si	si	si	si	no	-	no	no
G03XC01	Raloxifene	Reduction in risk of invasive breast cancer in postmenopausal women with osteoporosis and reduction in risk of invasive breast cancer in postmenopausal women at high risk for invasive breast cancer	si	si	si	si	si	si	si	si	si	si	si
G04BE03	Sildenafil citrate	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	si	si	si	si	si	si	si	si	si	si	si
H01AC01	Somatropin	Treatment of short stature in patients with Noonan syndrome	si	no	si	si	si	si	si	si	si	si	si
H01AC01	Somatropin	Treatment of short bowel syndrome in patients receiving specialized nutritional support	si	no	si	si	si	si	si	si	si	si	si
H01AC05	Mecasermin rinfabate	Treatment of growth failure in children with severe primary IGF-1 deficiency (Primary IGFD) or with growth hormone (GH) gene deletion who have developed neutralizing antibodies to growth hormone	si	no	no	-	no	no	-	no	-	no	no
H01CA03	Histrelin	Treatment of central precocious puberty	si	no	no	-	no	si	si	si	si	no	no
H01CB03	Lanreotide	Long-term treatment of acromegalic patients who have had an inadequate response to or cannot be treated with surgery and/or radiotherapy	si	no	no	-	si	si	si	si	si	no	si
H05BX01	Cinacalcet	Treatment of hypercalcemia in patients with parathyroid carcinoma	si	no	si	si	si	no	-	si	si	no	no
H01AC03	Mecasermin	Long-term treatment of growth failure in children with severe primary IGF-1 deficiency (Primary IGFD) or with growth hormone (GH) gene deletion who have developed neutralizing antibodies to growth hormone.	si	si	si	si	si	no	-	no	-	no	no
H01AX01	Pegvisomant	Treatment of acromegaly	si	si	si	si	si	no	-	si	si	si	si
J05AB06	Ganciclovir	Treatment of acute herpetic keratitis (dendritic ulcers)	si	no	si	si	si	si	si	si	si	si	si
J05AB04	Ribavirin	Treatment of chronic hepatitis C among previously untreated pediatric patients at least three years of age or older.	si	si	si	si	si	si	si	si	si	si	si
J06BC	Botulism immune globulin	Indicated for treatment of infant botulism caused by type A or type B Clostridium botulinum.	si	no	no	-	no	no	-	no	-	no	no
J06BB04	Hepatitis B immune globulin (human)	Prevention of hepatitis B recurrence following liver transplantation in HBsAG-positive liver transplant patients	si	no	no	-	no	si	si	no	-	no	no

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APROVED ORPHAN DRUG			USA	EU	SPAIN		ITALY	ARGENTINA		CANADA		PERU	BRAZIL
ATC	GENERIC NAME	INDICATION	R	R	R	C (a)	R	R	C (b)	R	C (a)	R	R
J07	Vaccinia Immune Globulin (Human) Intravenous	Treatment and modification of aberrant infections induced by vaccinia virus that include its accidental implantation in eyes (except in cases of isolated keratitis), mouth, or other areas where vaccinia infection would constitute a special hazard; eczema vaccinatum; progressive vaccinia; severe generalized vaccinia, and vaccinia infections in individuals who have skin conditions such as burns, impetigo, varicella-zoster, or poison ivy; or in individuals who have eczematous skin lesions because of either the activity	si	no	no	-	no	si	si	no	-	no	no
J06BC	Botulism immune globulin	Indicated for treatment of infant botulism caused by type A or type B Clostridium botulinum.	si	no	no	-	no	no	-	no	-	no	no
J01DF01	Aztreonam lysinate (inhalation use)	To improve respiratory symptoms in cystic fibrosis (CF) patients with Pseudomonas aeruginosa (gram negative)	si	si	si	no	no	no	-	si	si	no	no
