



European Medicines Agency
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List of orphan-designated authorised medicines

(List current on 6 November 2008)

Medicines intended for the treatment, prevention or diagnosis of rare diseases (defined as those that affect fewer than five in 10,000 persons in the European Union) may be awarded an 'orphan designation' by the European Commission on the basis of a positive opinion from the European Medicines Agency's (EMA) Committee for Orphan Medicinal Products (COMP).

Orphan medicines are designated for broad medical conditions where development is possible for many therapeutic indications targeting specific groups of patients. The orphan indication refers to the potential use for diagnosis, prevention or treatment of the designated condition.

Orphan-designated medicines follow the centralised route of authorisation, which enables companies to obtain a Community marketing authorisation (valid in all Member States of the European Union) on the basis of a positive opinion from the EMA's Committee for Medicinal Products for Human Use (CHMP).

Below is a listing of medicines that have received both an orphan designation and a Community marketing authorisation, with a link to their European public assessment report (with the exception of Vidaza and Kuvan, which are currently in the decision-making procedure at the level of the European Commission). They are listed by year in which they received a Community marketing authorisation.

See also:

[Orphan-designated medicines in numbers \(p.18\)](#)

[List of authorised orphan-designated medicines per therapeutic area \(p.20\)](#)

2008

Thalidomide Pharmion (*thalidomide*)

Date of orphan medicinal product designation: 20 November 2001

Date of issue of marketing authorisation: 16 April 2008

Designated orphan indication: treatment of multiple myeloma

Authorised therapeutic indication: treatment of myeloma

Therapeutic area: haematology

Marketing authorisation holder: Pharmion Ltd

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/thalidomidepharmion/thalidomidepharmion.htm>

Volibris (*ambrisentan*)

Date of orphan medicinal product designation: 11 April 2005

Date of issue of marketing authorisation: 21 April 2008

Designated orphan indication: treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension

Authorised therapeutic indication: treatment of pulmonary arterial hypertension

Therapeutic area: cardiovascular and respiratory

Marketing authorisation holder: Glaxo Group Limited – UK

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/volibris/volibris.htm>

Firazyr (*icatibant acetate*)

Date of orphan medicinal product designation: 17 February 2003

Date of issue of marketing authorisation: 11 July 2008

Designated orphan indication: treatment of angioedema

Authorised therapeutic indication: treatment of hereditary angioedema

Therapeutic area: other

Marketing authorisation holder: Jerini AG – Germany

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/firazyr/firazyr.htm>

Ceplene (*histamine dihydrochloride*)

Date of orphan medicinal product designation: 11 April 2005

Date of issue of marketing authorisation: 7 October 2008

Designated orphan indication: treatment of acute myeloid leukaemia

Authorised therapeutic indication: treatment of adult patients with acute myeloid leukaemia in first remission concomitantly treated with interleukin-2

Therapeutic area: oncology

Marketing authorisation holder: EpiCept GmbH

European public assessment report: (will be available on the EMEA website when finalised)

Vidaza (*azacitidine*)

Date of orphan medicinal product designation: 6 February 2002

Date of issue of marketing authorisation: pending Commission Decision

Designated orphan indication: treatment of myelodysplastic syndromes

Recommended therapeutic indication: treatment of myelodysplastic syndromes and acute myeloid leukaemia in adults who are not eligible for haematopoietic stem-cell transplantation

Therapeutic area: oncology

Marketing authorisation holder: Celgene Europe Ltd

European public assessment report: (will be available on the EMEA website when marketing authorisation is granted)

Kuvan (*sapropterin dihydrochloride*)

Date of orphan medicinal product designation: 26 August 2005

Date of issue of marketing authorisation: pending Commission Decision

Designated orphan indication: treatment of hyperphenylalaninemia

Recommended therapeutic indication: treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients of four years of age and over with phenylketonuria (PKU) who have been shown to be responsive to such treatment. Kuvan is also indicated for the treatment of HPA in adult and paediatric patients with tetrahydrobiopterin (BH4) deficiency who have been shown to be responsive to such treatment

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Merck KGaA

European public assessment report: (will be available on the EMEA website when marketing authorisation is granted)

2007

Diacomit (*stiripentol*)

Date of orphan medicinal product designation: 5 December 2001

Date of issue of marketing authorisation: 4 January 2007

Designated orphan indication: treatment of severe myoclonic epilepsy in infancy

Authorised therapeutic indication: use in conjunction with clobazam and valproate as adjunctive therapy of refractory generalised tonic-clonic seizures in patients with severe myoclonic epilepsy in infancy (SMEI, Dravet's syndrome) whose seizures are not adequately controlled with clobazam and valproate

