| No | Orphan medicinal product | Therapeutic indication | Marketing authorisation holder | Related orphan designations |
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| 1 | ADCETRIS | ADCETRIS is indicated for the treatment of adult patients with relapsed or refractory CD30+ Hodgkin lymphoma (HL):<br>1.following autologous stem cell transplant (ASCT) or<br>2.following at least two prior therapies when ASCT or multi-agent chemotherapy is not a treatment option.<br><br>ADCETRIS is indicated for the treatment of adult patients with CD30+ HL at increased risk of relapse or progression following ASCT (see section 5.1).<br><br>ADCETRIS is indicated for the treatment of adult patients with relapsed or refractory systemic anaplastic large cell lymphoma (sALCL).<br><br>ADCETRIS is indicated for the treatment of adult patients with CD30+ cutaneous T-cell lymphoma (CTCL) after at least 1 prior systemic therapy. | Takeda Pharma A/S | EU/3/08/595 (active)<br>EU/3/08/596 (active)<br>EU/3/11/939 (active) |
| 2 | Adempas | Chronic thromboembolic pulmonary hypertension (CTEPH)<br><br>Adempas is indicated for the treatment of adult patients with WHO Functional Class (FC) II to III with<br>•inoperable CTEPH,<br>•persistent or recurrent CTEPH after surgical treatment, to improve exercise capacity.<br><br>Pulmonary arterial hypertension (PAH)<br><br>Adempas, as monotherapy or in combination with endothelin receptor antagonists, is indicated for the treatment of adult patients with pulmonary arterial hypertension (PAH) with WHO Functional Class (FC) II to III to improve exercise capacity.<br>Efficacy has been shown in a PAH population including aetiologies of idiopathic or heritable PAH or PAH associated with connective tissue disease. | Bayer AG | EU/3/07/518 (active) |
| 3 | Afinitor | Hormone receptor positive advanced breast cancer<br>Afinitor is indicated for the treatment of hormone receptor positive, HER2/neu negative advanced breast cancer, in combination with exemestane, in postmenopausal women without symptomatic visceral disease after recurrence or progression following a non steroidal aromatase inhibitor.<br><br>Neuroendocrine tumours of pancreatic origin<br>Afinitor is indicated for the treatment of unresectable or metastatic, well or moderately differentiated neuroendocrine tumours of pancreatic origin in adults with progressive disease.<br><br>Neuroendocrine tumours of gastrointestinal or lung origin<br>Afinitor is indicated for the treatment of unresectable or metastatic, well-differentiated (Grade 1 or Grade 2) non-functional neuroendocrine tumours of gastrointestinal or lung origin in adults with progressive disease (see sections 4.4 and 5.1).<br><br>Renal cell carcinoma<br>Afinitor is indicated for the treatment of patients with advanced renal cell carcinoma, whose disease has progressed on or after treatment with VEGF targeted therapy. | Novartis Europharm Limited | EU/3/07/449 (removed) |
| 4 | Aldurazyme | Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis I to treat the non-neurological manifestations of the disease | Genzyme Europe B.V. | EU/3/01/022 (removed) |

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| 5 | Alofisel | Alofisel is indicated for the treatment of complex perianal fistulas in adult patients with non-active/mildly active luminal Crohn's disease, when fistulas have shown an inadequate response to at least one conventional or biologic therapy. Alofisel should be used after conditioning of fistula. | Takeda Pharma A/S | EU/3/09/667 (active) |
| 6 | ALPROLIX | Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency).<br><br>ALPROLIX can be used for all age groups. | Swedish Orphan Biovitrum AB (publ) | EU/3/07/453 (active) |
| 7 | AMGLIDIA | AMGLIDIA is indicated for the treatment of neonatal diabetes mellitus, for use in newborns, infants and children.<br>Sulphonylureas like AMGLIDIA have been shown to be effective in patients with mutations in the genes coding for the β-cell ATP-sensitive potassium channel and chromosome 6q24-related transient neonatal diabetes mellitus. | AMMTeK | EU/3/15/1589 (active) |
| 8 | Arzerra | Previously untreated chronic lymphocytic leukaemia (CLL):<br>Arzerra in combination with chlorambucil or bendamustine is indicated for the treatment of patients with CLL who have not received prior therapy and who are not eligible for fludarabine-based therapy.<br><br>Relapsed CLL:<br>Arzerra is indicated in combination with fludarabine and cyclophosphamide for the treatment of adult patients with relapsed CLL.<br><br>Refractory CLL:<br>Arzerra is indicated for the treatment of CLL in patients who are refractory to fludarabine and alemtuzumab. | Novartis Europharm Limited | EU/3/08/581 (active) |
| 9 | Atriance | Nelarabine is indicated for the 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.<br>Due to the small patient populations in these disease settings, the information to support these indications is based on limited data. | Novartis Europharm Limited | EU/3/05/293 (removed) |
| 10 | Bavencio | Bavencio is indicated as monotherapy for the treatment of adult patients with metastatic Merkel cell carcinoma (MCC). | Merck Europe B.V. | EU/3/15/1590 (active) |
| 11 | Besponsa | BESPONSA is indicated as monotherapy for the treatment of adults with relapsed or refractory CD22-positive B cell precursor acute lymphoblastic leukaemia (ALL). Adult patients with Philadelphia chromosome positive (Ph+) relapsed or refractory B cell precursor ALL should have failed treatment with at least 1 tyrosine kinase inhibitor (TKI). | Pfizer Europe MA EEIG | EU/3/13/1127 (active) |
| 12 | Blincyto | BLINCYTO is indicated as monotherapy for the treatment of adults with Philadelphia chromosome negative CD19 positive relapsed or refractory B precursor acute lymphoblastic leukaemia (ALL).<br><br>BLINCYTO is indicated as monotherapy for the treatment of paediatric patients aged 1 year or older with Philadelphia chromosome negative CD19 positive B cell precursor ALL which is refractory or in relapse after receiving at least two prior therapies or in relapse after receiving prior allogeneic hematopoietic stem cell transplantation. | Amgen Europe B.V. | EU/3/09/650 (active) |
| 13 | Bosulif | Bosulif is indicated for the treatment of adult patients with:<br>•newly diagnosed chronic phase (CP) Philadelphia chromosome-positive chronic myelogenous leukaemia (Ph+ CML).<br>•CP, accelerated phase (AP), and blast phase (BP) Ph+ CML previously treated with one or more tyrosine kinase inhibitor(s) [TKI(s)] and for whom imatinib, nilotinib and dasatinib are not considered appropriate treatment options. | Pfizer Europe MA EEIG | EU/3/10/762 (removed) |