L01BB06	Clofarabine	Treatment of pediatric patients 1 to 21 years old with relapsed or refractory acute lymphoblastic leukemia after at least two prior regimens	si	si	si	si	si	no	-	no	-	no	no
L01XE06	Dasatinib	Treatment of chronic myeloid leukaemia	si	si	si	si	si	si	si	si	si	si	si
L04AA25	Eculizumab	Treatment of paroxysmal nocturnal haemoglobinuria	si	si	si	si	si	no	-	si	si	no	no
L01XE01	Imatinib mesylate	Treatment of patients with Kit (CD117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumors (GIST)	si	si	si	si	si	si	si	si	si	si	si
L01XE01	Imatinib mesylate	Treatment of adult patients with aggressive mastocytosis (ASM) without the D816V c-Kit mutation or with c-Kit mutational status unknown	si	si	si	si	si	si	si	si	si	si	si
L01XE01	Imatinib mesylate	Treatment of adult patients with myelodysplastic/myeloproliferative diseases (MDS/MPD) associated with PDGFR (platelet-derived growth factor receptor) gene re-arrangements	si	si	si	si	si	si	si	si	si	si	si
L01XE01	Imatinib mesylate	Treatment of adult patients with hypereosinophilic syndrome (HES) and/or chronic eosinophilic leukemia (CEL) who have the FIP1L1-PDGFR1± fusion kinase (mutational analysis or FISH demonstration of CHIC2 allele deletion) and for patients with HES and/or CEL who are FIP1L1-PDGFR1± fusion kinase negative or unknown	si	si	si	si	si	si	si	si	si	si	si
L01XE01	Imatinib mesylate	Treatment of adult patients with relapsed or refractory Philadelphia chromosome positive acute lymphoblastic leukemia (Ph+ALL)	si	si	si	si	si	si	si	si	si	si	si

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APROVED ORPHAN DRUG			USA	EU	SPAIN		ITALY	ARGENTINA		CANADA		PERU	BRAZIL
ATC	GENERIC NAME	INDICATION	R	R	R	C (a)	R	R	C (b)	R	C (a)	R	R
L01XE01	Imatinib mesylate	Treatment of adult patients with unresectable, recurrent and/or metastatic dermatofibrosarcoma protuberans (DFSP)	si	si	si	si	si	si	si	si	si	si	si
L01XE01	Imatinib mesylate	Treatment of chronic myeloid leukaemia	si	si	si	si	si	si	si	si	si	si	si
L01BB07	Nelarabine	Treatment of acute lymphoblastic leukaemia	si	si	si	si	si	no	-	si	si	no	no
L01XE08	Nilotinib	For the use for chronic phase (CP) and accelerated phase (AP) Philadelphia chromosome positive chronic myelogenous leukemia (CML) in adult patients resistant to or intolerant to prior therapy that included Gleevec®(imatinib)	si	si	si	si	si	si	si	si	si	si	si
L01XC10	Ofatumumab	Treatment of chronic lymphocytic leukemia (CLL) refractory to alemtuzumab and fludarabine	si	si	si	si	no	no	-	no	-	no	no
L01XD01	Porfimer	Treatment of high-grade dysplasia in Barrett's Esophagus	si	si	no	-	no	no	-	si	si	no	no
L04AC	Rilonacept	Treatment of Cryopyrin-Assisted Periodic Syndromes (CAPS)	si	si	no	-	no	no	-	no	-	no	no
L01XE05	Sorafenib	Treatment of renal cell carcinoma	si	si	si	si	si	si	si	si	si	si	si
L01XE09	Temsirolimus	Treatment of renal cell carcinoma	si	si	si	si	si	si	no	si	si	no	no
L04AX02	Thalidomide	Treatment of patients with newly diagnosed multiple myeloma	si	si	no	-	si	si	si	no	-	si	si
L01AB01	Busulfan	Conditioning treatment prior to hematopoietic progenitor cell transplantation	si	si	si	si	si	si	si	si	si	no	no
L01AD01	polifeprosan 20 with carmustine	Expanding the indication to include patients with malignant glioma undergoing primary surgical resection.	si	si	si	si	no	si	si	no	-	no	no
L01XC06	Cetuximab	For use in combination with radiation therapy, for the treatment of locally orregionally advanced squamous cell carcinoma of the head and neck (SCCHN) and for use as a single agent for the treatment of patients with recurrent or metastatic SCCHN for whom prior platinum-based therapy	si	si	si	si	si	no	-	si	si	si	si