Therapeutic area: nervous system

Marketing authorisation holder: BIOCDEX

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/diacomit/diacomit.htm>

Elaprase (*iduronate-2-sulfatase*)

Date of orphan medicinal product designation: 11 December 2001

Date of issue of marketing authorisation: 8 January 2007

Designated orphan indication: treatment of mucopolysaccharidosis, type II (Hunter syndrome)

Authorised therapeutic indication: long-term treatment of patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II)

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Shire Human Genetic Therapies AB

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/elaprase/elaprase.htm>

Inovelon (*rufinamide*)

Date of orphan medicinal product designation: 9 September 2004

Date of issue of marketing authorisation: 16 January 2007

Designated orphan indication: treatment of Lennox-Gastaut syndrome

Authorised therapeutic indication: as adjunctive therapy in the treatment of seizures associated with Lennox-Gastaut syndrome in patients aged four years and older

Therapeutic area: nervous system

Marketing authorisation holder: Esai Limited

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/inovelon/inovelon.htm>

Cystadane (*betaine anhydrous*)

Date of orphan medicinal product designation: 9 July 2001

Date of issue of marketing authorisation: 15 February 2007

Designated orphan indication: treatment of homocystinuria

Authorised therapeutic indication: adjunctive treatment of homocystinuria involving deficiencies or defects in:

- cystathionine beta-synthase (CBS)
- 5,10-methylene-tetrahydrofolate reductase (MTHFR)
- cobalamin cofactor metabolism (cbl)

Cystadane should be used as a supplement to other therapies, such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), folate and a specific diet

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Orphan Europe SARL

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/cystadane/cystadane.htm>

Revlimid [3-(4'-aminoisoindoline-1'-one)-1-piperidine-2,6-dione]

Date of orphan medicinal product designation: 12 December 2003

Date of issue of marketing authorisation: 14 June 2007

Designated orphan indication: treatment of multiple myeloma

Authorised therapeutic indication: treatment in combination with dexamethasone of multiple myeloma patients who have received at least one prior therapy

Therapeutic area: oncology

Marketing authorisation holder: Celgene Europe Limited – UK

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/revlimid/revlimid.htm>

Soliris (*eculizumab*)

Date of orphan medicinal product designation: 17 October 2003

Date of issue of marketing authorisation: 20 June 2007

Designated orphan indication: treatment of paroxysmal nocturnal haemoglobinuria

Authorised therapeutic indication: treatment of paroxysmal nocturnal haemoglobinuria

Therapeutic area: haematology

Marketing authorisation holder: Alexion Europe SAS

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/soliris/soliris.htm>

Siklos (*hydroxyurea*)

Date of orphan medicinal product designation: 9 July 2003

Date of issue of marketing authorisation: 29 June 2007

Designated orphan indication: treatment of sickle cell syndrome

Authorised therapeutic indication: prevention of recurrent painful vaso-occlusive crises including acute chest syndrome in paediatric and adult patients suffering from symptomatic Sickle Cell Syndrome.

Therapeutic area: haematology

Marketing authorisation holder: Addmedica SAS

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/siklos/siklos.htm>

Increlex (*mecasermin*)

Date of orphan medicinal product designation: 26 August 2005

Date of issue of marketing authorisation: 3 August 2007

Designated orphan indication: treatment of primary insulin-like growth factor-1 deficiency due to molecular or genetic defects

Authorised therapeutic indication: treatment of growth failure

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Tercica Europe Limited

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/increlex/increlex.htm>

Atriance (*nelarabine*)

Date of orphan medicinal product designation: 16 June 2005

Date of issue of marketing authorisation: 22 August 2007

Designated orphan indication: treatment of acute lymphoblastic leukaemia

Authorised therapeutic indication: treatment of patients with T-cell acute lymphoblastic leukaemia (T-ALL) and T-cell lymphoblastic lymphoma (T-LBL) whose disease has not responded to or has relapsed following treatment with at least two chemotherapy regimens

Therapeutic area: oncology

Marketing authorisation holder: Glaxo Group Limited

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/atricance/atricance.htm>

Gliolan (*5-aminolevulinic hydrochloride*)

Date of orphan medicinal product designation: 13 November 2002

Date of issue of marketing authorisation: 7 September 2007

Designated orphan indication: intra-operative photodynamic diagnosis of residual glioma

Authorised therapeutic indication: visualisation of malignant tissue during surgery for malignant glioma (WHO grade III and IV)

Therapeutic area: oncology

Marketing authorisation holder: Medac

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/gliolan/gliolan.htm>

Yondelis (*ecteinascidin 743*)

Date of orphan medicinal product designation: 30 May 2001

Date of issue of marketing authorisation: 17 September 2007

Designated orphan indication: treatment of soft tissue sarcoma

Authorised therapeutic indication: treatment of patients with advanced soft tissue sarcoma, after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents

Therapeutic area: oncology

Marketing authorisation holder: PharmaMar SA

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/yondelis/yondelis.htm>

Torisel (*temsirolimus*)

Date of orphan medicinal product designation: 6 April 2006

Date of issue of marketing authorisation: 19 November 2007

Designated orphan indication: treatment of renal cell carcinoma

Authorised therapeutic indication: first-line treatment of patients with advanced renal cell carcinoma who have at least three of six prognostic risk factors

Therapeutic area: oncology

Marketing authorisation holder: Wyeth Europa Limited – United Kingdom

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/torisel/torisel.htm>

Tasigna (*nilotinib*)

Date of orphan medicinal product designation: 22 May 2006

Date of issue of marketing authorisation: 19 November 2007

Designated orphan indication: treatment of chronic myeloid leukaemia

Authorised therapeutic indication: treatment of adults with chronic phase and accelerated phase Philadelphia chromosome positive chronic myelogenous leukaemia (CML) with resistance or intolerance to prior therapy, including imatinib

Therapeutic area: oncology

Marketing authorisation holder: Novartis Europharm Limited – UK

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/tasigna/tasigna.htm>

2006

Naglazyme (*N-acetylgalacto-samine 4-sulfatase*)

Date of orphan medicinal product designation: 14 February 2001

Date of issue of marketing authorisation: 24 January 2006

Designated orphan indication: treatment mucopolysaccharidosis VI (MPS VI) or Maroteaux-Lamy syndrome

Authorised therapeutic indication: long-term enzyme replacement therapy in patients with a confirmed diagnosis of mucopolysaccharidosis VI (MPS VI; N-acetylgalactosamine 4-sulfatase deficiency; Maroteaux-Lamy syndrome)

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: BioMarin Europe Ltd

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/naglazyme/naglazyme.htm>

Myozyme (*recombinant human acid alpha-glucosidase*)

Date of orphan medicinal product designation: 14 February 2001

Date of issue of marketing authorisation: 29 March 2006

Designated orphan indication: treatment of glycogen storage disease type II (Pompe's disease)

Authorised therapeutic indication: long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of Pompe's disease (acid alpha-glucosidase deficiency)

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Genzyme Europe BV

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/myozyme/myozyme.htm>

Evoltra [2-chloro-9-[2-deoxy-2-fluoro-β-D-arabinofura-nosyl]adenite]

Date of orphan medicinal product designation: 5 February 2002

Date of issue of marketing authorisation: 29 May 2006

Designated orphan indication: treatment of acute lymphoblastic leukaemia

Authorised therapeutic indication: treatment of acute lymphoblastic leukaemia (ALL) in paediatric patients who have relapsed or who are refractory after receiving at least two prior regimens and where there is no other treatment option anticipated to result in a durable response

Therapeutic area: oncology

Marketing authorisation holder: Genzyme Europe B.V.

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/evoltra/evoltra.htm>

Sutent [(Z)-N-[2-(Diethylamino)ethyl]-5-[(5-fluoro-2-oxo-1,2-dihydro-3H-indol-3-ylidene)methyl]-2,4-dimethyl-1H-pyrrole-3-carboxamide (S)-2-hydroxysuccinate]¹

Date of issue of marketing authorisation: 19 July 2006

Designated orphan indications: treatment of malignant gastrointestinal stromal tumours and treatment of renal cell carcinoma

Authorised therapeutic indication: treatment of unresectable and/or metastatic malignant gastrointestinal stromal tumour (GIST) after failure of imatinib mesylate treatment due to resistance or intolerance. Treatment of advanced and/or metastatic renal cell carcinoma (MRCC)

Therapeutic area: oncology

Marketing authorisation holder: Pfizer Limited

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/sutent/sutent.htm>

Nexavar (sorafenib tosylate)

Date of orphan medicinal product designation: 29 July 2004

Date of issue of marketing authorisation: 19 July 2006

Designated orphan indication: treatment of renal cell carcinoma

Authorised therapeutic indication: treatment of patients with advanced renal cell carcinoma who have failed prior interferon-alpha- or interleukin-2-based therapy or who are considered unsuitable for such therapy

Therapeutic area: oncology

¹ This product is no longer an orphan. It was originally an orphan designated on 10 March 2005. Upon request by the marketing authorisation holder, Sutent has now been removed from the Community Register of Orphan Medicinal Products.