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| 14 | Brineura | Brineura is indicated for the treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease, also known as tripeptidyl peptidase 1 (TPP1) deficiency. | BioMarin International Limited | EU/3/13/1118 (active) |
| 15 | Bronchitol | Bronchitol is indicated for the treatment of cystic fibrosis (CF) in adults aged 18 years and above as an add-on therapy to best standard of care. | Pharmaxis Pharmaceuticals Limited | EU/3/05/325 (active) |
| 16 | Busilvex | Busilvex 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.<br>Busilvex following fludarabine (FB) is indicated as conditioning treatment prior to haematopoietic progenitor cell transplantation (HPCT) in adult patients who are candidates for a reduced-intensity conditioning (RIC) regimen.<br>Busilvex followed by cyclophosphamide (BuCy4) or melphalan (BuMel) is indicated as conditioning treatment prior to conventional haematopoietic progenitor cell transplantation in paediatric patients. | Pierre Fabre Médicament | EU/3/00/011 (removed) |
| 17 | Cablivi | Cablivi is indicated for the treatment of adults experiencing an episode of acquired thrombotic thrombocytopenic purpura (aTTP), in conjunction with plasma exchange and immunosuppression. | Ablynx N.V. | EU/3/09/629 (active) |
| 18 | Carbaglu | Carbaglu is indicated in treatment of<br>- hyperammonaemia due to N-acetylglutamate synthase primary deficiency.<br>- hyperammonaemia due to isovaleric acidaemia.<br>- hyperammonaemia due to methymalonic acidaemia.<br>- hyperammonaemia due to propionic acidaemia. | Orphan Europe S.A.R.L. | EU/3/00/007 (removed)<br>EU/3/08/575 (active)<br>EU/3/08/576 (active)<br>EU/3/08/577 (active) |
| 19 | Cayston | Cayston is indicated for the suppressive therapy of chronic pulmonary infections due to *Pseudomonas aeruginosa* in patients with cystic fibrosis (CF) aged 6 years and older. Consideration should be given to official guidance on the appropriate use of antibacterial agents. | Gilead Sciences Ireland UC | EU/3/04/204 (active) |
| 20 | Ceplene | Ceplene maintenance therapy is Indicated for adult patients with acute myeloid leukaemia (AML) in first remission concomitantly treated with interleukin-2 (IL-2). The efficacy of Ceplene has not been fully demonstrated in patients older than age 60. | Noventia Pharma Srl | EU/3/05/272 (active) |
| 21 | Cerdelga | Cerdelga is indicated for the long-term treatment of adult patients with Gaucher disease type 1 (GD1), who are CYP2D6 poor metabolisers (PMs), intermediate metabolisers (IMs) or extensive metabolisers (EMs). | Genzyme Europe B.V. | EU/3/07/514 (active) |
| 22 | Chenodeoxycholic acid Leadiant | Chenodeoxycholic acid is indicated for the treatment of inborn errors of primary bile acid synthesis due to sterol 27 hydroxylase deficiency (presenting as cerebrotendinous xanthomatosis (CTX)) in infants, children and adolescents aged 1 month to 18 years and adults. | Leadiant GmbH | EU/3/14/1406 (active) |
| 23 | Coagadex | Coagadex is indicated for the treatment and prophylaxis of bleeding episodes and for perioperative management in patients with hereditary factor X deficiency.<br><br>Coagadex is indicated in all age groups. | Bio Products Laboratory Ltd | EU/3/07/471 (active) |
| 24 | Cometriq | Cometriq is indicated for the treatment of adult patients with progressive, unresectable locally advanced or metastatic medullary thyroid carcinoma.<br>For patients in whom Rearranged during Transfection (RET) mutation status is not known or is negative, a possible lower benefit should be taken into account before individual treatment decision | Ipsen Pharma | EU/3/08/610 (active) |
| 25 | Cresemba | Treatment of<br>- invasive aspergillosis<br>- mucormycosis in patients for whom amphotericin B is inappropriate<br>Consideration should be given to official guidance on the appropriate use of antifungal agents. | Basilea Medical Ltd | EU/3/14/1276 (active)<br>EU/3/14/1284 (active) |
| 26 | CRYSVITA | CRYSVITA is indicated for the treatment of X-linked hypophosphataemia with radiographic evidence of bone disease in children 1 year of age and older and adolescents with growing skeletons. | Kyowa Kirin Holdings B.V. | EU/3/14/1351 (active) |

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| 27 | Cyramza | Cyramza in combination with paclitaxel is indicated for the treatment of adult patients with advanced gastric cancer or gastro-oesophageal junction adenocarcinoma with disease progression after prior platinum and fluoropyrimidine chemotherapy.<br><br>Cyramza monotherapy is indicated for the treatment of adult patients with advanced gastric cancer or gastro-oesophageal junction adenocarcinoma with disease progression after prior platinum or fluoropyrimidine chemotherapy, for whom treatment in combination with paclitaxel is not appropriate.<br><br>Cyramza, in combination with FOLFIRI (irinotecan, folinic acid, and 5 fluorouracil), is indicated for the treatment of adult patients with metastatic colorectal cancer (mCRC) with disease progression on or after prior therapy with bevacizumab, oxaliplatin and a fluoropyrimidine.<br><br>Cyramza in combination with docetaxel is indicated for the treatment of adult patients with locally advanced or metastatic non-small cell lung cancer with disease progression after platinum-based chemotherapy. | Eli Lilly Nederland B.V. | EU/3/12/1004 (removed) |
| 28 | Cystadane | Adjunctive treatment of homocystinuria, involving deficiencies or defects in:<br>- cystathionine beta-synthase (CBS),<br>- 5,10-methylene-tetrahydrofolate reductase (MTHFR),<br>- cobalamin cofactor metabolism (cbl).<br>Cystadane should be used as supplement to other therapies such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), folate and a specific diet. | Orphan Europe S.A.R.L. | EU/3/01/045 (removed) |
| 29 | Cystadrops | Cystadrops is indicated for the treatment of corneal cystine crystal deposits in adults and children from 2 years of age with cystinosis. | Orphan Europe S.A.R.L. | EU/3/08/578 (active) |
| 30 | Dacogen | Dacogen is indicated for the treatment of adult patients with newly diagnosed de novo or secondary acute myeloid leukaemia (AML), according to the World Health Organisation (WHO) classification, who are not candidates for standard induction chemotherapy. | Janssen-Cilag International NV | EU/3/06/370 (active) |
| 31 | Darzalex | DARZALEX is indicated:<br>•in combination with bortezomib, melphalan and prednisone for the treatment of adult patients with newly diagnosed multiple myeloma who are ineligible for autologous stem cell transplant.<br>•as monotherapy for the treatment of adult patients with relapsed and refractory multiple myeloma, whose prior therapy included a proteasome inhibitor and an immunomodulatory agent and who have demonstrated disease progression on the last therapy.<br>•in combination with lenalidomide and dexamethasone, or bortezomib and dexamethasone, for the treatment of adult patients with multiple myeloma who have received at least one prior therapy. | Janssen-Cilag International NV | EU/3/13/1153 (active) |
| 32 | Defitelio | Treatment of severe hepatic veno-occlusive disease (VOD) also known as sinusoidal obstructive syndrome (SOS) in haematopoietic stem-cell transplantation (HSCT) therapy.<br>It is indicated in adults and in adolescents, children and infants over 1 month of age. | Gentium S.r.I. | EU/3/04/212 (active) |
| 33 | Deltyba | Deltyba is indicated for use as part of an appropriate combination regimen for pulmonary multi-drug resistant tuberculosis (MDR-TB) in adult patients when an effective treatment regimen cannot otherwise be composed for reasons of resistance or tolerability.<br>Consideration should be given to official guidance on the appropriate use of antibacterial agents. | Otsuka Novel Products GmbH | EU/3/07/524 (active) |
| 34 | Diacomit | Diacomit is indicated for use in conjunction with clobazam and valproate as adjunctive therapy of refractory generalized 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. | Biocodex | EU/3/01/071 (removed) |