L01XE06	Dasatinib	Treatment of adults with Philadelphia chromosome-positive acute lymphoblastic leukemia with resistance or intolerance to prior therapy	si	si	si	si	si	si	si	si	si	si	si

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APROVED ORPHAN DRUG			USA	EU	SPAIN		ITALY	ARGENTINA		CANADA		PERU	BRAZIL
ATC	GENERIC NAME	INDICATION	R	R	R	C (a)	R	R	C (b)	R	C (a)	R	R
L01XE08	Nilotinib	Treatment of gastro intestinal stromal tumours	si	si	si	si	si	si	si	si	si	si	si
L01XX05	Hydroxyurea	Treatment of sickle cell syndrome	si	si	no	-	no	si	si	si	si	si	no
L01XX23	Mitotane	Treatment of adrenal cortical carcinoma	si	si	si	si	si	no	-	si	si	no	si
L01XX32	Bortezomib	Treatment of multiple myeloma patients who have received at least one prior therapy	si	si	si	si	si	si	si	si	si	si	si
L04AX04	lenalidomide	Treatment of patients with transfusion dependant anemia due to low or intermediate-1 risk myelodysplastic syndromes associated with a deletion 5 q cytogenetic abnormality with or without additional cytogenetic abnormalities	si	si	si	si	si	si	si	si	si	si	no
L01BC07	Azacitidine	Treatment of patients with the following myelodysplastic syndrome subtypes: refractory anemia or refractory anemia with ringed sideroblasts (if accompanied by neutropenia or thrombocytopenia and requiring transfusions), refractory anemia with excess blasts, refractory anemia with excess blasts in transformation, and chronic myelomonocytic leukemia	si	si	si	si	no	si	si	no	-	no	si
L01AA09	Bendamustine hydrochloride	Treatment of patients with chronic lymphocytic leukemia	si	no	no	-	no	no	-	no	-	no	no
L01AX03	Temozolomide	Treatment of adult patients with newly diagnosed glioblastoma multiforme concomitantly with radiotherapy and then as maintenance treatment	si	si	si	si	si	si	si	si	si	si	si
L01BA04	Pemetrexed disodium	Treatment of patients with malignant pleural mesothelioma whose disease is either unresectable or who are otherwise not candidates for curative surgery	si	si	si	si	si	no	-	si	si	si	si
L01BB04	Cladribine	Treatment of indolent non-Hodgkin's lymphoma	si	si	si	si	si	si	si	si	si	no	si
L01BB06	Clofarabine	Treatment of pediatric patients 1 to 21 years old with relapsed or refractory acute lymphoblastic leukemia after at least two prior regimens	si	si	si	si	si	no	-	no	-	no	no
L01BC08	Decitabine	for treatment of patients with myelodysplastic syndromes (MDS) including previously treated and untreated, de novo and secondary MDS of all French-American-British subtypes (refractory anemia, refractory anemia with ringed sideroblasts, refractory anemia with excess blasts, refractory anemia with excess blasts in transformation, and chronic myelomonocytic leukemia) and intermediate-1, intermediate-2, and high-risk International Prognostic	si	no	no	-	no	no	-	no	-	si	si
L01DB01	Doxorubicin HCL liposome injection	For use in combination with bortezomib for the treatment of patients with multiple myeloma who have not previously received bortezomib and have at least one prior therapy.	si	no	no	-	no	si	si	no	-	no	no
L01CX01	Trabectedin	Treatment of soft tissue sarcoma	no	si	si	si	si	no	-	no	-	no	no
L01XC06	Cetuximab	For use in combination with radiation therapy, for the treatment of locally or regionally advanced squamous cell carcinoma of the head and neck (SCCHN) and for use as a single agent for the treatment of patients with recurrent or metastatic SCCHN for whom prior platinum-based therapy has	si	si	si	si	si	no	-	si	si	si	si