Marketing authorisation holder: Bayer Healthcare AG – Germany

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/nexavar/nexavar.htm>

Savene (*dexrazoxane*)

Date of orphan medicinal product designation: 19 September 2001

Date of issue of marketing authorisation: 28 July 2006

Designated orphan indication: treatment of anthracycline extravasations

Authorised therapeutic indication: treatment of anthracycline extravasation

Therapeutic area: other

Marketing authorisation holder: TopoTarget A/S

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/savene/savene.htm>

Thelin (*sitaxentan sodium*)

Date of orphan medicinal product designation: 21 October 2004

Date of issue of marketing authorisation: 10 August 2006

Designated orphan indication: treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension

Authorised therapeutic indication: treatment of patients with pulmonary arterial hypertension classified as WHO functional class III, to improve exercise capacity

Therapeutic area: cardiovascular and respiratory

Marketing authorisation holder: Encysive (UK) Ltd

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/thelin/thelin.htm>

Exjade [*4-(3,5-bis(hydroxy-phenyl)-1,2,4 triazol-1-yl) benzoic acid*]

Date of orphan medicinal product designation: 13 March 2002

Date of issue of marketing authorisation: 28 August 2006

Designated orphan indication: treatment of chronic iron overload requiring chelation therapy

Authorised therapeutic indication: treatment of chronic iron overload due to frequent blood transfusions (≥ 7 ml/kg/month of packed red blood cells) in patients with beta thalassaemia major aged six years and older. Treatment of chronic iron overload due to blood transfusions when deferoxamine therapy is contraindicated or inadequate in the following patient groups:

- patients with other anaemias
- patients aged two to five years
- patients with beta thalassaemia major with iron overload due to infrequent blood transfusions (< 7 ml/kg/month of packed red blood cells)

Therapeutic area: haematology

Marketing authorisation holder: Novartis Europharm Limited

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/exjade/exjade.htm>

Sprycel (*dasatinib*)

Date of orphan medicinal product designation: 23 December 2005

Date of issue of marketing authorisation: 20 November 2006

Designated orphan indications: treatment of acute lymphoblastic leukaemia and treatment of chronic myeloid leukaemia

Authorised therapeutic indication: treatment of adults with Philadelphia chromosome positive (Ph+) acute lymphoblastic leukaemia (ALL) and lymphoid blast CML with resistance or intolerance to prior therapy. Treatment of adults with chronic, accelerated or blast phase chronic myeloid leukaemia (CML) with resistance or intolerance to prior therapy including imatinib mesylate

Therapeutic area: oncology

Marketing authorisation holder: Bristol-Myers Squibb Pharma EEIG

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/sprycel/sprycel.htm>

2005

Prialt (*ziconotide*)

Date of orphan medicinal product designation: 9 July 2001

Date of issue of marketing authorisation: 21 February 2005

Designated orphan indication: treatment of chronic pain requiring intraspinal analgesia

Authorised therapeutic indication: treatment of severe, chronic pain in patients who require intrathecal (IT) analgesia

Therapeutic area: nervous system

Marketing authorisation holder: Eisai Limited

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/prialt/prialt.htm>

Orfadin (*nitisinone*)

Date of orphan medicinal product designation: 29 December 2000

Date of issue of marketing authorisation: 21 February 2005

Designated orphan indication: treatment of tyrosinaemia type I

Authorised therapeutic indication: treatment of patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Swedish Orphan International AB

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/orfadin/orfadin.htm>

Xyrem (*sodium oxybate*)

Date of orphan medicinal product designation: 3 February 2003

Date of issue of marketing authorisation: 13 October 2005

Designated orphan indication: treatment of narcolepsy

Authorised therapeutic indication: treatment of narcolepsy with cataplexy in adult patients

Therapeutic area: nervous system

Marketing authorisation holder: UCB Pharma Ltd

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/xyrem/xyrem.htm>

Revatio (*sildenafil citrate*)

Date of orphan medicinal product designation: 12 December 2003

Date of issue of marketing authorisation: 28 October 2005

Designated orphan indication: treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension

Authorised therapeutic indication: treatment of patients with pulmonary arterial hypertension classified as WHO functional class III, to improve exercise capacity

Therapeutic area: cardiovascular and respiratory

Marketing authorisation holder: Pfizer Limited

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/revatio/revatio.htm>

2004

Photobarr [*porfimer sodium (for use with photodynamic therapy)*]

Date of orphan medicinal product designation: 6 March 2002

Date of issue of marketing authorisation: 25 March 2004

Designated orphan indication: treatment of high-grade dysplasia in Barrett's Oesophagus

Authorised therapeutic indication: photodynamic therapy (PDT) for ablation of high-grade dysplasia (HGD) in patients with Barrett's Oesophagus (BO)

Therapeutic area: oncology

Marketing authorisation holder: Axcan Pharma International BV

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/photoBarr/photoBarr.htm>

Litak (*cladribine*)