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| 35 | Elaprase | Elaprase is indicated for the long-term treatment of patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II). | Shire Human Genetic Therapies AB | EU/3/01/078 (removed) |
| 36 | Esbriet | Esbriet is indicated in adults for the treatment of mild to moderate Idiopathic Pulmonary Fibrosis (IPF). | Roche Registration GmbH | EU/3/04/241 (active) |
| 37 | Evoltra | Treatment of acute lymphoblastic leukaemia (ALL) in paediatric patients who have relapsed or are refractory after receiving at least two prior regimens and where there is no other treatment option anticipated to result in a durable response. Safety and efficacy have been assessed in studies of patients ≤ 21 years old at initial diagnosis | Genzyme Europe B.V. | EU/3/01/082 (removed) |
| 38 | Exjade | 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 6 years and older.<br>Treatment of chronic iron overload due to blood transfusions when deferoxamine therapy is contraindicated or inadequate in the following patient groups:<br>- in paediatric patients with beta thalassaemia major with iron overload due to frequent blood transfusions (>=7 ml/kg/month of packed red blood cells) aged 2 to 5 years,<br>- in adult and paediatric patients with beta thalassaemia major with iron overload due to infrequent blood transfusions (<7 ml/kg/month of packed red blood cells) aged 2 years and older,<br>- in adult and paediatric patients with other anaemias aged 2 years and older.<br>Treatment of chronic iron overload requiring chelation therapy when deferoxamine therapy is contraindicated or inadequate in patients with non-transfusion-dependent thalassaemia syndromes aged 10 years and older. | Novartis Europharm Limited | EU/3/02/092 (removed) |
| 39 | Fabrazyme | Fabrazyme is indicated for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency).<br>Fabrazyme is indicated in adults, children and adolescents aged 8 years and older. | Genzyme Europe B.V. | EU/3/00/003 (removed) |
| 40 | Farydak | Farydak, in combination with bortezomib and dexamethasone, is indicated for the treatment of adult patients with relapsed and/or refractory multiple myeloma who have received at least two prior regimens including bortezomib and an immunomodulatory agent. | Novartis Europharm Limited | EU/3/12/1063 (active) |
| 41 | Firazyr | Firazyr is indicated for symptomatic treatment of acute attacks of hereditary angioedema (HAE) in adults, adolescents and children aged 2 years and older, with C1-esterase-inhibitor deficiency. | Shire Pharmaceuticals Ireland Limited | EU/3/03/133 (active) |
| 42 | Firdapse | Symptomatic treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults. | BioMarin International Limited | EU/3/02/124 (active) |
| 43 | Galafold | Galafold is indicated for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation (see the tables in section 5.1). | Amicus Therapeutics UK Ltd | EU/3/06/368 (active) |
| 44 | Gazyvaro | Chronic Lymphocytic Leukaemia (CLL)<br><br>Gazyvaro in combination with chlorambucil is indicated for the treatment of adult patients with previously untreated chronic lymphocytic leukaemia (CLL) and with comorbidities making them unsuitable for full-dose fludarabine based therapy (see section 5.1).<br><br>Follicular Lymphoma (FL)<br><br>Gazyvaro in combination with bendamustine followed by Gazyvaro maintenance is indicated for the treatment of patients with follicular lymphoma (FL) who did not respond or who progressed during or up to 6 months after treatment with rituximab or a rituximab-containing regimen. | Roche Registration GmbH | EU/3/12/1054 (active)<br>EU/3/15/1504 (active) |
| 45 | Gliolan | Gliolan is indicated in adult patients for visualisation of malignant tissue during surgery for malignant glioma (WHO grade III and IV). | medac Gesellschaft für klinische Spezialpräparate mbH | EU/3/02/121 (removed) |

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| 46 | Glivec | Glivec is indicated for the treatment of<br>- 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.<br>- adult and paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy, or in accelerated phase or blast crisis.<br>- adult patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) integrated with chemotherapy.<br>- adult patients with relapsed or refractory Ph+ ALL as monotherapy.<br>- adult patients with myelodysplastic/myeloproliferative diseases (MDS/MPD) associated with platelet-derived growth factor receptor (PDGFR) gene re-arrangements.<br>- adult patients with advanced hypereosinophilic syndrome (HES) and/or chronic eosinophilic leukaemia (CEL) with FIP1L1-PDGFRa rearrangement.<br>The effect of Glivec on the outcome of bone marrow transplantation has not been determined.<br>Glivec is indicated for<br>- the treatment of adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumours (GIST).<br>- the adjuvant treatment of adult patients who are at significant risk of relapse following resection of Kit (CD117)-positive GIST. Patients who have a low or very low risk of recurrence should not receive adjuvant treatment.<br>- the treatment of adult patients with unresectable dermatofibrosarcoma protuberans (DFSP) and adult patients with recurrent and/or metastatic DFSP who are not eligible for surgery.<br>In adult and paediatric patients, the effectiveness of Glivec is based on overall haematological and cytogenetic response rates and progression-free survival in CML, on haematological and cytogenetic response rates in Ph+ ALL, MDS/MPD, on haematological response rates in HES/CEL and on objective response rates in adult patients with unresectable and/or metastatic GIST and DFSP and on recurrence-free survival in adjuvant GIST. The experience with Glivec in patients with MDS/MPD associated with PDGFR gene re-arrangements is very limited (see section 5.1). Except in newly diagnosed chronic phase CML, there are no controlled trials demonstrating a clinical benefit or increased survival for these diseases. | Novartis Europharm Limited | EU/3/01/021 (removed)<br>EU/3/01/061 (removed)<br>EU/3/05/304 (removed)<br>EU/3/05/305 (removed)<br>EU/3/05/320 (removed)<br>EU/3/05/340 (removed) |
| 47 | Glybera | Glybera is indicated for adult patients diagnosed with familial lipoprotein lipase deficiency (LPLD) and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. The diagnosis of LPLD has to be confirmed by genetic testing. The indication is restricted to patients with detectable levels of LPL protein. | uniQure biopharma B.V. | EU/3/04/194 (active) |
| 48 | Granupas | Indicated for use as part of an appropriate combination regimen for multi-drug resistant tuberculosis in adults and paediatric patients from 28 days of age and older when an effective treatment regimen cannot otherwise be composed for reasons of resistance or tolerability. Consideration should be given to official guidance on the appropriate use of antibacterial agents. | Eurocept International B.V. | EU/3/10/826 (active) |
| 49 | Hetlioz | HETLIOZ is indicated for the treatment of Non-24-Hour Sleep-Wake Disorder (Non-24) in totally blind adults. | Vanda Pharmaceuticals Limited | EU/3/10/841 (active) |
| 50 | Holoclar | Treatment of adult patients with moderate to severe limbal stem cell deficiency (defined by the presence of superficial corneal neovascularisation in at least two corneal quadrants, with central corneal involvement, and severely impaired visual acuity), unilateral or bilateral, due to physical or chemical ocular burns. A minimum of 1-2 mm2 of undamaged limbus is required for biopsy. | Chiesi Farmaceutici S.p.A. | EU/3/08/579 (active) |

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| 51 | Iclusig | Iclusig is indicated in adult patients with<br>• chronic phase, accelerated phase, or blast phase chronic myeloid leukaemia (CML) who are resistant to dasatinib or nilotinib; who are intolerant to dasatinib or nilotinib and for whom subsequent treatment with imatinib is not clinically appropriate; or who have the T315I mutation<br>• Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) who are resistant to dasatinib; who are intolerant to dasatinib and for whom subsequent treatment with imatinib is not clinically appropriate; or who have the T315I mutation. | Incyte Biosciences Distribution B.V. | EU/3/09/715 (active)<br>EU/3/09/716 (active) |
| 52 | IDELVION | Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency).<br><br>IDELVION can be used for all age groups. | CSL Behring GmbH | EU/3/09/723 (active) |
| 53 | Ilaris | Periodic fever syndromes<br>Ilaris is indicated for the treatment of the following autoinflammatory periodic fever syndromes in adults, adolescents and children aged 2 years and older:<br><br>Cryopyrin-associated periodic syndromes<br>Ilaris is indicated for the treatment of cryopyrin-associated periodic syndromes (CAPS) including:<br>•Muckle-Wells syndrome (MWS),<br>•Neonatal-onset multisystem inflammatory disease (NOMID) / chronic infantile neurological, cutaneous, articular syndrome (CINCA),<br>•Severe forms of familial cold autoinflammatory syndrome (FCAS) / familial cold urticaria (FCU) presenting with signs and symptoms beyond cold-induced urticarial skin rash.<br><br>Tumour necrosis factor receptor associated periodic syndrome (TRAPS)<br>Ilaris is indicated for the treatment of tumour necrosis factor (TNF) receptor associated periodic syndrome (TRAPS).<br><br>Hyperimmunoglobulin D syndrome (HIDS)/mevalonate kinase deficiency (MKD)<br>Ilaris is indicated for the treatment of hyperimmunoglobulin D syndrome (HIDS)/mevalonate kinase deficiency (MKD).<br><br>Familial Mediterranean fever (FMF)<br>Ilaris is indicated for the treatment of Familial Mediterranean Fever (FMF). Ilaris should be given in combination with colchicine, if appropriate.<br><br>Ilaris is also indicated for the treatment of:<br>Still's disease<br>Ilaris is indicated for the treatment of active Still's disease including adult-onset Still's disease (AOSD) and systemic juvenile idiopathic arthritis (SJIA) in patients aged 2 years and older who have responded inadequately to previous therapy with non-steroidal anti-inflammatory drugs (NSAIDs) and systemic corticosteroids. Ilaris can be given as monotherapy or in combination with methotrexate.<br>Gouty arthritis<br>Ilaris is indicated for the symptomatic treatment of adult patients with frequent gouty arthritis attacks (at least 3 attacks in the previous 12 months) in whom non-steroidal anti-inflammatory drugs (NSAIDs) and colchicine are contraindicated, are not tolerated, or do not provide an adequate response, and in whom repeated courses of corticosteroids are not appropriate. | Novartis Europharm Limited | EU/3/07/439 (removed) |