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Anexo 1: Comparación de listas de MH

APROVED ORPHAN DRUG			USA	EU	SPAIN		ITALY	ARGENTINA		CANADA		PERU	BRAZIL
ATC	GENERIC NAME	INDICATION	R	R	R	C (a)	R	R	C (b)	R	C (a)	R	R
L01XD04	5-aminolevulinic acid hydrochloride	Intra-operative photodynamic diagnosis of residual glioma	no	no	no	-	no	no	-	si	si	no	si
L01XE01	Imatinib mesylate	Treatment of adult patients with aggressive mastocytosis (ASM) without the D816V c-Kit mutation or with c-Kit mutational status unknown	si	si	si	si	si	si	si	si	si	si	si
L01XE04	Sunitinib	Treatment of malignant gastrointestinal stromal tumours	si	si	si	si	si	si	si	si	si	si	si
L01XE05	Sorafenib	Treatment of unresectable hepatocellular carcinoma	si	si	si	si	si	si	si	si	si	si	si
L01XE08	Nilotinib	Treatment of gastro intestinal stromal tumours	si	si	si	si	si	si	si	si	si	si	si
L01XX05	Hydroxyurea	Treatment of sickle cell syndrome	si	si	no	-	no	si	si	si	si	si	no
L01XX23	Mitotane	Treatment of adrenal cortical carcinoma	si	si	si	si	si	no	-	si	si	no	si
L01XX35	Anagrelide	Treatment of essential thrombocythaemia	si	si	si	si	si	si	si	si	si	no	si
L01XX27	Arsenic trioxide	Treatment of acute promyelocytic leukaemia	si	si	si	si	si	si	no	no	-	no	si
L01XX33	celecoxib	Treatment of Familial Adenomatous Polyposis	si	si	si	si	si	si	si	si	si	si	si
L01XX38	Vorinostat	Treatment of cutaneous manifestations in patients with cutaneous T-cell lymphoma (CTCL) who have progressive, persistent or recurrent disease on or following two systemic therapies	si	no	no	-	no	no	-	si	si	no	no
L04AB02	Infliximab	For reducing signs and symptoms and inducing and maintaining clinical remission in pediatric patients with moderately to severely active Crohn's disease who have had an inadequate response to conventional therapy	si	si	si	si	si	si	si	si	si	si	si
L04AB04	Adalimumab	Treatment of Juvenile Idiopathic Arthritis	si	si	si	si	si	no	-	si	si	si	si
L01XE10	Everolimus	Treatment of renal cell carcinoma	si	si	si	si	no	si	si	si	si	no	si
L03AX16	Plerixafor	Treatment to mobilize progenitor cells prior to stem cell transplantation	si	si	si	si	no	no	-	no	-	no	si
L01BA05	Pralatrexate	Treatment of patients with relapsed or refractory peripheral T-cell lymphoma	si	no	no	-	no	no	-	no	-	no	no
L01XX39	Romidepsin	Treatment of cutaneous T-cell lymphoma (CTCL) in patients who have received at least one prior systemic therapy	si	no	no	-	no	no	-	no	-	no	no
L03AX15	Mifamurtide	Treatment of osteosarcoma	no	no	si	no	no	no	-	no	-	no	no
L04AC04	Riloncept	Treatment of cryopyrin-associated periodic syndromes (Familial Cold Urticaria Syndrome (FCUS), Muckle-Wells Syndrome (MWS), and Neonatal Onset Multisystem Inflammatory Disease (NOMID), also known as Chronic Infantile Neurological Cutaneous Articular Syndrome (CINCA)	no	si	no	-	no	no	-	no	-	no	no
L01XE09	Temsirolimus	Treatment of mantle cell lymphoma	si	si	si	si	si	si	no	si	si	no	si
L01AC01	Thiotepa	Conditioning treatment prior to haematopoietic progenitor cell transplantation	si	si	si	no	no	si	no	no	-	no	no

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L01CX01	Trabectedin	Treatment of ovarian cancer	no	si	si	si	si	si	si	si	si	si	si
L04AC	Rilonacept	Treatment of Cryopyrin-Assisted Periodic Syndromes (CAPS)	si	no	no	-	no	no	-	no	-	no	no
L04AD02	Tacrolimus	Prophylaxis of organ rejection in patients receiving allogeneic heart transplants	si	no	no	-	no	si	si	si	si	si	si