Date of orphan medicinal product designation: 19 September 2001

Date of issue of marketing authorisation: 14 April 2004

Designated orphan indication: treatment of indolent non-Hodgkin's lymphoma

Authorised therapeutic indication: treatment of hairy cell leukaemia

Therapeutic area: oncology

Marketing authorisation holder: Lipomed GmbH

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/litak/litak.htm>

Lysodren (*mitotane*)

Date of orphan medicinal product designation: 12 June 2002

Date of issue of marketing authorisation: 28 April 2004

Designated orphan indication: treatment of adrenal cortical carcinoma

Authorised therapeutic indication: symptomatic treatment of advanced (unresectable, metastatic or relapsed) adrenal cortical carcinoma

Therapeutic area: oncology

Marketing authorisation holder: Laboratoire HRA Pharma

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/lysodren/lysodren.htm>

Pedea (*ibuprofen*)

Date of orphan medicinal product designation: 14 February 2001

Date of issue of marketing authorisation: 29 July 2004

Designated orphan indication: treatment of patent ductus arteriosus

Authorised therapeutic indication: treatment of a haemodynamically significant patent ductus arteriosus in preterm newborn infants less than 34 weeks of gestational age

Therapeutic area: cardiovascular and respiratory

Marketing authorisation holder: Orphan Europe SARL

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/pedea/pedea.htm>

Wilzin (*zinc acetate dihydrate*)

Date of orphan medicinal product designation: 31 July 2001

Date of issue of marketing authorisation: 13 October 2004

Designated orphan indication: treatment of Wilson's disease

Authorised therapeutic indication: treatment of Wilson's disease

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Orphan Europe SARL

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/wilzin/wilzin.htm>

Xagrid (*anagrelide hydrochloride*)

Date of orphan medicinal product designation: 29 September 2000

Date of issue of marketing authorisation: 16 November 2004

Designated orphan indication: treatment of essential thrombocythaemia

Authorised therapeutic indication: reduction of elevated platelet counts in at-risk essential thrombocythaemia patients who are intolerant to their current therapy or whose elevated platelet counts are not reduced to an acceptable level by their current therapy

Therapeutic area: haematology

Marketing authorisation holder: Shire Pharmaceuticals Contracts Ltd

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/xagrid/Xagrid.htm>

2003

Carbaglu (*N-carbamyl-L-glutamic acid*)

Date of orphan medicinal product designation: 28 October 2000

Date of issue of marketing authorisation: 24 January 2003

Designated orphan indication: treatment of N-acetylglutamate synthetase (NAGS) deficiency

Authorised therapeutic indication: treatment of hyperammonaemia due to N-acetylglutamate synthetase deficiency

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Orphan Europe SARL

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/carbaglu/carbaglu.htm>

Aldurazyme (*laronidase*)

Date of orphan medicinal product designation: 14 February 2001

Date of issue of marketing authorisation: 10 June 2003

Designated orphan indication: treatment of mucopolysaccharidosis type I

Authorised therapeutic indication: long-term enzyme replacement therapy in patients with a confirmed diagnosis of mucopolysaccharidosis I (MPS I; a [alpha]-L-iduronidase deficiency) to treat the non-neurological manifestations of the disease

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Genzyme Europe BV

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/aldurazyme/aldurazyme.htm>

Busilvex (*busulfan*)

Date of orphan medicinal product designation: 29 December 2000

Date of issue of marketing authorisation: 9 July 2003

Designated orphan indication: conditioning treatment prior to haematopoietic progenitor cell transplantation

Authorised therapeutic indication: followed by cyclophosphamide (BuCy2) is indicated as conditioning treatment prior to conventional haematopoietic progenitor cell transplantation (HPCT) in adult patients when the combination is considered the best available option. Followed by cyclophosphamide (BuCy4) or melphalan (BuMel) is indicated as conditioning treatment prior to conventional haematopoietic progenitor cell transplantation in paediatric patients

Therapeutic area: haematology

Marketing authorisation holder: Pierre Fabre Medicament

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/busilvex/busilvex.htm>

Ventavis (*iloprost*)

Date of orphan medicinal product designation: 29 December 2000

Date of issue of marketing authorisation: 16 September 2003

Designated orphan indication: 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 and chronic thromboembolic pulmonary hypertension

Authorised therapeutic indication: treatment of patients with primary pulmonary hypertension, classified as NYHA functional class III, to improve exercise capacity and symptoms

Therapeutic area: cardiovascular and respiratory

Marketing authorisation holder: Schering AG

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/ventavis/ventavis.htm>

Onsenal (*celecoxib*)