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| 54 | IMBRUVICA | IMBRUVICA as a single agent is indicated for the treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL).<br>IMBRUVICA as a single agent is indicated for the treatment of adult patients with previously untreated chronic lymphocytic leukaemia (CLL)<br>IMBRUVICA as a single agent or in combination with bendamustine and rituximab (BR) is indicated for the treatment of adult patients with CLL who have received at least one prior therapy.<br>IMBRUVICA as a single agent is indicated for the treatment of adult patients with Waldenström's macroglobulinaemia (WM) who have received at least one prior therapy, or in first line treatment for patients unsuitable for chemo-immunotherapy. | Janssen-Cilag International NV | EU/3/12/984 (active)<br>EU/3/13/1115 (active)<br>EU/3/14/1264 (active) |
| 55 | Imnovid | Imnovid in combination with dexamethasone is indicated in the treatment of adult patients with relapsed and refractory multiple myeloma who have received at least two prior treatment regimens, including both lenalidomide and bortezomib, and have demonstrated disease progression on the last therapy. | Celgene Europe B.V. | EU/3/09/672 (active) |
| 56 | INCRELEX | For the long-term treatment of growth failure in children and adolescents from 2 to 18 years with severe primary insulin-like growth factor-1 deficiency (Primary IGFD).Severe Primary IGFD is defined by:- height standard deviation score ≤ –3.0 and- basal IGF-1 levels below the 2.5th percentile for age and gender and- GH sufficiency.- Exclusion of secondary forms of IGF-1 deficiency, such as malnutrition, hypothyroidism, or chronic treatment with pharmacologic doses of anti-inflammatory steroids.Severe Primary IGFD includes patients with mutations in the GH receptor (GHR), post-GHR signaling pathway, and IGF-1 gene defects; they are not GH deficient, and therefore, they cannot be expected to respond adequately to exogenous GH treatment. It is recommended to confirm the diagnosis by conducting an IGF-1 generation test. | Ipsen Pharma | EU/3/06/373 (removed) |
| 57 | Inovelon | Adjunctive therapy in the treatment of seizures associated with Lennox-Gastaut syndrome in patients 1 year of age and older. | Eisai GmbH | EU/3/04/240 (active) |
| 58 | Jakavi | Myelofibrosis (MF)<br>Jakavi is indicated for the treatment of disease-related splenomegaly or symptoms in adult patients with primary myelofibrosis (also known as chronic idiopathic myelofibrosis), post polycythaemia vera myelofibrosis or post essential thrombocythaemia myelofibrosis.<br><br>Polycythaemia vera (PV)<br>Jakavi is indicated for the treatment of adult patients with polycythaemia vera who are resistant to or intolerant of hydroxyurea. | Novartis Europharm Limited | EU/3/08/572 (removed)<br>EU/3/09/620 (removed) |
| 59 | Jorveza | Jorveza is indicated for the treatment of eosinophilic esophagitis (EoE) in adults (older than 18 years of age). | Dr Falk Pharma GmbH | EU/3/13/1181 (active) |

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| 60 | Kalydeco | Film coated tablets:<br>Kalydeco tablets are indicated for the treatment of patients with cystic fibrosis (CF) aged 6 years and older and weighing 25 kg or more who have one of the following gating (class III) mutations in the CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N or S549R.<br><br>Kalydeco tablets are also indicated for the treatment of patients with cystic fibrosis (CF) aged 18 years and older who have an R117H mutation in the CFTR gene.<br>Kalydeco tablets are also indicated in a combination regimen with tezacaftor 100 mg/ivacaftor 150 mg tablets for the treatment of patients with cystic fibrosis (CF) aged 12 years and older who are homozygous for the F508del mutation or who are heterozygous for the F508del mutation and have one of the following mutations in the CFTR gene: P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272 26A→G, and 3849+10kbC→T.<br><br>Granules:<br>Kalydeco granules are indicated for the treatment of children with cystic fibrosis (CF) aged 12 months and older and weighing 7 kg to less than 25 kg who have one of the following gating (class III) mutations in the CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N or S549R. | Vertex Pharmaceuticals (Europe) Limited | EU/3/08/556 (active) |
| 61 | Kanuma | Kanuma is indicated for long-term enzyme replacement therapy (ERT) in patients of all ages with lysosomal acid lipase (LAL) Deficiency. | Alexion Europe SAS | EU/3/10/827 (active) |
| 62 | Ketoconazole HRA | Ketoconazole HRA is indicated for the treatment of endogenous Cushing's syndrome in adults and adolescents above the age of 12 years. | Laboratoire HRA Pharma | EU/3/12/965 (active) |
| 63 | Kolbam | Kolbam is indicated for the treatment of inborn errors in primary bile acid synthesis due to Sterol 27-hydroxylase (presenting as cerebrotendinous xanthomatosis, CTX) deficiency, 2- (or α-) methylacyl-CoA racemase (AMACR) deficiency or Cholesterol 7α-hydroxylase (CYP7A1) deficiency in infants, children and adolescents aged 1 month to 18 years and adults. | Retrophin Europe Limited | EU/3/09/683 (active)<br>EU/3/09/683 (active) |
| 64 | Kuvan | Kuvan is indicated for the treatment of hyperphenylalaninaemia (HPA) in adults and paediatric patients of all ages with phenylketonuria (PKU) who have been shown to be responsive to such treatment.<br>Kuvan is also indicated for the treatment of hyperphenylalaninaemia (HPA) in adults and paediatric patients of all ages with tetrahydrobiopterin (BH4) deficiency who have been shown to be responsive to such treatment. | BioMarin International Limited | EU/3/04/199 (active) |
| 65 | Kymriah | Kymriah is indicated for the treatment of:<br>- Paediatric and young adult patients up to 25 years of age with B cell acute lymphoblastic leukaemia (ALL) that is refractory, in relapse post transplant or in second or later relapse.<br>- Adult patients with relapsed or refractory diffuse large B cell lymphoma (DLBCL) after two or more lines of systemic therapy. | Novartis Europharm Limited | EU/3/14/1266 (active)<br>EU/3/16/1745 (active) |
| 66 | Kyprolis | Kyprolis in combination with either lenalidomide and dexamethasone or dexamethasone alone is indicated for the treatment of adult patients with multiple myeloma who have received at least one prior therapy. | Amgen Europe B.V. | EU/3/08/548 (active) |
| 67 | Lamzede | Enzyme replacement therapy for the treatment of non-neurological manifestations in patients with mild to moderate alpha mannosidosis. See sections 4.4 and 5.1. | Chiesi Farmaceutici S.p.A. | EU/3/04/260 (active) |
| 68 | Lartruvo | Lartruvo is indicated in combination with doxorubicin for the treatment of adult patients with advanced soft tissue sarcoma who are not amenable to curative treatment with surgery or radiotherapy and who have not been previously treated with doxorubicin | Eli Lilly Nederland B.V. | EU/3/15/1447 (active) |
| 69 | Ledaga | Ledaga is indicated for the topical treatment of mycosis fungoides-type cutaneous T-cell lymphoma (MF type CTCL) in adult patients. | Actelion Registration Ltd | EU/3/12/963 (active) |