L04AX04	Lenalidomide	Treatment of multiple myeloma	si	si	si	si	si	si	si	si	si	si	no
L01XC07	Bevacizumab	Treatment of glioblastoma with progressive disease following prior therapy	no	si	si	si	si	si	si	si	si	si	si
L04AC08	Canakinumab	Treatment of cryopyrin-associated periodic syndromes (CAPS), in adults and children 4 years of age and older.	no	si	si	no	si	no	-	si	si	no	no
L01XC07	Bevacizumab	Treatment of renal cell carcinoma in combination with interferon alfa	no	si	si	si	si	si	si	si	si	si	si
M01AC06	Meloxicam	For relief of the signs and symptoms of pauciarticular or polyarticular course Juvenile Rheumatoid Arthritis in patients 2 years of age or older	si	no	si	si	si	si	si	si	si	si	si
M02AB01	Capsaicin	Management of neuropathic pain associated with postherpetic neuralgia	si	si	si	si	si	si	si	si	no	-	no
M04AC01	Colchicine	Treatment of familial Mediterranean fever	si	no	si	si	si	si	si	si	si	si	si
N02BG08	Ziconotide (intraspinal use)	Treatment of chronic pain requiring intraspinal analgesia	si	si	si	si	si	no	-	no	-	no	no
N03AF03	Rufinamide	Treatment of Lennox-Gastaut syndrome	no	si	si	si	si	no	-	no	-	no	no
N03AX17	Stiripentol	Treatment of severe myoclonic epilepsy in infancy	no	si	no	-	no	no	-	no	-	no	no
N04BC07	Apomorphine HCl	For the acute, intermittent treatment of hypomobility, "off" episodes ("end-of-dose-wearing-off" and unpredictable "on/off" episodes) associated with advanced Parkinson's disease	si	no	no	-	no	si	si	no	-	no	si
N03AG04	Vigabatrin	For infantile spasms (IS) - 1 month to 2 years of age.	si	no	si	si	si	si	si	si	si	no	si
N07XX05	Amifampridine	Treatment of Lambert-Eaton myasthenic syndrome	no	si	no	-	no	no	-	no	-	no	no
N07XX04	Oxybate	Treatment of narcolepsy	si	si	si	si	si	no	-	si	si	no	no
N06BC01	Caffeine citrate	Treatment of primary apnoea of premature newborns	si	si	no	-	no	si	si	no	-	no	no
P01AB02	Tinidazole	Treatment of giardiasis caused by G. duodenalis (also termed G. lamblia) in both adults and pediatric patients older than three years of age	si	no	no	-	si	si	si	no	-	si	si
P01AB02	Tinidazole	Treatment of intestinal amebiasis and amebic liver abscess caused by E. histolytica in both adults and pediatric patients older than three years of age. It is not indicated for the treatment of asymptomatic cyst passage.	si	no	no	-	si	si	si	no	-	si	si
P01BC01	Quinine Sulfate	Treatment of uncomplicated Plasmodium falciparum malaria	si	no	no	-	no	si	si	si	si	si	si
P01BE52	Artemether/Lumefantrine	Treatment of malaria in patients of 5 kg bodyweight or above with acute, uncomplicated malaria due to Plasmodium falciparum or mixed infections including P. falciparum	si	no	si	no	no	si	no	no	-	si	si
S01BA15	Fluocinolone	Treatment of chronic non-infectious uveitis affecting the posterior segment of the eye	si	no	no	-	no	no	-	no	-	no	no

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V03AC03	Deferasirox	Conditioning treatment prior to hematopoietic progenitor cell transplantation for the treatment of chronic iron overload in patients with transfusion-dependent anaemias	si	si	si	si	si	si	si	si	si	si	si
V03AF02	Dexrazoxane	Treatment of anthracycline extravasations	si	si	si	si	si	si	si	si	si	si	si
V04CJ01	Thyrotropin alfa	For use as an adjunctive treatment for radioiodine ablation of thyroid tissue remnants in patients who have undergone thyroidectomy for well-differentiated thyroid cancer and who do not have evidence of metastatic thyroid cancer	si	si	si	si	si	no	-	si	si	si	si