Date of orphan medicinal product designation: 20 November 2001

Date of issue of marketing authorisation: 17 October 2003

Designated orphan indication: treatment of familial adenomatous polyposis (FAP)

Authorised therapeutic indication: reduction of the number of adenomatous intestinal polyps in familial adenomatous polyposis (FAP), as an adjunct to surgery and further endoscopic surveillance

Therapeutic area: gastro-enterology

Marketing authorisation holder: Pharmacia-Pfizer EEIG

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/onsenal/onsenal.htm>

2002

Trisenox (*arsenic trioxide*)

Date of orphan medicinal product designation: 18 October 2000

Date of issue of marketing authorisation: 5 March 2002

Designated orphan indication: treatment of acute promyelocytic leukaemia (APL)

Authorised therapeutic indication: induction of remission and consolidation in adult patients with relapsed/refractory acute promyelocytic leukaemia (APL), characterised by the presence of the t(15;17) translocation and/or the presence of the pro-myelocytic leukaemia/retinoic-acid-receptor-alpha (PML/RAR-alpha) gene. Previous treatment should have included a retinoid and chemotherapy

Therapeutic area: oncology

Marketing authorisation holder: Cephalon Europe

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/trisenox/trisenox.htm>

Tracleer (*bosentan*)

Date of orphan medicinal product designation: 16 February 2001

Date of issue of marketing authorisation: 15 May 2002

Designated orphan indication: treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension

Authorised therapeutic indication: treatment of pulmonary arterial hypertension (PAH) to improve exercise capacity and symptoms in patients with grade III functional status. Efficacy has been shown in: primary (idiopathic and familial) PAH; PAH secondary to scleroderma without significant interstitial pulmonary disease and PAH associated with congenital systemic-to-pulmonary shunts and Eisenmenger's physiology. Tracleer is also indicated to reduce the number of new digital ulcers in patients with systemic sclerosis and ongoing digital ulcer disease.

Therapeutic area: cardiovascular and respiratory

Marketing authorisation holder: Actelion Registration Limited

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/tracleer/tracleer.htm>

Somavert (*pegvisomant*)

Date of orphan medicinal product designation: 14 February 2001

Date of issue of marketing authorisation: 13 November 2002

Designated orphan indication: treatment of acromegaly

Authorised therapeutic indication: treatment of patients with acromegaly who have had an inadequate response to surgery and/or radiation therapy and in whom an appropriate medical treatment with somatostatin analogues did not normalise IGF-I concentrations or was not tolerated

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Pfizer Limited

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/somavert/somavert.htm>

Zavesca [*miglustat 1,5-(Butylimino)-1,5-dideoxy, D-glucitol (OGT 918), A*]

Date of orphan medicinal product designation: 18 October 2000

Date of issue of marketing authorisation: 20 November 2002

Designated orphan indication: treatment of Gaucher disease

Authorised therapeutic indication: oral treatment of mild to moderate type 1 Gaucher disease; may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Actelion Registration Ltd

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/zavesca/zavesca.htm>

2001

Fabrazyme [*agalsidase beta (recombinant human alfa galactosidase)*]

Date of orphan medicinal product designation: 8 August 2000

Date of issue of marketing authorisation: 3 August 2001

Designated orphan indication: treatment of Fabry disease

Authorised therapeutic indication: long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (a-galactosidase A deficiency)

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Genzyme Europe B.V.

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/fabrazyme/fabrazyme.htm>

Replagal (*α-galactosidase A*)

Date of orphan medicinal product designation: 8 August 2000

Date of issue of marketing authorisation: 3 August 2001

Designated orphan indication: treatment of Fabry disease

Authorised therapeutic indication: long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (a-galactosidase A deficiency)

Therapeutic area: endocrinology and metabolism

Marketing authorisation holder: Shire Human Genetics Therapies AB

European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/replagal/replagal.htm>

Glivec (*imatinib mesylate*)

Date of orphan medicinal product designation: 14 February 2001

Date of issue of marketing authorisation: 7 November 2001

Designated orphan indications: treatment of:

- chronic myeloid leukaemia
- malignant gastrointestinal stromal tumours
- dermatofibrosarcoma protuberans
- acute lymphoblastic leukaemia
- myelodysplastic/myeloproliferative diseases
- chronic eosinophilic leukaemia
- hypereosinophilic syndrome

Authorised therapeutic indication: treatment of adult and paediatric patients with newly diagnosed Philadelphia chromosome (bcr-abl) positive (Ph+) chronic myeloid leukaemia (CML) for whom bone marrow transplantation is not considered as the first line of treatment. It is also indicated for the treatment of adult and paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy, or in accelerated phase or blast crisis

Glivec has also received a marketing authorisation as an orphan drug for the following indications:

- treatment of adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumours (GIST)
- treatment of adult patients with unresectable dermafibrosarcoma protuberans (DFSP) and adult patients with recurrent and/or metastatic DFSP who are not eligible for surgery
- treatment of adults patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) integrated with chemotherapy
- treatment of adult patients with myelodysplastic/myeloproliferative diseases (MDS/MPD) associated with PDGFR gene rearrangements
- treatment of adult patients with hypereosinophilic syndrome (HES) and chronic eosinophilic leukaemia (CEL)

Therapeutic area: oncology, haematology

Marketing authorisation holder: Novartis Europharm Limited

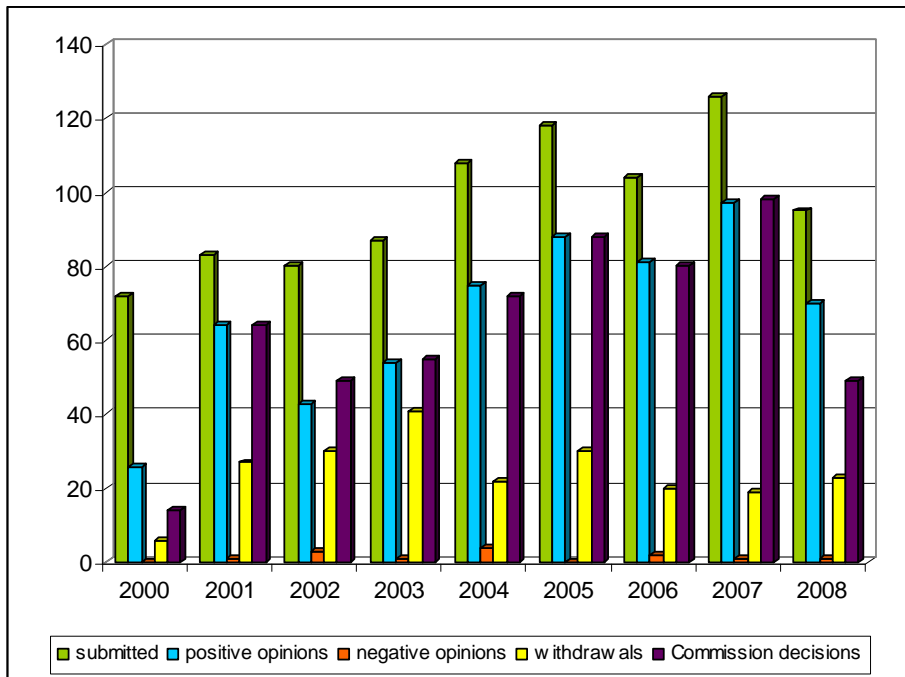
European public assessment report:

<http://www.emea.europa.eu/humandocs/Humans/EPAR/glivec/glivec.htm>

Orphan-designated medicines in numbers

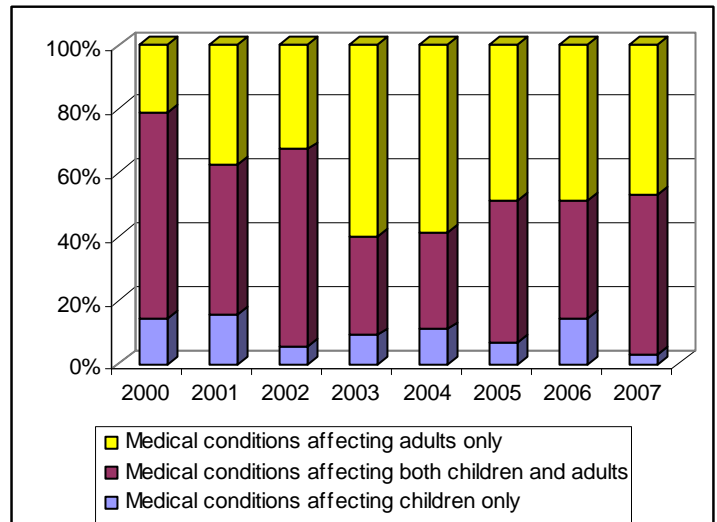
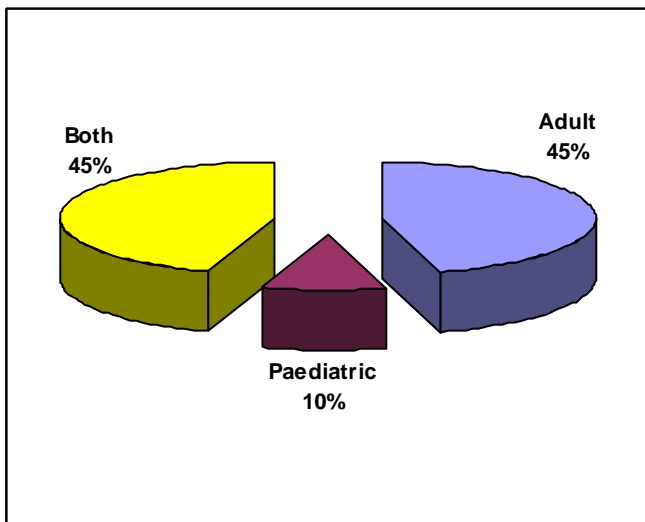
Status of orphan applications