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| 70 | Lenvima | LENVIMA is indicated as monotherapy for the treatment of adult patients with progressive, locally advanced or metastatic, differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma (DTC), refractory to radioactive iodine (RAI).<br><br>LENVIMA is indicated as monotherapy for the treatment of adult patients with advanced or unresectable hepatocellular carcinoma (HCC) who have received no prior systemic therapy | Eisai Europe Limited | EU/3/13/1119 (removed)<br>EU/3/13/1121 (removed) |
| 71 | Litak | treatment of hairy cell leukaemia | Lipomed GmbH | EU/3/01/055 (removed) |
| 72 | Lutathera | Lutathera is indicated for the treatment of unresectable or metastatic, progressive, well differentiated (G1 and G2), somatostatin receptor positive gastroenteropancreatic neuroendocrine tumours (GEP NETs) in adults. | Advanced Accelerator Applications | EU/3/07/523 (active) |
| 73 | Lynparza | 50 mg hard capsules:<br>Lynparza is indicated as monotherapy for the maintenance treatment of adult patients with platinum sensitive relapsed BRCA mutated (germline and/or somatic) high grade serous epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in response (complete response or partial response) to platinum based chemotherapy.<br><br>100 mg film coated tablets:<br>150 mg film coated tablets:<br><br>Lynparza is indicated as monotherapy for the maintenance treatment of adult patients with platinum sensitive relapsed high grade epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in response (complete or partial) to platinum based chemotherapy. | AstraZeneca AB | EU/3/07/501 (removed) |
| 74 | Lysodren | Symptomatic treatment of advanced (unresectable, metastatic or relapsed) adrenal cortical carcinoma.<br>The effect of Lysodren on non-functional adrenal cortical carcinoma is not established. | Laboratoire HRA Pharma | EU/3/02/102 (removed) |
| 75 | Mepact | MEPACT is indicated in children, adolescents and young adults for the treatment of high-grade resectable non-metastatic osteosarcoma after macroscopically complete surgical resection. It is used in combination with post-operative multi-agent chemotherapy. Safety and efficacy have been assessed in studies of patients 2 to 30 years of age at initial diagnosis | Takeda France SAS | EU/3/04/206 (active) |
| 76 | Mepsevii | Mepsevii is indicated for the treatment of non-neurological manifestations of Mucopolysaccharidosis VII (MPS VII; Sly syndrome). | Ultragenyx Germany GmbH | EU/3/12/973 (active) |
| 77 | Mozobil | Mozobil is indicated in combination with granulocyte-colony stimulating factor (G-CSF) to enhance mobilisation of haematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in adult patients with lymphoma and multiple myeloma whose cells mobilise poorly. | Genzyme Europe B.V. | EU/3/04/227 (active) |
| 78 | Myalepta | Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients:<br>•with confirmed congenital generalised LD (Berardinelli-Seip syndrome) or acquired generalised LD (Lawrence syndrome) in adults and children 2 years of age and above<br>•with confirmed familial partial LD or acquired partial LD (Barraquer-Simons syndrome), in adults and children 12 years of age and above for whom standard treatments have failed to achieve adequate metabolic control. | Aegerion Pharmaceuticals B.V. | EU/3/12/1022 (active)<br>EU/3/12/1023 (active)<br>EU/3/12/1024 (active)<br>EU/3/12/1025 (active) |
| 79 | Mylotarg | MYLOTARG is indicated for combination therapy with daunorubicin (DNR) and cytarabine (AraC) for the treatment of patients age 15 years and above with previously untreated, *de novo* CD33 positive acute myeloid leukaemia (AML), except acute promyelocytic leukaemia (APL). | Pfizer Europe MA EEIG | EU/3/00/005 (active) |
| 80 | Myozyme | Myozyme is indicated for long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of Pompe disease (acid α-glucosidase deficiency).<br>Myozyme is indicated in adults and paediatric patients of all ages. | Genzyme Europe B.V. | EU/3/00/018 (removed) |

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| 81 | Naglazyme | Naglazyme is indicated for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis VI (MPS VI; N-acetylgalactosamine 4-sulfatase deficiency; Maroteaux-Lamy syndrome).<br>A key issue is to treat children aged <5 years suffering from a severe form of the disease, even though children <5 years were not included in the pivotal phase 3 study. Limited data are available in patients < 1 year of age. | BioMarin International Limited | EU/3/01/025 (removed) |
| 82 | Natpar | Natpar is indicated as adjunctive treatment of adult patients with chronic hypoparathyroidism who cannot be adequately controlled with standard therapy alone. | Shire Pharmaceuticals Ireland Limited | EU/3/13/1210 (active) |
| 83 | Nexavar | Hepatocellular carcinoma<br>Nexavar is indicated for the treatment of hepatocellular carcinoma.<br>Renal cell carcinoma<br>Nexavar is indicated for the treatment of patients with advanced renal cell carcinoma who have failed prior interferon-alpha or interleukin-2 based therapy or are considered unsuitable for such therapy.<br>Differentiated thyroid carcinoma<br>Nexavar is indicated for the treatment of patients with progressive, locally advanced or metastatic, differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma, refractory to radioactive iodine. | Bayer AG | EU/3/04/207 (removed)<br>EU/3/06/364 (removed)<br>EU/3/13/1199 (active)<br>EU/3/13/1200 (active) |
| 84 | NexoBrid | NexoBrid is indicated for removal of eschar in adults with deep partial- and full-thickness thermal burns. | MediWound Germany GmbH | EU/3/02/107 (active) |
| 85 | Ninlaro | NINLARO in combination with lenalidomide and dexamethasone is indicated for the treatment of adult patients with multiple myeloma who have received at least one prior therapy. | Takeda Pharma A/S | EU/3/11/899 (active) |
| 86 | Nplate | Nplate is indicated for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). | Amgen Europe B.V. | EU/3/05/283 (active) |
| 87 | OCALIVA | OCALIVA is indicated for the treatment of primary biliary cholangitis (also known as primary biliary cirrhosis) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA. | Intercept Pharma Ltd | EU/3/10/753 (active) |
| 88 | Ofev | Indicated in adults for the treatment of Idiopathic Pulmonary Fibrosis (IPF). | Boehringer Ingelheim International GmbH | EU/3/13/1123 (active) |
| 89 | Onivyde | Treatment of metastatic adenocarcinoma of the pancreas, in combination with 5 fluorouracil (5 FU) and leucovorin (LV), in adult patients who have progressed following gemcitabine based therapy. | Les Laboratoires Servier | EU/3/11/933 (active) |
| 90 | Onpattro | Onpattro is indicated for the treatment of hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis) in adult patients with stage 1 or stage 2 polyneuropathy. | Alnylam Netherlands B.V. | EU/3/11/857 (active) |
| 91 | Onsenal | reduction of the number of adenomatous intestinal polyps in familial polyposis, as an adjunct to surgery and further endoscopic surveillance | Pfizer Limited | EU/3/01/070 (removed) |
| 92 | Opsumit | Opsumit, as monotherapy or in combination, is indicated for the long-term treatment of pulmonary arterial hypertension (PAH) in adult patients of WHO Functional Class (FC) II to III. Efficacy has been shown in a PAH population including idiopathic and heritable PAH, PAH associated with connective tissue disorders, and PAH associated with corrected simple congenital heart disease. | Janssen-Cilag International NV | EU/3/11/909 (active) |
| 93 | Orfadin | Treatment of adult and paediatric (in any age range) patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT 1) in combination with dietary restriction of tyrosine and phenylalanine. | Swedish Orphan Biovitrum International AB | EU/3/00/012 (removed) |
| 94 | Orphacol | Treatment of inborn errors in primary bile acid synthesis due to 3β-Hydroxy-$\Delta^5$-$C_{27}$-steroid oxidoreductase deficiency or $\Delta^4$3-Oxosteroid-5β-reductase deficiency in infants, children and adolescents aged 1 month to 18 years and adults. | Laboratoires CTRS | EU/3/02/127 (active) |
| 95 | OXERVATE | Treatment of moderate (persistent epithelial defect) or severe (corneal ulcer) neurotrophic keratitis in adults. | Dompé farmaceutici S.p.A. | EU/3/15/1586 (active) |
| 96 | Pedea | Treatment of a haemodynamically significant patent ductus arteriosus in preterm newborn infants less than 34 weeks of gestational age | Orphan Europe S.A.R.L. | EU/3/01/020 (removed) |

