

Tradename	Active substance	Designated orphan indication	Sponsor of designation	Market Authorization date
ALDURAZYME	Laronidase (INN)	Treatment of Mucopolysaccharidosis, type I	Genzyme B.V.	10/06/2003
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	Treatment of homocystinuria	Orphan Europe	15/02/07
DIACOMIT	Stiripentol	Treatment of severe myoclonic epilepsy in infancy	Biocodex	04/01/07
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

GLIVEC	Imatinib mesilate (INN)	Treatment of chronic myeloid leukaemia	Novartis Europharm Limited	07/11/2001
		Treatment of malignant gastrointestinal stromal tumours		07/11/2001
		Treatment of acute lymphoblastic leukaemia		07/11/2001
		Treatment of chronic eosinophilic leukaemia and the hypereosinophilic syndrome		07/11/2001
		Treatment of dermatofibrosarcoma protuberans		07/11/2001
		Treatment of myelodysplastic / myeloproliferative diseases		07/11/2001
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	Bayer Healthcare AG	19/07/2006
ONSENAL	Celecoxib (INN)	Treatment of Familial Adenomatous Polyposis	Pharmacia-Pfizer EEIG	17/10/2003
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
SAVENE	Dexrazoxane (INN)	Treatment of anthracycline extravasations	TopoTarget A/S	28/07/2006

SOMAVERT	Pegvisomant (INN)	Treatment of acromegaly	Pfizer Limited	13/11/2002
SPRYCEL	Dasatinib (INN)	Treatment of chronic myeloid leukaemia Treatment of acute lymphoblastic leukaemia	Bristol-Myers Squibb Pharma EEIG	20/11/2006 20/11/2006
SUTENT	Sunitinib malate INN=sunitinib	Treatment of malignant gastrointestinal stromal tumours Treatment of renal cell carcinoma	Pfizer Limited	19/07/2006
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 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	Schering AG	16/09/2003
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 Development Ltd	16/11/2004
XYREM	Sodium oxybate (INN)	Treatment of narcolepsy	UCB Pharma Ltd.	13/10/2005
ZAVESCA	Miglustat (INN)	Treatment of Gaucher Disease	Actelion Registration Limited	20/11/2002