- A total of 873 applications have been submitted to date for the designation of orphan medicines.
- The Committee for Orphan Medicinal Products (COMP) has adopted 598 positive opinions on orphan designation.
- A total of 218 applications have been withdrawn and 13 received a negative COMP opinion.
- A total of 569 medicines have been awarded orphan-designation status by the European Commission.



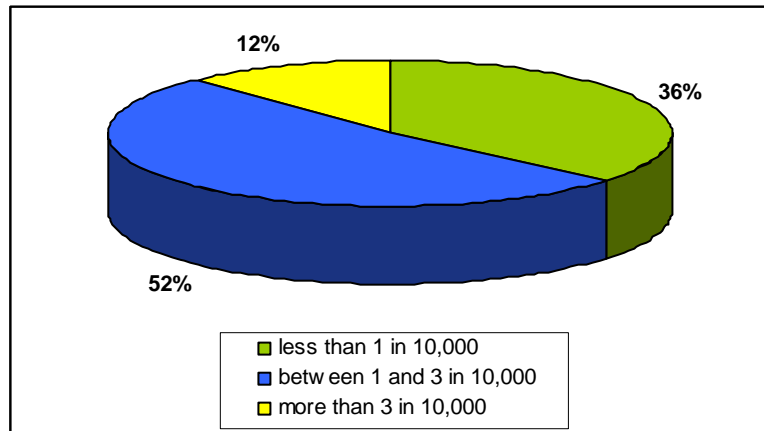
Use by population

- More than half of the medicines that have received a positive opinion on orphan designation are for conditions that affect children.



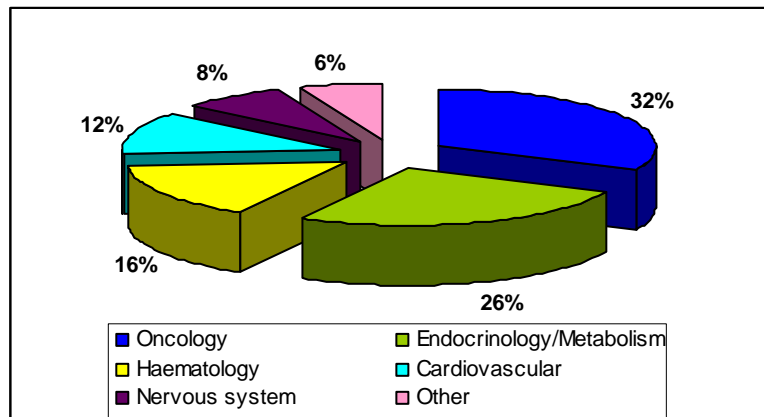
Prevalence of orphan-designated conditions

- The majority of patients needing orphan medicines suffer from diseases that affect between one and three in 10,000 people.



Distribution of orphan marketing authorisation applications by therapeutic area

- Orphan-designated medicines to treat cancer have received more marketing authorisations from the European Commission than those for any other therapeutic area.



List of authorised orphan-designated medicines per therapeutic area ²

(Listed by year in which the marketing authorisation was granted.)

Oncology

2008	Ceplene
2007	Atriance Gliolan Yondelis Torisel Tasigna
2006	Evoltra Sutent Nexavar Sprycel
2004	Photobarr Litak Lysodren
2003	Onsenal
2002	Trisenox
2001	Glivec

Endocrinology/metabolism

2007	Elaprase Cystadane Increlex
2006	Naglazyme Myozyme
2005	Orfadin
2004	Wilzin
2003	Carbaglu Aldurazyme
2002	Somavert Zavesca
2001	Fabrazyme Replagal

Haematology

2008	Thalidomide
2007	Revlimid Soliris Siklos
2006	Exjade
2004	Xagrid
2003	Busilvex
2001	Glivec

Cardiovascular & respiratory

2008	Volibris
2006	Thelin
2005	Revatio
2004	Pedea
2003	Ventavis
2002	Tracleer

Nervous system

2007	Diacomit Inovelon
2005	Prialt Xyrem

Other

2008	Firazyr
2006	Savene
2002	Tracleer

² Medicines can be classified in more than one therapeutic area.