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| 97 | Peyona | Treatment of primary apnoea of premature newborns. | Chiesi Farmaceutici S.p.A. | EU/3/03/132 (active) |
| 98 | PhotoBarr | Ablation of high-grade dysplasia (HGD) in patients with Barrett's Oesophagus (BO) | Pinnacle Biologics B.V. | EU/3/02/086 (removed) |
| 99 | Plenadren | Treatment of adrenal insufficiency in adults. | Shire Services BVBA | EU/3/06/372 (active) |
| 100 | PREVYMIS | PREVYMIS is indicated for prophylaxis of cytomegalovirus (CMV) reactivation and disease in adult CMV-seropositive recipients [R+] of an allogeneic haematopoietic stem cell transplant (HSCT).<br><br>Consideration should be given to official guidance on the appropriate use of antiviral agents. | Merck Sharp & Dohme B.V. | EU/3/11/849 (active) |
| 101 | Prialt | Treatment of severe, chronic pain in adults who require intrathecal (IT) analgesia | RIEMSER Pharma GmbH | EU/3/01/048 (removed) |
| 102 | Procysbi | PROCYSBI is indicated for the treatment of proven nephropathic cystinosis. Cysteamine reduces cystine accumulation in some cells (e.g. leukocytes, muscle and liver cells) of nephropathic cystinosis patients and, when treatment is started early, it delays the development of renal failure. | Chiesi Farmaceutici S.p.A. | EU/3/10/778 (active) |
| 103 | Qarziba | Qarziba is indicated for the treatment of high-risk neuroblastoma in patients aged 12 months and above, who have previously received induction chemotherapy and achieved at least a partial response, followed by myeloablative therapy and stem cell transplantation, as well as patients with history of relapsed or refractory neuroblastoma, with or without residual disease. Prior to the treatment of relapsed neuroblastoma, any actively progressing disease should be stabilised by other suitable measures.<br>In patients with a history of relapsed/refractory disease and in patients who have not achieved a complete response after first line therapy, Qarziba should be combined with interleukin-2 (IL-2). | EUSA Pharma (UK) Limited | EU/3/12/1062 (active) |
| 104 | Ravicti | RAVICTI is indicated for use as adjunctive therapy for chronic management of adult and paediatric patients ≥2 months of age with urea cycle disorders (UCDs) including deficiencies of carbamoyl phosphate-synthase-I (CPS), ornithine carbamoyltransferase (OTC), argininosuccinate synthetase (ASS), argininosuccinate lyase (ASL), arginase I (ARG) and ornithine translocase deficiency hyperornithinaemia-hyperammonaemia homocitrullinuria syndrome (HHH) who cannot be managed by dietary protein restriction and/or amino acid supplementation alone.<br><br>RAVICTI must be used with dietary protein restriction and, in some cases, dietary supplements (e.g., essential amino acids, arginine, citrulline, protein-free calorie supplements). | Horizon Pharma Ireland Limited | EU/3/10/733 (active)<br>EU/3/10/734 (active)<br>EU/3/10/735 (active)<br>EU/3/10/736 (active)<br>EU/3/10/737 (active)<br>EU/3/10/738 (active) |
| 105 | Raxone | Raxone is indicated for the treatment of visual impairment in adolescent and adult patients with Leber's Hereditary Optic Neuropathy (LHON). | Santhera Pharmaceuticals (Deutschland) GmbH | EU/3/07/434 (active) |
| 106 | Replagal | Replagal is indicated for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry Disease (α-galactosidase A deficiency). | Shire Human Genetic Therapies AB | EU/3/00/002 (removed) |
| 107 | Revatio | <u>Adults</u><br>Treatment of adult patients with pulmonary arterial hypertension classified as WHO functional class II and III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and pulmonary hypertension associated with connective tissue disease.Revatio solution for injection is for the treatment of adult patients (≥ 18 years) with pulmonary arterial hypertension who are currently prescribed oral Revatio and who are temporarily unable to take oral therapy, but are otherwise clinically and haemodynamically stable.<br><br><u>Paediatric population</u><br>Treatment of paediatric patients aged 1 year to 17 years old with pulmonary arterial hypertension. Efficacy in terms of improvement of exercise capacity or pulmonary haemodynamics has been shown in primary pulmonary hypertension and pulmonary hypertension associated with congenital heart disease. | Pfizer Europe MA EEIG | EU/3/03/178 (removed) |

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| 108 | Revestive | Revestive is indicated for the treatment of patients aged 1 year and above with Short Bowel Syndrome. Patients should be stable following a period of intestinal adaptation after surgery. | Shire Pharmaceuticals Ireland Limited | EU/3/01/077 (active) |
| 109 | Revlimid | Multiple myeloma<br>Revlimid as monotherapy is indicated for the maintenance treatment of adult patients with newly diagnosed multiple myeloma who have undergone autologous stem cell transplantation.<br><br>Revlimid as combination therapy is indicated for the treatment of adult patients with previously untreated multiple myeloma who are not eligible for transplant.<br><br>Revlimid in combination with dexamethasone is indicated for the treatment of multiple myeloma in adult patients who have received at least one prior therapy.<br><br>Myelodysplastic syndromes<br>Revlimid as monotherapy is indicated for the treatment of adult patients with transfusion-dependent anemia due to low- or intermediate-1-risk myelodysplastic syndromes associated with an isolated deletion 5q cytogenetic abnormality when other therapeutic options are insufficient or inadequate.<br><br>Mantle cell lymphoma<br>Revlimid as monotherapy is indicated for the treatment of adult patients with relapsed or refractory mantle cell lymphoma | Celgene Europe B.V. | EU/3/03/177 (removed)<br>EU/3/04/192 (active)<br>EU/3/11/924 (active) |
| 110 | Revolade | Revolade is indicated for chronic immune (idiopathic) thrombocytopenic purpura (ITP) patients aged 1 year and above who are refractory to other treatments (e.g. corticosteroids, immunoglobulins) (see sections 4.2 and 5.1)..<br><br>Revolade is indicated in adult patients with chronic hepatitis C virus (HCV) infection for the treatment of thrombocytopenia, where the degree of thrombocytopenia is the main factor preventing the initiation or limiting the ability to maintain optimal interferon-based therapy (see sections 4.4 and 5.1).<br><br>Revolade is indicated in adult patients with acquired severe aplastic anaemia (SAA) who were either refractory to prior immunosuppressive therapy or heavily pretreated and are unsuitable for haematopoietic stem cell transplantation (see section 5.1). | Novartis Europharm Limited | EU/3/07/467 (removed) |
| 111 | Rilonacept Regeneron | indicated for the treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) with severe symptoms, including Familial Cold Autoinflammatory Syndrome (FCAS) and Muckle-Wells Syndrome (MWS), in adults and children aged 12 years and older | Regeneron UK Limited | EU/3/07/456 (removed) |
| 112 | Rubraca | Rubraca is indicated as monotherapy treatment of adult patients with platinum sensitive, relapsed or progressive, BRCA mutated (germline and/or somatic), high-grade epithelial ovarian, fallopian tube, or primary peritoneal cancer, who have been treated with two or more prior lines of platinum based chemotherapy, and who are unable to tolerate further platinum based chemotherapy. | Clovis Oncology UK Limited | EU/3/12/1049 (active) |
| 113 | Rydapt | Rydapt is indicated:<br>•in combination with standard daunorubicin and cytarabine induction and high dose cytarabine consolidation chemotherapy, and for patients in complete response followed by Rydapt single agent maintenance therapy, for adult patients with newly diagnosed acute myeloid leukaemia (AML) who are FLT3 mutation positive;<br>•as monotherapy for the treatment of adult patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with associated haematological neoplasm (SM AHN), or mast cell leukaemia (MCL). | Novartis Europharm Limited | EU/3/04/214 (active)<br>EU/3/10/765 (active) |
| 114 | Savene | Savene is indicated for the treatment of anthracycline extravasation in adults. | Clinigen Healthcare Limited | EU/3/01/059 (removed) |