V03AB23	Acetylcysteine	For the use of Acetadote(registered TM) Injection, administered intravenously within 8 to 10 hours after ingestion of a potentially hepatotoxic quantity of acetaminophen, to prevent or lessen hepatic injury	si	no	no	-	si	si	no	si	si	si	si
V03AF04	Levoleucovorin calcium O LEVOFOLINATO CALCICO	Levoleucovorin rescue is indicated after high-dose methotrexate therapy in osteosarcoma. Levoleucovorin is also indicated to diminish the toxicity and counteract the effects of impaired methotrexate elimination and of inadvertent overdosage of folic acid antagonists	si	no	no	-	no	no	-	no	-	no	si
V10XA53	Tositumomab and iodine I 131 tositumomab	Treatment of patients with CD20 positive, follicular, non-Hodgkin's lymphoma, with and without transformation, whose disease is refractory to Rituximab and has relapsed following chemotherapy	si	no	no	-	no	no	-	si	si	no	no
sin código	Collagenase Clostridium Histolyticum	Treatment of adult patients with Dupuytren's contracture with a palpable cord	no	no	no	-	no	no	-	no	-	no	no
sin código	Dalfampridine	Treatment to improve walking in patients with multiple sclerosis (MS)	si	no	no	-	no	no	-	no	-	no	no
sin código	Ecallantide	Treatment of acute attacks of hereditary angioedema in patients 16 years of age and older	no	no	no	-	no	no	-	no	-	no	no
sin código	Human Fibrinogen Concentrate, Pasteurized	Treatment of acute bleeding episodes in patients with congenital fibrinogen deficiency (afibrinogenemia and hypofibrinogenemia)	no	no	no	-	si	si	si	si	si	no	si
sin código	Diethylenetriamin epentaacetic acid (DTPA)	Treatment of internal contamination with plutonium, americium or curium	si	no	no	-	no	no	-	no	-	no	no
sin código	Iron(III)-hexacyanoferrate (II)	Treatment of patients with known or suspected internal contamination with radioactive cesium and/or radioactive or non-radioactive thallium to increase their rates of elimination	si	no	no	-	no	no	-	no	-	no	no
sin código	Poractant Alfa	Treatment (rescue) of respiratory distress syndrome in premature	si	no	si	si	no	no	-	no	-	si	no
sin código	Poractant Alfa	Treatment (rescue) of respiratory distress syndrome in premature infants.	si	si	si	si	no	no	-	no	-	si	no

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sin código	Trypan Blue	For use as an aid in ophthalmic surgery by staining the epiretinal membranes during ophthalmic surgical vitrectomy procedures, facilitating removal of the tissue	si	no	no	-	no	no	-	si	si	si	no
sin código	Benzoate/phenyl acetate	Adjunctive therapy in the treatment of acute hyperammonemia and associated encephalopathy in patients with deficiencies in enzymes of the urea cycle.	si	no	no	-	no	no	-	no	-	no	no
sin código	Multi-vitamin infusion without vitamin K	Prevention of vitamin deficiency and thrombotic complications in people receiving home parenteral nutrition who also receive warfarin-type anticoagulant therapy	si	no	no	-	no	no	-	no	-	no	no
sin código	C1-Esterase-Inhibitor, Human, Pasteurized	Treatment of acute attacks of hereditary angioedema.	no	no	no	-	no	no	-	si	si	no	no
sin código	Biocarbonate infusate	Use as a replacement solution in Continuous Renal Replacement Therapy (CRRT) to replace water and to correct electrolytes and acid-base imbalances in adults and children	si	no	no	-	no	si	si	si	si	no	no
sin código	Velaglucerase alfa	Treatment of Gaucher disease	si	si	si	no	no	no	-	si	si	no	no

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