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| 115 | Scenesse | Scenesse is indicated for prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP). | Clinuvel UK Limited | EU/3/08/541 (active) |
| 116 | Signifor | Treatment of adult patients with acromegaly for whom surgery is not an option or has not been curative and who are inadequately controlled on treatment with another somatostatin analogue.<br><br>Treatment of adult patients with Cushing's disease for whom surgery is not an option or for whom surgery has failed.<br><br>The 60 mg strength is only to be used in the treatment of acromegaly. | Novartis Europharm Limited | EU/3/09/670 (active)<br>EU/3/09/671 (active) |
| 117 | Siklos | Siklos is indicated for the prevention of recurrent painful vaso-occlusive crises including acute chest syndrome in adults, adolescents and children older than 2 years suffering from symptomatic Sickle Cell Syndrome | Addmedica | EU/3/03/154 (removed) |
| 118 | Sirturo | SIRTURO is indicated for use as part of an appropriate combination regimen for pulmonary multidrug resistant tuberculosis (MDR TB) in adult patients when an effective treatment regimen cannot otherwise be composed for reasons of resistance or tolerability. Consideration should be given to official guidance on the appropriate use of antibacterial agents. | Janssen-Cilag International NV | EU/3/05/314 (active) |
| 119 | Soliris | Soliris is indicated in adults and children for the treatment of:<br>- Paroxysmal nocturnal haemoglobinuria (PNH). Evidence of clinical benefit is demonstrated in patients with haemolysis with clinical symptom(s) indicative of high disease activity, regardless of transfusion history.<br>- Atypical haemolytic uremic syndrome (aHUS).<br><br>Soliris is indicated in adults for the treatment of:<br>- Refractory generalized myasthenia gravis (gMG) in patients who are anti-acetylcholine receptor (AChR) antibody-positive. | Alexion Europe SAS | EU/3/03/166 (active)<br>EU/3/09/653 (active)<br>EU/3/14/1304 (active) |
| 120 | SomaKit TOC | This medicinal product is for diagnostic use only.<br><br>After radiolabelling with gallium (68Ga) chloride solution, the solution of gallium (68Ga) edotreotide obtained is indicated for Positron Emission Tomography (PET) imaging of somatostatin receptor overexpression in adult patients with confirmed or suspected well-differentiated gastro-enteropancreatic neuroendocrine tumours (GEP-NET) for localizing primary tumours and their metastases. | Advanced Accelerator Applications | EU/3/15/1450 (active) |
| 121 | Somavert | Treatment of adult 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 normalize IGF-I concentrations or was not tolerated. | Pfizer Europe MA EEIG | EU/3/01/023 (removed) |
| 122 | Spinraza | Spinraza is indicated for the treatment of 5q Spinal Muscular Atrophy. | Biogen Netherlands B.V. | EU/3/12/976 (active) |
| 123 | Sprycel | SPRYCEL is indicated for the treatment of adult patients with:<br>- newly diagnosed Philadelphia chromosome positive (Ph+) chronic myelogenous leukaemia (CML) in the chronic phase.<br>- chronic, accelerated or blast phase CML with resistance or intolerance to prior therapy including imatinib mesilate.<br>- Ph+ acute lymphoblastic leukaemia (ALL) and lymphoid blast CML with resistance or intolerance to prior therapy.<br>SPRYCEL is indicated for the treatment of paediatric patients with:<br>- newly diagnosed Ph+ CML in chronic phase (Ph+ CML CP) or Ph+ CML CP resistant or intolerant to prior therapy including imatinib. | Bristol-Myers Squibb Pharma EEIG | EU/3/05/338 (removed)<br>EU/3/05/339 (removed) |
| 124 | Strensiq | Strensiq is indicated for long-term enzyme replacement therapy in patients with paediatric-onset hypophosphatasia to treat the bone manifestations of the disease. | Alexion Europe SAS | EU/3/08/594 (active) |

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| 125 | Strimvelis | Strimvelis is indicated for the treatment of patients with severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID), for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available. | Orchard Therapeutics (Netherlands) B.V. | EU/3/05/313 (active) |
| 126 | Sutent | *Gastrointestinal stromal tumour (GIST)*<br>Sutent is indicated for the treatment of unresectable and/or metastatic malignant gastrointestinal stromal tumour (GIST) in adults after failure of imatinib treatment due to resistance or intolerance.<br>*Metastatic renal cell carcinoma (MRCC)*<br>Sutent is indicated for the treatment of advanced/metastatic renal cell carcinoma (MRCC) in adults.<br>*Pancreatic neuroendocrine tumours (pNET)*<br>Sutent is indicated for the treatment of unresectable or metastatic, well-differentiated pancreatic neuroendocrine tumours (pNET) with disease progression in adults.<br>Experience with Sutent as first-line treatment is limited. | Pfizer Europe MA EEIG | EU/3/05/267 (removed) |
| 127 | Sylvant | Sylvant is indicated for the treatment of adult patients with multicentric Castleman's disease (MCD) who are human immunodeficiency virus (HIV) negative and human herpesvirus 8 (HHV 8) negative. | Janssen-Cilag International NV | EU/3/07/508 (active) |
| 128 | Symkevi | Symkevi is indicated in a combination regimen with ivacaftor 150 mg tablets for the treatment of patients with cystic fibrosis (CF) aged 12 years and older who are homozygous for the *F508del* mutation or who are heterozygous for the *F508del* mutation and have one of the following mutations in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene: *P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272 26A→G, and 3849+10kbC→T*. | Vertex Pharmaceuticals (Europe) Limited | EU/3/17/1828 (active) |
| 129 | TAKHZYRO | TAKHZYRO is indicated for routine prevention of recurrent attacks of hereditary angioedema (HAE) in patients aged 12 years and older. | Shire Pharmaceuticals Ireland Limited | EU/3/15/1551 (active) |
| 130 | Tasigna | Tasigna is indicated for the treatment of:<br>- adult and paediatric patients with newly diagnosed Philadelphia chromosome positive chronic myelogenous leukaemia (CML) in the chronic phase,<br>- adult patients with chronic phase and accelerated phase Philadelphia chromosome positive CML with resistance or intolerance to prior therapy including imatinib. Efficacy data in patients with CML in blast crisis are not available,<br>- paediatric patients with chronic phase Philadelphia chromosome positive CML with resistance or intolerance to prior therapy including imatinib. | Novartis Europharm Limited | EU/3/06/375 (active) |
| 131 | Tegsedi | Tegsedi is indicated for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR). | Akcea Therapeutics UK Ltd. | EU/3/14/1250 (active) |
| 132 | Tepadina | Tepadina is indicated, in combination with other chemotherapy medicinal products:<br>- with or without total body irradiation (TBI), as conditioning treatment prior to allogeneic or autologous haematopoietic progenitor cell transplantation (HPCT) in haematological diseases in adult and paediatric patients;<br>- when high dose chemotherapy with HPCT support is appropriate for the treatment of solid tumours in adult and paediatric patients. | ADIENNE S.r.l. | EU/3/06/424 (active) |
| 133 | Thalidomide Celgene | In combination with melphalan and prednisone as first line treatment of patients with untreated multiple myeloma, aged >= 65 years or ineligible for high dose chemotherapy.<br>Prescribed and dispensed according to the Thalidomide Celgene Pregnancy Prevention Programme | Celgene Europe B.V. | EU/3/01/067 (removed) |
| 134 | Thelin | Treatment of patients with pulmonary arterial hypertension (PAH) classified as WHO functional class III, to improve exercise capacity. Efficacy has been shown in primary pulmonary hypertension and in pulmonary hypertension associated with connective tissue disease. | Pfizer Limited | EU/3/04/234 (removed) |

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| 135 | TOBI Podhaler | TOBI Podhaler is indicated for the suppressive therapy of chronic pulmonary infection due to Pseudomonas aeruginosa in adults and children aged 6 years and older with cystic fibrosis. Consideration should be given to official guidance on the appropriate use of antibacterial agents. | Novartis Europharm Limited | EU/3/03/140 (active) |
| 136 | Torisel | Renal cell carcinoma<br><br>Torisel is indicated for the first-line treatment of adult patients with advanced renal cell carcinoma (RCC) who have at least three of six prognostic risk factors.<br><br>Mantle cell lymphoma<br><br>Torisel is indicated for the treatment of adult patients with relapsed and/or refractory mantle cell lymphoma (MCL). | Pfizer Europe MA EEIG | EU/3/06/365 (removed)<br>EU/3/06/420 (active) |
| 137 | Tracleer | Treatment of pulmonary arterial hypertension (PAH) to improve exercise capacity and symptoms in patients with WHO functional class III. Efficacy has been shown in:<br>• Primary (idiopathic and heritable) pulmonary arterial hypertension<br>• Pulmonary arterial hypertension secondary to scleroderma without significant interstitial pulmonary disease<br>• Pulmonary arterial hypertension associated with congenital systemic-to-pulmonary shunts and Eisenmenger's physiology<br>Some improvements have also been shown in patients with pulmonary arterial hypertension WHO functional class II.<br>Tracleer is also indicated to reduce the number of new digital ulcers in patients with systemic sclerosis and ongoing digital ulcer disease. | Janssen-Cilag International NV | EU/3/01/019 (removed)<br>EU/3/03/139 (removed) |
| 138 | Translarna | Translarna is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 2 years and older (see section 5.1). Efficacy has not been demonstrated in non-ambulatory patients.<br>The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing (see section 4.4). | PTC Therapeutics International Limited | EU/3/05/278 (active) |
| 139 | Trisenox | TRISENOX is indicated for induction of remission, and consolidation in adult patients with:<br>•Newly diagnosed low-to-intermediate risk acute promyelocytic leukaemia (APL) (white blood cell count, ≤ 10 x 103/µl) in combination with all trans retinoic acid (ATRA)<br>•Relapsed/refractory acute promyelocytic leukaemia (APL)(Previous treatment should have included a retinoid and chemotherapy)<br>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.<br><br>The response rate of other acute myelogenous leukaemia subtypes to arsenic trioxide has not been examined. | Teva B.V. | EU/3/00/008 (removed) |
| 140 | Unituxin | Unituxin is indicated for the treatment of high-risk neuroblastoma in patients aged 12 months to 17 years, who have previously received induction chemotherapy and achieved at least a partial response, followed by myeloablative therapy and autologous stem cell transplantation (ASCT). It is administered in combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), interleukin-2 (IL-2), and isotretinoin. | United Therapeutics Europe Ltd | EU/3/11/879 (removed) |

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| 141 | Venclyxto | Venclyxto in combination with rituximab is indicated for the treatment of adult patients with chronic lymphocytic leukaemia (CLL) who have received at least one prior therapy.<br><br>Venclyxto monotherapy is indicated for the treatment of CLL:<br>• in the presence of 17p deletion or TP53 mutation in adult patients who are unsuitable for or have failed a B cell receptor pathway inhibitor, or<br>• in the absence of 17p deletion or TP53 mutation in adult patients who have failed both chemoimmunotherapy and a B cell receptor pathway inhibitor. | AbbVie Deutschland GmbH & Co. KG | EU/3/12/1080 (removed) |
| 142 | Ventavis | Treatment of patients with primary pulmonary hypertension, classified as NYHA functional class III, to improve exercise capacity and symptoms. | Bayer AG | EU/3/00/014 (removed) |
| 143 | Verkazia | Treatment of severe vernal keratoconjunctivitis (VKC) in children from 4 years of age and adolescents. | Santen Oy | EU/3/06/360 (active) |
| 144 | Vidaza | Vidaza is indicated for the treatment of adult patients who are not eligible for haematopoietic stem cell transplantation (HSCT) with:<br>- intermediate-2 and high-risk myelodysplastic syndromes (MDS) according to the International Prognostic Scoring System (IPSS),<br>- chronic myelomonocytic leukaemia (CMML) with 10-29 % marrow blasts without myeloproliferative disorder,<br>- acute myeloid leukaemia (AML) with 20-30 % blasts and multi-lineage dysplasia, according to World Health Organisation (WHO) classification.<br>- AML with >30% marrow blasts according to the WHO classification. | Celgene Europe B.V. | EU/3/01/084 (active)<br>EU/3/07/509 (active) |
| 145 | Vimizim | Vimizim is indicated for the treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome, MPS IVA) in patients of all ages. | BioMarin International Limited | EU/3/09/657 (active) |
| 146 | Volibris | Volibris is indicated for treatment of pulmonary arterial hypertension (PAH) in adult patients of WHO Functional Class (FC) II to III, including use in combination treatment (see section 5.1). Efficacy has been shown in idiopathic PAH (IPAH) and in PAH associated with connective tissue disease. | GlaxoSmithKline (Ireland) Limited | EU/3/05/273 (removed) |
| 147 | Votubia | Renal angiomyolipoma associated with tuberous sclerosis complex (TSC)<br>Votubia is indicated for the treatment of adult patients with renal angiomyolipoma associated with tuberous sclerosis complex (TSC) who are at risk of complications (based on factors such as tumour size or presence of aneurysm, or presence of multiple or bilateral tumours) but who do not require immediate surgery.<br>The evidence is based on analysis of change in sum of angiomyolipoma volume.<br>Subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis complex (TSC) Votubia is indicated for the treatment of adult and paediatric patients with subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis complex (TSC) who require therapeutic intervention but are not amenable to surgery.<br>The evidence is based on analysis of change in SEGA volume. Further clinical benefit, such as improvement in disease-related symptoms, has not been demonstrated. | Novartis Europharm Limited | EU/3/10/764 (active) |
| 148 | VPRIV | VPRIV is indicated for long-term enzyme replacement therapy (ERT) in patients with type 1 Gaucher disease. | Shire Pharmaceuticals Ireland Limited | EU/3/10/752 (active) |
| 149 | Vyndaqel | Vyndaqel is indicated for the treatment of transthyretin amyloidosis in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment. | Pfizer Europe MA EEIG | EU/3/06/401 (active) |
| 150 | Vyxeos | Vyxeos is indicated for the treatment of adults with newly diagnosed, therapy-related acute myeloid leukaemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC). | Jazz Pharmaceuticals Ireland Ltd | EU/3/11/942 (active) |
| 151 | Wakix | Wakix is indicated in adults for the treatment of narcolepsy with or without cataplexy. | Bioprojet Pharma | EU/3/07/459 (active) |
| 152 | Wilzin | Treatment of Wilson's disease | Orphan Europe S.A.R.L. | EU/3/01/050 (removed) |

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| 153 | Xagrid | Xagrid is indicated for the reduction of elevated platelet counts in at risk essential thrombocythaemia (ET) patients who are intolerant to their current therapy or whose elevated platelet counts are not reduced to an acceptable level by their current therapy.<br>An at risk patient<br>An at risk essential thrombocythaemia patient is defined by one or more of the following features:<br>- > 60 years of age or<br>- a platelet count > 1000 x 109/l or<br>- a history of thrombo-haemorrhagic events. | Shire Pharmaceuticals Ireland Limited | EU/3/00/010 (removed) |
| 154 | Xaluprine | Treatment of acute lymphoblastic leukaemia (ALL) in adults, adolescents and children. | Nova Laboratories Limited | EU/3/09/628 (active) |
| 155 | Xermelo | Xermelo is indicated for the treatment of carcinoid syndrome diarrhoea in combination with somatostatin analogue (SSA) therapy in adults inadequately controlled by SSA therapy. | Ipsen Pharma | EU/3/09/661 (active) |
| 156 | Xyrem | Treatment of cataplexy in adult patients with narcolepsy | UCB Pharma S.A. | EU/3/02/131 (removed) |
| 157 | Yescarta | YESCARTA is indicated for the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) and primary mediastinal large B-cell lymphoma (PMBCL), after two or more lines of systemic therapy. | Kite Pharma EU B.V. | EU/3/14/1393 (active)<br>EU/3/15/1553 (active) |
| 158 | Yondelis | Yondelis is indicated for the treatment of adult patients with advanced soft tissue sarcoma, after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents. Efficacy data are based mainly on liposarcoma and leiomyosarcoma patients.<br>Yondelis in combination with pegylated liposomal doxorubicin (PLD) is indicated for the treatment of patients with relapsed platinum-sensitive ovarian cancer. | Pharma Mar S.A. | EU/3/01/039 (removed)<br>EU/3/03/171 (active) |
| 159 | Zalmoxis | Zalmoxis is indicated as adjunctive treatment in haploidentical haematopoietic stem cell transplantation (HSCT) of adult patients with high-risk haematological malignancies. | MolMed S.p.A. | EU/3/03/168 (active) |
| 160 | Zavesca | Zavesca is indicated for the oral treatment of adult patients with mild to moderate type 1 Gaucher disease. Zavesca may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable.<br>Zavesca is indicated for the treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type C disease. | Janssen-Cilag International NV | EU/3/00/006 (removed)<br>EU/3/06/351 (active) |
| 161 | Zejula | Zejula is indicated as monotherapy for the maintenance treatment of adult patients with platinum sensitive relapsed high grade serous epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in response (complete or partial) to platinum based chemotherapy. | TESARO U.K. Limited | EU/3/10/760 (active) |